Filed pursuant to Rule 424(b)(4)
Registration No. 333-249074

PROSPECTUS

10,000,000 Shares

Common Stock

This is the initial public offering of shares of our common stock. We are offering 10,000,000 shares of our common stock. Prior to this offering, there has been no public market for our common stock. Our common stock has been approved for listing on The Nasdaq Global Select Market under the symbol "PRAX." The initial public offering price of our common stock is $19.00 per share.

We are an "emerging growth company" under applicable Securities and Exchange Commission rules and will be subject to reduced public company reporting requirements for this prospectus and future filings.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

Our business and an investment in our common stock involve significant risks. These risks are described under the caption "Risk Factors" beginning on page 13 of this prospectus.

	Per Share	*Total*
Public offering price	**$ 19.00**	**$190,000,000**
Underwriting discounts(1)	**$ 1.33**	**$ 13,300,000**
Proceeds, before expenses, to Praxis Precision Medicines, Inc.	**$ 17.67**	**$176,700,000**

(1) See "Underwriting (Conflicts of Interest)" beginning on page 218 of this prospectus for additional information regarding underwriting compensation.

The underwriters may also purchase up to an additional 1,500,000 shares from us at the public offering price, less the underwriting discount, within 30 days from the date of this prospectus to cover overallotments.

The underwriters expect to deliver the shares against payment in New York, New York on October 20, 2020.

Book-running Managers

Cowen **Evercore ISI** **Piper Sandler**

Lead Manager

Wedbush PacGrow

Co-Manager

Blackstone Capital Markets

October 15, 2020.

TABLE OF CONTENTS

Through and including November 9, 2020 (25 days after the commencement of this offering), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the obligation of dealers to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

You should rely only on the information contained in this prospectus or in any free writing prospectus we file with the Securities and Exchange Commission, or the SEC. Neither we nor the underwriters have authorized anyone to provide you with information other than that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are offering to sell, and seeking offers to buy, common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover page of this prospectus, or other earlier date stated in this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock.

For investors outside of the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any

jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms or other independent sources that we believe to be reliable sources. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. We are responsible for all of the disclosure contained in this prospectus, and we believe that these sources are reliable; however, we have not independently verified the information contained in such publications. While we are not aware of any misstatements regarding any third-party information presented in this prospectus, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties and are subject to change based on various factors, including those discussed under the section entitled “Risk Factors” and elsewhere in this prospectus. Some data are also based on our good faith estimates.

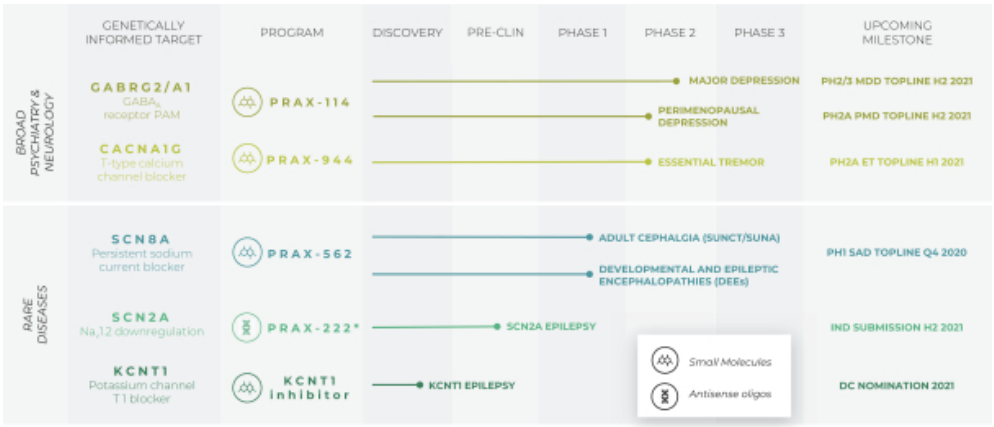
PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including our consolidated financial statements and the related notes included elsewhere in this prospectus. You should also consider, among other things, the matters described in the sections entitled "Risk Factors," "Business" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," in each case appearing elsewhere in this prospectus. Unless the context otherwise requires, we use the terms "Praxis," "Company," "we," "us" and "our" in this prospectus to refer to Praxis Precision Medicines, Inc.

Company Overview

We are a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system, or CNS, disorders characterized by neuronal imbalance. Normal brain function requires a delicate balance of excitation and inhibition in neuronal circuits, which, when dysregulated, can lead to abnormal function and disease. We are applying insights into the genetic mutations that drive excitation-inhibition imbalance in diseases to select biological targets for severe pediatric epilepsies and more broadly for prevalent psychiatric diseases and neurologic disorders. We apply a deliberate and pragmatic precision approach, leveraging a suite of translational tools including novel transgenic and predictive translational animal models and electrophysiology markers, to enable an efficient path to proof-of-concept in patients. We have three product candidates currently in clinical development. We intend to develop differentiated therapies that can deliver long-term benefits to human health by meaningfully impacting patients and society.

Our insights into genetic mutations resulting in neuronal imbalance have enabled us to develop a pipeline addressing prevalent psychiatric and neurologic conditions and rare diseases, with the ability to expand into additional indications. We have established a broad portfolio, including five disclosed programs across multiple CNS disorders, including depression, epilepsy, movement disorders and pain syndromes. We expect multiple topline clinical trial readouts from our three clinical-stage product candidates prior to the end of 2021 and anticipate the launch of a new clinical development program in 2021. Below is a summary of our portfolio of programs, organized by their initial therapeutic focus.



* PRAX-222 is a collaboration with Ionis Pharmaceuticals, Inc., or Ionis, and RogCon Inc., or RogCon. Ionis is eligible to receive royalties as a percentage of net product sales worldwide in the low-20s.

We own global commercialization rights for all of our product candidates, and we are party to collaboration and license agreements with Purdue Neuroscience Company, Ionis and RogCon, under which we could be obligated to pay certain fees, milestone and other conditional payments and cost reimbursements.

PRAX-114

We are developing PRAX-114, an extrasynaptic-preferring $GABA_A$ receptor positive allosteric modulator, or PAM, for the treatment of patients suffering from major depressive disorder, or MDD, and perimenopausal depression, or PMD. PRAX-114 is under development as a potentially differentiated treatment as both a monotherapy and adjunctive therapy for both the acute and maintenance setting. We have a multi-cohort, three-part Phase 2a clinical trial ongoing in Australia, and in Part A of the trial, we observed marked improvements in depression scores in MDD patients within two weeks of treatment. We expect complete topline data from Part B of the trial in the second half of 2021 and from longer-term dosing in MDD patients in Part C of the trial in the fourth quarter of 2020. We are planning to initiate a Phase 2/3 trial in the United States and Australia in the fourth quarter of 2020, which is intended to satisfy one of two registrational trials required by the U.S. Food and Drug Administration, or the FDA, to support clinical efficacy for the treatment of MDD, and we expect topline data in the second half of 2021.

There is significant unmet medical need in MDD and PMD with over 22 million individuals suffering from depressive symptoms in the U.S. Current pharmacological interventions suffer from multiple shortcomings including slow onset of efficacy, low response rates and side effects that limit patient compliance. PRAX-114 targets an increasingly well-understood neuronal circuit in the brain that we believe, when properly modulated, can result in a robust antidepressant effect with an advantageous safety and tolerability profile.

We believe that our PRAX-114 program has several advantages as compared to currently available therapies and product candidates in the $GABA_A$ PAM therapeutic class:

- **Wider Therapeutic Window**. We have determined that PRAX-114 is an approximately 10.5-fold more potent PAM of the extrasynaptic form of $GABA_A$ receptors compared to the synaptic form. By preferentially modulating extrasynaptic $GABA_A$ receptors, we believe PRAX-114 has the potential to mediate antidepressant and anxiolytic activity without the significant sedation observed with less selective neuroactive steroids.
- **Patient-Centric Dosing.** We believe the ability to administer PRAX-114 with or without food is key for clinical and commercial success in MDD and is critical for a patient-centric therapeutic, as many patients with depression suffer from appetite disturbance. We have observed fast absorption of PRAX-114 within one to three hours of dosing and a robust pharmacokinetic, or PK, profile across multiple trials. Based on clinical findings to date, we believe that PRAX-114 does not need to be taken with food to achieve therapeutic exposure, whereas other $GABA_A$ PAMs may require food to achieve therapeutic exposure.
- **Sustained Administration**. After consultation with the FDA and other stakeholders in MDD and PMD therapy, we designed our Phase 2/3 trial of PRAX-114 to include 28-day nightly dosing to evaluate patients at both 14 days to assess the rapidity and robustness of response and 28 days to evaluate durability of effect. We believe that having a dosing paradigm consistent with the duration of depressive episodes will provide the most substantial benefit to patients in controlling their disease, further differentiating PRAX-114 from other $GABA_A$ PAMs.
- **Indication Expansion.** Because PRAX-114 has demonstrated a novel pharmacology and, to date, has a well-tolerated profile, we believe PRAX-114 is suitable for potential development across a wide-range of indications in psychiatry and neurology, providing sizable expansion opportunities to explore in addition to MDD.

PRAX-944

We are developing PRAX-944, a potentially differentiated selective small molecule inhibitor of T-type calcium channels, for the treatment of Essential Tremor, or ET. We have evaluated the safety and tolerability of PRAX-944 in over 150 healthy volunteers in five separate clinical trials. In these trials, we have studied the safety of PRAX-944 modified release formulation with titration up to 120mg/day and no maximum tolerated dose, or MTD, has been identified. We are currently conducting a Phase 2a proof-of-concept open-label trial in ET patients. Preliminary site data from five patients of the low dose cohort showed tremor reduction, which seems to compare favorably to the standard of care agents. We plan to announce topline data, including a high dose cohort, in the first half of 2021.

There is a large body of clinical, preclinical and genetic evidence that points to the involvement of T-type calcium channels in the cerebello-thalamo-cortical circuit as a main driver of ET. ET is the most common movement disorder, affecting up to seven million patients in the United States, which is seven times more individuals compared to Parkinson's tremor. ET is a progressive and debilitating movement disorder with action tremors that significantly disrupt daily living. There is a high unmet need for ET patients given the limited treatment options, with only one approved pharmacotherapy that is poorly tolerated, resulting in high discontinuation rates and the need for invasive brain surgeries.

Successful development of T-type calcium channel modulators in ET likely requires a PK profile with a blunted maximum drug exposure, or Cmax, and thoughtful clinical trial design and endpoint selection. We have designed our development program to include careful selection of clinical endpoints, a modified release formulation and dose titration strategy. We believe our modified release formulation for PRAX-944 is positioned to be a differentiated therapy in ET.

Because of the gatekeeper role of T-type calcium channels in regulating neuronal firing patterns in multiple neuronal circuits, we believe PRAX-944 is suitable for potential development across a wide-range of indications in psychiatry and neurology, providing sizable expansion opportunities in addition to ET.

PRAX-562

Our lead rare disease product candidate and third clinical program, PRAX-562, is the first selective, persistent sodium current blocker in development for the treatment of a broad range of rare, devastating CNS disorders, such as severe pediatric epilepsy and adult cephalgia. To date, PRAX-562 has demonstrated pharmacological activity in *in-vivo* models with significantly improved tolerability, suggesting a potentially improved therapeutic index compared to other sodium channel blockers.

We have initiated a Phase 1 trial of PRAX-562 in Australia to evaluate the safety, tolerability, PK and effects on an EEG biomarker in up to 129 adult healthy volunteers. We anticipate Phase 1 single ascending dose, or SAD, topline safety data in the fourth quarter of 2020. The clinical development plan for PRAX-562 encompasses exploring the broad potential for the mechanism of action in rare diseases through proof-of-concept trials in Short-lasting Unilateral Neuralgiform headache attacks with Conjunctival injection and Tearing, or SUNCT, and Short-lasting Unilateral Neuralgiform headache with Autonomic symptoms, or SUNA, two rare types of cephalgia, and then expanding into a range of rare pediatric Development and Epileptic Encephalopathies, or DEEs.

Preclinical Programs

In addition to our clinical programs, we have one preclinical program and one disclosed discovery program in development for severe genetic epilepsies. We continue to evaluate additional rare disease program opportunities. We anticipate submitting an Investigational New Drug, or IND, application for one of these programs in the second half of 2021.

Our Approach

Each of our programs is based on four key principles that we believe will both increase the probability of success and allow us to efficiently translate insights into high-impact therapies for patients and society:

1. *Focus on therapeutic targets identified through human genetics.*
2. *Utilize translational tools to validate the potential of our targets and product candidates.*
3. *Pursue efficient, rigorous clinical development paths to proof-of-concept in humans.*
4. *Apply patient-centric development strategies.*

Our Team and Investors

We have attracted a talented team of scientists and researchers in genetics and biology, chemistry and translational medicine as well as business leaders with established track records of successfully executing innovative drug discovery and development programs. Our Chief Executive Officer, Marcio Souza, most recently served as Chief Operating Officer at PTC Therapeutics, Inc. and was instrumental in the development and commercialization of multiple approved products while at NPS Pharmaceuticals, Inc., Shire Human Genetic Therapies Inc. and Sanofi Genzyme Corporation. Our Chief Medical Officer, Bernard Ravina, M.D., previously Chief Medical Officer at Voyager Therapeutics, Inc., is a neurologist and movement disorder specialist who brings decades of neurologic drug development experience from roles at Biogen, the University of Rochester and the NIH's Institute of Neurological Disorders and Stroke. Our Chief Financial Officer, Stuart Chaffee, Ph.D., co-founded Kymera Therapeutics, Inc. and has held multiple senior roles in finance, business development and corporate strategy at Biogen Inc., Zafgen, Inc. and Amgen Inc.

To date, we have raised approximately $210 million from premier life science investment institutions, including Blackstone Life Sciences, Vida Ventures, Novo Holdings, Eventide Asset Management, Avoro Capital Advisors, Surveyor Capital (a Citadel company), Point72, Cormorant Asset Management, Qatar Investment Authority (QIA), Irving Investors, Adage Capital Management, Verition Fund Management, OCV Partners and Ample Plus Fund.

Our Strategy

Our goal is to translate genetic insights into high-impact therapies for millions of people suffering from rare or prevalent CNS disorders characterized by neuronal imbalance. Key components of our strategy include:

- *Advance PRAX-114 toward regulatory approval and commercialization as a potentially differentiated monotherapy and adjunctive therapy for MDD and PMD in both acute and maintenance settings.*
- *Advance PRAX-944 toward regulatory approval and commercialization as a potentially differentiated therapy for ET.*
- *Advance our three disclosed rare disease programs and build our franchise of candidates addressing rare diseases such as DEEs based on precision medicine principles.*
- *Maximize the value of our product candidates through select indication expansion.*
- *Advance our understanding of genetics and neuronal imbalance to maintain our leadership and continue to build our pipeline.*
- *Build a sales and marketing infrastructure to reach prescribers in the United States and maximize the reach of our products globally, alone or in collaboration with others.*

Risks Associated with Our Business

Our ability to implement our business strategy is subject to numerous risks, as more fully described in the section entitled "Risk Factors" immediately following this prospectus summary. These risks include, among others:

- We are a clinical-stage biopharmaceutical company and we have incurred significant losses since our inception. We anticipate that we will continue to incur significant losses for the foreseeable future.
- We will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product discovery and development programs or commercialization efforts.
- Our business substantially depends upon the successful development of PRAX-114, PRAX-944 and PRAX-562. If we are unable to obtain regulatory approval for, and successfully commercialize, PRAX-114, PRAX-944 or PRAX-562, our business may be materially harmed.
- Clinical development involves a lengthy, complex and expensive process, with an uncertain outcome. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and the results of our clinical trials, which to date have primarily been conducted in Australia and New Zealand, may not satisfy the requirements of the FDA or comparable foreign regulatory authorities.
- The markets for PRAX-114 for MDD and PMD, PRAX-944 for ET, PRAX-562 for multiple rare neurological conditions and any other product candidates we may develop may be smaller than we expect.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following regulatory approval, if obtained.
- On May 20, 2020, we received a cease and desist demand letter from Sage Therapeutics, Inc., or Sage, claiming that we improperly accessed and benefited from Sage confidential information in connection with the in-license of our PRAX-114 development program as a result of our employment or engagement of former Sage employees and consultants. While we believe there is no merit to these claims and intend to defend our position, an adverse result could harm our business and result of operations.
- We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.
- We expect to depend on collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.
- Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.
- We have entered into, and may enter into, license or other collaboration agreements that impose certain obligations on us. If we fail to comply with our obligations under such agreements with third parties, we could lose license rights that may be important to our business.

- Our executive officers, directors and their affiliates and our principal stockholders own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Corporate Information

We were incorporated under the laws of the State of Delaware on September 22, 2015. Our principal executive office is located at One Broadway, 16th Floor, Cambridge, MA 02142, and our telephone number is (617) 300-8460. Our website address is www.praxismedicines.com. We do not incorporate the information on or accessible through our website into this prospectus, and you should not consider any information on, or that can be accessed through, our website as part of this prospectus.

Implications of Being an Emerging Growth Company

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act, as amended, or the JOBS Act, enacted in April 2012. As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in the registration statement for our initial public offering;
- reduced disclosure about our executive compensation arrangements;
- no non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the U.S. Securities and Exchange Commission. We may choose to take advantage of some but not all of these exemptions. We have taken advantage of reduced reporting requirements in the registration statement of which this prospectus is a part. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. Additionally, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption and, therefore, while we are an emerging growth company we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies.

THE OFFERING	
Common stock offered by us	10,000,000 shares.
Common stock to be outstanding immediately after this offering	36,749,675 shares (38,249,675 shares if the underwriters exercise their option to purchase additional shares in full).
Underwriters' option to purchase additional shares	We have granted a 30-day option to the underwriters to purchase up to an aggregate of 1,500,000 additional shares of common stock from us to cover over-allotments, if any, at the public offering price, less underwriting discounts and commissions, on the same terms as set forth in this prospectus.
Use of proceeds	We estimate that we will receive net proceeds from the sale of shares of our common stock in this offering of approximately $173.4 million, or $199.9 million if the underwriters exercise their option to purchase additional shares in full, based on an initial public offering price of $19.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. The principal purposes of this offering are to create a public market for our common stock and thereby facilitate future access to the public equity markets, increase our visibility in the marketplace and obtain additional capital. We currently intend to use the net proceeds from this offering along with cash on hand to (i) advance PRAX-114 into and through the completion of our Phase 2/3 trial in MDD, which is intended to satisfy one of two registrational trials required by the FDA to support clinical efficacy, and to complete Part B (PMD) and Part C (longer-term dosing in MDD) of our ongoing Phase 2a clinical trial for PRAX-114; (ii) complete our ongoing Phase 2a clinical trial and a Phase 2/3 randomized, controlled clinical trial for PRAX-944 in ET; (iii) complete our ongoing Phase 1 healthy volunteer trial and the first patient trial for PRAX-562 and (iv) advance other programs in our pipeline and support working capital and other general corporate purposes. For a more complete description of our intended use of the proceeds from this offering, see "Use of Proceeds."
Risk factors	You should carefully read the "Risk Factors" section of this prospectus for a discussion of factors that you

	should consider before deciding to invest in our common stock.
Conflicts of Interest	Certain affiliates of Blackstone Securities Partners L.P., an underwriter in this offering, beneficially own more than 10% of our outstanding preferred equity prior to the consummation of this offering. As a result, Blackstone Securities Partners L.P. is deemed to have a "conflict of interest" within the meaning of Rule 5121 of the Financial Industry Regulatory Authority, Inc., or FINRA Rule 5121. Accordingly, this offering is being made in compliance with the applicable requirements of FINRA Rule 5121. A qualified independent underwriter is not necessary for this offering pursuant to FINRA Rule 5121(a)(1)(A). See "Underwriting (Conflicts of Interest)."
Directed Share Program	At our request, the underwriters have reserved for sale, at the initial public offering price, up to 5% of the shares of our common stock offered by this prospectus (excluding the shares of common stock that may be issued upon the underwriters' exercise of their over-allotment option), for sale at the public offering price to individuals, including our officers, directors and employees, as well as friends and family members of our officers and directors. If purchased by persons who are not officers or directors, the shares will not be subject to a lock-up restriction. If purchased by any officer or director, the shares will be subject to a 180-day lock-up restriction. The number of shares available for sale to the general public, referred to as the general public shares, will be reduced to the extent that these persons purchase all or a portion of the reserved shares. Any reserved shares not so purchased will be offered by the underwriters to the general public on the same basis as the other shares offered by this prospectus. Likewise, to the extent demand by these persons exceeds the number of shares reserved for sale in the program, and there are remaining shares available for sale to these persons after the general public shares have first been offered for sale to the general public, then such remaining shares may be sold to these persons at the discretion of the underwriters. For further information regarding our directed share program,

	see “Certain Relationships and Related Party Transactions” and “Underwriting (Conflicts of Interest).”
Nasdaq Global Select Market symbol	“PRAX”

The number of shares of our common stock after this offering is based on 26,749,675 shares of our common stock issued as of September 30, 2020, including 25,067,977 shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering and 8,763 shares of unvested restricted common stock as of September 30, 2020, and excludes:

- 5,814,944 shares of common stock issuable upon the exercise of stock options outstanding as of September 30, 2020 under the 2017 Stock Incentive Plan, at a weighted average exercise price of $6.20 per share;
- 6,051 shares of common stock reserved for future issuance as of September 30, 2020 under the 2017 Stock Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- 3,271,028 shares of our common stock that became available for future issuance under the 2020 Stock Option and Incentive Plan, or the 2020 Plan, upon the effectiveness of the registration statement of which this prospectus forms a part (including 37,615 shares of our common stock issuable upon the exercise of stock options we have agreed to grant under our 2020 Plan upon the effectiveness of the registration statement of which this prospectus is a part, at an exercise price equal to the initial public offering price); and
- 327,102 shares of our common stock that became available for future issuance under the 2020 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

Unless otherwise indicated, all information in this prospectus reflects or assumes the following:

- the filing of our amended and restated certificate of incorporation upon the closing of this offering and the effectiveness of our amended and restated by-laws upon the closing of this offering;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 25,067,977 shares of common stock upon the closing of this offering;
- no exercise of outstanding options described above;
- a one-for-2.14 reverse split of our common stock effected on October 8, 2020; and
- no exercise by the underwriters of their option to purchase up to 1,500,000 additional shares of common stock in this offering.

SUMMARY CONSOLIDATED FINANCIAL DATA

You should read the following summary consolidated financial data together with our consolidated financial statements and unaudited interim condensed consolidated financial statements and the related notes appearing elsewhere in this prospectus and the "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus. We have derived the consolidated statement of operations data for the years ended December 31, 2018 and 2019 from our audited consolidated financial statements appearing elsewhere in this prospectus. The consolidated statement of operations data for the six months ended June 30, 2019 and 2020 and the consolidated balance sheet data as of June 30, 2020 have been derived from our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair statement of the financial information in those statements. Our historical results are not necessarily indicative of the results that may be expected in any future period and our operating results for the six months ended June 30, 2020 are not necessarily indicative of the results that may be expected for the full year ended December 31, 2020 or any other interim periods or any future year or period.

	YEAR ENDED DECEMBER 31,		SIX MONTHS ENDED JUNE 30,	
	2018	2019	2019	2020
			(Unaudited)	
	(In thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Operating expenses:				
Research and development	$ 18,820	$ 29,557	$ 14,512	$ 15,918
General and administrative	3,899	6,232	3,129	4,121
Total operating expenses	22,719	35,789	17,641	20,039
Loss from operations	(22,719)	(35,789)	(17,641)	(20,039)
Total other income (expense):				
Interest income (expense), net	(35)	193	123	133
Other expense	(3,648)	–	–	–
Total other income (expense), net	(3,683)	193	123	133
Loss before provision for (benefit from) income taxes	(26,402)	(35,596)	(17,518)	(19,906)
Provision for (benefit from) income taxes	133	(84)	–	(8)
Net loss	$(26,535)	$(35,512)	$(17,518)	$(19,898)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,296)	(5,170)	(2,184)	(4,103)
Loss on conversion of convertible notes	(392)	–	–	–
Gain on repurchase of redeemable convertible preferred stock	–	–	–	493
Net loss attributable to common stockholders	$(29,223)	$(40,682)	$(19,702)	$(23,508)

	YEAR ENDED DECEMBER 31,		SIX MONTHS ENDED JUNE 30,	
	2018	2019	2019	2020
			(Unaudited)	
	(In thousands, except share and per share data)			
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (22.52)	$ (26.60)	$ (13.38)	$ (14.37)
Weighted average common shares outstanding, basic and diluted(1)	1,297,633	1,529,629	1,472,484	1,635,913
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(1)		$ (2.68)		$ (1.16)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)(1)		13,270,761		17,177,274

(1) See Note 13 to our consolidated financial statements and Note 9 to our unaudited interim condensed consolidated financial statements appearing elsewhere in this prospectus for details on the calculation of basic and diluted net loss per share attributable to common stockholders and unaudited pro forma basic and diluted net loss per share attributable to common stockholders.

	AS OF JUNE 30, 2020 (Unaudited)		
	ACTUAL	PRO FORMA(2)	PRO FORMA AS ADJUSTED(3)
	(In thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 19,748	$ 129,998	$ 303,411
Working capital(1)	$ 12,283	$ 122,533	$ 296,317
Total assets	$ 22,653	$ 132,903	$ 305,931
Redeemable convertible preferred stock	$ 118,190	$ –	$ –
Accumulated deficit	$(104,085)	$ (104,085)	$ (104,085)
Total stockholders' (deficit) equity	$(104,084)	$ 124,356	$ 297,756

(1) We define working capital as current assets less current liabilities. See our consolidated financial statements and unaudited interim condensed consolidated financial statements and related notes appearing elsewhere in this prospectus for further details regarding our current assets and current liabilities.

(2) The pro forma consolidated balance sheet data give effect to: (i) the sale and issuance of 19,444,453 shares of our Series C-1 redeemable convertible preferred stock in the third quarter of 2020 for gross cash proceeds of $110.3 million and (ii) the automatic conversion of all outstanding shares of our redeemable convertible preferred stock, including our Series C-1 redeemable convertible preferred stock, into an aggregate of 25,067,977 shares of common stock upon the consummation of this offering.

(3) The pro forma as adjusted balance sheet data give further effect to the issuance and sale of 10,000,000 shares of our common stock in this offering at the initial public offering price of $19.00 per share after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all other information in this prospectus, including our consolidated financial statements and unaudited interim condensed consolidated financial statements and related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before investing in our common stock. Any of the risk factors we describe below could adversely affect our business, financial condition or results of operations. The market price of our common stock could decline if one or more of these risks or uncertainties actually occur, causing you to lose all or part of the money you paid to buy our common stock. Additional risks that we currently do not know about or that we currently believe to be immaterial may also impair our business. Certain statements below are forward-looking statements. See the section titled "Special Note Regarding Forward-Looking Statements" appearing elsewhere in this prospectus.

Risks Related to Our Financial Position and Need for Additional Capital

We are a clinical-stage biopharmaceutical company and we have incurred significant losses since our inception. We anticipate that we will continue to incur significant losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue to date, and we will continue to incur significant research and development and other expenses related to our clinical development and ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. Since our inception, we have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical trials. Our financial condition and operating results, including net losses, may fluctuate significantly from quarter to quarter and year to year. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance. Additionally, net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' (deficit) equity and working capital. Our net losses were $35.5 million and $26.5 million for the years ended December 31, 2019 and 2018, respectively, and $19.9 million and $17.5 million for the six months ended June 30, 2020 and 2019, respectively. As of June 30, 2020, we had an accumulated deficit of $104.1 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for our product candidates in our initial and potential additional indications as well as for other product candidates.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials, including in expanded geographies such as the United States or Europe, for PRAX-114, PRAX-944 and PRAX-562 for our initial and potential additional indications;
- initiate and continue research and development, including preclinical, clinical, and discovery efforts for any future product candidates;
- seek to identify additional product candidates;
- seek regulatory approvals for PRAX-114, PRAX-944 and PRAX-562, or any other product candidates that successfully complete clinical development;
- add operational, financial and management information systems and personnel, including personnel to support our product candidate development and help us comply with our obligations as a public company;

- hire and retain additional personnel, such as clinical, quality control, scientific, commercial and administrative personnel;
- maintain, expand and protect our intellectual property portfolio;
- establish sales, marketing, distribution, manufacturing, supply chain and other commercial infrastructure in the future to commercialize various products for which we may obtain regulatory approval;
- add equipment and physical infrastructure to support our research and development;
- acquire or in-license other product candidates and technologies;
- incur increased costs as a result of operating as a public company.

Our expenses could increase beyond our expectations if we are required by the U.S. Food and Drug Administration, or the FDA, or other regulatory authorities to perform clinical trials in addition to those that we currently expect, or if there are any delays in establishing appropriate manufacturing arrangements for or in completing our clinical trials or the development of any of our product candidates.

We will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product discovery and development programs or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the preclinical and clinical development of our current and future programs. If we are able to gain marketing approval for product candidates that we develop, including any indication for which we are developing or may develop PRAX-114, PRAX-944 and PRAX-562, we will require significant additional amounts of cash in order to launch and commercialize such product candidates to the extent that such launch and commercialization are not the responsibility of a future collaborator that we may contract with in the future. In addition, other unanticipated costs may arise in the course of our development efforts. Because the design and outcome of our planned and anticipated clinical trials is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of any product candidates we develop.

Our future capital requirements depend on many factors, including:

- the scope, progress, results and costs of researching and developing PRAX-114, PRAX-944 and PRAX-562 for our initial and potential additional indications, as well as other product candidates we may develop;
- the timing and uncertainty of, and the costs involved in, obtaining marketing approvals for PRAX-114, PRAX-944 and PRAX-562 for our initial and potential additional indications, and other product candidates we may develop and pursue;
- the number of future product candidates that we may pursue and their development requirements;
- the number of jurisdictions in which we plan to seek regulatory approvals;
- if approved, the costs of commercialization activities for PRAX-114, PRAX-944 and PRAX-562 for any approved indications, or any other product candidate that receives regulatory approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of regulatory approval, revenue, if any, received from commercial sales of PRAX-114, PRAX-944 and PRAX-562 for any approved indications or any other product candidates;

- the extent to which we in-license or acquire rights to other products, product candidates or technologies;
- our headcount growth and associated costs as we expand our research and development, increase our office space, and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights, including enforcing and defending intellectual property related claims; and
- the ongoing costs of operating as a public company.

We cannot be certain that additional funding will be available on acceptable terms, or at all. We have no committed source of additional capital and if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. Any of our current or future license agreements may also be terminated if we are unable to meet the payment or other obligations under the agreements.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents as of June 30, 2020 and the $110.3 million gross cash proceeds from the sale and issuance of our Series C-1 redeemable convertible preferred stock, will enable us to fund our operating expenses and capital expenditure requirements through at least the next 18 months. Our estimate may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to increase in connection with our planned operations. Unless and until we can generate a substantial amount of revenue from our product candidates, we expect to finance our future cash needs through public or private equity offerings, debt financings, collaborations, licensing arrangements or other sources, or any combination of the foregoing. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans.

To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, your ownership interest may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

If we raise additional funds through collaborations or marketing, distribution, licensing and royalty arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

We are in the early stages of clinical drug development and have a very limited operating history and no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.

We are a clinical-stage biopharmaceutical company with a very limited operating history, focused on translating genetic insights into the development of high-impact therapies for people with prevalent, as well as rare, CNS disorders characterized by neuronal imbalance. We commenced operations in 2016, have no products approved for commercial sale and have not generated any revenue from product sales. Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We are conducting Phase 1 or Phase 2a clinical trials for our PRAX-114, PRAX-944 and PRAX-562 programs, and have not initiated clinical trials for any of our other current product candidates. To date, our clinical trials have been conducted only in Australia and New Zealand, and we have not initiated or completed a pivotal clinical trial, obtained marketing approval for any product candidates, manufactured a commercial scale product or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Our short operating history as a company makes any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage biopharmaceutical companies in rapidly evolving fields, and we have not yet demonstrated an ability to successfully overcome such risks and difficulties. If we do not address these risks and difficulties successfully, our business will suffer.

Drug development is a highly uncertain undertaking and involves a substantial degree of risk.

We have no products approved for commercial sale. To obtain revenues from the sales of our product candidates that are significant or large enough to achieve profitability, we must succeed, either alone or with third parties, in developing, obtaining regulatory approval for, manufacturing, and marketing therapies with significant commercial success. Our ability to generate revenue and achieve profitability depends on many factors, including:

- completing research and preclinical and clinical development of our product candidates;
- obtaining regulatory approvals and marketing authorizations for product candidates for which we successfully complete clinical development and clinical trials;
- developing a sustainable and scalable manufacturing process for our product candidates, as well as establishing and maintaining commercially viable supply relationships with third parties that can provide adequate products and services to support clinical activities and commercial demand of our product candidates;
- identifying, assessing, acquiring and/or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- launching and successfully commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;
- obtaining and maintaining an adequate price for our product candidates in the countries where our products are commercialized;
- obtaining adequate reimbursement for our product candidates from payors;
- obtaining market acceptance of our product candidates as viable treatment options;
- addressing any competing technological and market developments;
- receiving milestone and other payments under any future collaboration arrangements;
- maintaining, protecting, expanding and enforcing our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when we will be able to generate any meaningful revenue or achieve or maintain profitability, if ever. In addition, our expenses could increase beyond our current expectations if we are required by the FDA or foreign regulatory agencies to perform studies in addition to those that we currently anticipate, or if there are any delays in any of our or our future collaborators' clinical trials or the development of any of our product candidates. Even if one or more of our product candidates is approved for commercial sale, we anticipate incurring significant costs associated with launching and commercializing any approved product candidate and ongoing compliance efforts.

Due to the significant resources required for the development of our programs, and depending on our ability to access capital, we must prioritize development of certain product candidates. Moreover, we may expend our limited resources on programs that do not yield a successful product candidate or fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We currently have three product candidates in clinical trials. Together, the development of these programs and product candidates require significant capital investment. Due to the significant resources required for the development of our programs and product candidates, we must focus our programs and product candidates on specific diseases and disease pathways and decide which product candidates to pursue and advance and the amount of resources to allocate to each. Our drug development strategy is to clinically test and seek regulatory approval for our product candidates in indications in which we believe there is the most evidence that we will be able to efficiently generate proof-of-concept data. We then intend to expand to clinical testing and seek regulatory approvals in other neurological and psychiatric disease indications based on genetic and mechanistic overlap with the primary indication. However, even if our product candidates are able to gain regulatory approval in one indication, there is no guarantee that we will be able to expand to other indications, and we may expend significant resources in seeking such approvals.

In addition, we may focus resources on pursuing indications outside of neurology based on the same strategic approach (e.g., human genetics, mechanistic rationale, the availability of translational tools, clinical development path, commercial opportunity) we utilize in determining on which of our discovery programs to focus. Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates or therapeutic areas may not lead to the development of any viable commercial product and may divert resources away from better opportunities. Additionally, we may reprioritize product candidate development plans and activities at any time and delay or terminate development of any product candidates we identify. For example, we have prioritized developing PRAX-144 for major depressive disorder, or MDD, ahead of perimenopausal depression, or PMD. Similarly, our potential decisions to delay, terminate, or collaborate with third parties in respect of certain programs may subsequently also prove to be suboptimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the viability or market potential of any of our programs or product candidates or misread trends in the biopharmaceutical industry, in particular for neurological and psychiatric diseases, our business, financial condition, and results of operations could be materially adversely affected. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases and disease pathways that may later prove to have greater commercial potential than those we choose to pursue or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights.

Risks Related to Research and Development and the Biopharmaceutical Industry

Our business substantially depends upon the successful development of PRAX-114, PRAX-944 and PRAX-562. If we are unable to obtain regulatory approval for, and successfully commercialize, PRAX-114, PRAX-944 or PRAX-562, our business may be materially harmed.

We currently have no products approved for sale and are investing the majority of our efforts and financial resources in the development of our lead product candidates, PRAX-114 for the treatment of MDD and PMD and PRAX-944 for the treatment of Essential Tremor, or ET. We have also commenced a first-in-human trial of PRAX-562 in healthy volunteers. We plan to initiate a Phase 2 trial for Short-lasting Unilateral Neuralgiform headache with Conjunctival injection and Tearing, or SUNCT, and Short-lasting Unilateral Neuralgiform headache attacks with Autonomic symptoms, or SUNA, to demonstrate clinical proof-of-concept and then subsequently expand into severe pediatric epilepsies. Successful continued development and ultimate regulatory approval of PRAX-114, PRAX-944 and PRAX-562 for our initial and potential additional indications is critical to the future success of our business. We will need to raise sufficient funds for, and successfully enroll and complete, our clinical development programs of PRAX-114 for the treatment of MDD and PMD, PRAX-944 for the treatment of ET, and possibly other diseases, and PRAX-562 for the treatment of a broad range of rare, devastating central nervous system, or CNS, disorders, such as severe pediatric epilepsy and adult cephalgia.

Before we can generate any revenue from sales of PRAX-114, PRAX-944, PRAX-562 or any of our other product candidates, we must undergo additional preclinical and clinical development, regulatory review and approval in one or more jurisdictions. To date, our clinical trials have been conducted exclusively in Australia and, for PRAX-944, in New Zealand as well. We are planning to pursue clinical trials in the United States for all of our clinical programs. We have not submitted Investigational New Drug applications, or INDs, for any of our product candidates with the FDA. In addition, if one or more of our product candidates are approved, we must ensure access to sufficient commercial manufacturing capacity and conduct significant marketing efforts in connection with any commercial launch. These efforts will require substantial investment, and we may not have the financial resources to continue development of our product candidates or commercialization of any products.

We may experience setbacks that could delay or prevent regulatory approval of our product candidates or our ability to commercialize any products, including:

- negative or inconclusive results from our preclinical studies or clinical trials or the clinical trials of others for product candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;
- product-related side effects experienced by subjects in our clinical trials or by individuals using drugs or therapeutics similar to our product candidates;
- delays in submitting INDs in the United States or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators or institutional review boards to commence a clinical trial, or a suspension or termination of a clinical trial once commenced;
- if the FDA or comparable foreign authorities do not accept the earlier preclinical and clinical trial work, then we may need to conduct additional preclinical or clinical trials beyond that which we currently have planned and significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our drug candidates and may harm our business and results of operations;

- conditions imposed by the FDA or comparable foreign authorities regarding the scope or design of our clinical trials;
- delays in contracting with clinical sites or enrolling subjects in clinical trials, including due to the COVID-19 pandemic;
- delays or interruptions in the supply of materials necessary for the conduct of our clinical trials;
- regulators or institutional review boards, or IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or other comparable regulatory authorities may disagree with our clinical trial design, including with respect to dosing levels administered in our planned clinical trials, which may delay or prevent us from initiating our clinical trials with our originally intended trial design;
- delays in reaching, or failure to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organizations, or CROs, which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the number of subjects required for clinical trials of any product candidates may be larger than we anticipate or subjects may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third-party contractors for preclinical studies or clinical trials may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or take actions that could cause clinical sites or clinical investigators to drop out of the trial, which may require that we add new clinical trial sites or investigators;
- due to the impact of the COVID-19 pandemic, we may experience some delays and interruptions to our preclinical studies and clinical trials, we may experience delays or interruptions to our manufacturing supply chain, or we could suffer delays in reaching, or we may fail to reach, agreement on acceptable terms with third-party service providers on whom we rely;
- greater than anticipated clinical trial costs, including as a result of delays or interruptions that could increase the overall costs to finish our clinical trials as our fixed costs are not substantially reduced during delays;
- we may elect to, or regulators, IRBs, Data Safety Monitoring Boards, or DSMBs, or ethics committees may require that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- we may not have the financial resources available to begin and complete the planned trials, or the cost of clinical trials of any product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate to initiate or complete a given clinical trial;
- the FDA or other comparable foreign regulatory authorities may require us to submit additional data such as long-term toxicology studies, or impose other requirements before permitting us to initiate a clinical trial, including because the FDA has not reviewed our preclinical or clinical data, to date, having been developed outside the United States in Australia and New Zealand;
- inability to compete with other therapies;
- poor efficacy of our product candidates during clinical trials;
- unfavorable FDA or other regulatory agency inspection and review of clinical trial sites or manufacturing facilities;

- unfavorable product labeling associated with any product approvals and any requirements for a Risk Evaluation and Mitigation Strategy, or REMS, that may be required by the FDA or comparable requirements in other jurisdictions to ensure the benefits of an individual product outweigh its risks;
- unfavorable acceptance of our clinical trial data by the patient or medical communities or third-party payors;
- failure of our third-party contractors or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- delays related to the impact or the spread of COVID-19 or other pandemics, including the impact of COVID-19 on the FDA's, or similar foreign regulatory agency's, ability to continue its normal operations;
- delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our technology in particular; or
- varying interpretations of data by the FDA and similar foreign regulatory agencies.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and our manufacturing, marketing, distribution and sales efforts or that of any future collaborator.

In addition, of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application, such as a new drug application, or NDA, to the FDA, and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval for PRAX-144, PRAX-944 or PRAX-562 for any indication, any such approval may be subject to limitations on the indications or uses or patient populations for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure that we will successfully develop or commercialize PRAX-114, PRAX-944 or PRAX-562 for any indication. If we or any of our future collaborators are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize PRAX-114, PRAX-944 or PRAX-562 for our initial or potential additional indications, we may not be able to generate sufficient revenue to continue our business. In addition, our failure to demonstrate positive results in our clinical trials in any indication for which we are developing PRAX-114, PRAX-944 and PRAX-562 could adversely affect our development efforts for PRAX-114, PRAX-944 and PRAX-562 in other indications.

Clinical development involves a lengthy, complex and expensive process, with an uncertain outcome. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and the results of our clinical trials, which to date have primarily been conducted in Australia and New Zealand, may not satisfy the requirements of the FDA or comparable foreign regulatory authorities.

To obtain the requisite regulatory approvals to commercialize any product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our product candidates are safe and effective in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. In particular, in the U.S. where we hope to advance our product development efforts in the future, the general approach for FDA approval of a new drug is dispositive data from two well-controlled, Phase 3 clinical trials of the relevant drug in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete. A product candidate can fail at any stage of testing, even after observing promising signs of activity in earlier preclinical studies or clinical trials. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are

completed. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or emergence of unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that commence clinical trials are never approved as products and there can be no assurance that any of our future clinical trials will ultimately be successful or support further clinical development of PRAX-114, PRAX-944, PRAX-562 or any of our other product candidates. Product candidates that appear promising in the early phases of development may fail to reach the market for several reasons, including:

- preclinical studies or clinical trials may show the product candidates to be less effective than expected (e.g., a clinical trial could fail to meet its primary endpoint(s)) or to have unacceptable side effects or toxicities;
- failure to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful;
- failure to receive the necessary regulatory approvals;
- manufacturing issues, formulation issues, pricing or reimbursement issues or other factors that make a product candidate uneconomical; and
- the proprietary rights of others and their competing products and technologies that may prevent one of our product candidates from being commercialized.

In addition, differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials. Moreover, clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in clinical trials have nonetheless failed to obtain marketing approval of their products.

Additionally, some of our trials may be open-label studies, where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. For example, prior MDD studies have exhibited a high placebo effect. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. Therefore, it is possible that positive results observed in open-label trials will not be replicated in later placebo-controlled trials. To date, we have conducted some trials as open-label trials, including with PRAX-114 and PRAX-944. Additionally, the trial design differences and placebo effects that may be possible in clinical research for the indications we are studying may make it difficult to extrapolate the results of earlier clinical trials to later clinical trials or to interpret the clinical data in any of our trials.

The standards that foreign regulatory authorities and the FDA use when regulating us require judgment and can change, which makes it difficult to predict with certainty how they will be applied. Although we are initially focusing our efforts on development of small molecule drug products, we intend to develop a potential antisense oligonucleotide candidate for genetic epilepsies and may in the future pursue development of biological products, each of which could make us subject to additional regulatory requirements. Any analysis we perform of data from preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent

regulatory approval. Our clinical trials have primarily been conducted in Australia and New Zealand. The FDA's acceptance of data from clinical trials outside of the United States is subject to certain conditions, including that the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with good clinical practice, that the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful, and that the trials are conducted in compliance with all applicable U.S. laws and regulations. If the FDA or comparable foreign regulatory authorities do not accept earlier preclinical or clinical data, we may need to conduct additional preclinical studies or clinical trials.

We may also encounter unexpected delays or increased costs due to new government regulations. Examples of such regulations include future legislation or administrative action, or changes in foreign regulatory authority or FDA policy during the period of product development and regulatory review. It is impossible to predict whether legislative changes will be enacted, or whether foreign or FDA regulations, guidance or interpretations will be changed, or what the impact of such changes, if any, may be. In the United States, where we plan to develop our candidates in the future, the FDA may also require a panel of experts, referred to as an Advisory Committee, to deliberate on the adequacy of the safety and efficacy data to support approval. The opinion of the Advisory Committee, although not binding on the FDA, may have a significant impact on our ability to obtain approval of any product candidates that we develop.

If we seek to continue conducting clinical trials in foreign countries or pursue marketing approvals in foreign jurisdictions, we must comply with numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing and third-party reimbursement. The foreign regulatory approval process varies among countries and jurisdictions and may include all of the risks associated with FDA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Moreover, the time required to obtain approval may differ jurisdiction-to-jurisdiction from that required to obtain FDA approval. Approval by foreign regulatory authorities does not ensure approval by the FDA and, similarly, approval by the FDA does not ensure approval by regulatory authorities outside the United States.

Successful completion of clinical trials is a prerequisite to submitting a marketing application to foreign regulatory authorities or the FDA, for each product candidate and, consequently, the ultimate approval and commercial marketing of any product candidates. We may experience negative or inconclusive results, or regulators may be unwilling to accept preclinical or clinical data obtained in foreign jurisdictions, which may result in our deciding, or our being required by regulators, to conduct additional clinical studies or trials or abandon some or all of our product development programs, which could have a material adverse effect on our business.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following regulatory approval, if obtained.

Undesirable side effects caused by PRAX-114, PRAX-944, PRAX-562 or any future product candidate could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. In addition, many compounds that have initially showed promise in clinical or earlier stage testing are later found to cause undesirable or unexpected side effects that prevented further development of the compound. Additionally, the composition of our product candidates or learnings in preclinical studies or clinical trials may result in contraindications for any product candidates for which we may obtain regulatory approval.

If unacceptable side effects arise in the development of our product candidates, we, foreign regulatory authorities, or, in the future, the FDA, the IRBs, DSMBs or independent ethics committees at

the institutions in which our trials are conducted could suspend or terminate our preclinical studies or clinical trials or foreign regulatory authorities or the FDA could order us to cease preclinical studies or clinical trials or deny approval of our product candidates for any or all targeted indications.

Treatment-emergent side effects that are deemed to be drug-related could also affect subject recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Undesirable side effects in one of our clinical trials for our product candidates in one indication could adversely affect enrollment in clinical trials, regulatory approval and commercialization of our product candidates in other indications. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. Any of these occurrences may harm our business, financial condition and prospects significantly.

Moreover, clinical trials of our product candidates are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any future collaborator, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. Clinical trials by their nature utilize a sample of the potential patient population. With a limited number of patients, rare and severe side effects of our product candidates may only be uncovered with a significantly larger number of patients exposed to the product candidate. If our product candidates receive marketing approval and we or others identify undesirable side effects caused by such product candidates (or any other similar products) after such approval, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw or limit their approval of such product candidates;
- regulatory authorities may require the addition of labeling statements, such as a Boxed Warning or contraindications;
- we may be required to change the way such product candidates are distributed or administered, or change the labeling of the product candidates;
- FDA may require a REMS plan to mitigate risks, which could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools, and regulatory authorities in other jurisdictions may require comparable risk mitigation plans;
- we may be subject to regulatory investigations and government enforcement actions;
- the FDA or a comparable foreign regulatory authority may require us to conduct additional clinical trials or costly post-marketing testing and surveillance to monitor the safety and efficacy of the product;
- we could be sued and held liable for injury caused to individuals exposed to or taking our product candidates; and
- our reputation may suffer.

For example, we are developing PRAX-114, an extrasynaptic-preferring $GABA_A$ receptor positive allosteric modulator, or PAM, for the treatment of patients suffering from MDD and PMD. There have been documented cases of approved $GABA_A$ receptor modulators leading to addiction and having the potential for abuse. To date, there has been no indication of this side effect for PRAX-114 in our clinical trials; however, in any such instance, we would be subject to the risks outlined above, which would impact our ability to achieve or maintain market acceptance.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidates and could substantially increase the costs of commercializing our product candidates, if approved, and significantly impact our ability to successfully commercialize our product candidates and generate revenues.

If we encounter difficulties enrolling patients in our future clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion.

Patient enrollment is affected by many factors, including:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- in the case clinical trials focused on rare disease, the small size of the patient population and the potential of a patient being undiagnosed or misdiagnosed;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- competing clinical trials and clinicians' and patients' perceptions as to the potential advantages and risks of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications that we are investigating;
- the impacts of the COVID-19 pandemic on clinical trial sites, personnel and patient travel;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or might require us to abandon one or more clinical trials altogether. Delays in patient enrollment may result in increased costs, affect the timing or outcome of the planned clinical trials, product candidate development and approval process and jeopardize our ability to seek and obtain the regulatory approval required to commence product sales and generate revenue, which could prevent completion of these trials, adversely affect our ability to advance the development of our product candidates, cause the value of our company to decline and limit our ability to obtain additional financing if needed.

We are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, such as www.ClinicalTrials.gov in the United States, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available, and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary, interim or topline data from our clinical trials. These interim updates are based on a preliminary analysis of then-available data, and the results

and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously reported. As a result, topline data should be viewed with caution until the final data are available. In addition, we may report interim analyses of only certain endpoints rather than all endpoints. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse changes between interim data and final data could significantly harm our business and prospects. Some of our trials may be open-label studies, where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. For example, prior MDD studies have exhibited a high placebo effect. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. Therefore, it is possible that positive results observed in open-label trials will not be replicated in later placebo-controlled trials. Additionally, the trial design differences and placebo effects that may be possible in clinical research for the indications we are studying may make it difficult to extrapolate the results of earlier clinical trials to later clinical trials or to interpret the clinical data in any of our trials. Further, additional disclosure of interim data by us or by our competitors in the future could result in volatility in the price of our common stock.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is typically selected from a more extensive amount of available information. You or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the preliminary or topline data that we report differ from late, final or actual results, or if others, including the FDA and comparable foreign regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize our product candidates may be harmed, which could harm our business, financial condition, results of operations and prospects.

The markets for PRAX-114 for MDD and PMD, PRAX-944 for ET, PRAX-562 for multiple rare neurological conditions and any other product candidates we may develop may be smaller than we expect.

Our estimates of the potential market opportunity for PRAX-114 for the treatment of MDD and PMD, PRAX-944 for the treatment of ET, PRAX-562 for the treatment of multiple rare neurological conditions, including epilepsy, cephalgias and pain, as well as any other product candidates, include several key assumptions based on our industry knowledge, industry publications and third-party research reports. There can be no assurance that any of these assumptions are, or will remain, accurate. If the actual market for PRAX-114, PRAX-944 and PRAX-562 for these or other indications, or for any other product candidate we may develop, is smaller than we expect, our revenues, if any, may be limited and it may be more difficult for us to achieve or maintain profitability.

We may not be successful in our efforts to identify or discover additional product candidates in the future.

Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development or commercialization for a number of reasons, including:

- our inability to design such product candidates with the pharmacological or pharmacokinetic properties that we desire; or
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be medicines that will receive marketing approval and achieve market acceptance.

Research programs to identify new product candidates require substantial technical, financial and human resources. If we are unable to identify suitable compounds for preclinical and clinical development, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

We may in the future conduct clinical trials for our product candidates in the United States, Europe or other jurisdictions, and the FDA, the European Medicines Agency, or EMA, and applicable foreign regulatory authorities may not accept data from trials conducted outside those respective jurisdictions.

We may in the future choose to conduct one or more of our clinical trials in the United States, Europe or in other foreign jurisdictions outside of Australia and New Zealand where our trials currently are being conducted for PRAX-114, PRAX-944 and PRAX-562. The acceptance of study data from preclinical studies and clinical trials conducted outside those jurisdictions may be subject to certain conditions for acceptance. For example, in cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice; and (ii) the trials were performed by clinical investigators of recognized competence and pursuant to Good Clinical Practices, or GCP, regulations. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory bodies, such as the EMA, have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA, EMA or any applicable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA, EMA or any applicable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction.

We plan to seek orphan drug designation for one or more of our product candidates, but we may be unable to obtain or maintain such a designation or the benefits associated with orphan drug status, including marketing exclusivity, which may cause our revenue, if any, to be reduced.

In the United States, under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested and granted by the FDA before a new NDA is submitted. In the United States, orphan drug designation entitles a party to financial

incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, it will disclose publicly the generic identity of the drug and its potential orphan use. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Such a designation may also be revoked by the FDA in certain circumstances, such as if the agency finds that the applicant's request for designation request omitted material information required under the Orphan Drug Act and its implementing regulations.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity. This means that the FDA may not approve any other marketing applications for the same drug and the same indication for seven years, except in limited circumstances such as a showing of clinical superiority to the product with orphan exclusivity or if FDA finds that the holder of the orphan exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan product to meet the needs of patients with the disease or condition for which the drug was designated. Furthermore, the FDA can waive orphan exclusivity if the applicant is unable to manufacture sufficient supply of the product subject to a period of orphan drug marketing exclusivity. Because we are developing PRAX-562 and PRAX-222 for indications we believe to be rare, we expect to pursue orphan designations for our candidates as applicable in the jurisdictions where development activities are planned.

A Breakthrough Therapy Designation by the FDA may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

We do not currently have Breakthrough Therapy Designation for any of our product candidates, but we may seek Breakthrough Therapy Designation for any product candidate that we plan to develop in the United States if we believe the qualifying criteria for such a designation can be met. A Breakthrough Therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as Breakthrough Therapies by the FDA are also eligible for accelerated approval and priority review.

The FDA has discretion to determine whether the criteria for a Breakthrough Therapy has been met and whether to grant a Breakthrough Therapy Designation to an investigational product. Accordingly, even if we believe a product candidate we develop meets the criteria for designation as a Breakthrough Therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even after granting Breakthrough Therapy Designation to our product candidates, the FDA may later decide that the drugs no longer meet the conditions for qualification and withdraw the designation.

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

We do not currently have Fast Track Designation for any of our product candidates, but we may seek such a designation for the product candidates we plan to develop in the United States if we

believe the qualifying criteria for such a designation have been met. If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. Many drugs that have received Fast Track Designation have failed to obtain regulatory approval.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, but a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants approval of a product candidate, comparable regulatory authorities in other jurisdictions, including Australia and Europe, must also approve the manufacturing, marketing and sale of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must also be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to governmental approval.

Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we or any partner we work with fail to comply with the regulatory requirements in international markets or fail to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Any of our current and future product candidates for which we, or any future collaborators, obtain regulatory approval in the future will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, our product candidates could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Any of our product candidates for which we, or any future collaborators, obtain regulatory approval, as well as the manufacturing processes, post-approval studies, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA, if approved in the United States, and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. We and our contract manufacturers will also be subject to user fees and periodic inspection by the FDA in the United States and other regulatory authorities, including similar regulatory authorities in foreign jurisdictions, to monitor compliance with these

requirements and the terms of any product approval we may obtain. Even if regulatory approval of a product candidate is granted, the approval may be subject to limitations on the indications or uses for which the product may be marketed or to the conditions of approval, including the potential requirement in the United States to implement a REMS.

Regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. In the United States, the FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use of approved drug products. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. If we, or any future collaborators, do not market any of our product candidates for which we, or they, receive regulatory approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing if it is alleged that we are doing so. In the United States, violation of the Federal Food, Drug and Cosmetic Act and other statutes relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws, including the federal False Claims Act, or the FCA.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on the manufacturing of such products;
- restrictions on the labeling or marketing of such products;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- exclusion from U.S. federal health care programs such as Medicare and Medicaid;
- suspension or withdrawal of regulatory approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Even if we, or any future collaborators, obtain regulatory approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue.

Once regulatory approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product

candidates for which we or they obtain regulatory approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive regulatory requirements, including under FDA authorities ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by regulatory authorities to monitor and ensure compliance with cGMPs. Despite our efforts to inspect and verify regulatory compliance, one or more of our third-party manufacturing vendors may be found on regulatory inspection to be not in compliance with cGMP requirements, which may result in shutdown of the third-party vendor or invalidation of drug product lots or processes. In some cases, a product recall may be warranted or required, which would materially affect our ability to supply and market our drug products.

Accordingly, assuming we, or any future collaborators, receive regulatory approval for one or more of our product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, or any future collaborators, are not able to comply with post-approval regulatory requirements, we, or any future collaborators, could have the regulatory approvals for our products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Changes in regulatory requirements or regulatory guidance or unanticipated events during our non-clinical studies and clinical trials of our product candidates may occur, which may result in changes to non-clinical studies and clinical trial protocols or the need for additional non-clinical studies and clinical trials, which could result in increased costs to us and could delay our development timelines.

Changes in regulatory requirements or regulatory guidance or unanticipated events during our non-clinical studies and clinical trials may force us to amend non-clinical studies and clinical trial protocols or the applicable regulatory authority may impose additional non-clinical studies and clinical trial requirements. Amendments or changes to our clinical trial protocols would generally require resubmission to the applicable regulatory authority and IRBs for review and approval, which may adversely impact the cost, timing or successful completion of clinical trials. These decisions may increase costs, and cause us not to meet expected timelines and, correspondingly, our business and financial prospects could be adversely affected. Similarly, amendments to our non-clinical studies may adversely impact the cost, timing or successful completion of those non-clinical studies. If we experience delays completing, or if we terminate, any of our non-clinical studies or clinical trials, or if we are required to conduct additional non-clinical studies or clinical trials, the development pathway, and ultimately the commercial prospects, for our product candidates may be harmed and our ability to generate product revenue from resulting products, if any, will be delayed.

We could be subject to product liability lawsuits based on the use of our product candidates in clinical testing or, if obtained, following our products' marketing approval and commercialization. Product liability lawsuits brought against us or any of our future collaborators could divert our

resources and attention, require us to cease clinical testing, cause us to incur substantial liabilities or limit commercialization of our product candidates.

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing and use of biopharmaceutical products. Currently, we have no products that have been approved for commercial sale; however, the use of our product candidates by us and any collaborators in clinical trials may expose us to liability claims. We will face an even greater risk if product candidates are approved by regulatory authorities and introduced commercially. Product liability claims may be brought against us or our partners if any product candidate we develop allegedly causes injury or is found to be otherwise unsuitable for human use during product testing, manufacturing, marketing or sale. Any such product liability claim may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. Such claims could be made by participants enrolled in our clinical trials, patients, health care providers, biopharmaceutical companies, our collaborators or others using, administering or selling any of our future approved products. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources.

Regardless of the merits or eventual outcome, product liability claims may result in:

- decreased demand for any of our future approved products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- significant litigation costs;
- substantial monetary awards to, or costly settlements with, patients or other claimants;
- product recalls or a change in the indications for which any approved drug products may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize our product candidates.

Although the clinical trial process is designed to identify and assess potential side effects, clinical development does not always fully characterize the safety and efficacy profile of a new medicine, and it is always possible that a drug, even after regulatory approval, may exhibit unforeseen side effects. If our product candidates were to cause adverse side effects during clinical trials or after approval, we may be exposed to substantial liabilities. Physicians and patients may not comply with any warnings that identify known potential adverse effects and patients who should not use our product candidates. If any of our product candidates are approved for commercial sale, we will be highly dependent upon consumer perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity associated with illness or other adverse effects resulting from physicians' or patients' use or misuse of our products or any similar products distributed by other companies.

Although we maintain product liability insurance coverage including clinical trial liability, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if we commercialize any product that receives regulatory approval. In

addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could harm our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from biopharmaceutical companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing and commercialization.

Specifically, we face competition with $GABA_A$ receptor modulator programs in development targeting MDD, including that of SAGE Therapeutics, as well as other programs in clinical development targeting other mechanisms of action and approved therapies such as selective serotonin reuptake inhibitors, or SSRIs; T-type calcium channel inhibitor programs in development targeting ET, including that of Jazz Pharmaceuticals, as well as other programs in clinical development targeting other mechanisms of action, and approved therapies, such as propranolol, and off-label therapies, such as primidone; and sodium channel blocker programs in development for DEE, including those of SK-Pharma and Xenon Pharmaceuticals, as well as other programs in clinical development targeting other mechanisms of action, and approved therapies including other existing ion channel blockers.

If any of these competitors or competitors for our other product candidates receive FDA approval before we do, our product candidates would not be the first treatment on the market, and our market share may be limited. In addition to competition from other companies targeting our target indications, any products we may develop may also face competition from other types of therapies.

Many of our current or potential competitors, either alone or with their strategic partners, have:

- greater financial, technical and human resources than we have at every stage of the discovery, development, manufacture and commercialization of products;
- more extensive experience in preclinical testing, conducting clinical trials, obtaining regulatory approvals, and in manufacturing, marketing and selling drug products;
- products that have been approved or are in late stages of development; and
- collaborative arrangements in our target markets with leading companies and research institutions.

Mergers and acquisitions in the biopharmaceutical industry may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are

more convenient or are less expensive than any products that we may develop. Furthermore, currently approved products could be discovered to have application for treatment of our targeted disease indications or similar indications, which could give such products significant regulatory and market timing advantages over our product candidates. Our competitors also may obtain FDA, EMA or other comparable foreign regulatory approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications that we are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, we could face litigation or other proceedings with respect to the scope, ownership, validity and/or enforceability of our patents relating to our competitors' products and our competitors may allege that our products infringe, misappropriate or otherwise violate their intellectual property. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

Risks Related to the Commercialization of our Product Candidates

Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Even if the FDA or a comparable foreign regulatory authority approves any of our product candidates, we will be subject to ongoing regulatory requirements in the applicable jurisdictions for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies and submission of safety, efficacy and other post-market information. In addition, we will be subject to continued compliance with cGMP and GCP requirements for any clinical trials that we conduct post-approval.

Manufacturers and their facilities are required to comply with extensive regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any marketing application, and previous responses to inspection observations. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and surveillance to monitor the safety and efficacy of the product candidate. In the United States, the FDA may also require a REMS program as a condition of approval of our product candidates, which could entail requirements for long-term patient follow-up, a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, we will have to comply with requirements including submissions of safety and other post-marketing information and reports and registration.

In the United States, the FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later

discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information or other restrictions; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the manufacturing of our products, the approved manufacturers or the manufacturing process;
- withdrawal of the product from the market or voluntary product recalls;
- requirements to conduct post-marketing studies or clinical trials;
- fines, restitution or disgorgement of profits or revenues;
- warning or untitled letters from the FDA or comparable notice of violations from foreign regulatory authorities;
- suspensions of any of our ongoing clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or withdrawal of marketing approvals;
- product seizure or detention or refusal to permit the import or export of products; and
- consent decrees, injunctions or the imposition of civil or criminal penalties.

Regulatory authorities strictly regulate marketing, labeling, advertising and promotion of products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. However, in the United States, companies may share truthful and not misleading information that is not inconsistent with the labeling. The FDA and other regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses and a company that is found to have improperly promoted off-label uses may be subject to significant liability. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may also lead to investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws. Accordingly, to the extent we receive marketing approval for one or more of our product candidates, we and our third-party partners will continue to expend time, money and effort in all areas of regulatory compliance, including promotional and labeling compliance, manufacturing, production, product surveillance and quality control.

The policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow to, or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Our commercial success depends upon attaining significant market acceptance of our drug product candidates, if approved, among physicians, patients, third-party payors and other members of the medical community.

Even if any of the product candidates we develop receives marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, such as Medicare and Medicaid programs and managed care organizations in the United States, and others in the medical community. In addition, the availability of coverage by third-party payors may be affected by existing and future health care reform measures designed to reduce the cost of health care. If the

product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable.

The degree of market acceptance of any product candidate, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- the ability to offer our products, if approved, for sale at competitive prices;
- relative convenience and ease of administration compared to alternative treatments;
- perceptions by the medical community, physicians, and patients, regarding the safety and effectiveness of our products and the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the size of the market for such product candidate, based on the size of the patient subsets that we are targeting, in the territories for which we gain regulatory approval;
- the recommendations with respect to our product candidates in guidelines published by various scientific organizations applicable to us and our product candidates;
- the strength of sales, marketing and distribution support;
- the timing of any such marketing approval in relation to other product approvals;
- any restrictions on concomitant use of other medications;
- support from patient advocacy groups;
- the ability to obtain sufficient third-party coverage and adequate reimbursement; and
- the prevalence and severity of any side effects.

If government and other third-party payors do not provide coverage and adequate reimbursement levels for any products we commercialize, market acceptance and commercial success would be reduced.

The successful commercialization of our product candidates in the United States will depend in part on the extent to which third-party payors, including governmental authorities and private health insurers, provide coverage and adequate reimbursement levels, as well as implement pricing policies favorable for our product candidates. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States and in other countries, patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. The availability of coverage and adequacy of reimbursement for our products by third-party payors, including government health care programs (e.g., Medicare, Medicaid, TRICARE), managed care providers, private health insurers, health maintenance organizations and other organizations is essential for most patients to be able to afford medical services and biopharmaceutical products such as our product candidates. Third-party payors decide which medications they will pay for and establish reimbursement levels.

In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS. CMS decides whether and to what extent our products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a

substantial degree. Factors payors consider in determining reimbursement are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Our ability to successfully commercialize our product candidates will depend in part on the extent to which coverage and adequate reimbursement for our products and related treatments will be available from third-party payors. Moreover, a payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. If coverage and adequate reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

In the United States, no uniform policy for coverage and reimbursement for products exists among third-party payors. Therefore, coverage and reimbursement for our products can differ significantly from payor to payor. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payor will pay for the product. One payor's determination to provide coverage for a product does not assure that other payors will also provide coverage and reimbursement for the product. Third-party payors may also limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication. We cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, our product candidates.

A decision by a third-party payor not to cover or not to separately reimburse for our medical products or therapies using our products could reduce physician utilization of our products once approved. Assuming there is coverage for our product candidates, or therapies using our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and reimbursement in the United States will be available for our current or future product candidates, or for any procedures using such product candidates, and any reimbursement that may become available may not be adequate or may be decreased or eliminated in the future.

Further, increasing efforts by third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates may nonetheless not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third-party payors in connection with the potential sale of any of our product candidates.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, in the European Union, Member States can restrict the range of medicinal products for which their national health insurance systems provide reimbursement and they can control the prices of medicinal products for human use. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. A Member State may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Approaches between Member States are diverging. For example, in France, effective market access will be supported by agreements with hospitals and products may be reimbursed by the Social Security Fund. The price of medicines is negotiated with the Economic Committee for Health Products, or CEPS. There can be no assurance that any country that has price controls or reimbursement limitations for biopharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our product candidates. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower.

Even if we obtain approval of any of our product candidates in the United States or Europe, we may never obtain approval or commercialize such products in other countries, which would limit our ability to realize their full market potential.

In order to market any products in the United States or European Union, we must establish and comply with numerous and varying regulatory requirements regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals outside of where our clinical trials currently have been conducted could result in significant delays, difficulties and costs for us and may require additional preclinical studies or clinical trials which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, our ability to realize the full market potential of our products will be harmed.

We currently have no marketing and sales organization and have no experience as a company in commercializing products, and we may have to invest significant resources to develop these capabilities. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may not be able to generate product revenue.

We have no internal sales, marketing or distribution capabilities, nor have we commercialized a product. If any of our product candidates ultimately receives regulatory approval, we expect to establish a marketing and sales organization with technical expertise and supporting distribution capabilities to commercialize each such product in major markets, which will be expensive and time consuming. We have no prior experience as a company in the marketing, sale and distribution of biopharmaceutical products and there are significant risks involved in building and managing a sales

organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may also choose to collaborate with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. We may not be able to enter into collaborations or hire consultants or external service providers to assist us in sales, marketing and distribution functions on acceptable financial terms, or at all. In addition, our product revenues and our profitability, if any, may be lower if we rely on third parties for these functions than if we were to market, sell and distribute any products that we develop ourselves. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we are not successful in commercializing our products, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses.

Risks Related to Ongoing Regulatory and Legal Compliance

Our relationships with healthcare providers and physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Although we do not currently have any products on the market, upon commercialization of our product candidates, if approved, we will be subject to additional health care statutory and regulatory requirements and oversight by federal and state governments in the United States as well as foreign governments in the jurisdictions in which we conduct our business. Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare providers, third-party payors, customers and others may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. In particular, the research of our product candidates, as well as the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business or financial arrangements.

The applicable federal, state and foreign healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual, or the purchase, lease, order, arrangement or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. The term remuneration has been interpreted broadly to include anything of value. Further, courts have found that if “one purpose” of renumeration is to induce referrals, the federal Anti-Kickback statute is violated. Violations are subject to significant civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment and exclusion from government

healthcare programs. In addition, a claim submitted for payment to any federal healthcare program that includes items or services that were made as a result of a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. The Anti-Kickback Statute has been interpreted to apply to arrangements between biopharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers, among others, on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;

- the federal civil and criminal false claims laws, including the FCA, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, false, fictitious or fraudulent claims for payment to, or approval by Medicare, Medicaid, or other federal healthcare programs; knowingly making, using or causing to be made or used, a false record or statement material to a false, fictitious or fraudulent claim or an obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding, decreasing or concealing an obligation to pay money to the federal government. A claim that includes items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim under the FCA. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. The FCA also permits a private individual acting as a "whistleblower" to bring qui tam actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery or settlement. When an entity is determined to have violated the FCA, the government may impose civil fines and penalties for each false claim, plus treble damages, and exclude the entity from participation in Medicare, Medicaid and other federal healthcare programs;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private), and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statement or representation, or making or using any false writing or document knowing the same to contain any materially false fictitious or fraudulent statement or entry in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA fraud provisions without actual knowledge of the statute or specific intent to violate it;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, certain requirements relating to the privacy, security and transmission of individually identifiable health information on certain covered healthcare providers, health plans and healthcare clearinghouses, known as covered entities, as well as their respective "business associates," those independent contractors or agents of covered entities that create, receive, maintain, transmit or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, there may be additional federal, state and non-U.S. laws which govern the privacy and security of health and other

personal information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts;

- the federal Physician Payments Sunshine Act, created under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or, collectively, the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to direct or indirect payments and other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made in the previous year to certain non-physician providers such as physician assistants and nurse practitioners;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous U.S. state, local and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers, and may be broader in scope than their federal equivalents; state and foreign laws that require biopharmaceutical companies to comply with the biopharmaceutical industry's voluntary compliance guidelines and other relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures or drug pricing; state and local laws that require the registration of biopharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information, some of which may be more stringent than those in the United States (such as the European Union, which adopted the General Data Protection Regulation, which became effective in May 2018) in certain circumstances, and may differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The distribution of biopharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of biopharmaceutical products.

If the FDA or a comparable foreign regulatory authority approves any of our product candidates, we will be subject to an expanded number of these laws and regulations and will need to expend resources to develop and implement policies and processes to promote ongoing compliance. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

It is possible that governmental and enforcement authorities will conclude that our business practices, including our arrangements with physicians and other healthcare providers, some of whom may receive stock options as compensation for services provided, may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to significant sanctions,

including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, reputational harm, exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restricting of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to similar penalties. Any action for violation of these laws, even if successfully defended, could cause us to incur significant legal expenses and divert management's attention from the operation of the business. In addition, the approval and commercialization of any product candidate we develop outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws. All of these could harm our ability to operate our business and our financial results.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

We may be subject to, or may in the future become subject to, US federal and state, and foreign laws and regulations imposing obligations on how we collect, use, disclose, store and process personal information. Our actual or perceived failure to comply with such obligations could result in liability or reputational harm and could harm our business. Ensuring compliance with such laws could also impair our efforts to maintain and expand our customer base, and thereby decrease our revenue.

In many activities, including the conduct of clinical trials, we may be subject to laws and regulations governing data privacy and the protection of health-related and other personal information. These laws and regulations govern our processing of personal data, including the collection, access, use, analysis, modification, storage, transfer, security breach notification, destruction and disposal of personal data.

The privacy and security of personally identifiable information stored, maintained, received or transmitted, including electronically, is subject to significant regulation in the United States and abroad. While we strive to comply with all applicable privacy and security laws and regulations, legal standards for privacy continue to evolve and any failure or perceived failure to comply may result in proceedings or actions against us by government entities or others, or could cause reputational harm, which could have a material adverse effect on our business.

Numerous foreign, federal and state laws and regulations govern collection, dissemination, use and confidentiality of personally identifiable health information, including state privacy and confidentiality laws (including state laws requiring disclosure of breaches), federal and state consumer protection and employment laws; HIPAA and European and other foreign data protection laws. These laws and regulations are increasing in complexity and number, and may change frequently and sometimes conflict. The European Union's omnibus data protection law, the General Data Protection Regulation, or GDPR, took effect in May 2018. The GDPR imposes numerous requirements on entities that process personal data in the context of an establishment in the European Economic Area, or EEA, or that process the personal data of data subjects who are located in the EEA. These requirements include, for example, establishing a basis for processing, providing notice to data subjects, developing procedures to vindicate expanded data subject rights, implementing appropriate technical and organizational measures to safeguard personal data and complying with restrictions on the cross-border transfer of personal data from the EEA to countries that the European Union does not consider

to have in place adequate data protection legislation, such as the United States. The GDPR additionally establishes heightened obligations for entities that process “special categories” of personal data, such as health data. Nearly all clinical trials involve the processing of these “special categories” of personal data, and thus processing of personal data collected during the course of clinical trials is subject to heightened protections under the GDPR. Violations of the GDPR can lead to penalties of up to $20 million or 4% of an entity’s annual turnover.

As a means to transfer personal data from the EEA to the U.S., U.S.-based companies may certify compliance with the privacy principles of the EU-U.S. Privacy Shield, or the Privacy Shield. Certification to the Privacy Shield, however, is not mandatory. If a U.S.-based company does not certify compliance with the Privacy Shield, it may rely on other authorized mechanisms to transfer personal data. Notably, the Privacy Shield is currently subject to challenge in the EU courts, and it is possible that it will be invalidated, which was the fate of its predecessor, the EU-U.S. Safe Harbor. In the event of invalidation of the Privacy Shield, U.S. companies that currently rely on the Privacy Shield as the basis for cross-border transfer of personal data will need to establish another basis for cross-border transfer of personal data.

HIPAA establishes a set of national privacy and security standards for the protection of individually identifiable health information, including protected health information, or PHI, by health plans, certain health care clearinghouses and health care providers that submit certain covered transactions electronically, or covered entities, and their “business associates,” which are persons or entities that perform certain services for, or on behalf of, a covered entity that involve creating, receiving, maintaining or transmitting PHI. While we are not currently a covered entity or business associate under HIPAA, we may receive identifiable information from these entities. Failure to receive this information properly could subject us to HIPAA’s criminal penalties, which may include fines up to $250,000 per violation and/or imprisonment. In addition, responding to government investigations regarding alleged violations of these and other laws and regulations, even if ultimately concluded with no findings of violations or no penalties imposed, can consume company resources and impact our business and, if public, harm our reputation.

In addition, various states, such as California and Massachusetts, have implemented similar privacy laws and regulations, such as the California Confidentiality of Medical Information Act, that impose restrictive requirements regulating the use and disclosure of health information and other personally identifiable information. In addition to fines and penalties imposed upon violators, some of these state laws also afford private rights of action to individuals who believe their personal information has been misused. California’s patient privacy laws, for example, provide for penalties of up to $250,000 and permit injured parties to sue for damages. In addition to the California Confidentiality of Medical Information Act, California also recently enacted the California Consumer Privacy Act of 2018, or CCPA, which was effective January 1, 2020. The CCPA has been characterized as the first “GDPR-like” privacy statute to be enacted in the United States because it mirrors a number of the key provisions of the EU General Data Protection Regulation. The CCPA establishes a new privacy framework for covered businesses in the State of California, by creating an expanded definition of personal information, establishing new data privacy rights for consumers imposing special rules on the collection of consumer data from minors and creating a new and potentially severe statutory damages framework for violations of the CCPA and for businesses that fail to implement reasonable security procedures and practices to prevent data breaches.

The interplay of federal and state laws may be subject to varying interpretations by courts and government agencies, creating complex compliance issues for us and potentially exposing us to additional expense, adverse publicity and liability. Further, as regulatory focus on privacy issues continues to increase and laws and regulations concerning the protection of personal information expand and become more complex, these potential risks to our business could intensify. The

legislative and regulatory landscape for privacy and data security continues to evolve, and there has been an increasing focus on privacy and data security issues which may affect our business. Failure to comply with current and future laws and regulations could result in government enforcement actions (including the imposition of significant penalties), criminal and/or civil liability for us and our officers and directors, private litigation and/or adverse publicity that negatively affects our business.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in U.S. and foreign regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the U.S. and in some foreign jurisdictions, there have been, and likely will continue to be, a number of legislative initiatives and regulatory changes regarding the healthcare system directed at broadening the availability of healthcare, improving the quality of healthcare, and containing or lowering the cost of healthcare. For example, in March 2010, the ACA was enacted, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacted the U.S. biopharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, expands the types of entities eligible for the 340B drug discount program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations; established annual fees and taxes on manufacturers of certain branded prescription drugs; and created a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (increased to 70% pursuant to the Bipartisan Budget Act of 2018, or BBA, effective as of January 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since its enactment, there have been numerous judicial, administrative, executive and legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Various portions of the ACA are currently undergoing legal and constitutional challenges in the United States Supreme Court; the Trump Administration has issued various Executive Orders which eliminated cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices; and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the ACA. Also, in December 2018, CMS issued a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program. Since then, the ACA risk adjustment program payment parameters have been updated annually. It is unclear whether the ACA will be overturned, repealed, replaced or further amended. We cannot predict what affect further changes to the ACA would have on our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, on August 2, 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs, including aggregate reductions of Medicare payments to

providers of up to 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030, unless additional Congressional action is taken. However, pursuant to the Coronavirus Aid, Relief and Economic Security Act, or CARES Act, these Medicare sequester reductions will be suspended from May 1, 2020 through December 31, 2020 due to the COVID-19 pandemic. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, the BBA, among other things, amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount (from 50% under the ACA to 70%) that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole”.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. Recently, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their products. Such scrutiny has resulted in several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, on March 10, 2020, the Trump administration sent “principles” for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. Additionally, the Trump administration previously released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contained proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. HHS has solicited feedback on some of these measures and has implemented others under its existing authority. For example, in May 2019, CMS issued a final rule that would allow Medicare Advantage Plans the option of using step therapy, a type of prior authorization, for Part B drugs beginning January 1, 2020. This final rule codified CMS’s policy change that was effective January 1, 2019. Although a number of these and other measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

At the state level in the United States, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biologic product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions on coverage or access could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates that we successfully commercialize or put pressure on our product pricing.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States or in any other jurisdictions. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the

adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

In the United States, inadequate funding for the FDA, the Securities and Exchange Commission, or the SEC, and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. Separately, in response to the COVID-19 pandemic, on March 10, 2020, the FDA announced its intention to temporarily postpone most inspections of foreign manufacturing facilities and along with routine surveillance inspections of domestic manufacturing facilities. On July 10, 2020, the FDA announced its goal of restarting domestic onsite inspections during the week of July 20 but such activities will depend on data about the virus' trajectory in a given state and locality and the rules and guidelines that are put in place by state and local governments. The FDA has developed a rating system to assist in determining when and where it is safest to conduct prioritized domestic inspections. In April 2020, the FDA stated that its New Drug Program was continuing to meet program user fee performance goals, but due to many agency staff working on COVID-19 activities, it was possible that the FDA would not be able to sustain that level of performance indefinitely. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, upon completion of this offering and in our operations as a public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

EU drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for our products in the European member states.

We ultimately intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of drugs is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our product candidates. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate coverage and

reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

Much like the federal Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of EU Member States, and in respect of the U.K. (which is longer a member of the EU), the U.K. Bribery Act of 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In addition, in most foreign countries, including the EEA, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for biopharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the EU do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales and the potential profitability of any of our product candidates in those countries would be negatively affected.

Legal, political and economic uncertainty surrounding the exit of the U.K. from the EU may be a source of instability in international markets, create significant currency fluctuations, adversely affect our operations in the U.K. and pose additional risks to our business, revenue, financial condition and results of operations.

On June 23, 2016, the U.K. held a referendum in which a majority of the eligible members of the electorate voted to leave the EU, commonly referred to as Brexit. Pursuant to Article 50 of the Treaty on EU, the U.K. ceased being a Member State of the EU on January 31, 2020. However, the terms of the withdrawal have yet to be fully negotiated. The implementation period began February 1, 2020 and will continue until December 31, 2020. During this 11-month period, the U.K. will continue to follow all of the EU's rules, the EU's pharmaceutical law remains applicable to the U.K. and the U.K.'s trading relationship will remain the same. However, regulations (including financial laws and regulations, tax and free trade agreements, intellectual property rights, data protection laws, supply chain logistics, environmental, health and safety laws and regulations, medicine licensing and regulations, immigration laws and employment laws), have yet to be addressed. This lack of clarity on future U.K. laws and

regulations and their interaction with the EU laws and regulations may negatively impact foreign direct investment in the U.K., increase costs, depress economic activity and restrict access to capital.

The uncertainty concerning the U.K.'s legal, political and economic relationship with the EU after Brexit may be a source of instability in the international markets, create significant currency fluctuations and/or otherwise adversely affect trading agreements or similar cross-border co-operation arrangements (whether economic, tax, fiscal, legal, regulatory or otherwise) beyond the date of Brexit.

These developments, or the perception that any of them could occur, may have a significant adverse effect on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and limit the ability of key market participants to operate in certain financial markets. In particular, it could also lead to a period of considerable uncertainty in relation to the U.K. financial and banking markets, as well as on the regulatory process in Europe. Asset valuations, currency exchange rates and credit ratings may also be subject to increased market volatility.

If the U.K. and the EU are unable to negotiate acceptable agreements or if other EU member states pursue withdrawal, barrier-free access between the U.K. and other EU member states or among the European Economic Area overall could be diminished or eliminated. The long-term effects of Brexit will depend on any agreements (or lack thereof) between the U.K. and the EU and, in particular, any arrangements for the U.K. to retain access to EU markets either during a transitional period from January 1, 2021 or more permanently.

Such a withdrawal from the EU is unprecedented, and it is unclear how the U.K.'s access to the European single market for goods, capital, services and labor within the EU, or single market, and the wider commercial, legal and regulatory environment, will impact our current and future operations (including business activities conducted by third parties and contract manufacturers on our behalf) and clinical activities in the U.K. In addition to the foregoing, our U.K. operations support our current and future operations and clinical activities in the EU and European Economic Area, or EEA, and these operations and clinical activities could be disrupted by Brexit.

We may also face new regulatory costs and challenges that could have an adverse effect on our operations. Depending on the terms of the U.K.'s withdrawal from the EU, the U.K. could lose the benefits of global trade agreements negotiated by the EU on behalf of its members, which may result in increased trade barriers that could make our doing business in the EU and the EEA more difficult. Since the regulatory framework in the U.K. covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit could materially impact the future regulatory regime with respect to the approval of our product candidates in the U.K. For instance, in November 2017, EU member states voted to move the EMA, the EU's regulatory body, from London to Amsterdam. Operations in Amsterdam commenced in March 2019, and the move itself may cause significant disruption to the regulatory approval process in Europe. It remains to be seen how, if at all, Brexit will impact regulatory requirements for product candidates and products in the U.K. Any delay in obtaining, or an inability to obtain, any regulatory approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the U.K. and/or the EU and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the U.K. and/or EU for our product candidates, which could significantly and materially harm our business. Even prior to any change to the U.K.'s relationship with the EU, the announcement of Brexit has created economic uncertainty surrounding the terms of Brexit and its consequences could adversely impact customer confidence resulting in customers reducing their spending budgets on our product candidates, if approved, which could adversely affect our business, financial condition, results of operations and could adversely affect the market price of our common stock.

Additional laws and regulations governing international operations could negatively impact or restrict our operations.

For our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The U.S. Foreign Corrupt Practices Act, or the FCPA, prohibits any U.S. individual or business entity from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the biopharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals and healthcare providers in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions. Recently the SEC and Department of Justice have increased their FCPA enforcement activities with respect to biotechnology and pharmaceutical companies. There is no certainty that all of our employees, agents or contractors, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information products classified for national security purposes, as well as certain products, technology and technical data relating to those products. If we continue and expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, the closing down of our facilities, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products, if approved, in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees and our business, prospects, operating results and financial condition. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could

exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Risks Related to Our Intellectual Property

Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

Our business will depend in large part on obtaining and maintaining patent, trademark and trade secret protection of our proprietary technologies and our product candidates, their respective components, synthetic intermediates, formulations, combination therapies and methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents that cover these activities and whether a court would issue an injunctive remedy. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected.

The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue, obtain, or maintain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third parties and are reliant on our licensors or licensees.

The strength of patents in the biotechnology and biopharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our technology, including our product candidates, or prevent others from designing around our claims. If the breadth or strength of protection provided by the patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced.

We cannot be certain that we were the first to file any patent application related to our technology, including our product candidates, and, if we were not, we may be precluded from obtaining patent protection for our technology, including our product candidates.

We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. Furthermore, for United States applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the United States Patent and Trademark Office, or USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. Similarly, for United States applications in which at least one claim is not entitled to a priority date before March 16, 2013, derivation proceedings can be instituted to determine whether the subject matter of a patent claim was derived from a prior inventor's disclosure.

We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent or patent application claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, would adequately protect our product candidates, or would be found by a court to be infringed by a competitor's technology or product. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that may issue that cover our products.

Recent or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. Under the enacted Leahy-Smith America Invents Act, or America Invents Act, enacted in 2013, the United States moved from a "first to invent" to a "first-to-file" system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently unclear as the USPTO only recently developed new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the "first-to-file" provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to the compositions of our product candidates but that are not covered by the claims of our patents or those of our licensors;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government in regards to any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents, as the case may be, or parts of our or their patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes which design around our patents or become hostile to us or the patents or patent applications on which they are named as inventors;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we have engaged in scientific collaborations in the past, and will continue to do so in the future. Such collaborators may develop adjacent or competing products to ours that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

We have entered into, and may enter into, license or other collaboration agreements that impose certain obligations on us. If we fail to comply with our obligations under such agreements with third parties, we could lose license rights that may be important to our business.

In connection with our efforts to expand our pipeline of product candidates, we may enter into certain licenses or other collaboration agreements in the future pertaining to the in-license of rights to

additional candidates. Such agreements may impose various diligence, milestone payment, royalty, insurance or other obligations on us. If we fail to comply with these obligations, our licensor or collaboration partners may have the right to terminate the relevant agreement, in which event we would not be able to develop or market the products covered by such licensed intellectual property.

Moreover, disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our product candidates, technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

In addition, we may have limited control over the maintenance and prosecution of these in-licensed patents and patent applications, or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by any future licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that is licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to patent protection, we rely heavily upon know-how and trade secret protection, as well as non-disclosure agreements and invention assignment agreements with our employees, consultants and third parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using physical and technological security measures. Such measures may not, for example, in the case of misappropriation of a trade secret by an employee or third-party with authorized access, provide adequate protection for our proprietary

information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, trade secrets may be independently developed by others in a manner that could prevent legal recourse by us. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any such information was independently developed by a competitor, our competitive position could be harmed.

In addition, courts outside the United States are sometimes less willing to protect trade secrets. If we choose to go to court to stop a third-party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology.

Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary technology by third parties. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets.

Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and biopharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and biopharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our product candidates, technologies or methods.

We also may be subject to other third party claims relating to alleged infringement of intellectual property or other proprietary rights, including breach of nondisclosure, nonuse, noncompetition and non-solicitation provisions, intellectual property assignment and ownership, and misuse or misappropriation of intellectual property, trade secrets and other confidential information, among others. If a court of competent jurisdiction finds us liable for any such claims, we may be prohibited from using certain intellectual property, trade secrets and confidential information, effectively blocking our ability to seek patent protection for our inventions and halting the progress of our clinical development and commercialization efforts. For example, on May 20, 2020, we received a cease and desist letter from Sage Therapeutics, Inc., or Sage, in which Sage alleges a claim that we improperly accessed and benefited from Sage confidential information in connection with the in-license of our PRAX-114 development program as a result of our employment or engagement of former Sage employees and consultants. We believe that there is no merit to these claims and intend to defend our position. However, an adverse result could harm our business and result of operations.

If a third-party claims that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third-party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third-party licenses its product rights to us, which it is not required to do;
- if a license is available from a third-party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products and any license that is available may be non-exclusive, which could result in our competitors gaining access to the same intellectual property; and
- redesigning our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure.

Our collaborators may assert ownership or commercial rights to inventions they develop from research we support or that we develop or otherwise arising from the collaboration.

We collaborate with several institutions, universities, medical centers, physicians and researchers in scientific matters and expect to continue to enter into additional collaboration agreements. In certain cases, we do not have written agreements with these collaborators, or the written agreements we have do not cover intellectual property rights. Also, we rely on numerous third parties to provide us with

materials that we use to conduct our research activities and develop our product candidates. If we cannot successfully negotiate sufficient ownership and commercial rights to any inventions that result from our use of a third-party collaborator's materials, or if disputes arise with respect to the intellectual property developed with the use of a collaborator's samples, or data developed in a collaborator's study, we may be limited in our ability to capitalize on the market potential of these inventions or developments.

Third parties may assert that we are employing their proprietary technology without authorization.

There may be third-party patents of which we are currently unaware with claims to compositions of matter, materials, formulations, methods of manufacture or methods for treatment that encompass the composition, use or manufacture of our product candidates. There may be currently pending patent applications of which we are currently unaware which may later result in issued patents that our product candidates or their use or manufacture may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patent were held by a court of competent jurisdiction to cover our product candidates, intermediates used in the manufacture of our product candidates or our materials generally, aspects of our formulations or methods of use, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible, or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and biopharmaceutical industries, we employ individuals who were previously employed at universities or other biotechnology or biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary

damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may not be successful in obtaining or maintaining necessary rights to develop any future product candidates on acceptable terms.

Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights.

Our product candidates may also require specific formulations to work effectively and efficiently and these rights may be held by others. We may develop products containing our compounds and pre-existing biopharmaceutical compounds. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Additionally, we sometimes collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of such program and our business and financial condition could suffer.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our current or future licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question or for other reasons. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

We may choose to challenge the patentability of claims in a third-party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-examination, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third-party's patent in patent opposition proceedings in the European Patent Office, or EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third-party alleging that the patent may be infringed by our product candidates or proprietary technologies.

In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to those owned by or in-licensed to us, we or, in the case of in-licensed technology, the licensor may have to participate in an interference or derivation proceeding declared by the USPTO to determine priority of invention in the United States. If we or one of our licensors is a party to an interference or derivation proceeding involving a U.S. patent application on inventions owned by or in-licensed to us, we may incur substantial costs, divert management's time and expend other resources, even if we are successful.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Litigation or interference proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be

compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In certain circumstances, even inadvertent noncompliance events may permanently and irrevocably jeopardize patent rights. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Any patents, if issued, covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensors initiate legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third-party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, inter partes review, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates.

Likewise, our current licensed or owned patents are expected to expire between 2029 and 2041, without taking into account any possible patent term adjustments or extensions. Our earliest patents may expire before, or soon after, our first product achieves marketing approval in the United States or foreign jurisdictions. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, results of operations, financial condition and prospects. Our current licensed or owned pending patent applications covering our proprietary technologies or our product candidates that if issued as patents are expected to expire from 2029 through 2041, without taking into

account any possible patent term adjustments or extensions. However, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of these patent applications.

Changes in patent law in the United States and in foreign jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 16, 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. On March 16, 2013, under the America Invents Act, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO on or after March 16, 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter-parties review and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biopharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively

expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of, and may require a compulsory license to, patents, trade secrets and other intellectual property protection, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our proprietary rights generally. The initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions such as patent term adjustments and/or extensions, may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not be

granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations and prospects could be materially harmed.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Risks Related to Our Dependence on Third Parties

We rely on third parties to assist in conducting our preclinical studies and clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or commercialize our product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

We have relied upon and plan to continue to rely on third parties, such as laboratories, CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our preclinical studies and clinical trials and expect to rely on these third parties to conduct preclinical studies and clinical trials of any other product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Although our reliance on these third parties for preclinical and clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. Moreover, human clinical research must comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and IRBs. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the regulatory authorities may require us to perform additional clinical trials before approving our product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, a regulatory authority will determine that any of our clinical trials comply with GCPs.

The third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These outside contractors may not assign as great a priority to our programs or pursue them as diligently as we would if we were undertaking such programs ourselves. These contractors may also have

relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical programs. If these third parties, including clinical investigators, do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

If our relationships with any third parties conducting our studies are terminated, we may be unable to enter into arrangements with alternative third parties on commercially reasonable terms, or at all. Switching or adding third parties to conduct our studies involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired preclinical and clinical development timelines. Although we carefully manage our relationships with third parties conducting our studies, we cannot assure that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material and adverse effect on our business, financial condition and results of operations.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or regulatory approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

Furthermore, we may also engage third parties to develop companion or complementary diagnostics for use in our clinical trials, as applicable, but such third parties may not be successful in developing such companion or complementary diagnostics, furthering the difficulty in identifying patients with the targeted eligibility criteria for our clinical trials. If we are required to develop companion or complementary diagnostics and are unable to do so or unable to obtain any required regulatory clearance or approval of those diagnostics, this could compromise our ability to seek participation in the U.S. in certain of the FDA's expedited review and development programs, including those that may accelerate clinical development and regulatory timelines, and could limit our ability to seek regulatory approval for our product candidates.

The manufacture of our product candidates is complex, and we may encounter difficulties in production. We currently rely completely on third parties to formulate and manufacture our preclinical, clinical trial and commercial drug supplies. The development and commercialization of any of our product candidates could be stopped, delayed or made less profitable if those third parties fail to provide us with sufficient quantities of such drug supplies or fail to do so at acceptable quality levels, including in accordance with rigorously enforced regulatory requirements or contractual obligations, and our operations could be harmed as a result.

The processes involved in manufacturing our drug product candidates are complex, expensive, highly-regulated and subject to multiple risks. Further, as product candidates are developed through preclinical studies to late-stage clinical trials towards approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could require the conduct of bridging studies and could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials. We have limited experience in drug formulation or

manufacturing. Currently, we rely on an extensive network of contract manufacturers, and in some cases sole source suppliers, for the production of our product candidates for current and planned clinical trials.

In order to conduct clinical trials of our product candidates, or supply commercial products, if approved, we will need to manufacture them in large quantities. Our contract development and manufacturing organizations, or CDMOs, may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If our CDMOs are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing and clinical trials of that product candidate may be delayed or become infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business. The same risk would apply to our internal manufacturing facilities, should we in the future decide to build internal manufacturing capacity. In addition, building internal manufacturing capacity would carry significant risks in terms of being able to plan, design and execute on a complex project to build manufacturing facilities in a timely and cost-efficient manner, and the resources associated with ensuring the ongoing regulatory compliance of such manufacturing facilities would be significant.

In addition, the manufacturing process for any products that we may develop is subject to FDA, EMA, and foreign regulatory authority approval processes and continuous oversight, and we will need to contract with manufacturers who can meet all applicable FDA, EMA and foreign regulatory authority requirements, including complying with current good manufacturing practices, or cGMPs, on an ongoing basis. Although our agreements with our CDMOs require them to perform according to certain cGMP requirements such as those relating to quality control, quality assurance and qualified personnel, we cannot control the ability of our CDMOs to implement and maintain these standards. If we or our third-party manufacturers are unable to reliably produce products to specifications acceptable to the FDA, EMA or other regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CDMOs will be able to manufacture the approved product to specifications acceptable to the FDA, EMA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product, or to meet potential future demand. Any of these challenges could delay completion of clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates, impair commercialization efforts, increase our cost of goods and have an adverse effect on our business, financial condition, results of operations and growth prospects.

If any third-party manufacturer of our product candidates is unable to increase the scale of its production of our product candidates, and/or increase the product yield of its manufacturing, then our costs to manufacture the product may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of our product candidates that we may develop, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of the product. The transition to larger scale production could prove difficult. In addition, if our third party manufacturers are not able to optimize their manufacturing processes to increase the product yield for our product candidates, or if they are unable to produce increased amounts of our product candidates while maintaining the quality of the product, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operation.

We depend on third-party suppliers for key raw materials used in our manufacturing processes, some of which are our sole source of supply, and the loss of these third-party suppliers or their inability to supply us with adequate raw materials could harm our business.

We rely on our CDMOs to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical trials. We do not have, nor do we expect to enter into, any agreements for the commercial production of these raw materials, and we do not expect to have any control over the process or timing of our CDMOs' acquisition of raw materials needed to produce our product candidates. Any significant delay in the supply of a product candidate or the raw material components thereof for an ongoing clinical trial due to a manufacturer's need to replace a third-party supplier of raw materials could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates. Additionally, if our future manufacturers or we are unable to purchase these raw materials to commercially produce any of our product candidates that gains regulatory approvals, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates.

Furthermore, for those third-party suppliers who may be our sole source of supply of certain materials, we may not have arrangements in place for a redundant or second-source supply of any such materials in the event any of our current suppliers cease their operations for any reason. Establishing additional or replacement suppliers for the raw materials used in our product candidates, if required, may not be accomplished quickly. If we are able to find a replacement supplier, such replacement supplier would need to be qualified and may require additional regulatory inspection or approval, which could result in further delay.

We expect to depend on collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.

We may seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. For example, we have a collaboration with Ionis Pharmaceuticals, Inc. to further our development of product candidates and to enhance our research efforts directed to developing a product candidate for the treatment of epilepsy. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, biotechnology companies and academic institutions. If we enter into any such arrangements with any third parties, we will likely have shared or limited control over the amount and timing of resources that our collaborators dedicate to the development or potential commercialization of any product candidates we may seek to develop with them. Our ability to generate revenue from these arrangements with commercial entities will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs, or any product candidates we may develop, pose the following risks to us:

- collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not properly obtain, maintain, enforce or defend intellectual property or proprietary rights relating to our product candidates or research programs, or may use our proprietary information in such a way as to expose us to potential litigation or other intellectual property related proceedings, including proceedings challenging the scope, ownership, validity and enforceability of our intellectual property;

- collaborators may own or co-own intellectual property covering our product candidates or research programs that results from our collaboration with them, and in such cases, we may not have the exclusive right to commercialize such intellectual property or such product candidates or research programs;
- we may need the cooperation of our collaborators to enforce or defend any intellectual property we contribute to or that arises out of our collaborations, which may not be provided to us;
- collaborators may control certain interactions with regulatory authorities, which may impact our ability to obtain and maintain regulatory approval of our products candidates;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or research programs or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborators may decide to not pursue development and commercialization of any product candidates we develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates or research programs if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators may restrict us from researching, developing or commercializing certain products or technologies without their involvement;
- collaborators with marketing and distribution rights to one or more product candidates may not commit sufficient resources to the marketing and distribution of such product candidates;
- we may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control;
- collaborators may grant sublicenses to our technology or product candidates or undergo a change of control and the sublicensees or new owners may decide to take the collaboration in a direction which is not in our best interest;
- collaborators may become bankrupt, which may significantly delay our research or development programs, or may cause us to lose access to valuable technology, know-how or intellectual property of the collaborator relating to our products, product candidates or research programs;
- key personnel at our collaborators may leave, which could negatively impact our ability to productively work with our collaborators;
- collaborations may require us to incur short and long-term expenditures, issue securities that dilute our stockholders or disrupt our management and business;
- if our collaborators do not satisfy their obligations under our agreements with them, or if they terminate our collaborations with them, we may not be able to develop or commercialize product candidates as planned;

- collaborations may require us to share in development and commercialization costs pursuant to budgets that we do not fully control and our failure to share in such costs could have a detrimental impact on the collaboration or our ability to share in revenue generated under the collaboration;
- collaborations may be terminated in their entirety or with respect to certain product candidates or technologies and, if so terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates or technologies; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our development or commercialization program under such collaboration could be delayed, diminished or terminated.

We may face significant competition in seeking appropriate collaborations. Recent business combinations among biotechnology and pharmaceutical companies have resulted in a reduced number of potential collaborators. In addition, the negotiation process is time-consuming and complex, and we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

If we enter into collaborations to develop and potentially commercialize any product candidates, we may not be able to realize the benefit of such transactions if we or our collaborator elects not to exercise the rights granted under the agreement or if we or our collaborator are unable to successfully integrate a product candidate into existing operations and company culture. The failure to develop and commercialize a product candidate pursuant to our agreements with our current or future collaborators could prevent us from receiving future payments under such agreements, which could negatively impact our revenues. In addition, if our agreement with any of our collaborators terminates, our access to technology and intellectual property licensed to us by that collaborator may be restricted or terminate entirely, which may delay our continued development of our product candidates utilizing the collaborator's technology or intellectual property or require us to stop development of those product candidates completely. We may also find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. Many of the risks relating to product development, regulatory approval, and commercialization described in this "Risk Factors" section also apply to the activities of our collaborators and any negative impact on our collaborators may adversely affect us.

We may acquire businesses or products, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions or alliances.

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and

marketing any new products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

Risks Related to Employee Matters, Managing Our Business and Operations

Our business is subject to economic, political, regulatory and other risks associated with international operations.

Our business is subject to risks associated with conducting business internationally. Some of our suppliers and collaborative relationships are located outside the United States. Accordingly, our future results could be harmed by a variety of factors, including:

- economic weakness, including inflation, or political instability in particular non-U.S. economies and markets;
- differing and changing regulatory requirements in non-U.S. countries;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- difficulties in compliance with non-U.S. laws and regulations;
- changes in non-U.S. regulations and customs, tariffs and trade barriers;
- changes in non-U.S. currency exchange rates and currency controls;
- changes in a specific country's or region's political or economic environment;
- trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or non-U.S. governments;
- negative consequences from changes in tax laws;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- difficulties associated with staffing and managing international operations, including differing labor relations
- potential liability under the FCPA, U.K. Bribery Act of 2010 or comparable foreign laws;
- business interruptions resulting from geo-political actions, including war and terrorism, natural disasters including earthquakes, typhoons, floods and fires, or health epidemics such as COVID-19; and
- cyberattacks, which are growing in frequency, sophistication and intensity, and are becoming increasingly difficult to detect.

These and other risks associated with our planned international operations may materially adversely affect our ability to attain profitable operations.

A pandemic, epidemic or outbreak of an infectious disease in the United States or worldwide may adversely affect our business.

If a pandemic, epidemic or outbreak of an infectious disease occurs in the United States or worldwide our business may be adversely affected. In December 2019, a novel strain of coronavirus named SARS-CoV-2 was identified in Wuhan, China. This virus continues to spread globally, including in the United States and the disease it causes, COVID-19, has been declared a pandemic by the World

Health Organization. The COVID-19 pandemic has impacted the global economy and may impact our operations, including the potential interruption of our clinical trial activities, regulatory reviews and our supply chain. For example, the COVID-19 pandemic may delay enrollment in our clinical trials due to prioritization of hospital resources toward the outbreak or other factors, and some patients may be unwilling to enroll in our trials or be unable to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services, which would delay our ability to conduct clinical trials or release clinical trial results and could delay our ability to obtain regulatory approval and commercialize our product candidates. Furthermore, the spread of the virus may affect the operations of key governmental agencies, such as the FDA, which may delay the development or approval process for our product candidates.

The spread of an infectious disease, including COVID-19, may also result in the inability of our suppliers to deliver components or raw materials on a timely basis or at all. In addition, hospitals may reduce staffing and reduce or postpone certain treatments in response to the spread of an infectious disease. Such events may result in a period of business disruption, and in reduced operations, or doctors and medical providers may be unwilling to participate in our clinical trials, any of which could materially affect our business, financial condition and results of operations. We continue to closely monitor the COVID-19 pandemic as we evolve our business continuity plans, clinical development plans and response strategy. The extent to which the COVID-19 pandemic impacts our business will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of the novel coronavirus and the actions to contain the coronavirus or treat its impact, among others. At present, we are not experiencing significant impact or delays from the COVID-19 pandemic on our business, operations and, if approved, commercialization plans. In addition, we have taken steps to mitigate against COVID-19 pandemic-related delays, and may take additional measures, intended to help minimize the risk of the virus to our employees, including temporarily requiring all employees to work remotely, suspending all non-essential travel worldwide for our employees, and discouraging employee attendance at industry events and in-person work-related meetings, which could negatively affect our business.

A significant outbreak of other infectious diseases in the future also could result in a widespread health crisis that could adversely affect the economies and financial markets worldwide, resulting in an economic downturn that could impact our business, financial condition and results of operations.

We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.

Our success depends, and will likely continue to depend, upon our ability to hire, retain the services of our current executive officers, principal consultants and others, including Marcio Souza, our President and Chief Executive Officer, Bernard Ravina, our Chief Medical Officer, and Stuart Chaffee, our Chief Financial Officer. We have entered into employment agreements with Mr. Souza, Dr. Ravina and Dr. Chaffee, but they may terminate their employment with us at any time. The loss of their services might impede the achievement of our research, development and commercialization objectives. We do not maintain “key person” insurance for any of our executives or other employees.

Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years. Replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully.

Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous

pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategies. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

We only have a limited number of employees to manage and operate our business. If we are unable to hire to retain adequate personnel, then we may not be able to meet our operational goals.

As of September 30, 2020, we had 50 full-time employees and one part-time employee. Our focus on the clinical development of PRAX-114, PRAX-944 and PRAX-562 requires us to optimize cash utilization and to manage and operate our business in a highly efficient manner. We cannot ensure that we will be able to hire and/or retain adequate staffing levels to develop PRAX-114, PRAX-944 and PRAX-562 or to run our operations and/or to accomplish all of the objectives that we otherwise would seek to accomplish.

Our employees, independent contractors, consultants, collaborators and contract research organizations may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators and contract research organizations may engage in fraudulent conduct or other illegal activity. Misconduct by those parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates:

- regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities;
- manufacturing standards;
- federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities; and
- laws that require the accurate reporting of financial information or data.

Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of product materials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this kind of activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, disgorgement, integrity oversight and reporting obligations, possible exclusion from participation in the United States in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings

and curtailment of our operations, any of which could have a material adverse effect on our ability to operate our business and our results of operations.

We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of regulatory affairs and sales, marketing and distribution, as well as to support our public company operations. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Moreover, our expected growth could require us to relocate to a different geographic area of the country. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion or relocation of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion or relocation of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

Cyber-attacks or other failures in our telecommunications or information technology systems, or those of our collaborators, contract research organizations, third-party logistics providers, distributors or other contractors or consultants, could result in information theft, data corruption and significant disruption of our business operations.

We, our collaborators, our CROs, third-party logistics providers, distributors and other contractors and consultants utilize information technology, or IT, systems and networks to process, transmit and store electronic information in connection with our business activities. As use of digital technologies has increased, cyber incidents, including third parties gaining access to employee accounts using stolen or inferred credentials, computer malware, viruses, spamming, phishing attacks or other means, and deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have increased in frequency and sophistication. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. These threats pose a risk to the security of our, our collaborators', our CROs', third-party logistics providers', distributors' and other contractors' and consultants' systems and networks, and the confidentiality, availability and integrity of our data. There can be no assurance that we will be successful in preventing cyber-attacks or successfully mitigating their effects. Similarly, there can be no assurance that our collaborators, CROs, third-party logistics providers, distributors and other contractors and consultants will be successful in protecting our clinical and other data that is stored on their systems. Any cyber-attack, data breach or destruction or loss of data could result in a violation of applicable U.S. and international privacy, data protection and other laws, and subject us to litigation and governmental investigations and proceedings by federal, state and local regulatory entities in the United States and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. Further, our general liability insurance and corporate risk program may not cover all potential claims to which we are exposed and may not be adequate to indemnify us for all liability that may be imposed; and could have a material adverse effect on our business and prospects. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials for any of our product candidates

could result in delays in our development and regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, we may suffer reputational harm or face litigation or adverse regulatory action as a result of cyber-attacks or other data security breaches and may incur significant additional expense to implement further data protection measures.

We rely on a set of cloud-based software services and access these services via the Internet for the vast majority of our computing, storage, bandwidth and other services. Any disruption of or interference with our use of our cloud-based services would negatively affect our operations and could seriously harm our business.

We use several distributed computing infrastructure platforms for business operations, or what is commonly referred to as "cloud" computing services and we access these services via the Internet. Any transition of the cloud services currently provided by an existing vendor to another cloud provider would be difficult to implement and will cause us to incur significant time and expense. Given this, any significant disruption of or interference with our use of these cloud computing services would negatively impact our operations and our business would be seriously harmed. If our employees or partners are not able to access our cloud computing services or encounter difficulties in doing so, we may experience business disruption. The level of service provided by our cloud computing vendors, including the ability to secure our confidential information and the confidential information of third parties that is shared with us, may also impact the perception of our company and could seriously harm our business and reputation and create liability for us. If a cloud computing service that we use experiences interruptions in service regularly or for a prolonged basis, or other similar issues, our business could be seriously harmed.

In addition, a cloud computing service may take actions beyond our control that could seriously harm our business, including:

- discontinuing or limiting our access to its platform;
- increasing pricing terms;
- terminating or seeking to terminate our contractual relationship altogether; or
- modifying or interpreting its terms of service or other policies in a manner that impacts our ability to run our business and operations.

Our cloud computing services have broad discretion to change and interpret its terms of service and other policies with respect to us, and those actions may be unfavorable to us. Our cloud computing services may also alter how we are able to process data on the platform. If a cloud computing services makes changes or interpretations that are unfavorable to us, our business could be seriously harmed.

Our efforts to protect the information shared with us may be unsuccessful due to the actions of third parties, software bugs or other technical malfunctions, employee error or malfeasance or other factors. In addition, third parties may attempt to fraudulently induce employees or users to disclose information to gain access to our data or third-party data entrusted to us. If any of these events occur, our or third-party information could be accessed or disclosed improperly. Some partners or collaborators may store information that we share with them on their own computing system. If these third parties fail to implement adequate data-security practices or fail to comply with our policies, our data may be improperly accessed or disclosed. And even if these third parties take all of these steps, their networks may still suffer a breach, which could compromise our data.

Any incidents where our information is accessed without authorization, or is improperly used, or incidents that violate our policies, could damage our reputation and our brand and diminish our competitive position. In addition, affected parties or government authorities could initiate legal or regulatory action against us over those incidents, which could cause us to incur significant expense

and liability or result in orders or consent decrees forcing us to modify our business practices. Concerns over our privacy practices, whether actual or unfounded, could damage our reputation and brand and deter users, advertisers and partners from using our products and services. Any of these occurrences could seriously harm our business.

We are also subject to many federal, state and foreign laws and regulations, including those related to privacy, rights of publicity, data protection, content regulation, intellectual property, health and safety, competition, protection of minors, consumer protection, employment and taxation. These laws and regulations are constantly evolving and may be interpreted, applied, created or amended in a manner that could seriously harm our business

We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.

We may be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts if we are unable to obtain additional funding to support our current operating plan.

As of June 30, 2020, we had $19.7 million of cash and cash equivalents. To date, we have primarily financed our operations with proceeds from sales of our redeemable convertible preferred stock and the issuance of convertible debt. We have incurred recurring losses since our inception, including net losses of $35.5 million for the year ended December 31, 2019 and $19.9 million for the six months ended June 30, 2020. We expect to continue to generate operating losses for the foreseeable future as we continue to invest significantly in the research and development of our programs. As a result, there is a significant degree of uncertainty as to how long our existing cash and cash equivalents will be sufficient to fund our operations. These conditions raise substantial doubt about our ability to continue as a going concern for a period of at least one year from the date our consolidated financial statements are issued, and our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited consolidated financial statements included elsewhere in this prospectus.

We are seeking the anticipated proceeds from this offering to provide additional funding for our operations. Even if the offering is consummated, we may be required to obtain additional funding whether through private or public equity transactions, debt financings or other capital sources, including collaborations with other companies or other strategic transactions and such additional funding may not be available on terms we find acceptable or favorable. There is inherent uncertainty associated with these fundraising activities and they are not considered probable. If we are unable to obtain sufficient capital to continue to advance our programs, we would be forced to delay, reduce or eliminate our research and development programs and any future commercialization efforts. Accordingly, our plans do not alleviate substantial doubt of our ability to continue as a going concern for a period of at least one year after the date our consolidated financial statements are issued.

Nevertheless, our consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. We will need to raise additional capital in this offering and/or otherwise to fund our future operations and remain as a going concern. However, we cannot guarantee that we will be able to obtain sufficient additional funding in this offering or otherwise or that such funding, if available, will be obtainable on terms favorable to us. In the event that we are unable to obtain sufficient additional funding, there can be no assurance that we will be able to continue as a going concern.

Legislation or other changes in U.S. tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury

Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many changes have been made to applicable tax laws and changes are likely to continue to occur in the future.

For example, the Tax Cuts and Jobs Act, or the TCJA, was enacted in 2017 and made significant changes to corporate taxation, including the reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for net operating losses from taxable years beginning after December 31, 2017 to 80% of current year taxable income and the elimination of net operating loss carrybacks generated in taxable years ending after December 31, 2017 (though any such net operating losses may be carried forward indefinitely) and the modification or repeal of many business deductions and credits. In addition, on March 27, 2020, President Trump signed into law the "Coronavirus Aid, Relief, and Economic Security Act" or the CARES Act, which included certain changes in tax law intended to stimulate the U.S. economy in light of the COVID-19 public health emergency, including temporary beneficial changes to the treatment of net operating losses, interest deductibility limitations and payroll tax matters.

It cannot be predicted whether, when, in what form or with what effective dates new tax laws may be enacted, or regulations and rulings may be enacted, promulgated or issued under existing or new tax laws, which could result in an increase in our or our shareholders' tax liability or require changes in the manner in which we operate in order to minimize or mitigate any adverse effects of changes in tax law or in the interpretation thereof.

Our ability to use our U.S. net operating loss carryforwards and certain other U.S. tax attributes may be limited.

Our ability to use our U.S. federal and state net operating losses to offset potential future taxable income and related income taxes that would otherwise be due is dependent upon our generation of future taxable income, and we cannot predict with certainty when, or whether, we will generate sufficient taxable income to use all of our net operating losses.

Unused losses for the tax year beginning before January 1, 2018 and prior tax years will carry forward to offset future taxable income, if any, until such unused losses expire. Unused losses generated for the tax year beginning after December 31, 2017, under new tax legislation will not expire and may be carried forward indefinitely, and generally may not be carried back to prior taxable years, except that, under the CARES Act, net operating losses generated in 2018, 2019 and 2020 may be carried back five taxable years. Additionally, for taxable years beginning after December 31, 2020, the deductibility of such U.S. federal net operating losses is limited to 80% of our taxable income in any future taxable year. In addition, both our current and our future unused losses may be subject to limitation under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if we undergo an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period. We may have experienced such ownership changes in the past, and we may experience ownership changes in the future as a result of this offering or subsequent shifts in our stock ownership, some of which are outside our control.

Risks Related to Our Common Stock and This Offering

There has been no prior public market for our common stock, the stock price of our common stock may be volatile or may decline regardless of our operating performance, an active trading market for our common stock may not develop and you may not be able to resell your shares at or above the initial public offering price.

Prior to this offering there has been no public market for shares of our common stock. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not

active. The initial public offering price for our common stock was determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of the common stock after the offering. Although our common stock has been approved for listing on The Nasdaq Global Select Market, an active or liquid market in our common stock may not develop upon the completion of this offering or, if it does develop, it may not be sustainable. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price.

Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic collaborations or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment, completion or results of our current Phase 2a clinical trials of PRAX-114 and PRAX-944 and current Phase 1 trial of PRAX-562;
- any delay in identifying and advancing a clinical candidate for our other programs;
- any delay in our regulatory filings for PRAX-114, PRAX-944, PRAX-562 or our future product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays, suspensions or terminations in future preclinical studies or clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval of PRAX-114, PRAX-944, PRAX-562 or any other product candidate or the failure of a regulatory authority to accept data from preclinical studies or clinical trials conducted in other countries;
- changes in laws or regulations applicable to PRAX-114, PRAX-944, PRAX-562 or any other product candidate, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- our failure to commercialize our product candidates, if approved;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of PRAX-114, PRAX-944, PRAX-562 or any other product candidate;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- actual or anticipated variations in quarterly operating results;

- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry, or product candidates in particular, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- changes in the structure of the healthcare payment systems;
- overall performance of the equity markets;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the market for biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, including as a result of the COVID-19 pandemic. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Furthermore, future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our executive officers, directors and their affiliates and our principal stockholders own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Based on shares outstanding as of September 30, 2020 and immediately following the completion of this offering, our executive officers, directors and their affiliates and our principal stockholders will beneficially hold, in the aggregate, approximately 48.6% of our outstanding voting stock, excluding any shares purchased in this offering. These stockholders, acting together, would be able to significantly influence all matters requiring stockholder approval. These stockholders acquired their shares of common stock (including shares of common stock issuable upon the conversion of preferred stock) for

less than the price of the shares of common stock being acquired in this offering, and these stockholders may have interests, with respect to their common stock, that are different from those of investors in this offering and the concentration of voting power among these stockholders may have an adverse effect on the price of our common stock. This concentration of ownership control may adversely affect the market price of our common stock by:

- delaying, deferring or preventing a change in control;
- entrenching our management and the board of directors;
- impeding a merger, consolidation, takeover or other business combination involving us that other stockholders may desire; and/or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

See the “Principal Stockholders” section of this prospectus for more information regarding the ownership of our outstanding common stock by our executive officers, directors, principal stockholders and their affiliates.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price will be substantially higher than the pro forma as adjusted net tangible book value per share of our common stock after this offering. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the pro forma as adjusted net tangible book value per share after this offering. As a result, investors purchasing common stock in this offering will incur immediate dilution of $10.91 per share, based on the initial public offering price of $19.00 per share, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering and the initial public offering price. Further, investors purchasing common stock in this offering will contribute approximately 47.8% of the total amount invested by stockholders since our inception, but will own only approximately 27.2% of the shares of common stock outstanding after this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less when they purchased their shares than the price offered to the public in this offering. To the extent outstanding options are exercised, there will be further dilution to new investors. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see the section entitled “Dilution.”

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, or EGC, as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For as long as we continue to be an EGC, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not EGCs, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an EGC for up to five years following the year in

which we complete this offering, although circumstances could cause us to lose that status earlier. We will remain an EGC until the earlier of (i) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least $1.07 billion or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed $700.0 million as of the prior June 30th, and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in this prospectus. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, EGCs can also delay adopting new or revised accounting standards until such time as those standards apply to private companies, which may make our financial statements less comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which will require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and The Nasdaq Global Select Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial reporting controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits EGCs to implement many of these requirements over a longer period and up to five years from the pricing of this offering. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have an adverse effect on our business. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our stock.

We will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an EGC, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We could be an EGC for up to five years. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to restatements of our financial statements and require us to incur the expense of remediation.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon completion of this offering, we will become subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

Substantial amounts of our outstanding shares may be sold into the market when lock-up or market standoff periods end. If there are substantial sales of shares of our common stock, the price of our common stock could decline.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of September 30, 2020, upon the completion of this offering we will have outstanding a total of 36,749,675 shares of common stock. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable without restriction in the public market immediately following this offering.

The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus, subject to earlier release of all or a portion of the shares subject to such agreements by the representatives of the underwriters in this offering in their sole discretion. After the lock-up agreements expire, based upon the number of shares of common stock, on an as-converted basis, outstanding as of September 30, 2020, up to an additional 26,737,873 shares of common stock will be eligible for sale in the public market. Approximately 68% of these additional shares are beneficially held by directors, executive officers and their affiliates and will be subject to certain limitations of Rule 144 under the Securities Act of 1933, as amended, or the Securities Act.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our existing equity compensation plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. Additionally, the number of shares of our common stock reserved for issuance under our 2020 Stock Option and Incentive Plan, or the 2020 Plan, will automatically increase on January 1 of each year, beginning on January 1, 2021, by 5% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors or compensation committee. Moreover, the number of shares of our common stock reserved for issuance under our 2020 Employee Stock Purchase Plan will automatically increase on January 1 of each year, beginning on January 1, 2021, by the lesser of 327,102 shares of common stock, 1% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors or compensation committee. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution.

After this offering, the holders of 25,067,977 shares of our common stock will be entitled to rights with respect to the registration of their shares under the Securities Act as provided under the terms of an investors' rights agreement between us and the holders of our redeemable convertible preferred stock, subject to the 180-day lock-up agreements described above. See "Description of Capital Stock—Registration Rights." Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

We have broad discretion in the use of our existing cash and cash equivalents and the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of our existing cash and cash equivalents and the net proceeds from this offering, including for any of the purposes described in the section entitled "Use of Proceeds," and you will not have the opportunity as part of your investment decision to assess whether such proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of our existing cash and cash equivalents and the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our existing cash and cash equivalents and the net proceeds from this offering in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Affiliates of Blackstone Securities Partners L.P., an underwriter in this offering, will have an interest in this offering beyond customary underwriting discounts and commissions.

Certain affiliates of Blackstone Securities Partners L.P., an underwriter in this offering, beneficially own more than 10% of our outstanding preferred equity prior to the consummation of this offering. Therefore, Blackstone Securities Partners L.P. is deemed to have a conflict of interest within the meaning of Rule 5121 of the Financial Industry Regulatory Authority, Inc., or FINRA Rule 5121. Accordingly, this offering is being conducted in accordance with FINRA Rule 5121. See "Underwriting (Conflicts of Interest)" for additional information.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws, which are to become effective upon the completion of this offering, contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the board of directors acting pursuant to a resolution approved by the affirmative vote of a majority of the directors then in office;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue convertible preferred stock on terms determined by the board of directors without stockholder approval and which convertible preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Our bylaws to be effective upon the consummation of this offering designate certain courts as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our bylaws that will become effective upon the completion of this offering provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our bylaws (in each case, as they may be amended from time to time) or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein; provided, however, that this exclusive forum provision will not apply to any causes of action arising under the Securities Act or the Exchange Act. Our bylaws further provide that, unless we consent in writing to an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. We have chosen the United States District Court for the District of Massachusetts as the exclusive forum for such Securities Act causes of action because our principal executive offices are located in Cambridge, Massachusetts. In addition, our amended and restated bylaws will provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions. We recognize that the forum selection clause in our bylaws may impose additional litigation costs on stockholders in pursuing any such claims, particularly if the stockholders do not reside in or near the State of Delaware or the Commonwealth of Massachusetts, as applicable. Additionally, the forum selection clause in our bylaws may limit our stockholders' ability to bring a claim in a forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. The Court of Chancery of the State of Delaware or the United States District Court for the District of Massachusetts may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

Our issuance of additional capital stock in connection with financings, acquisitions, investments, our stock incentive plans or otherwise will dilute all other stockholders.

We expect to issue additional capital stock in the future that will result in dilution to all other stockholders. We expect to grant equity awards to employees, directors and consultants under our stock incentive plans. We may also raise capital through equity financings in the future. As part of our business strategy, we may acquire or make investments in complementary companies, products or

technologies and issue equity securities to pay for any such acquisition or investment. Any such issuances of additional capital stock may cause stockholders to experience significant dilution of their ownership interests and the per share value of our common stock to decline.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the sections entitled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," and "Business," contains express or implied forward-looking statements that are based on our management's belief and assumptions and on information currently available to our management. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the success, cost and timing of our product development activities and clinical trials;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates;
- the ability to license additional intellectual property relating to our product candidates from third parties and to comply with our existing license agreements and collaboration agreements;
- the ability and willingness of our third-party research institution collaborators to continue research and development activities relating to our product candidates;
- our ability to commercialize our products in light of the intellectual property rights of others;
- our ability to obtain funding for our operations, including funding necessary to complete further development and commercialization of our product candidates;
- the commercialization of our product candidates, if approved;
- our plans to research, develop and commercialize our product candidates;
- future agreements with third parties in connection with the commercialization of our product candidates and any other approved product;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates;
- the pricing and reimbursement of our product candidates, if approved;
- regulatory developments in the United States and foreign countries;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act, enacted in April 2012;
- our use of the proceeds from this offering;
- the development of major public health concerns, including the novel coronavirus outbreak or other pandemics arising globally, and the future impact of it and COVID-19 on our clinical trials, business operations and funding requirements; and
- other risks and uncertainties, including those listed under the caption "Risk Factors."

In some cases, forward-looking statements can be identified by terminology, such as “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue,” or the negative of these terms or other comparable terminology. These statements are only predictions. You should not place undue reliance on forward-looking statements because they involve known and unknown risks, uncertainties and other factors, which are, in some cases, beyond our control and which could materially affect results. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk Factors” and elsewhere in this prospectus. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this prospectus and the documents that we reference in this prospectus and have filed with the Securities and Exchange Commission as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should therefore not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. We are responsible for all of the disclosure contained in this prospectus, and we believe that these sources are reliable; however, we have not independently verified the information contained in such publications.

USE OF PROCEEDS

We estimate that our net proceeds from the sale of shares of our common stock in this offering will be approximately $173.4 million, or $199.9 million if the underwriters exercise in full their option to purchase additional shares, based on the initial public offering price of $19.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, including the proceeds from our Series C-1 redeemable convertible preferred stock financing completed in August 2020, as follows:

- approximately $70 to $80 million to advance PRAX-114 into and through the completion of our Phase 2/3 trial in MDD, which is intended to satisfy one of two registrational trials required by the FDA to support clinical efficacy, and to complete Part B (PMD) and Part C (longer-term dosing in MDD) of our ongoing Phase 2a clinical trial for PRAX-114;
- approximately $30 to $40 million to complete our ongoing Phase 2a clinical trial and a Phase 2/3 randomized, controlled clinical trial for PRAX-944 in ET;
- approximately $20 to $30 million to complete our ongoing Phase 1 healthy volunteer trial and the first patient trial for PRAX-562; and
- the remainder for advancement of other programs in our pipeline and support of working capital and other general corporate purposes.

Based on our current plans, we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to fund our operations for at least the next 18 months. We have based this estimate on assumptions that may prove to be incorrect, and we could use our available capital resources sooner than we currently expect.

This expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the closing of this offering or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual expenditures and the extent of clinical development may vary significantly depending on numerous factors, including the progress of our development, the status of and results from preclinical studies or clinical trials we may commence in the future, as well as any collaborations that we may enter into with third parties for our product candidates, and any unforeseen cash needs. We may also use a portion of the net proceeds to in-license, acquire or invest in additional businesses, technologies, products or assets, although currently we have no specific agreements, commitments or understandings in this regard. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Pending our use of proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation instruments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock. We intend to retain all available funds and any future earnings to fund the growth and development of our business. We do not intend to pay cash dividends to our stockholders in the foreseeable future. In addition, any future financing instruments could preclude us from paying dividends. Any future determination to pay dividends will be made at the discretion of our board of directors subject to applicable laws, and will depend upon, among other factors, our results of operations, financial condition, contractual restrictions and capital requirements. Investors should not purchase our common stock with the expectation of receiving cash dividends.

CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of June 30, 2020:

- on an actual basis;
- on a pro forma basis to give effect to: (i) the sale and issuance of 19,444,453 shares of our Series C-1 redeemable convertible preferred stock in the third quarter of 2020 for gross cash proceeds of $110.3 million, (ii) the automatic conversion of all outstanding shares of our redeemable convertible preferred stock, including our Series C-1 redeemable convertible preferred stock, into an aggregate of 25,067,977 shares of common stock upon the consummation of this offering and (iii) the filing and effectiveness of our amended and restated certificate of incorporation upon the completion of this offering; and
- on a pro forma as adjusted basis to give further effect to the issuance and sale of 10,000,000 shares of our common stock in this offering at the initial public offering price of $19.00 per share after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read the information in this table together with our unaudited interim condensed consolidated financial statements and the related notes appearing elsewhere in this prospectus, as well as the sections of this prospectus captioned “Selected Consolidated Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.”

	AS OF JUNE 30, 2020		
		(Unaudited)	
	ACTUAL	PRO FORMA	PRO FORMA AS ADJUSTED
	(In thousands, except share and per share data)		
Cash and cash equivalents	$ 19,748	$ 129,998	$ 303,411
Redeemable convertible preferred stock, $0.0001 par value; 36,724,132 shares authorized, 34,199,861 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	$ 118,190	$ –	$ –
Stockholders’ (deficit) equity:			
Preferred stock, $0.0001 par value; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted	–	–	–
Common stock, $0.0001 par value; 50,000,000 shares authorized, 1,670,070 shares issued and 1,648,165 shares outstanding, actual; 150,000,000 shares authorized, 26,738,047 shares issued and 26,716,142 shares outstanding, pro forma; 150,000,000 shares authorized, 36,738,047 shares issued and 36,716,142 shares outstanding, pro forma as adjusted	1	3	4
Additional paid-in capital	–	228,438	401,837
Accumulated deficit	(104,085)	(104,085)	(104,085)
Total stockholders’ (deficit) equity	(104,084)	124,356	297,756
Total	$ 14,106	$ 124,356	$ 297,756

This table excludes:

- 3,220,201 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2020 under the 2017 Stock Incentive Plan, at a weighted average exercise price of $4.07 per share;
- 584,384 shares of common stock reserved for future issuance as of June 30, 2020 under the 2017 Stock Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- 3,271,028 shares of our common stock that became available for future issuance under the 2020 Stock Option and Incentive Plan, or the 2020 Plan, upon the effectiveness of the registration statement of which this prospectus forms a part (including 37,615 shares of our common stock issuable upon the exercise of stock options we have agreed to grant under our 2020 Plan upon the effectiveness of the registration statement of which this prospectus is a part, at an exercise price equal to the initial public offering price); and
- 327,102 shares of our common stock that became available for future issuance under the 2020 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of common stock and the pro forma as adjusted net tangible book value per share of common stock immediately after this offering.

Our historical net tangible book value (deficit) as of June 30, 2020 was $(104.5) million, or $(62.55) per share of common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and the carrying value of our redeemable convertible preferred stock. Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 1,670,070 issued shares of our common stock, which include 21,905 shares of unvested restricted common stock, as of June 30, 2020.

Our pro forma net tangible book value as of June 30, 2020 was $124.0 million, or $4.64 per share of common stock. Pro forma net tangible book value is the amount of our total tangible assets less our total liabilities, after having given effect to: (i) the sale and issuance of 19,444,453 shares of our Series C-1 redeemable convertible preferred stock in the third quarter of 2020 for gross cash proceeds of $110.3 million and (ii) the automatic conversion of all outstanding shares of our redeemable convertible preferred stock, including our Series C-1 redeemable convertible preferred stock, into an aggregate of 25,067,977 shares of common stock upon the consummation of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of issued shares of our common stock as of June 30, 2020, after having given effect to the pro forma adjustments described above.

After giving further effect to the sale and issuance of 10,000,000 shares of our common stock in this offering at the initial public offering price of $19.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of June 30, 2020 would have been $297.4 million, or $8.09 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $3.45 to existing stockholders and immediate dilution of $10.91 in pro forma as adjusted net tangible book value per share to new investors participating in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors.

The following table illustrates this dilution on a per share basis to new investors:

Initial public offering price per share		$ 19.00
Historical net tangible book value (deficit) per share as of June 30, 2020	$(62.55)	
Increase per share attributable to pro forma adjustments	67.19	
Pro forma net tangible book value per share as of June 30, 2020	4.64	
Increase in pro forma net tangible book value per share attributable to new investors participating in this offering	3.45	
Pro forma as adjusted net tangible book value per share after this offering		8.09
Dilution per share to new investors participating in this offering		$ 10.91

If the underwriters exercise their option in full to purchase 1,500,000 additional shares of common stock in this offering, our pro forma as adjusted net tangible book value per share after this offering would be $8.47, representing an immediate increase in pro forma as adjusted net tangible book value per share of $0.38 to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $10.53 to investors participating in this offering, based on the initial public

offering price of $19.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, on the pro forma as adjusted basis described above as of June 30, 2020, the total number of shares of common stock purchased from us on an as converted to common stock basis, the total consideration paid or to be paid and the average price per share paid or to be paid by existing stockholders and by investors participating in this offering at the initial public offering price of $19.00 per share before deducting underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing common stock in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	SHARES PURCHASED		TOTAL CONSIDERATION		AVERAGE PRICE PER SHARE
	NUMBER	PERCENTAGE	AMOUNT	PERCENTAGE	
			(In thousands)		
Existing stockholders	26,738,047	72.8%	$ 207,362	52.2%	$ 7.76
Investors participating in this offering	10,000,000	27.2	190,000	47.8	19.00
Total	36,738,047	100.0%	$397,362	100.0%	$ 10.82

The table assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to 69.9% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors participating in the offering would be increased to 30.1% of the total number of shares outstanding after this offering.

The above discussion and tables are based on issued shares of our common stock, which include 21,905 shares of unvested restricted common stock, as of June 30, 2020 and exclude:

- 3,220,201 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2020 under the 2017 Stock Incentive Plan, at a weighted average exercise price of $4.07 per share;
- 584,384 shares of common stock reserved for future issuance as of June 30, 2020 under the 2017 Stock Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- 3,271,028 shares of our common stock that became available for future issuance under the 2020 Stock Option and Incentive Plan, or the 2020 Plan, upon the effectiveness of the registration statement of which this prospectus forms a part (including 37,615 shares of our common stock issuable upon the exercise of stock options we have agreed to grant under our 2020 Plan upon the effectiveness of the registration statement of which this prospectus is a part, at an exercise price equal to the initial public offering price); and
- 327,102 shares of our common stock that became available for future issuance under the 2020 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

New investors will experience further dilution if new options or warrants are issued under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

SELECTED CONSOLIDATED FINANCIAL DATA

We have derived the consolidated statement of operations data for the years ended December 31, 2018 and 2019 and the consolidated balance sheet data as of December 31, 2018 and 2019 from our audited consolidated financial statements appearing elsewhere in this prospectus. The consolidated statement of operations data for the six months ended June 30, 2019 and 2020 and the consolidated balance sheet data as of June 30, 2020 have been derived from our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair statement of the financial information in those statements. You should read the following selected consolidated financial data together with our consolidated financial statements and unaudited interim condensed consolidated financial statements and the related notes appearing elsewhere in this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus. The selected consolidated financial data contained in this section are not intended to replace our consolidated financial statements and the related notes. Our historical results are not necessarily indicative of the results that may be expected in any future period and our operating results for the six months ended June 30, 2020 are not necessarily indicative of the results that may be expected for the full year ended December 31, 2020 or any other interim periods or any future year or period.

	YEAR ENDED DECEMBER 31,		SIX MONTHS ENDED JUNE 30,	
	2018	2019	2019	2020
			(Unaudited)	
	(In thousands, except share and per share data)			
Consolidated Statement of Operations Data:				
Operating expenses:				
Research and development	$ 18,820	$ 29,557	$ 14,512	$ 15,918
General and administrative	3,899	6,232	3,129	4,121
Total operating expenses	22,719	35,789	17,641	20,039
Loss from operations	(22,719)	(35,789)	(17,641)	(20,039)
Total other income (expense):				
Interest income (expense), net	(35)	193	123	133
Other expense	(3,648)	–	–	–
Total other income (expense), net	(3,683)	193	123	133
Loss before provision for (benefit from) income taxes	(26,402)	(35,596)	(17,518)	(19,906)
Provision for (benefit from) income taxes	133	(84)	–	(8)
Net loss	$(26,535)	$(35,512)	$(17,518)	$(19,898)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,296)	(5,170)	(2,184)	(4,103)
Loss on conversion of convertible notes	(392)	–	–	–
Gain on repurchase of redeemable convertible preferred stock	–	–	–	493
Net loss attributable to common stockholders	$(29,223)	$(40,682)	$(19,702)	$(23,508)

	YEAR ENDED DECEMBER 31,		SIX MONTHS ENDE JUNE 30,	
	2018	2019	2019	202
			(Unaudited)	
	(In thousands, except share and per share data)			
Net loss per share attributable to common stockholders, basic and diluted(1)	$ (22.52)	$ (26.60)	$ (13.38)	$ (
Weighted average common shares outstanding, basic and diluted(1)	1,297,633	1,529,629	1,472,484	1,63
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)(1)		$ (2.68)		$
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)(1)		13,270,761		17,17

(1) See Note 13 to our consolidated financial statements and Note 9 to our unaudited interim condensed consolidated financial statements appearing elsewhere in this prospectus for details on the calculation of basic and diluted net loss per share attributable to common stockholders and unaudited pro forma basic and diluted net loss per share attributable to common stockholders.

	AS OF DECEMBER 31,		AS OF JUNE 30, 2020
	2018	2019	(Unaudited)
	(In thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 17,950	$ 44,815	$ 19,748
Working capital(1)	$ 13,981	$ 38,678	$ 12,283
Total assets	$ 19,829	$ 47,694	$ 22,653
Redeemable convertible preferred stock	$ 55,720	$ 121,121	$ 118,190
Accumulated deficit	$ (41,365)	$ (81,009)	$ (104,085)
Total stockholders' deficit	$ (41,038)	$ (81,008)	$ (104,084)

(1) We define working capital as current assets less current liabilities. See our consolidated financial statements and unaudited interim condensed consolidated financial statements and related notes appearing elsewhere in this prospectus for further details regarding our current assets and current liabilities.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the "Selected Consolidated Financial Data" section of this prospectus and our consolidated financial statements and unaudited interim condensed consolidated financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system, or CNS, disorders characterized by neuronal imbalance. Normal brain function requires a delicate balance of excitation and inhibition in neuronal circuits, which, when dysregulated, leads to abnormal function and disease. We are applying insights into the genetic mutations that drive excitation-inhibition imbalance in diseases to select biological targets for severe pediatric epilepsies and more broadly for prevalent psychiatric diseases and neurologic disorders. We apply a deliberate and pragmatic precision approach, leveraging a suite of translational tools including novel transgenic and predictive translational animal models and electrophysiology markers, to enable an efficient path to proof-of-concept in patients. Through this approach, we have established a broad portfolio, including five disclosed programs across multiple CNS disorders, including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. We intend to develop differentiated therapies that can deliver long-term benefits to human health by meaningfully impacting patients and society. We expect multiple topline clinical trial readouts from all three programs prior to the end of 2021 and anticipate the launch of a new clinical development program in 2021.

Our most advanced programs, PRAX-114 and PRAX-944, are currently in Phase 2 development. PRAX-114 is being developed for the treatment of major depressive disorder and perimenopausal depression. Together, these conditions affect more than 22 million patients in the United States. PRAX-944 is a selective small molecule inhibitor of T-type calcium channels for the treatment of essential tremor, a progressive and debilitating movement disorder affecting up to seven million people in the United States. In addition, we have initiated a Phase 1 trial of PRAX-562, a persistent sodium current blocker, for the treatment of a broad range of rare, devastating CNS disorders, such as severe pediatric epilepsy and adult cephalgia. In addition to our clinical programs, we have two disclosed preclinical product candidates in development for severe genetic epilepsies.

We were incorporated in 2015 and commenced operations in 2016. Since inception, we have devoted substantially all of our resources to developing our preclinical and clinical product candidates, building our intellectual property portfolio, business planning, raising capital and providing general and administrative support for these operations. We employ a "virtual" research and development model, relying heavily upon external consultants, collaborators and contract research organizations to conduct our preclinical and clinical activities. Since inception, we have financed our operations primarily with proceeds from the issuance of convertible debt and sales of our Series A redeemable convertible preferred stock, Series B redeemable convertible preferred stock, Series B-1 redeemable convertible preferred stock, Series C redeemable convertible preferred stock and Series C-1 redeemable convertible preferred stock.

We are a development stage company and we have not generated any revenue from product sales, and do not expect to do so for several years, if at all. All of our programs are still in preclinical

and clinical development. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our product candidates, if approved. We have incurred recurring operating losses since inception, including net losses of $26.5 million and $35.5 million for the years ended December 31, 2018 and 2019, respectively, and $17.5 million and $19.9 million for the six months ended June 30, 2019 and 2020, respectively. As of June 30, 2020, we had an accumulated deficit of $104.1 million. We expect to incur significant expenses and operating losses for the foreseeable future as we expand our research and development activities. In addition, our losses from operations may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- advance our lead product candidates, PRAX-114 and PRAX-944, to late stage clinical trials;
- advance our PRAX-562 product candidate to Phase 2 clinical trials;
- advance our preclinical programs to clinical trials;
- further invest in our pipeline;
- further invest in our manufacturing capabilities;
- seek regulatory approval for our investigational medicines;
- maintain, expand, protect and defend our intellectual property portfolio;
- acquire or in-license technology;
- secure facilities to support continued growth in our research, development and commercialization efforts;
- take temporary precautionary measures to help minimize the risk of COVID-19 to our employees;
- increase our headcount to support our development efforts and to expand our clinical development team; and
- incur additional costs and headcount associated with operating as a public company upon the completion of this offering.

In addition, as we progress toward marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of June 30, 2020, we had cash and cash equivalents of $19.7 million. In the third quarter of 2020, we sold and issued shares of Series C-1 redeemable convertible preferred stock for gross cash proceeds of $110.3 million. We believe that our existing cash and cash equivalents, combined with the cash proceeds from the sale and issuance of our Series C-1 redeemable convertible preferred stock and the anticipated net proceeds from this offering, will enable us to fund our operating expenses and capital expenditure requirements through at least the next 18 months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "—Liquidity and Capital Resources."

Without giving effect to the anticipated net proceeds from this offering, based on our current operating plan, we believe we have sufficient cash and cash equivalents on hand to support current operations into the second half of 2021. We have concluded that this circumstance raises substantial doubt about our ability to continue as a going concern for at least one year from the date that our unaudited interim condensed consolidated financial statements for the six months ended June 30, 2020 were issued. See Note 1 to our unaudited interim condensed consolidated financial statements appearing at the end of this prospectus for additional information on our assessment. To finance our operations, we will need to raise additional capital, which cannot be assured. If we are unable to obtain additional funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. We expect to seek additional funding through private or public equity transactions, debt financings or other capital sources, including collaborations with other companies or other strategic transactions.

COVID-19 Business Update

With the global spread of the ongoing COVID-19 pandemic in 2020, we have implemented business continuity plans designed to address and mitigate the impact of the COVID-19 pandemic on our employees and our business, including our preclinical studies and clinical trials. Our operations are considered an essential business and we are continuing to operate during this period. We have taken measures to secure our research and development activities. While we are experiencing limited financial impacts at this time, given the global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic, our business, financial condition and results of operations could be materially adversely affected. We continue to closely monitor the COVID-19 pandemic as we evolve our business continuity plans, clinical development plans and response strategy.

In addition, while we have taken and are continuing to take steps to mitigate against COVID-19 pandemic-related delays, our planned clinical trials may be affected by the COVID-19 pandemic, including (i) delays or difficulties in enrolling and retaining patients in our planned clinical trials, including patients that may not be able or willing to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services; (ii) delays or difficulties in clinical site initiation, including difficulties in recruiting and retaining clinical site investigators and clinical site staff; (iii) diversion or prioritization of healthcare resources away from the conduct of clinical trials and towards the COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials, and because, who, as healthcare providers, may have heightened exposure to COVID-19 and adversely impact our clinical trial operations; (iv) interruption of our future clinical supply chain or key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal, state/provincial or municipal governments, employers and others; and (v) limitations in outsourced third-party resources that would otherwise be focused on the conduct of our planned clinical trials, including because of sickness of third-party personnel or their families, or the desire of third-party personnel to avoid contact with large groups of people.

Financial Operations Overview

Revenue

We have not generated any revenue since inception and do not expect to generate any revenue from the sale of products for several years, if at all. If our development efforts for our current or future product candidates are successful and result in marketing approval or collaboration or license agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from collaboration or license agreements that we may enter into with third parties.

Operating Expenses

Research and Development Expense

The nature of our business and primary focus of our activities generate a significant amount of research and development costs. Research and development expenses represent costs incurred by us for the following:

- costs to develop our portfolio;
- discovery efforts leading to development candidates;
- clinical development costs for our programs; and
- costs to develop our manufacturing technology and infrastructure.

The costs above comprise the following categories:

- personnel-related expenses, including salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with third parties, such as consultants, investigative sites and contract research organizations, or CROs, that conduct our preclinical and clinical studies and in-licensing arrangements;
- costs incurred to maintain compliance with regulatory requirements;
- costs incurred with third-party contract manufacturing organizations, or CMOs, to acquire, develop and manufacture materials for preclinical and clinical studies; and
- depreciation, amortization and other direct and allocated expenses, including rent, insurance and other operating costs, incurred as a result of our research and development activities.

We expense research and development costs as incurred. We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors and our clinical investigative sites. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our consolidated balance sheets as prepaid expenses or accrued research and development expenses. Non-refundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized, even when there is no alternative future use for the research and development. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

A significant portion of our research and development costs have been external costs. We track direct external research and development expenses to specific programs upon commencement. Due to the number of ongoing programs and our ability to use resources across several projects, indirect or shared operating costs incurred for our research and development programs, such as personnel, facility costs and certain consulting costs, are not recorded or maintained on a program-specific basis.

Our major programs, PRAX-114, PRAX-944 and PRAX-562, are those for which we have initiated clinical activities. Our discovery-stage programs are those which are at an earlier point in the development process. The following table reflects our research and development expenses, including direct program-specific expenses summarized by major program, discovery-stage program costs and indirect or shared operating costs recognized as research and development expenses during each period presented (in thousands):

	Year Ended December 31,		Six Months Ended June 30,	
	2018	2019	2019	2020
			(Unaudited)	
PRAX-114	$ 4,795	$ 7,192	$ 4,881	$ 5,060
PRAX-944	2,286	4,035	2,779	1,577
PRAX-562	4,469	4,276	2,259	1,642
Discovery-stage programs	1,954	5,909	730	1,896
Personnel-related (including stock-based compensation)	2,337	5,398	2,541	4,345
Other indirect research and development expenses	2,979	2,747	1,322	1,398
Total research and development expenses	$18,820	$29,557	$14,512	$15,918

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will continue to increase in the foreseeable future as we advance our product candidates through the development phase, and as we continue to discover and develop additional product candidates, build manufacturing capabilities and expand into additional therapeutic areas.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales or licensing of our product candidates. This is due to the numerous risks and uncertainties associated with drug development, including the uncertainty of:

- our ability to add and retain key research and development personnel;
- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to successfully complete clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize, our product candidates;
- our successful enrollment in and completion of clinical trials;
- the costs associated with the development of any additional product candidates we identify in-house or acquire through collaborations;
- our ability to discover, develop and utilize biomarkers to demonstrate target engagement, pathway engagement and the impact on disease progression of our product candidates;
- our ability to establish and maintain agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if our product candidates are approved;

- the terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder;
- our ability to obtain and maintain patent, trade secret and other intellectual property protection and regulatory exclusivity for our product candidates if and when approved;
- our receipt of marketing approvals from applicable regulatory authorities;
- our ability to commercialize products, if and when approved, whether alone or in collaboration with others; and
- the continued acceptable safety profiles of the product candidates following approval.

A change in any of these variables with respect to the development of any of our product candidates would significantly change the costs, timing and viability associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to delay our planned start of clinical trials or require us to conduct clinical trials or other testing beyond those that we currently expect, or if we experience significant delays in enrollment in any of our planned clinical trials, we could be required to expend significant additional financial resources and time to complete our clinical development activities. We may never obtain regulatory approval for any of our product candidates. Drug commercialization will take several years and millions of dollars in development costs.

General and Administrative Expense

General and administrative expenses consist primarily of personnel-related costs, including salaries, benefits and stock-based compensation, for personnel in our executive, finance, legal, business development and administrative functions. General and administrative expenses also include legal fees relating to corporate matters; professional fees for accounting, auditing, tax and administrative consulting services; insurance costs; administrative travel expenses; and facility-related expenses, which include direct depreciation costs and allocated expenses for office rent and other operating costs. These costs relate to the operation of the business, unrelated to the research and development function, or any individual program. Costs to secure and defend our IP are expensed as incurred and are classified as general and administrative expenses.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support the expected growth in our research and development activities and the potential commercialization of our product candidates. We also expect to incur increased expenses associated with being a public company, including increased costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance costs and investor and public relations costs. We also expect to incur additional IP-related expenses as we file patent applications to protect innovations arising from our research and development activities.

Total Other Income (Expense)

Interest Income

Interest income consists of interest earned on our cash and cash equivalents.

Interest Expense

Interest expense consists of interest incurred on our convertible promissory notes.

Other Expense

Other expense consists of fluctuations in the fair value of financial instruments that were measured at fair value, including our Series B redeemable convertible preferred stock tranche obligation, an anti-dilution obligation related to our license agreement with Purdue Neuroscience Company, and conversion features associated with our convertible notes. All of these financial instruments were settled during the year ended December 31, 2018.

Income Taxes

Since our inception, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in each year or for our earned research and development tax credits, due to our uncertainty of realizing a benefit from those items. As of December 31, 2018 and 2019, we had U.S. federal and state net operating loss carryforwards which may be available to offset future taxable income and which would begin to expire in 2035. As of December 31, 2018 and 2019, we also had federal and state research and development tax credit carryforwards which may be available to offset future income tax liabilities and which would begin to expire in 2031.

Income taxes are determined at the applicable tax rates adjusted for non-deductible expenses, research and development tax credits and other permanent differences. Our income tax provision may be significantly affected by changes to our estimates. The income tax provision and benefit recognized for the years ended December 31, 2018 and 2019 and the six months ended June 30, 2020 related to income tax associated with our operations in Australia.

Results of Operations

Comparison of the Years Ended December 31, 2018 and 2019

The following table summarizes our consolidated statements of operations for each period presented (in thousands):

	Year Ended December 31,		Change
	2018	2019	
Operating expenses:			
Research and development	$ 18,820	$ 29,557	$ 10,737
General and administrative	3,899	6,232	2,333
Total operating expenses	22,719	35,789	13,070
Loss from operations	(22,719)	(35,789)	(13,070)
Total other income (expense):			
Interest income	92	193	101
Interest expense	(127)	–	127
Other expense	(3,648)	–	3,648
Total other income (expense), net	(3,683)	193	3,876
Loss before provision for (benefit from) income taxes	(26,402)	(35,596)	(9,194)
Provision for (benefit from) income taxes	133	(84)	(217)
Net loss	$(26,535)	$(35,512)	$ (8,977)

Research and Development Expense

The following table summarizes our research and development expenses for each period presented, along with the changes in those items (in thousands):

	Year Ended December 31,		Change
	2018	2019	
PRAX-114	$ 4,795	$ 7,192	$ 2,397
PRAX-944	2,286	4,035	1,749
PRAX-562	4,469	4,276	(193)
Discovery-stage programs	1,954	5,909	3,955
Personnel-related (including stock-based compensation)	2,337	5,398	3,061
Other indirect research and development expenses	2,979	2,747	(232)
Total research and development expenses	$18,820	$29,557	$10,737

Research and development expenses increased $10.7 million from $18.8 million for the year ended December 31, 2018, to $29.6 million for the year ended December 31, 2019. The increase in research and development expenses was primarily attributable to the following:

- $4.0 million increase in discovery-stage program expense, primarily for our PRAX-222 program which commenced during the year ended December 31, 2019 and incurred $4.3 million of research and development expense, including $2.2 million upon our acquisition of the underlying in-process research and development assets in September 2019 and $0.6 million of an up-front payment and research activity reimbursements under a collaboration agreement for that program;
- $3.1 million increase in personnel-related costs due to increased headcount;
- $2.4 million increase in expense related to our PRAX-114 program, including a $2.1 million increase in outsourced research and development spend and a $0.3 million increase in consulting costs, as we started enrolling patients in a Phase 2a clinical trial during the year ended December 31, 2019;
- $1.7 million increase in expense related to our PRAX-944 program, driven by a $1.5 million increase in outsourced research and development and CRO spend and a $0.2 million increase in consulting costs, as the program progressed into a Phase 2a clinical trial during the year ended December 31, 2019;
- $0.2 million offsetting decrease in other indirect research and development expenses, driven by a $0.9 million decrease in outsourced research and development and CRO spend as well as consulting costs not allocated to a specific program, offset by a $0.7 million increase in facility, office, software and other overhead costs due to increased research and development headcount; and
- $0.2 million offsetting decrease in expense related to our PRAX-562 program, primarily driven by the timing of our outsourced research and development activities.

General and Administrative Expense

General and administrative expenses increased $2.3 million from $3.9 million for the year ended December 31, 2018 to $6.2 million for the year ended December 31, 2019. The increase in general and administrative expenses was primarily attributable to the following:

- $1.5 million increase in personnel-related costs driven by increased headcount;

- $0.7 million increase in professional fees including legal and consulting services, driven by a $0.4 million increase in legal fees, primarily related to intellectual property filings, as we expand our research and development activities, and a $0.3 million increase in consulting costs, primarily in the accounting and business development functions; and
- $0.1 million increase in facilities, office and other general and administrative expenses to support the increase in our operating activities.

Total Other Income (Expense), Net

Total other income (expense), net for the year ended December 31, 2018 was $(3.7) million, compared to $0.2 million for the year ended December 31, 2019. The change was primarily attributable to a $3.6 million loss recognized during the year ended December 31, 2018 as a result of fluctuations in the fair value of financial instruments that were measured at fair value, including our Series B redeemable convertible preferred stock tranche obligation, an anti-dilution obligation related to our license agreement with Purdue Neuroscience Company and conversion features associated with our convertible notes. All of these financial instruments were settled during the year ended December 31, 2018.

Comparison of the Six Months Ended June 30, 2019 and 2020

The following table summarizes our consolidated statements of operations for each period presented (in thousands):

	Six Months Ended June 30,		Change
	2019	2020	
	(Unaudited)		
Operating expenses:			
Research and development	$ 14,512	$ 15,918	$ 1,406
General and administrative	3,129	4,121	992
Total operating expenses	17,641	20,039	2,398
Loss from operations	(17,641)	(20,039)	(2,398)
Total other income:			
Interest income	123	133	10
Total other income	123	133	10
Loss before provision for (benefit from) income taxes	(17,518)	(19,906)	(2,388)
Benefit from income taxes	—	(8)	(8)
Net loss	$(17,518)	$(19,898)	$(2,380)

Research and Development Expense

The following table summarizes our research and development expenses for each period presented, along with the changes in those items (in thousands):

	Six Months Ended June 30,		Change
	2019	2020	
	(Unaudited)		
PRAX-114	$ 4,881	$ 5,060	$ 179
PRAX-944	2,779	1,577	(1,202)
PRAX-562	2,259	1,642	(617)
Discovery-stage programs	730	1,896	1,166
Personnel-related (including stock-based compensation)	2,541	4,345	1,804
Other indirect research and development expenses	1,322	1,398	76
Total research and development expenses	$14,512	$15,918	$ 1,406

Research and development expenses increased $1.4 million from $14.5 million for the six months ended June 30, 2019, to $15.9 million for the six months ended June 30, 2020. The increase in research and development expenses was primarily attributable to the following:

- $1.8 million increase in personnel-related costs due to increased headcount;
- $1.2 million increase in discovery-stage program expense, primarily for our PRAX-222 program which commenced in September 2019 and incurred $1.4 million of research and development expense during the six months ended June 30, 2020, primarily driven by outsourced research and development and CRO spend;
- $0.2 million increase in expense related to our PRAX-114 program, driven by a $1.1 million increase in outsourced clinical spend for our ongoing Phase 2a clinical trial for this program, partially offset by a $0.6 million decrease due to a drug product manufacturing campaign completed in the prior year and a $0.2 million decrease related to expenses incurred during the six months ended June 30, 2019 in support of our investigational new drug application;
- $0.1 million increase in other indirect research and development expenses, primarily driven by an increase in facility, office, software and other overhead costs due to increased research and development headcount;
- $1.2 million offsetting decrease in expense related to our PRAX-944 program, driven by a $1.0 million decrease in outsourced research and development and CRO spend, primarily related to toxicology work performed during the six months ended June 30, 2019 to prepare for our Phase 2a clinical trial and a drug product manufacturing campaign that was executed during the six months ended June 30, 2019, as well as a $0.2 million decrease in consulting costs; and
- $0.6 million offsetting decrease in our PRAX-562 program, driven by a $0.7 million decrease in outsourced research and development and CRO spend due to expenses incurred during the six months ended June 30, 2019 to identify our drug product candidate, offset by a $0.1 million increase in consulting spend.

General and Administrative Expense

General and administrative expenses increased $1.0 million from $3.1 million for the six months ended June 30, 2019 to $4.1 million for the six months ended June 30, 2020. The increase in general and administrative expenses was primarily attributable to the following:

- $0.5 million increase in professional fees including legal and consulting services, driven by a $0.4 million increase in consulting costs, primarily in the accounting and business development functions, and a $0.1 million increase in legal fees, primarily related to intellectual property filings, as we expand our research and development activities;
- $0.4 million increase in personnel-related costs driven by increased headcount; and
- $0.1 million increase in facilities, office and other general and administrative expenses to support the increase in our operating activities.

Total Other Income

Total other income for each of the six months ended June 30, 2019 and 2020 was $0.1 million, comprised of interest income on our cash and cash equivalents.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have incurred significant losses in each period. We have not yet commercialized any of our product candidates, which are in various phases of preclinical and clinical development, and we do not expect to generate revenue from sales of any products for several years, if at all. To date, we have financed our operations primarily with proceeds from sales of our redeemable convertible preferred stock and the issuance of convertible debt.

In 2015 and 2016, we raised $1.0 million from the issuance of convertible promissory notes. In October 2016, April 2017 and July 2017, we raised an aggregate of $6.1 million from the sale of our Series A redeemable convertible preferred stock, net of issuance costs. In December 2017 and January 2018, we raised an aggregate of $3.0 million from the issuance of convertible promissory notes. In March and October 2018, we raised an aggregate of $36.8 million from the sale of our Series B redeemable convertible preferred stock, net of issuance costs. In June 2019, we raised $9.9 million from the sale of our Series B-1 redeemable convertible preferred stock, net of issuance costs. In November and December 2019, we raised an aggregate of $50.3 million from the sale of our Series C redeemable convertible preferred stock. In February and March 2020, we repurchased shares of our Series C redeemable convertible preferred stock for an aggregate cash repurchase price of $30.0 million. In April and May 2020, we raised an aggregate of $23.5 million from the sale of our Series C redeemable convertible preferred stock, net of issuance costs.

As of June 30, 2020, we had cash and cash equivalents of $19.7 million. In the third quarter of 2020, we raised an aggregate of approximately $110.1 million from the sale of our Series C-1 redeemable convertible preferred stock, net of issuance costs.

Historical Cash Flows

The following table provides information regarding our cash flows for each period presented (in thousands):

	Year Ended December 31,		Six Months Ended June 30,	
	2018	2019	2019	2020
			(Unaudited)	
Net cash provided by (used in):				
Operating activities	$(20,721)	$(33,420)	$(15,766)	$(18,513)
Investing activities	(63)	(103)	(74)	—
Financing activities	37,804	60,388	9,997	(6,554)
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 17,020	$ 26,865	$ (5,843)	$(25,067)

Operating Activities

Our cash flows from operating activities are greatly influenced by our use of cash for operating expenses and working capital requirements to support the business. We have historically experienced negative cash flows from operating activities as we invested in developing our portfolio, drug discovery efforts and related infrastructure. The cash used in operating activities resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital, which are primarily the result of increased expenses and timing of vendor payments.

During the year ended December 31, 2018, net cash used in operating activities of $20.7 million was primarily due to our $26.5 million net loss, partially offset by $4.4 million of non-cash charges and $1.5 million in changes in operating assets and liabilities.

During the year ended December 31, 2019, net cash used in operating activities of $33.4 million was primarily due to our $35.5 million net loss, partially offset by $1.3 million of non-cash charges and $0.7 million in changes in operating assets and liabilities.

During the six months ended June 30, 2019, net cash used in operating activities of $15.8 million was primarily due to our $17.5 million net loss, partially offset by $0.6 million of non-cash charges and $1.2 million in changes in operating assets and liabilities.

During the six months ended June 30, 2020, net cash used in operating activities of $18.5 million was primarily due to our $19.9 million net loss, partially offset by $0.8 million of non-cash charges and $0.6 million in changes in operating assets and liabilities.

Investing Activities

During the years ended December 31, 2018 and 2019 and the six months ended June 30, 2019, net cash used in investing activities related to the purchase of property and equipment. There were no cash flows from investing activities during the six months ended June 30, 2020.

Financing Activities

During the years ended December 31, 2018 and 2019, net cash provided by financing activities was $37.8 million and $60.4 million, respectively, consisting of net proceeds from the issuance of our Series B redeemable convertible preferred stock and proceeds from the issuance of a convertible note during the year ended December 31, 2018, and net proceeds from the issuance of our Series B-1 redeemable convertible preferred stock and our Series C redeemable convertible preferred stock during the year ended December 31, 2019.

During the six months ended June 30, 2019 and 2020, net cash provided by (used in) financing activities was $10.0 million and $(6.6) million, respectively, consisting of net proceeds from the issuance of our Series B-1 redeemable convertible preferred stock during the six months ended June 30, 2019, and net cash paid for the repurchase and issuance of shares of our Series C redeemable convertible preferred stock during the six months ended June 30, 2020.

Plan of Operation and Future Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing research and development activities, particularly as we advance the preclinical activities and clinical trials of our product candidates. In addition, upon the completion of this offering, we expect to incur additional costs associated with operating as a public company. As a result, we expect to incur substantial operating losses and negative operating cash flows for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- advance the clinical development of our PRAX-114, PRAX-944 and PRAX-562 product candidates;
- advance the development of any additional product candidates;
- conduct research and continue preclinical development of potential product candidates;
- make strategic investments in manufacturing capabilities;
- maintain our current intellectual property portfolio and opportunistically acquire complementary intellectual property;
- seek to obtain regulatory approvals for our product candidates;
- potentially establish a sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize any products for which we may obtain regulatory approval;
- add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and potential future commercialization efforts and to support our transition to a public company; and
- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, complex results, safety issues or other regulatory challenges.

As of June 30, 2020, we had cash and cash equivalents of $19.7 million. In the third quarter of 2020, we raised an aggregate of approximately $110.1 million from the sale of our Series C-1 redeemable convertible preferred stock, net of issuance costs. Based on our current operating plan, we believe that our existing cash and cash equivalents, combined with the cash proceeds from the sale and issuance of our Series C-1 redeemable convertible preferred stock and the anticipated net proceeds from this offering, will enable us to fund our operating expenses and capital expenditure requirements through at least the next 18 months. However, we have based this estimate on assumptions that may prove to be wrong and we could exhaust our capital resources sooner than we expect.

We are unable to estimate the exact amount of our working capital requirements, but based on our available cash resources, without giving effect to the anticipated net proceeds from this offering, we expect to have sufficient cash and cash equivalents on hand to support current operations into the second half of 2021. This circumstance raises substantial doubt about our ability to continue as a going concern for at least one year from the date that our unaudited interim condensed consolidated financial statements for the six months ended June 30, 2020 appearing at the end of this prospectus were issued. We will need to raise additional capital in this offering and/or otherwise to fund our future operations and remain as a going concern. However, we cannot guarantee that we will be able to obtain sufficient additional funding in this offering or otherwise or that such funding, if available, will be obtainable on terms satisfactory to us. In the event that we are unable to obtain sufficient additional funding, there can be no assurance that we will be able to continue as a going concern.

Because of the numerous risks and uncertainties associated with product development, and because the extent to which we may enter into collaborations with third parties for the development of our product candidates is unknown, we may incorrectly estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our funding requirements and timing and amount of our operating expenditures will depend on many factors, including, but not limited to:

- the scope, progress, results and costs of preclinical studies and clinical trials for our programs and product candidates;
- the number and characteristics of programs and technologies that we develop or may in-license;
- the costs and timing of future commercialization activities, including manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the costs necessary to obtain regulatory approvals, if any, for products in the United States and other jurisdictions, and the costs of post-marketing studies that could be required by regulatory authorities in jurisdictions where approval is obtained;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the continuation of our existing licensing arrangements and entry into new collaborations and licensing arrangements;
- the costs we incur in maintaining business operations;
- the costs associated with being a public company;
- the revenue, if any, received from commercial sales of any product candidates for which we receive marketing approval;
- the effect of competing technological and market developments;
- the impact of any business interruptions to our operations or to those of our manufacturers, suppliers or other vendors resulting from the COVID-19 pandemic or similar public health crisis; and
- the extent to which we acquire or invest in businesses, products and technologies, including entering into licensing or collaboration arrangements for product candidates, although we currently have no commitments or agreements to complete any such acquisitions or investments in businesses.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

Adequate additional funds may not be available to us on acceptable terms, or at all. We do not currently have any committed external source of funds. Market volatility resulting from the COVID-19 pandemic or other factors could also adversely impact our ability to access capital as and when needed. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Additional debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute your ownership interest.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2019 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods (in thousands):

	Payments Due by Period				
	Total	Less Than 1 Year	1 to 3 Years	3 to 5 Years	More Than 5 Years
Operating lease commitments(1)	$1,574	$ 783	$791	$ —	$ —
Total	$1,574	$ 783	$791	$ —	$ —

(1) We sublease building space in Cambridge, Massachusetts. Our sublease will expire on December 30, 2021. The amounts in the table above represent the fixed contractual lease obligations.

We have collaboration and license agreements with Purdue Neuroscience Company, or Purdue, RogCon Inc., or RogCon, and Ionis Pharmaceuticals, Inc., or Ionis, under which we could be obligated to pay certain fees, milestone payments and cost reimbursements.

Under our license agreement with Purdue, we are obligated to make future milestone payments of up to $33.0 million based on the achievement of specified development and sales milestones. Furthermore, we will pay Purdue royalties of a low-single-digit percentage of annual net sales of licensed products. Either party may terminate the license agreement for convenience or in the event of a material breach by the other party and failure to cure such breach within a certain period of time. If the agreement is voluntarily terminated by Purdue, our license rights will survive such termination and such rights will become fully paid, perpetual and irrevocable. As the agreement may be terminated for convenience, the payments are not included in the table above. See “Business—License Agreements—License Agreement with Purdue.”

Under our license agreement with RogCon, we will reimburse RogCon for its out-of-pocket costs incurred for activities performed under the license agreement. Additionally, we may pay RogCon a milestone payment of $3.0 million and profit share payments. The $3.0 million milestone payment will become due when the first profit share payment has become due and payable and certain contingent payments have become due and payable to Ionis under our collaboration agreement with Ionis. The profit share payments will be based on a low-double-digit percentage of net profits, depending on sales volume. Either party may terminate the license agreement for material breach or insolvency of the other party. Additionally, we may terminate for convenience with prior notice to RogCon. As such, we do not include variable and contingent payments under our agreement with RogCon in the table above as they are not fixed and estimable. See “Business—License Agreements—License Agreement with RogCon.”

Under our collaboration agreement with Ionis, we are obligated to reimburse any out-of-pocket costs incurred by Ionis related to research activities, identification of a development candidate and conducting an IND-enabling toxicology study. We also have an exclusive option to obtain the rights and license related to the development candidate following the completion of the IND-enabling toxicology study. If we exercise our development candidate option, we may be required to make additional payments to Ionis

including a license fee, development milestone payments, additional milestone payments and sales royalties or sublicense fees. However, we are not obligated to exercise our development candidate option and are able to terminate our collaboration agreement with Ionis for convenience. Either party may terminate the collaboration agreement upon material breach or insolvency of the other party or if Ionis is unable to identify a development candidate. Ionis may terminate if we fail to achieve a performance milestone. As such, payments due pursuant to the exercise of our development candidate option are contingent and therefore excluded from the table above as they are not fixed and estimable. See "Business—License Agreements—Ionis Collaboration Agreement."

We have agreements with certain vendors for various services, including services related to clinical operations and support, for which we are not contractually able to terminate for convenience and avoid any and all future obligations to the vendors. Under such agreements, we are contractually obligated to make certain payments to vendors to reimburse them for their unrecoverable outlays incurred prior to cancellation. The exact amounts of such obligations are dependent on the timing of termination and the exact terms of the relevant agreement and cannot be reasonably estimated. We do not include these payments in the table above as they are not fixed and estimable.

In addition, we enter into indemnification agreements and agreements containing indemnification provisions in the ordinary course of business. Pursuant to these agreements, we agree to indemnify, hold harmless and reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally our business partners, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to our products. The term of these indemnification agreements is generally perpetual upon execution of the agreement. The maximum potential amount of future payments we could be required to make under these indemnification agreements cannot be reasonably estimated and therefore is not included in the table above.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements and the notes to our unaudited interim condensed consolidated financial statements appearing elsewhere in this prospectus, we believe the following accounting policies used in the preparation of our consolidated financial statements require the most significant judgments and estimates.

Fair Value Measurements

Series B Preferred Stock Tranche Obligation

We determined that our obligation to issue additional shares of our Series B redeemable convertible preferred stock upon the occurrence of a specified clinical milestone event represented a

freestanding financial instrument. The resulting tranche liability was initially recorded at fair value, with gains and losses arising from changes in fair value recognized in other income (expense) in the consolidated statements of operations and comprehensive loss. The tranche liability was remeasured at each reporting period and upon the settlement of the obligation. The tranche liability was valued using a binomial model with significant inputs including the estimated future value of our Series B redeemable convertible preferred stock, the discount rate, estimated time from the initial closing of our Series B redeemable convertible preferred stock to the tranche closing, and probability of the tranche closing. The obligation was fully satisfied in October 2018 upon the second closing of our Series B redeemable convertible preferred stock, upon which the tranche liability was remeasured and reclassified to Series B redeemable convertible preferred stock.

Anti-Dilution Obligation

We concluded that our obligation to issue additional shares of our redeemable convertible preferred stock to Purdue to maintain a specified ownership percentage in our capital stock through issuances of our Series B redeemable convertible preferred stock represented a freestanding financial instrument. The resulting anti-dilution obligation was initially recorded at fair value, with gains and losses arising from changes in fair value recognized in other income (expense) in the consolidated statements of operations and comprehensive loss. The anti-dilution obligation was remeasured at each reporting period and upon the settlement of the obligation. The anti-dilution obligation was valued using a discounted cash flow model under the income approach, using significant inputs including the estimated future value of our Series B redeemable convertible preferred stock, discount rates, estimated time to liquidity and probability of each tranche closing. The anti-dilution obligation was settled in October 2018, upon which it was remeasured and reclassified to Series B redeemable convertible preferred stock.

Conversion Features

We issued two separate unsecured convertible promissory notes to an investor in 2017 and 2018 that would automatically convert, upon an equity financing of at least $2.0 million in gross proceeds, into shares of the type of equity securities issued in such financing, at a discount. We determined that the conversion features represented a derivative instrument which was initially recorded at fair value, with gains and losses arising from changes in fair value recognized in other income (expense) in the consolidated statements of operations and comprehensive loss. The conversion features were remeasured at each reporting period and upon settlement. The fair value of the conversion features was determined by calculating the fair values of the convertible promissory notes with and without the conversion features. The difference between the fair values of the convertible promissory notes in the “with” and “without” scenarios was determined to be the initial fair value of the conversion features. The valuation used significant inputs including the probability of various exit scenarios and discount rates. The conversion features were settled, and the notes converted into shares of Series B redeemable convertible preferred stock, upon the initial closing of our Series B redeemable convertible preferred stock in March 2018.

Research and Development Expenses and Related Accruals

Research and development expenses include costs directly attributable to the conduct of research and development programs, including personnel-related expenses such as salaries, benefits, and stock-based compensation expense; materials; supplies; manufacturing and external costs related to outside vendors engaged to conduct both preclinical studies and clinical trials; and the allocable portions of facility costs, such as rent, utilities, depreciation, and general support services. All costs associated with research and development activities are expensed as incurred.

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open

contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf, and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. When evaluating the adequacy of the accrued liabilities, we analyze progress of the studies or trials, including the phase or completion of events, invoices received and contracted costs. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with performing research services and preclinical and clinical studies;
- investigative sites or other providers in connection with preclinical and clinical studies;
- vendors in connection with preclinical and clinical development activities; and
- vendors related to product manufacturing, development and distribution of preclinical and clinical supplies.

The financial terms of our agreements with CROs are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of milestones. In accruing service fees, we estimate the time period over which services will be performed, enrollment of patients, number of sites activated and the level of effort expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period.

Stock-Based Compensation

We issue stock-based awards to employees and non-employees, generally in the form of stock options and restricted stock awards. We measure all stock-based awards granted to employees and non-employees as stock-based compensation expense at fair value in accordance with FASB ASC Topic 718, Compensation—Stock Compensation, or ASC 718.

For stock-based awards issued to employees, non-employees and members of the board of directors, or the Board, for their services on the Board, we measure the estimated fair value of the stock-based award on the date of the grant. We recognize compensation expense for those awards granted to employees and members of the Board over the requisite service period, which is generally the vesting period of the respective award. For non-employee awards, compensation expense is recognized as the services are provided, which is generally ratably over the vesting period. We issue stock-based awards with service-based vesting conditions and record the expense for these awards on a straight-line basis over the vesting period. To date, we have not issued any stock-based awards with performance or market-based vesting conditions. We account for forfeitures as they occur.

We classify stock-based compensation expense in our consolidated statements of operations and comprehensive loss in the same manner in which the award recipient’s salary or service payments are classified.

Fair Value of Stock-Based Awards

We determine the fair value of restricted stock based on the fair value of our common stock less purchase price. We estimate the fair value of our stock options using the Black-Scholes option pricing model, which requires inputs of subjective assumptions, including: (i) the expected volatility of our stock; (ii) the expected term of the award; (iii) the risk-free interest rate; (iv) expected dividends; and (v) the fair value of common stock. Due to the lack of company-specific historical and implied volatility data for our common stock, we determine the volatility for awards granted based on an analysis of reported data for a group of guideline publicly-traded companies that issued options with substantially similar terms. For this analysis, we select companies with comparable characteristics to ours including enterprise value, risk profiles and with historical share price information sufficient to meet the expected life of the stock-based awards. We determine expected volatility using a weighted average of the historical volatilities of the guideline group of companies. We will continue to apply this process until such a time as we have adequate historical data regarding the volatility of our own traded stock price. As permitted under ASC 718, we have elected to use the contractual term as the expected term for certain non-employee awards, on an award-by-award basis. For all other awards, we determined the expected term utilizing the “simplified” method whereby the expected term equals the average of the vesting term and the original contractual term of the option, on a weighted basis based on the vesting of each tranche. We utilize this method as we have insufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. For the determination of the risk-free interest rate, we utilize the U.S. Treasury yield curve for instruments in effect at the time of measurement with a term commensurate with the expected term assumption. The expected dividend yield is assumed to be zero as we have never paid dividends, and do not have current plans to pay any dividends on our common stock.

Determination of Fair Value of Common Stock

Given the absence of an active market for our common stock, the fair value of shares of common stock underlying our stock-based awards was determined on each grant date by the Board with input from management, considering our most recently available third-party valuations of common stock and the Board’s assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the grant date. Historically, these independent third-party valuations of our equity instruments were performed contemporaneously with identified value inflection points. The third-party valuations were prepared in accordance with the framework of the American Institute of Certified Public Accountants’ Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, or the Practice Aid. The Practice Aid identifies various available methods for allocating enterprise value across classes and series of capital stock to determine the estimated fair value of common stock at each valuation date.

In addition to considering the results of these third-party valuations, our Board considered various objective and subjective factors to determine the fair value of our equity instruments as of each grant date, which may be later than the most recently available third-party valuation date, including:

- the lack of liquidity of our equity as a private company;
- the prices of our redeemable convertible preferred stock sold to outside investors in arm’s length transactions, and the rights, preferences and privileges of our redeemable convertible preferred stock as compared to those of our common stock, including the liquidation preferences of our redeemable convertible preferred stock;
- the progress of our research and development efforts, including the status of preclinical and clinical studies for our product candidates;
- our stage of development and business strategy and the material risks related to our business and industry;

- the achievement of enterprise milestones, including entering into strategic collaborative and license agreements;
- the valuation of publicly-traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of peer companies;
- any external market conditions affecting the biotechnology industry and trends within the biotechnology industry;
- the likelihood of achieving a liquidity event, such as an initial public offering, or a sale of our company, given prevailing market conditions; and
- the analysis of initial public offerings and the market performance of similar companies in the biotechnology industry.

There are significant judgments and estimates inherent in these valuations. These judgments and estimates include assumptions regarding our future operating performance, the stage of development of our product candidates, the timing and probability of a potential initial public offering or other liquidity event and the determination of the appropriate valuation methodology at each valuation date. The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

Once a public trading market for our common stock has been established in connection with the consummation of this offering, it will no longer be necessary for our Board to estimate the fair value of our common stock in connection with our accounting for granted stock options and restricted stock, as the fair value of our common stock will be determined based on its trading price on The Nasdaq Global Select Market.

Valuation Methodologies

The option-pricing method, or OPM, treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceed the value of the liquidation preferences at the time of a liquidity event, such as a strategic sale or merger. The OPM uses the Black-Scholes option-pricing model to price the call options. Inputs to the model include the anticipated timing of a potential liquidity event and the estimated volatility of the equity securities.

Under the probability-weighted expected return method, or PWERM, the fair value of common stock is estimated based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. Present value is calculated using an appropriate risk-adjusted discount rate.

The hybrid method is a PWERM where the equity value in one of the scenarios is calculated using an OPM.

Third-party appraisals of our common stock were prepared as of March 13, 2018, June 24, 2019, May 1, 2020, August 1, 2020 and September 1, 2020. In each appraisal, the value of our equity was calibrated to a contemporaneous transaction in our redeemable convertible preferred stock. For the March 13, 2018 and June 24, 2019 appraisals, the OPM was used to back-solve to the most recent transaction in our preferred shares. For the May 1, 2020, August 1, 2020 and September 1, 2020 appraisals, the back-solve calculation used a hybrid PWERM. The hybrid PWERM considered three scenarios: an "early" IPO completed in 2020, a "late" IPO completed in 2021 and a "remain private"

scenario in which value is allocated using the OPM. An incremental discount for lack of marketability, or DLOM, is applied to the values of the common stock. The DLOM is estimated using a put option model which considers the expected time to liquidity and the volatility of the common shares.

These appraisals resulted in valuations of our common stock of $2.27 per share as of March 13, 2018, $3.30 per share as of June 24, 2019, $5.59 per share as of May 1, 2020, $8.27 per share as of August 1, 2020 and $8.91 per share as of September 1, 2020.

Grants of Stock-Based Awards

The following table summarizes by grant date and type of award, the number of stock-based awards granted between January 1, 2018 and the date of this prospectus, the per share exercise price, the fair value of common stock on each grant date and the per share estimated fair value of the awards, along with the fair value per award on the date of grant:

Grant Date	Type of Award	Number of Shares	Exercise Price per Share(1)	Fair Value of Common Stock per Share on Grant Date(1)	Weighted-Average Estimated Fair Value Per Share of Awards(2)
October 19, 2018	Options	1,128,318	2.27	2.27	1.65
September 24, 2019	Options	442,232	3.30	3.30	2.25
June 5, 2020	Options	1,683,265	5.59	5.59	4.02
August 19, 2020	Options	465,652	8.27	8.27	5.86
September 14, 2020	Options	2,146,701	8.91	8.91	6.40

(1) The exercise price per share and fair value of common stock per share represents the fair value of our common stock on the date of grant, as determined by the Board, after taking into account our most recently available contemporaneous valuation of our common stock as well as additional objective and subjective factors through the date of grant.

(2) Reflects the weighted-average fair value of options granted on each grant date, determined using the Black-Scholes option-pricing model.

We did not grant any shares of restricted common stock during this period.

JOBS Act and Emerging Growth Company Status

In April 2012, the JOBS Act was enacted. As an emerging growth company, or EGC, under the JOBS Act, we may delay the adoption of certain accounting standards until such time as those standards apply to private companies. Other exemptions and reduced reporting requirements under the JOBS Act for EGCs include presentation of only two years of audited financial statements in a registration statement for an initial public offering, an exemption from the requirement to provide an auditor's report on internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002, an exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation and less extensive disclosure about our executive compensation arrangements. Additionally, the JOBS Act provides that an EGC can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an EGC to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of the extended transition period and, therefore, while we are an EGC we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not EGCs, unless we choose to early adopt a new or revised accounting standard.

We will remain classified as an EGC until the earlier of: (i) the last day of our first fiscal year in which we have total annual gross revenues of $1.07 billion or more, (ii) the last day of the fiscal year

following the fifth anniversary of completion of this offering, (iii) the date on which we have issued more than $1.0 billion of non-convertible debt instruments during the previous three fiscal years or (iv) the date on which we are deemed a “large accelerated filer” under the rules of the SEC.

Recently Issued Accounting Pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus, such standards will not have a material impact on our financial statements or do not otherwise apply to our current operations.

Quantitative and Qualitative Disclosures About Market Risks

Interest Rate Fluctuation Risk

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our cash equivalents are invested in short-term U.S. Treasury obligations. However, because of the short-term nature of the instruments in our portfolio, an immediate change in market interest rates of 100 basis points would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

Foreign Currency Fluctuation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation Fluctuation Risk

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2018 or 2019 or the six months ended June 30, 2019 or 2020.

BUSINESS

Company Overview

We are a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system, or CNS, disorders characterized by neuronal imbalance. Normal brain function requires a delicate balance of excitation and inhibition in neuronal circuits, which, when dysregulated, can lead to abnormal function and disease. We are applying insights into the genetic mutations that drive excitation-inhibition imbalance in diseases to select biological targets for severe pediatric epilepsies and more broadly for prevalent psychiatric diseases and neurologic disorders. We apply a deliberate and pragmatic precision approach, leveraging a suite of translational tools including novel transgenic and predictive translational animal models and electrophysiology markers, to enable an efficient path to proof-of-concept in patients. Through this approach, we have established a broad portfolio, including five disclosed programs across multiple CNS disorders, including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. We expect multiple topline clinical trial readouts from all three programs prior to the end of 2021 and anticipate the launch of a new clinical development program in 2021. We intend to develop differentiated therapies that can deliver long-term benefits to human health by meaningfully impacting patients and society.

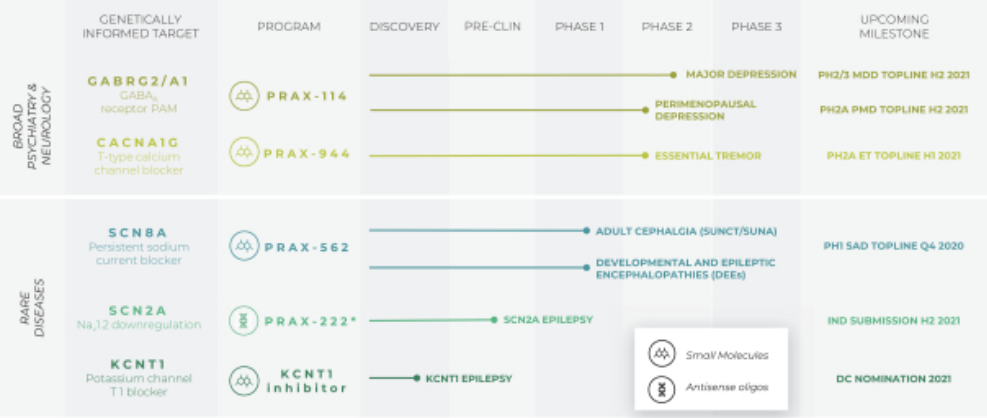
Our lead clinical program, PRAX-114, is an extrasynaptic-preferring $GABA_A$ receptor positive allosteric modulator, or PAM, for the treatment of patients suffering from major depressive disorder, or MDD, and perimenopausal depression, or PMD. Together, these conditions affect more than 22 million people in the United States, many of whom are not responsive to or are underserved by current treatments. PRAX-114 is under development as a potentially differentiated treatment as both a monotherapy and adjunctive therapy for both acute and maintenance settings. We believe that PRAX-114 has several advantages relative to currently available therapies and product candidates in the $GABA_A$ PAM therapeutic class, including a wider therapeutic window, patient-centric dosing and indication expansion opportunities. We have a multi-cohort, three-part Phase 2a clinical trial ongoing in Australia, and in Part A of the trial, we observed marked improvements in depression scores in MDD patients within two weeks of treatment. We expect complete topline data from Part B of the trial in the second half of 2021 and from longer-term dosing in MDD patients in Part C of the trial in the fourth quarter of 2020. We are planning to initiate a Phase 2/3 trial in the United States and Australia in the fourth quarter of 2020, which is intended to satisfy one of two registrational trials required by the U.S. Food and Drug Administration, or the FDA, to support clinical efficacy for the treatment of MDD, and we expect topline data in the second half of 2021.

Our second clinical program, PRAX-944, is a potentially differentiated selective small molecule inhibitor of T-type calcium channels, for the treatment of Essential Tremor, or ET. ET is a progressive and debilitating movement disorder with action tremors that significantly disrupt daily living, with an estimated prevalence of up to seven million patients in the United States. There is a high unmet need for ET patients given the limited treatment options, with only one approved pharmacotherapy that is poorly tolerated, resulting in high discontinuation rates and the need for invasive brain surgeries. Successful development of T-type calcium channel modulators in ET likely requires a PK profile with a blunted Cmax, which we believe positions our modified release formulation for PRAX-944 to be a differentiated therapy. In these trials, we have evaluated the safety and tolerability of PRAX-944 in over 150 healthy volunteers in five separate clinical trials. We have studied the safety of PRAX-944 modified release formulation with titration up to 120mg/day and no maximum tolerated dose, or MTD, has been identified. We are currently conducting a Phase 2a proof-of-concept open-label trial in ET patients. Preliminary site data from five patients of the low dose cohort showed tremor reduction, which seems to compare favorably to the standard of care agents. We plan to announce topline data, including a high dose cohort, in the first half of 2021.

Our lead rare disease product candidate and third clinical program, PRAX-562, is the first selective persistent sodium current blocker in development for the treatment of a broad range of rare,

devastating CNS disorders, such as severe pediatric epilepsy and adult cephalgia. To date, PRAX-562 has demonstrated pharmacological activity in *in-vivo* models with significantly improved tolerability, suggesting a potentially improved therapeutic index compared to other sodium channel blockers. We have initiated a Phase 1 trial of PRAX-562 in Australia to evaluate the safety, tolerability, pharmacokinetics, or PK, and effects on an EEG biomarker in up to 129 adult healthy volunteers. We anticipate Phase 1 single ascending dose, or SAD, topline safety data in the fourth quarter of 2020. In addition to our clinical programs, we have one preclinical program and one disclosed discovery program in development for severe genetic epilepsies and we continue to evaluate additional programs.

Below is a summary of our portfolio of programs, organized by their initial therapeutic focus addressing either broad psychiatric and neurologic conditions or rare diseases. We own global commercialization rights for all of our product candidates.



* PRAX-222 is a collaboration with Ionis Pharmaceuticals, or Ionis, and RogCon Inc. Ionis is eligible to receive royalties as a percentage of net product sales worldwide in the low-20s.

Our company was founded by scientific innovators Kiran Reddy, M.D., David Goldstein, Ph.D. and Steven Petrou, Ph.D., who have pioneered work to identify and characterize de novo mutations in several dozen genes believed to cause a number of forms of severe pediatric epilepsies. These genes regulate key neuronal circuits in the brain which, when dysregulated, can result in severe seizure phenotypes as well as comorbid developmental delays, cognitive deficits, sensory-motor issues and often early death. Further, based on our understanding of a body of preclinical and clinical evidence, we now believe that these genes also play critical roles in the predisposition to other more prevalent neurologic and psychiatric disorders, such as mood disorders, movement disorders, pain syndromes, autism, migraine and schizophrenia, making them attractive targets for therapeutic intervention for a wide range of CNS disorders.

We have attracted a talented team of scientists and researchers in genetics and biology, chemistry and translational medicine as well as business leaders with established track records of successfully executing innovative drug discovery and development programs. Our Chief Executive Officer, Marcio Souza, most recently served as Chief Operating Officer at PTC Therapeutics, Inc. and was instrumental in the development and commercialization of multiple approved products while at NPS Pharmaceuticals, Inc., Shire Human Genetic Therapies Inc. and Sanofi Genzyme Corporation. Our Chief Medical Officer, Bernard Ravina, M.D., previously Chief Medical Officer at Voyager Therapeutics,

Inc., is a neurologist and movement disorder specialist who brings decades of neurologic drug development experience from roles at Biogen, the University of Rochester and the NIH's Institute of Neurological Disorders and Stroke. Our Chief Financial Officer, Stuart Chaffee, Ph.D., co-founded Kymera Therapeutics, Inc. and has held multiple senior roles in finance, business development and corporate strategy at Biogen Inc., Zafgen, Inc. and Amgen Inc.

Our Approach

Each of our programs is based on four key principles that we believe will both increase the probability of success and allow us to efficiently translate insights into high-impact therapies for patients and society:

1. ***Focus on therapeutic targets identified through human genetics.*** Numerous CNS disorders are caused by an imbalance of excitation and inhibition in neuronal circuitry. By applying insights derived from the genetics of pediatric epilepsies, we have identified biological targets that we believe are implicated in determining neuronal excitability, not only in epilepsies, but also in a variety of more prevalent CNS disorders. For example, human genetics points to the relevance of the GABAergic system where mutations in $GABA_A$ receptors are associated with a number of rare pediatric epilepsies. The GABAergic system is also implicated in MDD, where enhancing $GABA_A$ activity is believed to be beneficial. As our understanding of the genetic underpinning of these disorders evolves, we plan to continually apply learnings to expand and advance our portfolio.
2. ***Utilize translational tools to validate the potential of our targets and product candidates.*** We leverage a number of translational tools to both confirm pharmacodynamic effects of our product candidates in the brain and establish on-mechanism effects, which we believe will result in an increased probability of success in the clinic. Our programs utilize target-specific EEG endpoints to serve as robust markers of pharmacological engagement of the drug target and novel transgenic animal models to assess the therapeutic activity of our molecules. We expect these tools, along with rigorous preclinical PK and pharmacodynamic characterization of our molecules will position us to more efficiently translate preclinical findings into clinical utility.
3. ***Pursue efficient, rigorous clinical development paths to proof-of-concept in humans***. Our development strategies are focused on defining efficient paths to demonstrate the safety and therapeutic activity of our programs in humans. We select indications that we believe will enable the early demonstration of desired effect in a relatively small patient sample and we focus on clinical endpoints that both minimize inter-patient variability and offer a clear connection between pharmacodynamic effects and clinical measures that are meaningful to patients, physicians and regulatory agencies. Our global network of contract research organizations, or CROs, and scientists affords us the flexibility to conduct research and development activities in diverse geographic locations to accelerate our development timelines and limit geographic risks.
4. ***Apply patient-centric development strategies.*** We pursue the development of candidates that address the treatment needs of patients and the treating community, including targeting the underlying disease pathology versus just symptom management. We intend to develop therapies that provide patients long-term relief from their disorders and significantly reduce the overall burden to patients and caregivers. Our development strategies are tailored to demonstrate these benefits.

Our Strategy

Our goal is to translate genetic insights into high-impact therapies for millions of people suffering from CNS disorders characterized by imbalance of neuronal excitation-inhibition. Key components of our strategy include:

- **Advance PRAX-114 in MDD and PMD toward regulatory approval and commercialization.** PRAX-114 is a potentially differentiated $GABA_A$ receptor PAM currently in Phase 2a development for the treatment of MDD and PMD. Based on early clinical data where we observed a marked improvement of depression scores in MDD patients within two weeks of treatment, we are planning to initiate a Phase 2/3 trial in the United States and Australia in the fourth quarter of 2020, which is intended to satisfy one of two registrational trials required by the FDA to support clinical efficacy for the treatment of MDD, and we expect topline data in the second half of 2021. We are conducting a Phase 2a trial in Australia in PMD and expect to announce topline data in the second half of 2021. We intend to develop PRAX-114 in the United States and in other countries as both a monotherapy and adjunctive therapy for MDD and PMD in both acute and maintenance settings. While we have been diligently pursuing our strategy to efficiently advance PRAX-114 towards regulatory approval and commercialization, there is no assurance that we will be successful in obtaining regulatory approval in an accelerated manner, or at all. For a detailed description of the risks related to the development and commercialization of our product candidates, please refer to the section entitled “Risk Factors” in this prospectus.
- **Advance PRAX-944 in ET toward regulatory approval and commercialization.** PRAX-944 is a potentially differentiated selective small molecule inhibitor of T-type calcium channels in development for ET. In these trials, we have evaluated the safety and tolerability of PRAX-944 in over 150 healthy volunteers in five separate clinical trials. We have studied the safety of PRAX-944 modified release formulation with titration up to 120mg/day and no MTD has been identified. We are currently conducting a Phase 2a proof-of-concept open-label trial in Australia and New Zealand in ET patients. Preliminary site data from five patients of the low dose cohort showed tremor reduction, which seems to compare favorably to the standard of care agents. We plan to announce topline data, including a high dose cohort, in the first half of 2021. While we have been diligently pursuing our strategy to efficiently advance PRAX-944 towards regulatory approval and commercialization, there is no assurance that we will be successful in obtaining regulatory approval in an accelerated manner, or at all. For a detailed description of the risks related to the development and commercialization of our product candidates, please refer to the section entitled “Risk Factors” in this prospectus.
- **Build a rare disease franchise.** We are advancing several programs for patients with rare diseases, such as genetically defined populations suffering from Developmental and Epileptic Encephalopathies, or DEEs, a group of disorders associated with severe and frequent seizures, developmental delays, cognitive decline and high mortality rates. We have three rare disease programs in our pipeline, including PRAX-562, which we believe represents the first selective persistent sodium current blocker in development for the treatment of a number of rare diseases with limited or no treatment options. We have an ongoing Phase 1 trial for PRAX-562 in healthy volunteers in Australia and anticipate topline from the SAD study by fourth quarter of 2020. We plan to initiate a Phase 2 trial for the treatment of Short-lasting Unilateral Neuralgiform headache attacks with Conjunctival injection and Tearing, or SUNCT, and Short-lasting Unilateral Neuralgiform headache with Autonomic symptoms, or SUNA, cephalgias. If we observe clinical proof-of-concept, we will then expand into DEEs. We believe that our additional programs for genetically-defined pediatric epilepsies will position Praxis as a leader in developing therapies for patients suffering from DEEs. Given the overlapping biology, phenotypic presentation and clinical execution considerations, we believe that we can translate learnings for the treatment of DEEs across our portfolio in order to more efficiently bring additional treatments to market.

- **Maximize the value of our product candidates through select indication expansion.** All of our clinical stage product candidates address targets with therapeutic potential beyond their lead indications. As these programs advance through the clinic, we will pragmatically evaluate indication expansion and consider subsequent clinical development that will expand the labels of our product candidates to encompass other compelling opportunities at a time when we determine to be most efficient.
- **Advance our understanding of genetics and neuronal imbalance to maintain our leadership and continue to build our pipeline.** Advances in the field of genetics continue to elucidate new insights into mutations that drive neuronal imbalance. Our team is deeply engaged in these efforts, which we believe will enable us to pursue a pipeline discovery and development strategy grounded in these learnings and coupled with our drug discovery, translational and clinical experience. As our knowledge base continues to grow, we believe our potential to deliver additional differentiated medicines for patients will grow as well.
- **Commercialize our products in the United States and globally.** To realize the full potential of our product candidates, we intend to build a sales and marketing infrastructure to reach prescribers in the United States. In order to capitalize on market opportunities outside the United States, we may pursue collaborations with reputable pharmaceutical companies that have established presences in key geographies.

BROAD PSYCHIATRY AND NEUROLOGY PROGRAMS

PRAX-114

We are developing PRAX-114, an extrasynaptic-preferring $GABA_A$ receptor positive allosteric modulator, or PAM, for the treatment of patients suffering from major depressive disorder, or MDD, and perimenopausal depression, or PMD. PRAX-114 is a potentially differentiated treatment as a monotherapy and adjunctive therapy for both acute and maintenance settings. We have a multi-cohort, three-part Phase 2a clinical trial ongoing in Australia. We observed marked improvements in depression scores in MDD patients in Part A of this trial within two weeks of treatment. We expect complete topline data from Part B of the trial in the second half of 2021 and from longer-term dosing in MDD patients in Part C of the trial in the fourth quarter of 2020. We are planning to initiate a Phase 2/3 trial in the United States and Australia in the fourth quarter of 2020, which is intended to satisfy one of two registrational trials required by the FDA to support clinical efficacy for the treatment of MDD, and we expect topline data in the second half of 2021.

There is significant unmet medical need in MDD and PMD with over 22 million individuals suffering from depressive symptoms in the U.S. Current pharmacological interventions suffer from multiple shortcomings including slow onset of efficacy, low response rates and side effects that limit patient compliance. PRAX-114 targets an increasingly well-understood neuronal circuit in the brain that we believe, when properly modulated, can result in a robust antidepressant effect with an advantageous safety and tolerability profile.

We believe that our PRAX-114 program has several advantages as compared to currently available therapies and product candidates in the $GABA_A$ PAM therapeutic class:

- **Wider Therapeutic Window**. We have determined that PRAX-114 is an approximately 10.5-fold more potent PAM of the extrasynaptic form of $GABA_A$ receptors compared to the synaptic form. By preferentially modulating extrasynaptic $GABA_A$ receptors, we believe PRAX-114 has the potential to mediate antidepressant and anxiolytic activity without the significant sedation observed with less selective neuroactive steroids.
- **Patient-Centric Dosing.** We believe the ability to administer PRAX-114 with or without food is key for clinical and commercial success in MDD and is critical for a patient-centric

therapeutic, as many patients with depression suffer from appetite disturbance. We have observed fast absorption of PRAX-114 within one to three hours of dosing and a robust PK profile across multiple trials. Based on clinical findings to date, we believe that PRAX-114 does not need to be taken with food to achieve therapeutic exposure, whereas other $GABA_A$ PAMs may require food to achieve therapeutic exposure.

- **Sustained Administration**. After consultation with the FDA and other stakeholders in MDD and PMD therapy, we designed our Phase 2/3 trial of PRAX-114 to include 28-day nightly dosing to evaluate patients at both 14 days to assess the rapidity and robustness of response and 28 days to measure durability of effect. We believe that having a dosing paradigm consistent with the duration of depressive episodes will provide the most substantial benefit to patients in controlling their disease, further differentiating PRAX-114 from other $GABA_A$ PAMs.
- **Indication Expansion.** Based on the novel pharmacology of PRAX-114 and its generally well-tolerated profile in clinical trials to date, we believe PRAX-114 is suitable for potential development across a wide-range of indications in psychiatry and neurology, providing sizable expansion opportunities to explore in addition to MDD.

Major Depressive Disorder

Major Depressive Disorder, or MDD, is a chronic psychiatric condition causing severe impairments that interfere with the ability to carry out life activities. An MDD episode is characterized by a period of at least two weeks of persistent depressed mood and/or the loss of interest or pleasure in activities, accompanied by sleep and appetite disturbance, fatigue, concentration difficulty, cognitive impairment, feelings of guilt, psychomotor retardation or agitation and suicidal ideation. MDD is one of the most prevalent psychiatric disorders. In the United States, approximately 19 million adults, or 7% of the adult population suffer from MDD, with episodes lasting on average six to eight months. It is estimated that MDD affects more than 300 million people worldwide. Moreover, the prevalence of depression has increased during the COVID-19 pandemic in the US and globally. In the United States, depression symptoms have increased by more than 3-fold overall during the COVID-19 pandemic. The most dramatic increases are reported in moderate, moderately severe and severe depression symptoms, with a 2.6-fold, 3.7-fold, and 7.5-fold rise, respectively, relative to a pre-COVID-19 pandemic period.

MDD is a chronic psychiatric condition that requires long-term treatment, with the ultimate goal of achieving remission. MDD is associated with an elevated risk of suicide, underscoring the need for rapid and effective treatment. The most explored pharmacological mechanisms for treating MDD target monoamine neurotransmitters. Drugs in this class include selective serotonin reuptake inhibitors, or SSRIs, serotonin and norepinephrine reuptake inhibitors, or SNRIs, bupropion and other monoaminergic medications. SSRIs and SNRIs are associated with significant side effects, including weight gain, sexual dysfunction, drowsiness, nausea, insomnia and discontinuation syndrome, which negatively impact treatment outcomes, quality of life and adherence in MDD patients.

Approximately seventy percent of MDD patients fail to respond to current first-line antidepressant treatments. Further, those patients that are responsive typically require approximately six to eight weeks of treatment to show a clinically meaningful response. Slow onset of action is a substantial unmet need in MDD, with some of the most commonly prescribed antidepressants showing a reduction in the Hamilton Depression Scale, or HAM-D, of approximately 6- to 8-points and a difference from placebo of approximately 1-2 points at Week 2 (Figure 1). Moreover, approximately 40% of patients on therapy discontinue treatment due to either a loss of response or adverse side effects. Finally, 33% of patients fail to respond after treatment with three or more different standard of care therapies.

Among the MDD patients who experience a response to treatment, the majority do not achieve remission. Even for patients deemed responsive, disease burden often persists through the presence of residual depression symptoms that lead to an ongoing negative impact on home, interpersonal and

occupational functioning, as well as a significantly increased risk of relapse of the full depressive syndrome and worse comorbid outcomes, including suicide.

Despite the numerous and long-standing antidepressant treatment options, there continues to be an unmet need for antidepressants that provide rapid onset of effect, higher remission rates, efficacy throughout the depressive episode, an improved tolerability profile and patient-centric dosing that is aligned with the clinical care and the course of MDD and its accompanying comorbid symptoms.

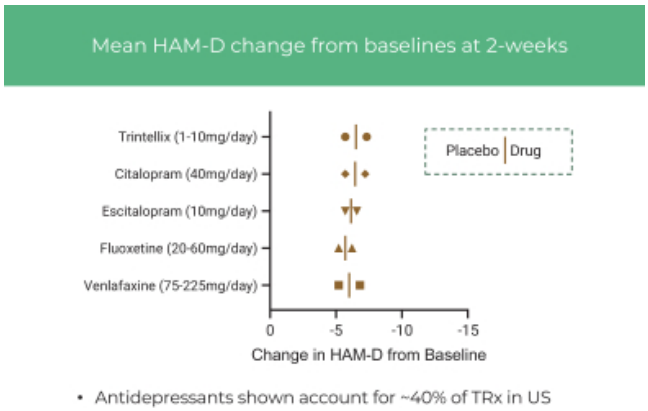


Figure 1. Reduction of HAM-D from baseline of commonly prescribed antidepressants and placebo at Week 2.

Perimenopausal depression

Perimenopause is the transition between the onset of hormonal and clinical features of menopause and the one-year period after the final menses. Perimenopause can last up to 10 years. Women with no lifetime history of major depression who have entered the perimenopause period are found to be twice as likely to develop significant depressive symptoms as women who have not entered the perimenopause period. Notably, the increased risk for depression during the perimenopausal transition has been observed to decline substantially after the final menstrual period.

There are over 30 million women in the United States between the ages of 45 and 59 years who are at risk of developing perimenopause symptoms, with an estimated three million developing mood symptoms such as depression, anxiety, irritability and suicidal ideation and behavior and an estimated 20 million women developing associated vasomotor symptoms or hot flushes. Notably, suicide rates are the highest among women 45 to 59 years of age and have increased by approximately 42% in recent decades.

Although primarily viewed as a reproductive transition, the symptoms of perimenopause are largely neuropsychiatric in nature. Neurological symptoms that emerge during perimenopause are indicative of disruption in multiple estrogen and progesterone-regulated systems such as thermoregulation, sleep, circadian rhythms and sensory processing and affect multiple domains of cognitive function. Perimenopausal depression also appears to impact the clinical symptomatology of menopause, with the presence of depression being associated with a greater degree of menopausal hot flushes than in women without perimenopausal depression.

There is substantial evidence that fluctuations in estrogen and progesterone, the precursor of the endogenous neuroactive steroid allopregnanolone, a $GABA_A$ receptor PAM, are in part responsible for

the mood changes, hot flushes and other neurologic symptoms of perimenopause. Similar to MDD, SSRIs and SNRIs have shown limited efficacy in treating perimenopausal depression. There remains an unmet medical need for effective treatment of core depression symptoms and associated physical symptoms of menopause.

A $GABA_A$ receptor PAM, like PRAX-114, that potentiates the activity of endogenous neuroactive steroids on $GABA_A$ receptors, may offer broader therapeutic benefit compared to current standard of care antidepressants.

$GABA_A$ in depression

Gamma-aminobutyric acid, or GABA, is the principal neurotransmitter mediating neuronal inhibition in the brain. Neurons that produce GABA, known as GABAergic neurons, are present throughout the brain, representing between 20 percent and 40 percent of all neurons depending on the region. Their primary role is to balance and fine tune excitatory neurotransmission of various neuronal circuits. Whole-exome sequencing has identified $GABA_A$ receptor mutation as an important cause in a range of neurological conditions, underscoring their importance as central regulators of excitatory and inhibitory balance in the brain.

It is well established that GABAergic signaling is impaired in patients with MDD and other stress-related mood disorders. GABA levels, levels of the GABA synthesizing enzyme GAD67, as well as $GABA_A$ receptor levels, have been shown to be reduced in brains of patients with MDD. In addition, decreased GABAergic neuron function, most notably in the prefrontal cortex, has been documented in MDD patients and in preclinical animal models of depression. Endogenous neuroactive steroids, such as allopregnanolone and pregnanolone or synthetic derivatives thereof, such as PRAX-114, are known to potentiate the activity of $GABA_A$ receptors. Both human and animal data reveal an important role for neuroactive steroids in these GABAergic deficits and levels of endogenous neuroactive steroids are decreased in individuals with MDD and PMD.

Of particular relevance to the PRAX-114 program is the more recently established link between GABAergic signaling, neuroactive steroid levels and stress—a well-established risk factor for MDD and other mood disorders. In preclinical models, exposure to chronic stress leads to reduced neuroactive steroid biosynthesis and reduced GABAergic inhibition in depression-relevant brain circuits. This ultimately results in increased anxiety and depression-like behaviors. In particular, it has been shown that stress causes long-lasting loss of GABAergic inhibition in the amygdala, a brain region central to the stress response involved in controlling emotions. This reduced inhibition causes increased activity of the amygdala and is associated with an exaggerated stress hormone response.

We believe that enhancing modulation of $GABA_A$ receptors in patients with depression and anxiety has the potential to restore normal function in these circuits, leading to broad applications in mood and anxiety disorders.

$GABA_A$ receptors: The target of PRAX-114

PRAX-114 is a small molecule neuroactive steroid that acts as a positive allosteric modulator of $GABA_A$ receptors. Positive allosteric modulators, or PAMs, are substances that bind to a receptor, such as $GABA_A$, to enhance that receptor's response to its endogenous ligand (or endogenous agonist). $GABA_A$ PAMs bind to a distinct site from endogenous GABA, an allosteric binding site, and do not activate the receptor in the absence of the ligand. Allosteric modulators are believed to have improved safety profiles and are less likely to result in tachyphylaxis, or decreasing drug response, as compared to agonists. GABA exerts its effects through binding to two types of $GABA_A$ receptors, synaptic and extrasynaptic receptors, which differ in their protein subunit composition, physical location on the cell surface and functional role in modulating neuronal circuits.

GABA$_A$ receptors are composed of five subunits which include two alpha, two beta and a fifth subunit (either gamma or delta) that is dependent on the type of receptor. Synaptic GABA$_A$ receptors, which are located in the synapse of neurons, contain a gamma subunit while GABA$_A$ receptors located outside of the synapse, referred to as extrasynaptic GABA$_A$ receptors, contain a delta subunit. Molecules that act as PAMs of only the synaptic GABA$_A$ receptor, such as benzodiazepines, bind to sites situated at the interface between the alpha and gamma subunits. Molecules that act as PAMs of both synaptic and extrasynaptic GABA$_A$ receptors, such as the neuroactive steroids allopregnanolone and PRAX-114, bind to sites situated at the interface between the alpha and beta subunits present in both types of receptors. The figure below displays the synaptic binding site for drugs such as benzodiazepines, and the distinct extrasynaptic and synaptic binding sites for neuroactive steroids, such as allopregnanolone and PRAX-114.

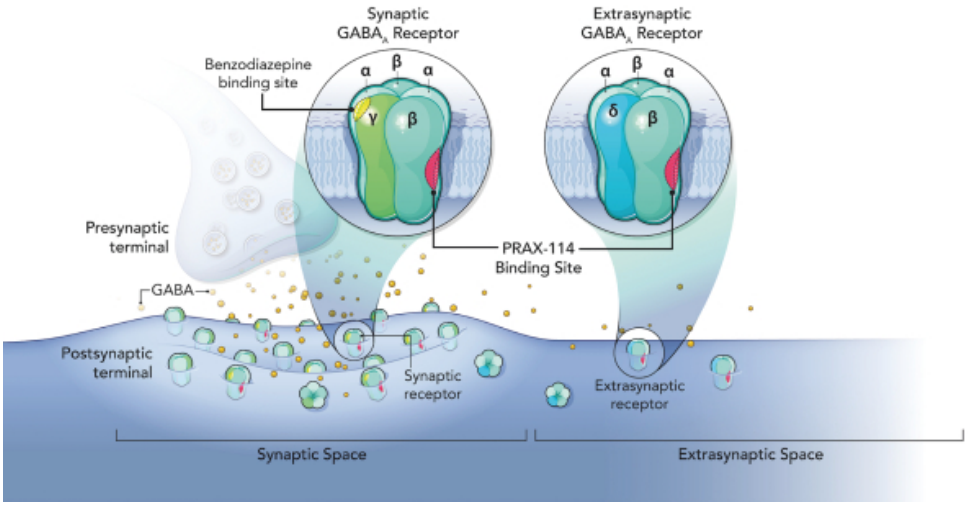


Figure 2. GABA$_A$ synaptic and extrasynaptic receptors differ in structure and function.

Synaptic and extrasynaptic GABA$_A$ receptors have distinct functions. Synaptic GABA$_A$ receptors are responsible for short-lasting, or phasic, inhibition of neurons in response to GABA release at the synapse. By contrast, extrasynaptic GABA$_A$ receptors drive continuous, or tonic, low-level inhibition of neurons in response to lower ambient levels of GABA outside of the synapse. While synaptic receptors can respond quickly to stimulation and network demand, extrasynaptic receptors have a broader modulatory role, serving to continuously modulate the overall excitability of neurons.

Molecules that act as PAMs of only the synaptic GABA$_A$ receptor, such as benzodiazepines and barbiturates, are used for sedation, sleep induction and anxiolysis, and have anticonvulsant and muscle relaxant properties. These drugs have potent and rapid onset of activity but have not demonstrated antidepressant effects.

Allopregnanolone is an endogenous neuroactive steroid and a PAM of both the extrasynaptic and synaptic GABA$_A$ receptors, which has been associated with antidepressant activity. However, allopregnanolone also has shown significant dose-limiting sedative activity, which we believe is likely mediated at least partially by its effects on synaptic GABA$_A$ receptors. Despite this limitation, a

formulation of allopregnanolone has been approved and is marketed as Zulresso to treat post-partum depression.

The distinct effects mediated by these classes of $GABA_A$ PAMs suggest that modulation of extrasynaptic $GABA_A$ receptors is responsible for the antidepressant effects demonstrated by allopregnanolone. One of the goals for a next generation neuroactive steroids, such as PRAX-114, is to preferentially modulate extrasynaptic $GABA_A$ receptors while minimizing the sedative impact from modulation of synaptic $GABA_A$ receptors.

PRAX-114 preference for extrasynaptic $GABA_A$ receptors

To assess the relative potency *in-vitro* of PRAX-114-mediated $GABA_A$ receptor activation for synaptic and extrasynaptic receptors, we measured the peak current induced by a low concentration of GABA (2 μM) in the presence of increasing concentrations of PRAX-114 in Chinese Hamster Ovary, or CHO, cells expressing either extrasynaptic (a4ß3d) or synaptic (a1ß2g2) human $GABA_A$ receptors. In this model, PRAX-114 potentiates the GABA-activated current of both extrasynaptic and synaptic $GABA_A$ receptors, but was approximately 6.4-fold more potent in potentiating the extrasynaptic form of the receptor than the synaptic form based on the concentration that gives half-maximal response, or EC_{50}. At a concentration that activates extrasynaptic $GABA_A$ receptors to the equivalent of full activation by the endogenous ligand GABA (~260 nM, 300% potentiation of 2μM GABA), PRAX-114 led to 10.5-fold greater potentiation of extrasynaptic $GABA_A$ receptors than synaptic $GABA_A$ receptors (29%) (Figure 3, Table 4).

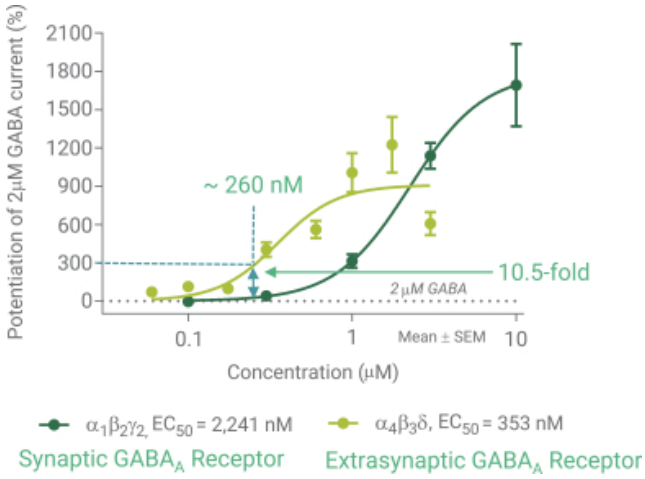


Figure 3. At 300 % extrasynaptic $GABA_A$ receptor potentiation (equivalent to ~ 100% activation by endogenous agonist GABA), PRAX-114 led to 10.5-fold greater potentiation of extrasynaptic $GABA_A$ receptors than synaptic $GABA_A$ receptors (29%).

In the same assay, at the same level of extrasynaptic $GABA_A$ receptor potentiation (300%), other $GABA_A$ receptor PAM neuroactive steroids in development, or on the market, demonstrated only 0.4 to 2.6 fold greater potentiation of extrasynaptic $GABA_A$ receptors, which compares unfavorably to the 10.5 fold observed for PRAX-114 (Table 4). Based on these assay conditions, we believe that the

differentiated preference at extrasynaptic $GABA_A$ receptors by PRAX-114 will allow it to achieve high levels of extrasynaptic GABAergic activation with improved tolerability.

	Dosing	a_4b_3d % potentiation (Equivalent of full activation by GABA)	$a_1b_2g_2$ % potentiation	Fold potentiation $a_4b_3d/a_1b_2g_2$
PRAX-114	**Oral**	**300%**	**29%**	**10.5**
Zuranolone	Oral	300%	117%	2.6
Ganaxolone	IV, Oral	300%	794%	0.4
Zulresso (brexanolone)	IV	300%	306%	1.0

Table 4. Comparison of the degree of *in-vitro* $GABA_A$ receptor potentiation achieved by PRAX-114 and other neuroactive steroid $GABA_A$ PAMs. $a_4ß_3d$: extrasynaptic $GABA_A$ receptors, $a_1ß_2g_2$: synaptic $GABA_A$ receptors.

PRAX-114 clinical development in depression

We have initiated clinical development for PRAX-114 in mood disorders. To date, two Phase 1 clinical trials of PRAX-114 have been completed in healthy volunteers. These studies in 82 healthy volunteers showed PRAX-114 to be generally well-tolerated, with dose-dependent pharmacodynamic activities. In our ongoing Phase 2a clinical trial in Australia, we have observed marked improvement in depression scores in MDD patients within two weeks of treatment, and a pharmacokinetics bridging study in healthy volunteers that is also ongoing. We are planning to initiate a Phase 2/3 trial in the United States and Australia in the fourth quarter of 2020, which is intended to satisfy one of two registrational trials required by the FDA to support clinical efficacy for the treatment of MDD, and we expect topline data in the second half of 2021.

Phase 1 SAD and MAD trials in healthy volunteers

We conducted a Phase 1 randomized, double-blind, placebo-controlled single ascending dose, or SAD, trial of PRAX-114 in healthy volunteers to evaluate safety and tolerability of PRAX-114. This trial enrolled 36 volunteers who were randomized into cohorts dosed with 1mg, 3mg, 10mg, 30mg or 60mg of PRAX-114 or placebo. PRAX-114 was generally well-tolerated and no serious adverse events, or SAEs, were reported in this trial.

We subsequently conducted a Phase 1 randomized, double-blind, placebo-controlled multiple ascending dose, or MAD, trial in healthy volunteers in Australia to evaluate the safety, tolerability and pharmacokinetics of PRAX-114 and to assess the effect of food on drug exposure. Thirty-six volunteers were randomized to receive daily doses of 15mg, 30mg or 60mg of PRAX-114 or placebo for 14 days. Ten additional volunteers in a food effect cohort received 30mg doses of PRAX-114 when they were in a fasted state or with a high-fat meal.

As part of our MAD trial, we measured the effect of PRAX-114 on the quantitative EEG, or qEEG, beta power, to understand the pharmacodynamic effect of PRAX-114 on $GABA_A$ receptor activation. An EEG is a real-time non-invasive measure of electrical activity of neurons in the brain. The frequency and amplitude of the detected electrical signals provide insights into brain function and brain state (e.g., awake, deep sleep, etc). qEEG, also called pharmaco-EEG, is a quantitative measure of the changes in brain activity in specific EEG frequency bands in response to treatment with a brain-active compound. Changes in beta power, specifically, are used as a pharmacodynamic biomarker of $GABA_A$ receptor activation in response to a brain active compound.

In both the Phase 1 SAD and MAD trials, we observed fast absorption of PRAX-114 within one to three hours of dosing and approximately dose-proportional increases in peak concentration and total

drug exposure. In the MAD trial, the half-life of the drug was between 12.2 and 14.8 hours, consistent with a once-daily dosing paradigm. Little or no accumulation of the drug was observed in the multiple dose trial over the ranges of doses tested.

We believe that the ability to administer PRAX-114 with or without food is key for clinical and commercial success in MDD, as many patients struggle with adherence to medication and forcing a dietary regimen would impose further complications in this vulnerable population. In the food effect cohort of the MAD trial, overall drug exposure as measured by area under the concentration curve, or AUC, of PRAX-114 increased by only 1.17-fold in the fed state versus in the fasted state. The primary effect of food was observed in the maximum drug exposure, or Cmax, which was 0.64-fold of that observed under fed conditions. These findings indicate that PRAX-114 does not need to be taken with food to achieve therapeutic exposures. According to results presented in a published patent application, Zuranolone, a $GABA_A$ PAM neuroactive steroid in development for treatment of MDD, exhibited a food effect that resulted in increases in Cmax of approximately 2.88-fold and in AUC of approximately 1.58-fold in the fed versus the fasted conditions (Table 5). We believe this food effect has led to the development of Zuranolone being carried out with a requirement for the compound to be administered together with a high-fat meal. We believe that the absence of a requirement that PRAX-114 be taken with food creates a potential competitive advantage.

Statistical Analysis of the Effect of High-fat Meal for PRAX-114 suspension (30mg)			Statistical Analysis of the Effect of High-fat Meal for Zuranolone capsules (30mg)		
PK Parameter	**Fed/Fasted Ratio**	**90% Confidence Interval**	**PK Parameter**	**Fed/Fasted Ratio**	**90% Confidence Interval**
Cmax (ng/mL)	0.64	**(0.46, 0.88)**	**Cmax (ng/mL)**	2.879	**(2.56, 3.28)**
AUC0-t (h*ng/mL)	1.17	**(0.91, 1.49)**	**AUC0-t (h*ng/mL)**	1.575	**(1.45, 1.69)**

WO2019/051264 A1 Patent

Table 5. Food effect clinical studies of PRAX-114 and Zuranolone (WO2019/051264 A1). Subjects were administered a high-fat meal 30 minutes prior to administration of the compound in both studies.

In the MAD trial, PRAX-114 was generally well-tolerated, with no SAEs reported. The reported treatment-emergent adverse events, or TEAEs, were mild to moderate and were consistent with those expected for the mechanism of action. The most common adverse event was somnolence, which is characterized by sleepiness or drowsiness, and was reported by 78% (7/9) of those subjects receiving the 60mg dose; all events of somnolence were mild in severity. Increases in sleepiness as measured by the Stanford Sleepiness Scale occurred between one- and three-hours post-dosing, consistent with the period of peak drug concentrations; sleepiness ratings at the 60mg dose were similar to placebo within 4 hours post-dose. We did not observe a maximally tolerated dose, or MTD.

In our Phase 1 multiple ascending dose trial of PRAX-114 in healthy volunteers in Australia, we also observed the following TEAEs, all of which were mild to moderate in severity:

- 60mg dose (n=9), we observed nervous system disorder TEAEs, of somnolence (77.8% of subjects), headache (33.3% of subjects), dizziness (55.6% of subjects) and hypoaesthesia, or diminished sense of touch (22.2% of subjects). Other TEAEs observed in more than one subject were euphoric mood (22.2% of subjects), hyperhidrosis, or excessive sweating (22.2% of subjects) and muscle twitching (22.2% of subjects).

- 30mg dose (n=9), we observed nervous system disorder TEAEs of somnolence (44.4% of subjects) and headache (22.2% of subjects). Other TEAEs observed in more than one subject were skin irritation (55.6% of subjects) and euphoric mood (22.2% of subjects).
- 15mg dose (n=9), the only TEAE observed in more than one subject was fatigue (22.2% of subjects).
- Placebo group (n=9), we observed fatigue (22.2% of subjects).

TEAEs appearing to be dose related were somnolence, dizziness, headache, euphoric mood and hypoaesthesia.

We measured changes in qEEG beta frequency power in these volunteers to assess the effect of PRAX-114 on $GABA_A$ receptors in the brain on days 1 and 14 of this trial. PRAX-114 produced increases in the power of the beta-frequency band which were strongly correlated with dose and PRAX-114 levels in the blood. In these healthy volunteers at one hour post-dose, PRAX-114 30mg resulted in an average increase in qEEG beta power of approximately 1.6-fold compared to baseline and 60mg resulted in an increase in this measure of 2.8-fold compared to baseline (Figure 6). The effects on the qEEG beta power were sustained at Day 14. These data show that PRAX-114 engaged $GABA_A$ receptors in the brain and produced consistent effects on qEEG within the first hour after dosing. This finding also supports comparison and translation of the pharmacologic activity and qEEG data from the pre-clinical studies, where a 1.6-fold increase in beta power was associated with robust activity in animal models of anxiety and depression and was used to inform dose selection in subsequent clinical trials.

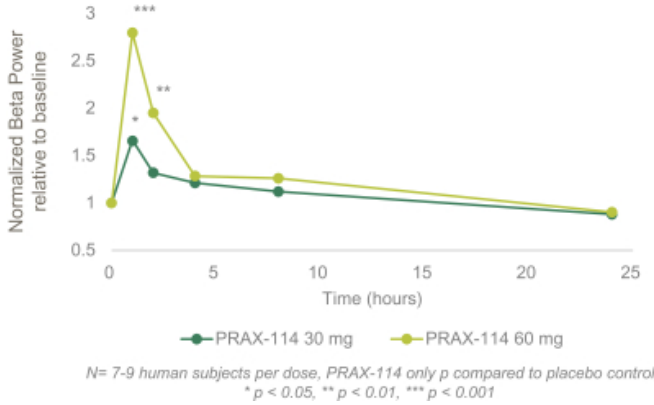


Figure 6. PRAX-114 (30mg and 60mg) showed a robust qEEG signal and target activation.

Notably, PRAX-114 showed increases in beta power up to 2.8-fold without achieving a MTD or demonstrating any SAEs. In a separate study, another molecule in development in the class resulted in degrees of sedation that were not tolerated at doses that resulted in increases in beta power by approximately 1.7-fold compared to baseline. This highlights the unique pharmacological profile of PRAX-114 and its ability to achieve high levels of GABAergic activation with improved tolerability.

We believe this improved tolerability profile of PRAX-114 offers the potential for a wider therapeutic window, increased adherence and a wider dose range for MDD patients.

Phase 2a trial in patients with depression

Based on the observed pharmacology in the Phase 1 trials, we are currently conducting a three-part, open-label, Phase 2a trial in Australia to assess the safety and efficacy of PRAX-114 in patients with moderate to severe MDD or PMD. We have completed Part A of this Phase 2a trial and Parts B and C are ongoing.

Part A results

Part A of the open-label trial included two weeks of treatment and was designed to evaluate the timing and magnitude of the antidepressant effects of PRAX-114 across a range of doses in patients with MDD. Patients were required to be between the ages of 18 and 65 and to have moderate to severe MDD for at least one month as defined by the Hamilton Depression Rating Scale, or HAM-D, score of 22 or higher. The HAM-D, one of the most widely-used clinical rating scales for depression, was the main assessment used to quantify levels of depression in these patients. The 17 items used for scoring this scale cover a wide range of symptoms typically found with depression including mood, suicidal thoughts, insomnia, anxiety, loss of appetite and weight loss. Patients with more severe depression have higher scores. The effect of PRAX-114 was measured by the change in the HAM-D score relative to baseline. Patients who had previously failed to respond to a standard of care antidepressant in their current episode were eligible for inclusion. In addition to HAM-D, other scales used included the Montgomery–Åsberg Depression Rating Scale, or MADRS, the Hamilton Anxiety Rating Scale, or HAM-A, and the Symptoms of Depression Questionnaire, or SDQ. MADRS is a 10 item rating scale designed to assess the severity of symptoms in a depressive illness. HAM-A is a 14 item scale widely used to measure the severity of anxiety symptoms, including both psychic anxiety (mental agitation and psychological distress) and somatic anxiety (physical complaints related to anxiety). SDQ is a 44 item self-reported scale designed to measure the severity of symptoms across several subtypes of depression, including irritability, anger attacks and anxiety.

Based on the Phase 1 data, we selected 3 doses: 45mg, 60mg or 80mg daily of PRAX-114. All three doses were expected to achieve exposures that would show clinical benefit based on the Phase 1 data and qEEG findings. The first week of treatment was conducted in an inpatient setting to facilitate daily efficacy and safety assessments and then patients were discharged and treated as outpatients for the second week. Patients were instructed to take PRAX-114 nightly before bed. Patients were not required to take PRAX-114 with food. Compliance was carefully monitored throughout the duration of the trial.

Thirty-three patients were enrolled and completed Part A before the COVID-19 pandemic. At baseline, patients had a mean HAM-D total score of 25, ranging from 20 to 33, indicating moderate to severe MDD. Twenty-six of the thirty-three participants had previously received an antidepressant during the current depressive episode but still had moderate to severe MDD. This failure to respond to initial antidepressant treatment has been associated with more severe and refractory depression. The remaining patients were not being treated with any antidepressant for the current episode before enrolling in the trial.

Dosing with PRAX-114 led to a marked improvement in the HAM-D score (Figure 7) within two weeks of treatment. After one week of treatment, least squares, or LS, mean improvements of 15 to 19 points from baseline were noted across the three dose groups. After two weeks of treatment, all 3 dose levels showed improvements from baseline of greater than 13 points with mean improvements from baseline of 14 to 16 points. Across all dose levels, two-thirds of patients were responders (defined as a >=50% reduction in HAM-D) or were clinically in remission (HAM-D<=7) at the end of the 14 day treatment period. Changes in other depression-related scales measured such as MADRS, HAM-A and SDQ were consistent with the changes in HAM-D. While the study was not powered to show differences between dose levels, there was no notable dose response observed, which is common amongst trials of antidepressants.

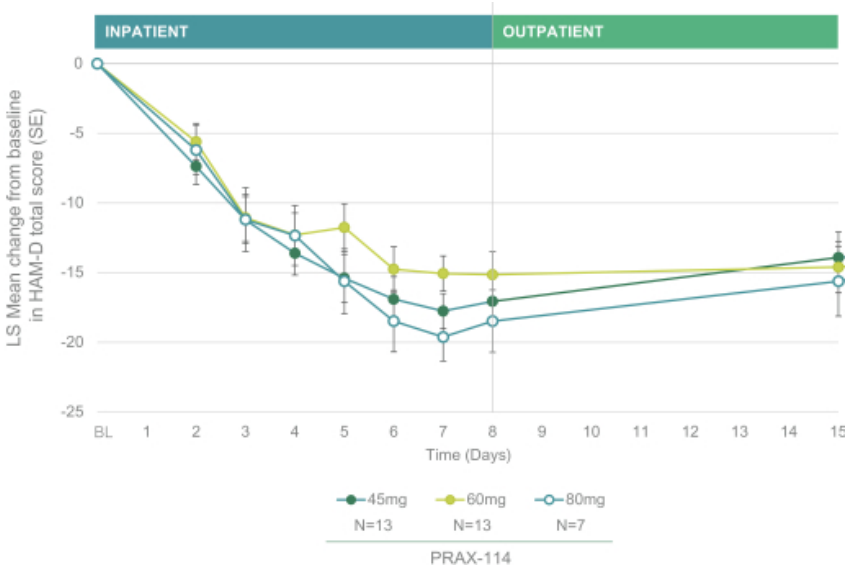


Figure 7. Reduction in HAM-D total score observed in MDD patients treated with PRAX-114.

After 14 days of treatment, patients were monitored for an additional 14 days. During this monitoring period, the core mood symptoms measured by the HAM-D generally remained stable with a slight increase in the insomnia item scores post-treatment.

While our Phase 2a trial is not placebo controlled, prior randomized placebo-controlled MDD trials provide important context for the interpretation of the clinical response. The marked improvements in HAM-D scores seen in MDD patients in Part A within two weeks of treatment compares favorably to published reports on changes in HAM-D scale in clinical trials of approved antidepressants such as vortioxetine and duloxetine, among others, which commonly take approximately six to eight weeks to reach a maximal efficacy and often fail to differentiate from placebo at two weeks. Moreover, mean HAM-D changes from baseline at Day 14 for the placebo group of these randomized controlled antidepressant trials are most often between 4-8 points. Even at the first post-dose assessment on Day 3, patients dosed with PRAX-114 had a mean decrease of over 11 points on the HAM-D scale, which compares favorably with the average changes reported in the placebo groups at Day 14 from randomized studies completed for recently approved antidepressants (Figure 8), and other common antidepressants after several weeks of dosing. The clinical data that we have generated to date, and that we expect to generate in the future, from our clinical studies will constitute the bulk of the data needed to support an application for marketing approval of PRAX-114. Unless we conduct head-to-head studies of PRAX-114 against other molecules as part of our future clinical trials and elect to include the resulting data in an application for regulatory approval, we would not expect to rely upon PRAX-114's potential differentiation from any other molecules in connection with submissions to the FDA or other regulatory agencies, as applicable, for approval or otherwise. As the data presented above is based on a cross-trial comparison and not a head-to-head clinical trial, such data may not be directly comparable due to differences in study protocols, conditions and patient populations. Accordingly, cross-trial comparisons may not be reliable predictors of the relative efficacy or other
benefits of PRAX-114 compared to other product candidates that may be approved or that are or were in development for MDD.

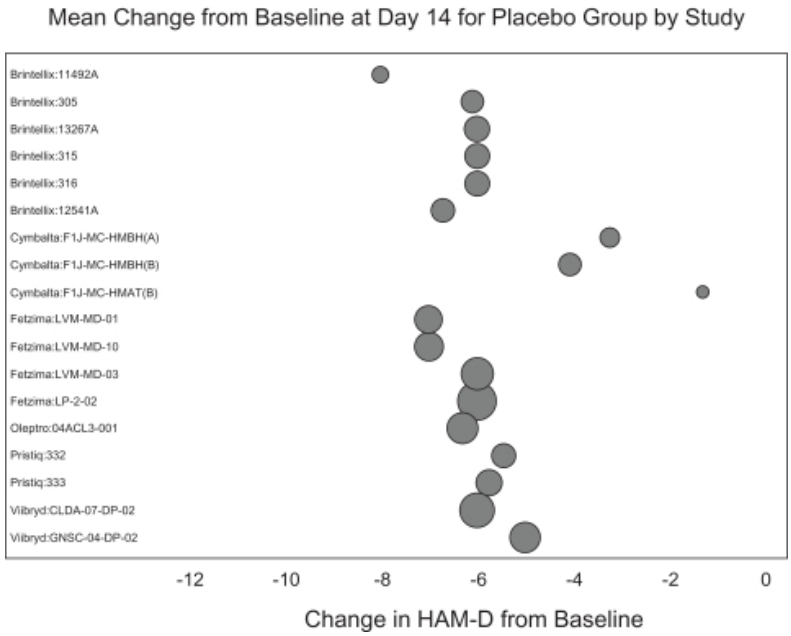


Figure 8. Change from baseline to Day 14 in HAM-D total score for the Placebo arm of selected randomized placebo-controlled studies of recently approved antidepressants. Bubble size is proportional to the sample size of the placebo group in each study. Across studies, the sample size in the placebo group ranged from 89 to 277.

PRAX-114 was generally well-tolerated across the dose range, including at the highest 80mg dose. This is consistent with an expected wider therapeutic window based on the preferential selectivity of PRAX-114 for extrasynaptic $GABA_A$ receptors. TEAEs were generally mild to moderate. Rates of somnolence, which is characterized by sleepiness or drowsiness, increased with dose, demonstrating a pharmacological effect which was somewhat mitigated by dosing at night versus the morning. With nighttime dosing, 12/33 patients (36%) noted somnolence post-dosing, which was generally time-limited and not experienced during the day time, substantially lower than the 78% reported in the Phase 1 60mg group with morning dosing. There were no SAEs or discontinuations and study drug cessation at the end of the treatment period was generally well-tolerated.

In this MDD part of the trial, we also observed the following TEAEs in at least 2 subjects per dose level:

- 80mg dose (n=7), headache (42.9% of subjects), somnolence (42.9% of subjects), dizziness (57.1% of subjects), feeling drunk (28.6% of subjects) and diarrhea (28.6% of subjects).
- 60mg dose (n=13), headache (46.2% of subjects), somnolence (53.8% of subjects), dizziness (30.8% of subjects), feeling drunk (23.1% of subjects) and constipation (23.1% of subjects).
- 45mg dose (n=13), headache (53.8% of subjects), somnolence (15.4% of subjects), fatigue (23.1% of subjects), vessel puncture site bruise from blood draws (15.4% of subjects), abdominal distension (15.4% of subjects) and upper respiratory tract infection (15.4% of subjects).

Across all dose levels studied, headache (48.5%), somnolence (36.4%), dizziness (24.2%), fatigue (15.2%), feeling drunk (15.2%), constipation (12.1%) and vessel site puncture bruise (12.1%) were reported in >10% of subjects.

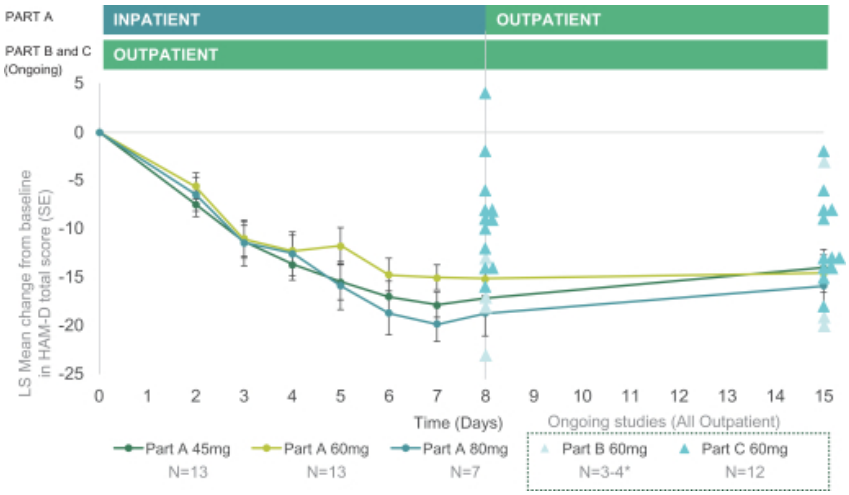
Parts B and C ongoing

We are currently conducting Part B of this trial in order to assess PRAX-114 in PMD patients. We are dosing up to twelve patients with PMD with 60mg of PRAX-114 nightly for 14 days on an outpatient basis. The dose for this part of the trial was selected based on the data from Part A. Inclusion criteria for Part B are similar to Part A and C, except that it requires participants to be females of 40 years of age or older with irregular menses and hot flushes. Part B will help to determine if PRAX-114 has an effect on broader menopausal symptoms, like hot flushes, in addition to confirming the antidepressant effect. We anticipate topline results by the second half of 2021.

Part C of this trial is also ongoing. The goal of Part C is to evaluate the impact of four-week dosing on the treatment effect observed in the two-week Part A in a full outpatient setting, a dosing paradigm that more closely parallels the standard of care approach to antidepressant treatment of MDD. We expect to enroll up to thirteen patients with MDD and dose PRAX-114 at 60mg nightly for four weeks. Inclusion criteria and efficacy endpoint measurements are the same as Part A. We expect to have complete topline data in the fourth quarter of 2020 that will inform our planned randomized, placebo-controlled trial, which we anticipate will also use four weeks of dosing.

Data from the ongoing Part B and Part C study indicate that 60mg of PRAX-114 dosed nightly shows a safety profile similar to Part A. The preliminary data show that dosing with PRAX-114 led to a marked improvement in the HAM-D score within two weeks of treatment. We expect complete topline data from Part B of the trial in the second half of 2021 and from longer-term dosing in MDD patients in Part C of the trial in the fourth quarter of 2020.

Moreover, greater than 70% of participants from the Part A, Part B and Part C study showed they had previously received an antidepressant during the current depressive episode but still had moderate to severe MDD, and preliminary data show greater than 60% of patients were responders or clinically in remission at two weeks. All Parts demonstrated a well-tolerated profile throughout treatment schedule.



* As of September 23, 2020, Part B data available for N=4 at Day-8 and N=3 at Day-15, trial ongoing

Figure 9. Data from ongoing trial showing reduction of HAM-D total score from baseline in patients with PMD (Part B) and MDD (Part C).

Pharmacokinetics bridging study

To date, we have conducted all studies with PRAX-114 using a liquid, suspension formulation. For our registrational studies and subsequent potential commercial use of PRAX-114, we have developed a tablet formulation.

In a clinical PK bridging study, PRAX-114 was administered as a solid dose (i.e., tablet) formulation in single ascending doses of 40mg, 60mg and 80mg and compared to PRAX-114 suspension administered at the 60mg dose. The PK of the tablet formulation was found to be comparable to the suspension formulation at the 60mg dose level (Figure 10). Administration of the 60mg tablet formulation under fasted conditions resulted in a median tmax of ~1.0 hour, Cmax of ~400ng/mL, AUCinf of ~2600 hr*ng/mL and t1/2 of ~11-12 hours, similar to the 60mg oral suspension. Exposure to PRAX-114 increased approximately proportional to dose.

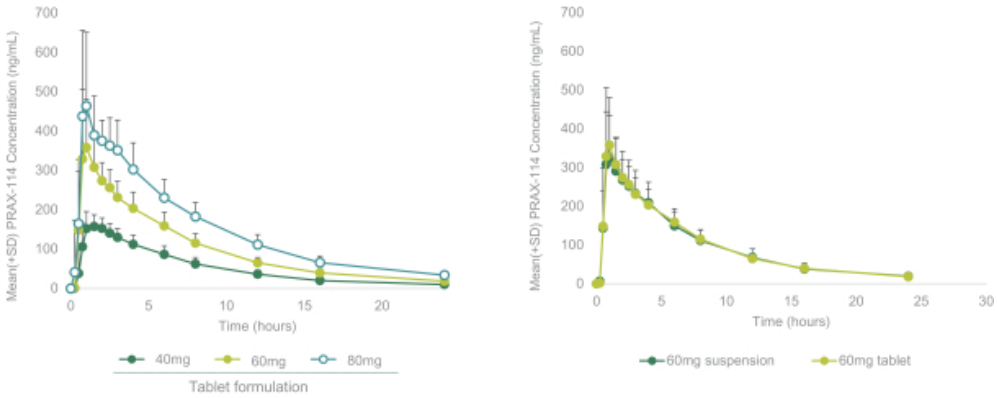


Figure 10. PRAX-114 dose-ranging pharmacokinetic study of tablet formulation and PRAX-114 pharmacokinetic bridging study of suspension and tablet formulations. Arithmetic means are displayed with standard deviations at each collection time point.

We are currently evaluating the effect of food on the solid formulation, and we expect to observe a similar effect of food to that seen with our suspension formulation. Like our suspension formulation, we expect that our solid formulation will not need to be taken with food to achieve targeted exposures.

Planned PRAX-114 clinical trials

We plan to submit an Investigational New Drug application, or IND, to the FDA and then initiate a randomized, double-blinded, placebo-controlled Phase 2/3 trial of PRAX-114 in the United States and other countries in approximately 200 moderate to severe MDD patients. Patients will be randomized 1:1 to receive nightly doses of PRAX-114 or placebo for 28 days in a fully outpatient setting. The primary efficacy endpoint will be change in the HAM-D score from baseline at Day 15. Patients will be required to be between the ages of 18 and 65 and have moderate to severe MDD for at least one month as defined by the HAM-D score of 23 or higher, and have at least one prior episode of MDD. We believe clinical conduct excellence is essential for a study in MDD that appropriately mitigates historical variability in placebo effect, and the current social and environmental changes caused by the COVID-19 pandemic. Our operational plan, amongst other aspects, includes utilizing sites with a track-record of high quality in the conduct of MDD trials, and a two-level subject quality process that includes independent clinical interviews for eligibility. A key secondary endpoint will be change in the HAM-D score after 28 days of treatment to assess the durability of effect of PRAX-114. In addition, we will also

evaluate changes in other depression-related scales. We intend to initiate this trial in the fourth quarter of 2020 with topline data anticipated in the second half of 2021. We believe this trial could satisfy one of two registrational trials required by the FDA to support clinical efficacy for the treatment of MDD.

Upon completion of Part B of our Phase 2a trial, we intend to have a meeting with regulators to discuss further development of PRAX-114 in PMD.

PRAX-114 preclinical data

The goal of our preclinical program was to establish the *in-vitro and in-vivo* pharmacological profiles, antidepressant potential and tolerability of PRAX-114. In addition, we evaluated translational pharmacodynamic biomarkers to inform clinical development.

Antidepressant activity

To determine the antidepressant-like activity of PRAX-114, we used the Wistar Kyoto, or WKY, rat model. The WKY rat is an inbred rat strain that has increased sensitivity to stress and displays a depressive-like phenotype that is resistant to SSRI and SNRI treatment. A common way to assess depressive-like symptoms in animals is a test known as the forced swim test, or FST. The FST is based on the natural behavior of an animal when placed in a container filled with water from which it cannot escape. The rat will first make efforts to escape by swimming or climbing, but eventually will exhibit floating behavior, which is an indication of behavioral despair and is seen as a surrogate for depression. WKY rats display longer time inactive (floating) over a given time period than normal rats as an indication of increased behavioral despair.

We administered oral doses of PRAX-114 or a placebo to WKY rats and evaluated performance on the FST. At all doses of PRAX-114 tested, 1mg/kg, 3mg/kg and 10mg/kg, we observed a significant reduction in immobility time compared to rats that received a placebo, which we believe reflects an anti-depressive-like reaction or activity of PRAX-114. Importantly, and as described below, at these doses, PRAX-114 did not impair or enhance overall spontaneous activity of the rats in independent assays in the same animals, which we believe indicates that PRAX-114 was generally well-tolerated at these doses.

Tolerability

A common model to assess sedation in rats is the measure of spontaneous locomotor activity, or sLMA. Dosing rats with sedatives dose-dependently reduces their spontaneous movement in this assay. In this model, doses of PRAX-114 up to 30mg/kg had no significant impact on spontaneous locomotion, while doses as low as 1mg/kg had significant antidepressant-like effects in the WKY rat model, demonstrating a wide therapeutic window in these models with preclinical activity at doses well below sedative doses.

We believe that the therapeutic window observed in our *in-vivo* assays is consistent with the preference of PRAX-114 for extrasynaptic $GABA_A$ potentiation observed *in-vitro*.

EEG as a pharmacodynamic biomarker

In our rat translational biomarker model, we administered PRAX-114 to wild-type rats at doses ranging from 1 to 20mg/kg to assess the impact on power in the beta frequency band. We found that PRAX-114 dose-dependently increased the power in the beta frequency band and these changes correlated with changes in plasma pharmacokinetics. This EEG biomarker was used to inform dose-selection for PRAX-114 clinical studies. In our Phase 1 MAD trial, healthy volunteers administered the 30mg dose of PRAX-114 displayed an approximately 1.6-fold increase in qEEG beta power compared to baseline. In our preclinical studies, doses (and plasma/brain concentrations) that induced a 1.6-fold increase in the beta frequency power in rats were associated with both robust preclinical activity in

animal models of depression and anxiety and good tolerability. Specifically, the PRAX-114 dose that is estimated to induce a 1.6-fold increase in EEG beta power activity in rats was efficacious in the rat WKY model of depression and the window between that dose that increased beta power by 1.6-fold increase in EEG and the dose that caused a 50% reduction of spontaneous locomotion in the sLMA sedation assay, or ED_{50}, was ~11-fold, based on brain concentrations. In addition, at this well-tolerated brain concentration, PRAX-114 was efficacious in animal models of anxiety including conditional emotional response, or CER, punished drinking, or Vogel, and elevated plus maze, or EPM (Figure 11).

In the figure below, the lower bound of the preclinical activity in animal models and EEG bars are determined by the brain exposure at the lowest dose at which significant activity was observed ($p < 0.05$). The lower bound of the tolerability bar represents the TC_{50} in the brain. The upper bound represents the mean brain concentration at the highest dose tested in a given assay.

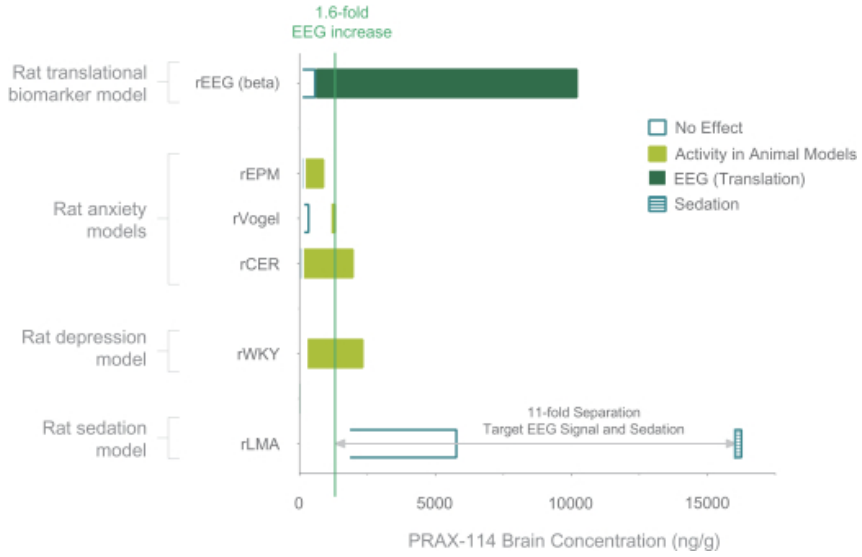


Figure 11. Summary of PRAX-114 preclinical data.

Based on our findings in preclinical models, we believe our initial results in humans are supportive of a wide therapeutic window which, in humans, begins at or below a daily dose of 30mg of PRAX-114 and extends to higher doses prior to the onset of potential dose-limiting somnolence or sedation. Our clinical studies to-date suggest that PRAX-114 doses up to 80mg, the highest we have tested in humans, remain generally well-tolerated. We have yet to identify the MTD.

PRAX-944

We are developing PRAX-944, a potentially differentiated selective small molecule inhibitor of T-type calcium channels, for the treatment of Essential Tremor, or ET. We have evaluated the safety and tolerability of PRAX-944 in over 150 healthy volunteers in five separate clinical trials. In these trials, we have studied the safety of PRAX-944 modified release formulation with titration up to 120mg/day and no MTD has been identified. We are currently conducting a Phase 2a proof-of-concept open-label trial in ET patients. Preliminary site data from five patients of the low dose cohort showed tremor reduction, which seems to compare favorably to the standard of care agents. We plan to announce topline data, including a high dose cohort, in the first half of 2021.

There is a large body of clinical, preclinical and genetic evidence that points to the involvement of T-type calcium channels in the cerebello-thalamo-cortical, or CTC, circuit, as a main driver of ET. ET is the most common movement disorder, affecting up to seven million patients in the United States, which is seven times more individuals compared to Parkinson's tremor. ET is a progressive and debilitating movement disorder with action tremors that significantly disrupt daily living. There is a high unmet need for ET patients given the limited treatment options, with only one approved pharmacotherapy that is poorly tolerated, resulting in high discontinuation rates and the need for invasive brain surgeries.

Successful development of T-type calcium channel modulators in ET likely requires a PK profile with a blunted Cmax and thoughtful clinical trial design and endpoint selection. We have designed our development program to include careful selection of clinical endpoints, a modified release formulation and dose titration strategy. We believe our modified release formulation for PRAX-944 is well positioned to be a differentiated therapy in ET.

Because of the gatekeeper role of T-type calcium channels in regulating neuronal firing patterns in multiple neuronal circuits, we believe PRAX-944 is suitable for potential development across a wide-range of indications in psychiatry and neurology, providing sizable expansion opportunities in addition to ET.

Essential Tremor

ET is the most common movement disorder, characterized by involuntary rhythmic movement in the upper limbs, with or without tremor in other body locations such as the head, vocal cords, or legs. ET is a day-time disease associated with debilitating tremors triggered when a patient voluntarily attempts to move. These tremors significantly disrupt daily living and are progressive in nature, with increases in tremor severity and amplitude commonly observed over the course of the disease. The upper-limb tremor can range from barely visible to greater than 20cm in amplitude.

Unlike Parkinson's disease, which is characterized by a rest tremor, the tremor of ET occurs with movement and therefore causes direct disability, as people are unable to perform basic, every-day functions such as writing, typing, drinking or feeding themselves. Given the debilitating physical challenges of the disease, ET has also been associated with high prevalence of comorbidities, including anxiety, depression and social phobia.

ET is a clinically well-recognized indication with defined diagnostic criteria established by The International Parkinson and Movement Disorders Society. ET affects between one and two percent of the worldwide population and approximately five percent of adults over 60 years of age. It is estimated that there are up to seven million individuals with ET in the United States, up to seven times more than the second most common movement disorder, Parkinson's disease.

Despite the prevalence and significant disease burden of ET, only a fraction of patients (an estimated one million based on claims data) are managed with pharmacological therapy, though an estimated 80% of those that are treated discontinue these medications due to limitations in efficacy and tolerability. We believe that the treated population will increase with the availability of new therapies with improved efficacy and tolerability.

Currently, there are only two drugs commonly used in ET. Propranolol, approved by the FDA in 1967, remains the only currently approved therapy for ET in the United States. A non-selective beta blocker, Propranolol is contraindicated for individuals with certain respiratory or cardiac issues, which are common comorbidities in the age group affected by ET. Primidone, an anticonvulsant used off-label, requires slow titration over six to eight weeks and can cause sedation and balance issue while accelerating osteoporosis with long-term use.

As a last line therapy, several thousand ET patients in the U.S. opt for invasive surgery each year. Interventions include gamma knife and focused ultrasound thalamotomy, where part of the thalamus involved in the CTC circuit is ablated, or deep brain stimulation, or DBS, where an electrode is implanted into the brain. These procedures are generally effective, but are associated with significant side effects and risk. Therefore, many patients who are eligible for surgical therapies do not elect to have these procedures.

A significant unmet need remains for the millions of ET patients that are not currently receiving treatment for their ET, or are underserved by existing treatment options. We believe that the relatively concentrated ET treatment setting composed of mainly neurology and movement disorder specialists would allow for the rapid adoption of a new treatment option that offered robust response rates and an improved tolerability profile.

Genetics of Essential Tremor

Our rationale for approaching ET through inhibition of T-type calcium channels is rooted in the genetics of epilepsy. CACNA1G, a gene that encodes for a particular isoform of T-type calcium channels, is one of the most significantly associated genes for generalized genetic epilepsy, or GGE. Some of these epilepsy patients also suffer from comorbid movement disorders such as tremor and ataxia. The odds of observing a T-type calcium channel mutation in the GGE population is 9 times of that of the healthy population. This supports the key role of T-type calcium channels in maintaining excitation and inhibition balance.

Additional human genetic data provide evidence for the role of T-type calcium channels in movement disorders. Whole exome sequencing of early-onset familial ET patients also identified mutations in CACNA1G that segregated with the tremor phenotype in multiple family pedigrees. Gain-of-function T-type calcium channel mutations have also been reported as causative variants for another phenotypically overlapping movement disorder called Cerebellar Ataxia. We believe this genetic link, along with the preclinical and clinical evidence, help confirm the role of T-type calcium channels in ET.

Role of T-type calcium channels in ET

T-type calcium channels function as the gatekeepers of neuronal firing patterns, controlling the switch between tonic and burst firing in the CTC circuit. The CTC circuit is a series of brain nuclei or neuron clusters, including the inferior olivary nucleus, cerebellar Purkinje cells, deep cerebellar nuclei, ventral motor thalamus and motor cortex, which work together in regulating coordinated movements and when disrupted generate tremor. All nuclei in this circuit contain pacemaker cells with inherent burst firing capability and express T-type calcium channels, which are known drivers of oscillatory burst firing.

T-type calcium channels are low voltage activated channels that respond to weak depolarization of neuronal membranes and are quickly inactivated (a closed state where the channel cannot be reopened for some time). The opening of T-type calcium channels leads to membrane depolarization, which activates voltage-activated sodium channels, leading to the formation of an action potential and neuronal firing. When only a small number of T-type calcium channels are activated, leading to small T-type calcium channel mediated membrane depolarizations, the neuron generally generates unitary action potentials, also called tonic firing. When the activity of T-type calcium channels is increased, either due to genetic mutations or other changes in network activity that recruit more T-type calcium

channels, a longer lasting depolarization is generated, resulting in high-frequency clusters of sodium channel driven action potentials, also called burst firing, as illustrated in the figure below.

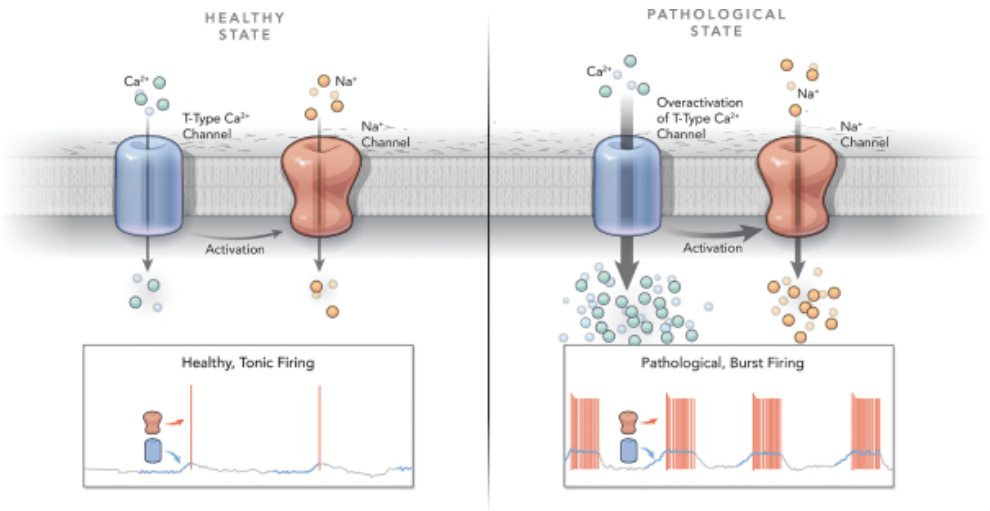


Figure 12. T-Type calcium channels are gatekeepers of neuronal firing patterns.

Neuroimaging of ET patients has consistently demonstrated that individual nuclei along the CTC circuit oscillate at tremor frequency and with strong coherence amongst the brain regions and movement in the affected muscles. Further, intraoperative real-time single-unit recordings of action potentials of individual neurons in the ventral motor thalamus of severe ET patients receiving DBS implants, in periods with and without tremors, further substantiates the central role of the CTC circuit and T-type calcium channels in ET (Figure 13). When no tremor was observed at rest, tonic firing was recorded in neurons of the ventral motor thalamus. During tremor, the same neurons fire in rhythmic bursts that are highly coherent with tremor activity. Furthermore, the emergence of action tremors coincided with the emergence of burst firing. Lesioning or DBS of the ventral motor thalamus has been shown to silence the oscillatory burst firing activity in the CTC circuit, resulting in significant tremor reduction. The strong temporal coordination between the tremors and burst firing, a neuronal firing pattern frequently gated by T-type calcium channel activity, strongly suggest that pharmacological inhibition of these channels may represent an effective pharmacological approach in ET.

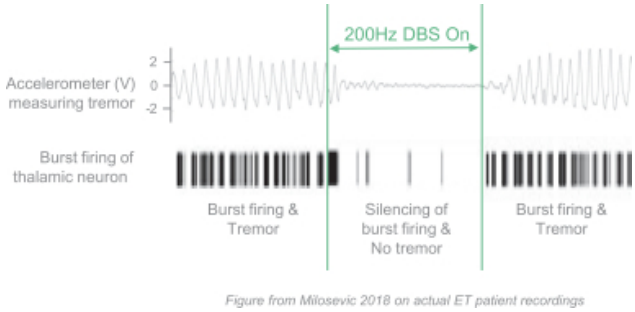


Figure 13. Thalamic neuron burst firing correlated with tremor activity in ET patients.

The role of the CTC circuit and T-type calcium channels has been further confirmed in animal models. A known pharmacological tremor model utilizes administration of harmaline, an alkaloid toxin, to animals. Harmaline, on administration to experimental animals such as rodents, induces an acute action tremor as well as rhythmic burst-firing activity in the CTC circuit similar to that observed in ET patients. We believe this model carries clinically predictive value, as compounds that improve tremor in ET patients clinically (e.g., propranolol, primidone) have also been shown to reduce harmaline-induced tremor preclinically; in contrast compounds that worsen tremor in patients (e.g., caffeine) also worsen tremor in this model. Similar to what's observed in ET patients, normalizing oscillatory activity in the CTC circuit, for example with DBS, reduces harmaline induced tremor in rodents. Pharmacological inhibition or genetic knockout of T-type calcium channels lead to resistance to harmaline-induced tremor.

PRAX-944 in Essential Tremor

We are advancing a modified release formulation of PRAX-944, a potent and selective small molecule inhibitor of T-type calcium channels, for the treatment of ET.

PRAX-944 Clinical Development in ET

We have evaluated the safety and tolerability of PRAX-944 in over 150 healthy volunteers in five separate clinical trials. In these trials, we have studied the safety of PRAX-944 modified release formulation with titration up to 120mg/day and no MTD has been identified. We are currently conducting a Phase 2a proof-of-concept open-label trial in Australia and New Zealand in ET patients. Preliminary site data from five patients of the low dose cohort showed tremor reduction, which seems to compare favorably to the standard of care agents. We plan to announce topline data, including a high dose cohort, in the first half of 2021.

Phase 1 trials in healthy volunteers using previous IR formulation

An immediate release, or IR, formulation of PRAX-944 was used in the initial Phase 1 trials and reached maximal plasma concentrations within one to three hours of dosing. Adverse events like nausea were associated with the high peak levels. This prompted the development of modified release, or MR, formulations, which can have slower absorption and improved tolerability.

Phase 1 trials in healthy volunteers using MR formulation

Our current clinical formulation of PRAX-944 is designed to release 80% of the drug product over seven hours. This slow release is desirable for diseases like ET where minimum, or trough concentrations, need to be maintained during the day when tremor is present for efficacy and for reducing side effects associated with high peak concentrations which could limit the ability to treat patients at therapeutic doses. The sustained exposure also enables once daily dosing (Figure 14).

In our Phase 1 multiple dose trial of the MR formulation of PRAX-944 in England, doses of 20mg and 40mg were generally well-tolerated over 8 days. Adverse events were transient and occurred at a rate similar to placebo. We observed the following TEAEs all of which were mild to moderate:

- 40mg dose (n=6): we observed the nervous system TEAEs of somnolence (33.3%), headache (33.3%) and dizziness (33.3%). The TEAEs also included fatigue (33.3%) and hot flash (33.3%). We observed ECG application site rash, EEG application site skin reaction, nausea, vision blurred, thermal burn (accidental) and euphoric mood in 16.7% of subject each.
- 20mg dose (n=6): we observed the nervous system disorder TEAEs of somnolence (16.7%) and headache (33.3%). We also observed nausea (33.3%), fatigue (16.7%), vomiting (16.7%) and dry throat (16.7%).
- Placebo (n=4): we observed the nervous system TEAEs of headache (25%), somnolence (25%) and dizziness (25%). We also observed fatigue (50%) and nausea (25%).

A single dose of 60mg was not tolerated in the single dose trial due to reports of nausea in five of six subjects and vomiting in three of six subjects. In the multiple dose 20mg and 40mg groups, three subjects

reported nausea with one subject also reporting vomiting; these events were mild in severity and resolved on Day 1 of dosing. No subjects reported nausea or vomiting after Day 1 of dosing (Figure 15). While a single dose of 60mg was not well tolerated, the average peak drug levels (138ng/mL) observed in the 40mg group after eight days of treatment were greater than those seen with the single 60mg dose (130ng/mL) on Day 1. Improved tolerability at higher concentrations following repeated dosing suggests that titration to higher doses might be a viable strategy to further improve the tolerability profile. To test this hypothesis, we are currently conducting a titration study in healthy volunteers evaluating up to 120mg of PRAX-944 daily dosing.

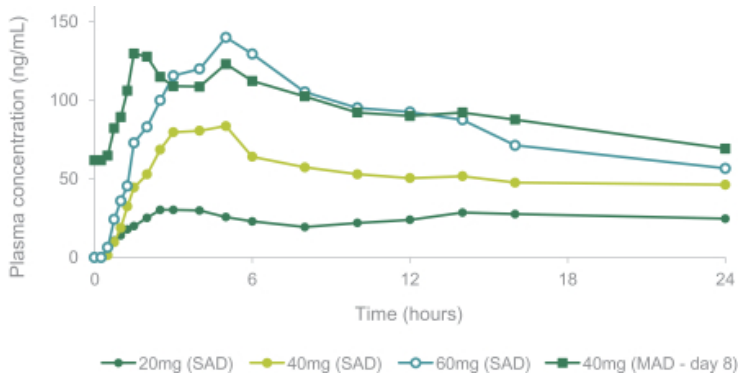


Figure 14. Sustained exposures were observed for the MR formulation of PRAX-944.

TEAEs	Placebo (n=4)	PRAX-944 20mg QD (n=6)	PRAX-944 40mg QD(n=6)
Headache	1	2	2
Somnolence	1	1	2
Dizziness	1	0	2
Fatigue	2	1	2
Rash*	0	0	1
Skin Reaction*	0	0	1
Nausea^	1	2	1
Vomiting^	0	1	0
Hot Flash	0	0	2
Vision Blurred	0	0	1
Thermal Burn**	0	0	1
Euphoric Mood	0	0	1
Dry Throat	0	1	0
Subjects with TEAEs	3	4	5

*Subject had application site rash from ECG stickers and application site skin reaction from EEG stickers
**Subject accidentally spilled hot custard

Patient Number	Dose (mg)	Adverse Event	Day 1 2 3 4 5 6 7 8
1207	20	Nausea^	
1208	20	Nausea^	
1208	20	Vomiting^	
1215	40	Nausea^	

Timing of TEAEs out of 8 days of dosing

^These events were mild in severity and resolved on Day 1 of dosing.

Figure 15. TEAEs observed in the PRAX-944 MAD trial were mild-moderate and transient.

Quantitative EEG studies in healthy volunteers were used to assess the effect of PRAX-944 on T-type calcium channels in the brain. One frequency band known to be driven by T-type calcium channel activation is the sigma frequency band (11 to 15 Hz) during non-rapid eye movement sleep, or NREM sleep. T-type calcium channels expressed in thalamic neurons are critically involved in the generation and modulation of these rhythmic thalamocortical oscillations during NREM sleep.

In our preclinical studies, dosing of normal rats with PRAX-944 led to robust and dose-dependent changes in EEG activity. Because similarly robust sigma frequency band changes after dosing with

PRAX-944 are observed during NREM sleep in rats and humans, our hypothesis is that the inhibition of this EEG signal can be used as a pharmacodynamic biomarker. Because the doses at which EEG changes observed in rats are similar to those that demonstrated activity in a preclinical model of essential tremor, or the harmaline model, we believe that this EEG biomarker can be used to estimate the dose of PRAX-944 that will produce a therapeutic effect in ET.

In our Phase 1 trial, 20mg and 40mg doses of PRAX-944 administered to healthy volunteers produced changes in the qEEG recordings of the sigma frequency band during NREM sleep consistent with those observed in rats. This indicated that PRAX-944 reached target levels in the brain needed to inhibit T-type calcium channels. Based on the overlap of these EEG changes with drug levels showing activity in the preclinical harmaline model, we believe that 20mg and 40mg doses of PRAX-944, which were generally well-tolerated in healthy volunteers without titration, have the potential to reduce tremor in patients with ET.

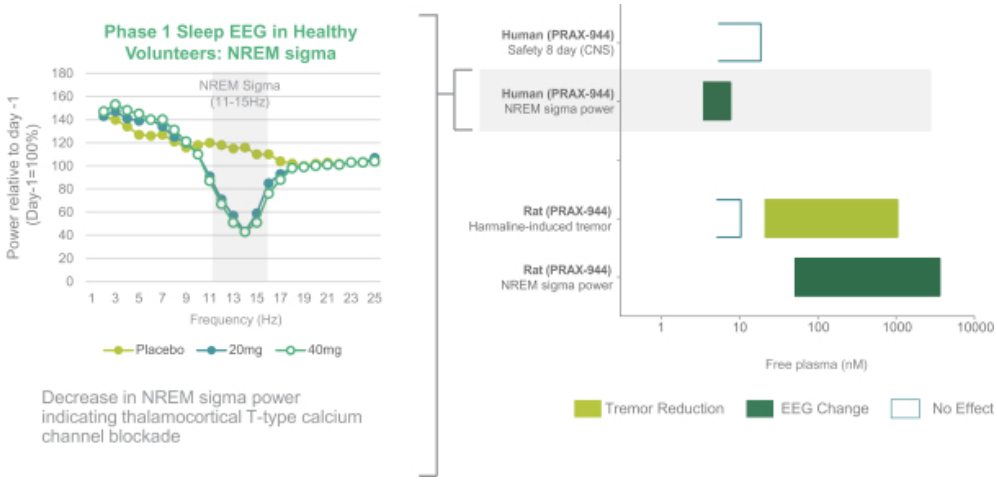


Figure 16. Exposures of PRAX-944 that decreased sigma band activity were generally well-tolerated in healthy volunteers and were associated with harmaline tremor reduction.

Titration trial in healthy volunteers

Considering that improved tolerability is the key unmet need in ET and that existing data suggest titration is a viable strategy to further improve PRAX-944's tolerability profile, we explored titration in healthy volunteers to dose levels as high as 120mg daily. Participants were randomized to PRAX-944 or placebo in a 3:1 ratio, starting at 20mg daily in the morning and titrated at 20mg increments up to 120mg daily with up to one week in between each dose increment to achieve steady-state plasma concentration and the collection of safety data.

Dosing has completed in this study and the analysis of the safety data to date suggests that with titration, PRAX-944 has been generally well tolerated up to 120mg. There were no SAEs and no severe AEs. The majority of AEs were mild, transient and resolved without intervention. There were no treatment related ECG or EEG abnormalities. To date, safety laboratory values have generally been within normal limits and there have been no dose dependent excursion from the normal range. Importantly, no MTD was identified. We believe this provides an advantage to PRAX-944 as it enables a wide dose range for optimizing efficacy and tolerability profile tailored to the key unmet need in ET as well as individual patient needs.

Phase 2a trial in patients with ET

We are currently conducting a Phase 2a proof-of-concept open-label trial in up to twelve patients per cohort with ET in Australia and New Zealand. Patients receive 20mg daily dosing of PRAX-944 for one week followed by 40mg daily dosing for the second week, taken in the morning. Based on the data from the titration trial in healthy volunteers, we are enrolling an additional cohort of ET patients dosing up to 120mg.

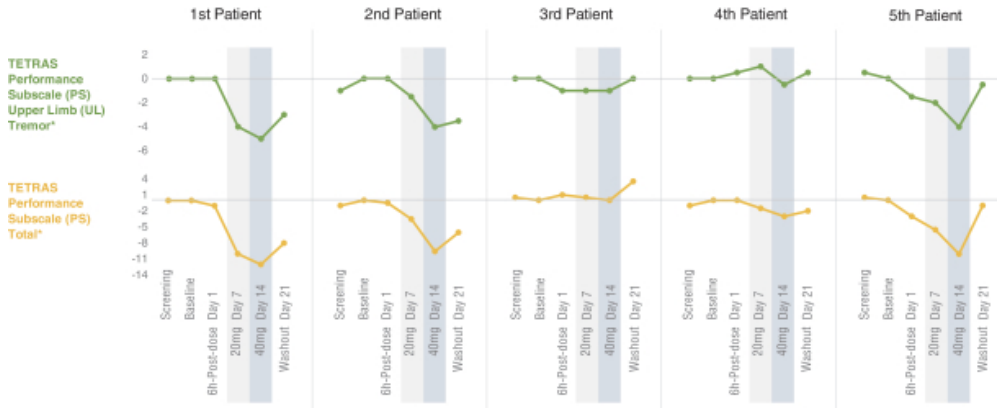
Studies in movement disorders require careful attention to methods for obtaining and scoring outcome measures. We are measuring changes in tremor with different, complementary approaches including components of the Essential Tremor Rating Scale, or TETRAS, Performance Scale and accelerometry. TETRAS is a widely used clinical rating scale that measures the severity of ET. It was based on similar clinical scales which have been used to support regulatory approval of neurosurgical treatments for severe ET. TETRAS has shown good measurement properties and dynamic range compared to other scales.

We are using change from baseline in the rating of upper limb, or UL, items of the TETRAS as the primary efficacy outcome in this proof-of-concept trial because all ET patients suffer from UL tremor. As the UL items drive most of the score on the overall TETRAS and are more reliably rated than other items on the scale, they are therefore expected to have the best signal to noise ratio. UL items have also been the basis of the most recent regulatory approval of neurosurgical treatments for severe ET. We have established rigorous procedures for training and for blinded scoring of efficacy, including centralized video assessment with randomization of the videos and masking to allow for objective ratings. We have also included the overall TETRAS performance scale, or TETRAS-PS, (both site and central video rating) and Kinesia ONE accelerometer, clinical global impression of severity and improvement (CGI-S and CGI-I) and the patient global impression of change (PGI-C) as secondary endpoints in the current open-label study to assess consistency of response across different endpoints.

In this trial, we are enrolling participants with well-established ET, as defined by the Movement Disorders Society, or MDS, Task Force for Tremor as an isolated tremor syndrome of bilateral UL action tremor with at least 3 years' duration. Patients are required to have a combined bilateral score of $^{3}10$ on the TETRAS UL items as confirmed by site investigator and central video review. This baseline severity provides a clear and measurable dynamic range for detecting a treatment response. Tremor severity will be evaluated before drug administration, after daily morning dosing of PRAX-944 20mg for 7 days (Day 7), following daily administration of PRAX-944 40mg for 7 additional days (Day 14) and one week after administration of PRAX-944 has been stopped (Day 21).

To date, five participants have been dosed in this trial with 20mg, followed by 40mg of PRAX-944. Preliminary site data of participants in this cohort showed stable tremor severity between screening and baseline visits. In addition, we observed tremor reduction at both dose levels which seems to compare favorably to the standard of care agents and consistency in response across different endpoint measurements (TETRAS-UL, TETRAS-PS, Kinesia ONE accelerometer score). Over the 14 days of dosing, three of the five participants had greater than or equal to a 4 point reduction in the primary endpoint for the study, TETRAS-UL score, which corresponds to a greater than 50% reduction in upper limb tremor amplitude. Improvement was also observed in the full TETRAS-PS, and

Kinesia ONE accelerometry scores. Across the three endpoint measures, the scores were also consistent for the two remaining patients that did not demonstrate a notable improvement at 20mg or 40mg.



* All values displayed are score reduction from baseline
Source: Praxis Data as of September 23, 2020; Complete Kinesia ONE data on the five participants pending

Figure 17. Preliminary site data from the Essential Tremor OL study (N=5)

PRAX-944 has been generally well tolerated in the ET patients dosed to date. There were no SAEs and no severe AEs. The majority of AEs were mild, transient and resolved without intervention. All five participants that were enrolled completed the full dosing schedule. There were no clinically significant ECG or laboratory abnormalities. There were no changes in the C-SSRS. Based on the observed safety profile in the titration HV trial and the ET open-label trial, we plan to include a high dose cohort in this on-going ET open-label trial dosing up to 120mg.

We plan to announce topline data, including the high dose cohort, from this Phase 2a trial in the first half of 2021.

Preclinical support for advancing PRAX-944

PRAX-944 has been shown preclinically to inhibit all three human T-type calcium channel isoforms, Cav3.1, Cav3.2 and Cav3.3, and has demonstrated high selectivity against L and N-type calcium channels, or Cav1.2 and Cav2.2, respectively, and other key ion channels important for normal physiology, such as the cardiac potassium channel human Ether-à-go-go-Related Gene, or hERG, and the voltage gated sodium channel Nav1.5. Robust selectivity and potency have been demonstrated across both exogenously expressed recombinant channels in a human cell line and naïve channels in isolated dorsal root ganglion, or DRG, neurons from rats using electrophysiological techniques.

HEK CELLS		RAT DRG NEURONS	
Channel	**IC_{50} (nM)**	**Channel**	**IC_{50} (nM)**
hCav3.1	**202**	**T-Type**	**50**
hCav3.2	**240**	N-Type	10,000
hCav3.3	**188**		
rCav1.2 (L-Type)	32,000		
rCav2.2 (N-Type)	11,000		
hNav1.5	100,000		
hERG	7,800		

Table 18. PRAX-944 is a potent and selective inhibitor of T-type calcium channels.

Consistent with the gatekeeper role of T-type calcium channels in neuronal firing patterns, a gain of function mutation of the T-type calcium channel Cav3.2 leads to pathological burst firing in thalamic neurons in a rat model known as the GAERS model. Administration of PRAX-944 resulted in complete suppression of the pathological burst-firing in thalamic neurons derived from the GAERS model.

We validated the therapeutic potential of PRAX-944 to treat ET using the harmaline-induced tremor model in rats. Administration of harmaline triggers ET-like tremors in experimental animals as well as pathological burst firing throughout the CTC circuit. We observed a large and dose-dependent decrease of harmaline-induced tremor in rats treated with PRAX-944 as compared to vehicle-treated animals, when measured as % increase of tremor from pre-harmaline baseline. This result served to both demonstrate the potential of PRAX-944 in ET and as independent evidence of the critical role of T-type calcium channels in tremor reduction.

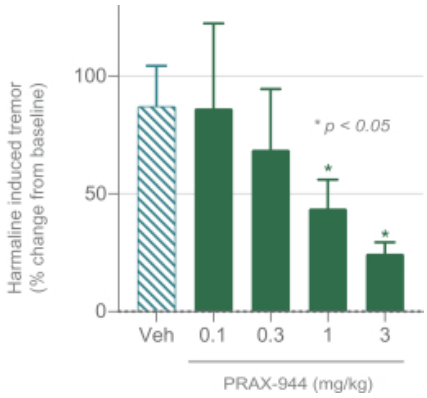


Figure 19. PRAX-944 led to a dose-dependent inhibition of tremors in the rat harmaline model.

EEG as a pharmacodynamic biomarker for dose selection

PRAX-944 robustly and dose-dependently decreased EEG power in the sigma frequency band during NREM sleep in rats. The effect of PRAX-944 on the EEG observed in rats when dosed with PRAX-944 indicates its ability to mediate the blockade of T-type calcium channels in the thalamocortical circuit, suggesting that this effect is a pharmacodynamic biomarker for PRAX-944. Because the doses at which the EEG changes are observed are similar to those that demonstrate tremor reduction in the harmaline model, we believe that this biomarker can be used to estimate the dose that could be effective in treating ET.

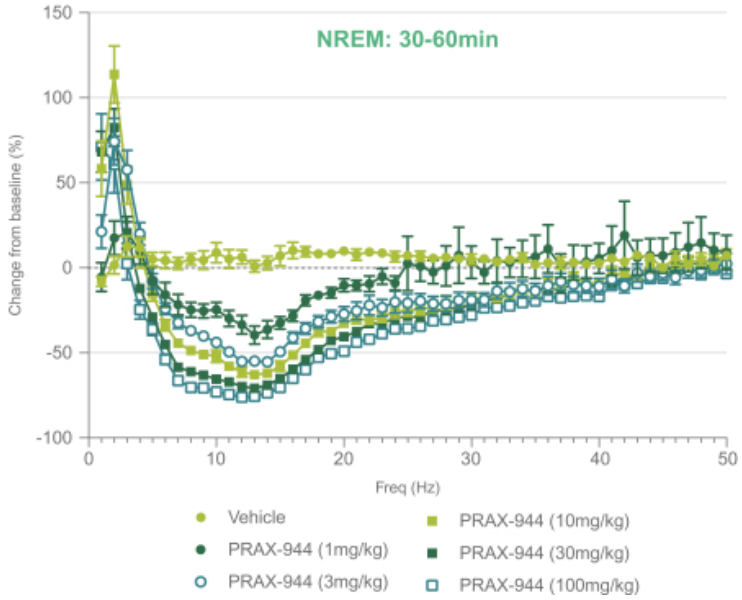


Figure 20. PRAX-944 decreased EEG sigma power during NREM sleep in rats.

RARE DISEASE PORTFOLIO

We are advancing several programs which we believe offer significant therapeutic benefits for rare disease populations over the current standard of care. We believe that all of the programs in our rare disease portfolio have the potential to be differentiated molecules, have foundational underpinnings in human genetics, utilize translational biomarker tools and have the potential for early clinical signal detection.

Our most advanced rare disease candidate is PRAX-562, which is currently in a Phase 1 trial in adult healthy volunteers in Australia. Its mechanism suggests that PRAX-562 has broad potential to treat many diseases of neuronal hyperexcitability. We are pursuing development in a subset of rare devastating diseases, initially cephalgias and pediatric epilepsies.

The remainder of our current rare disease portfolio consists of precision medicines approaches to address genetically defined populations suffering from Developmental and Epileptic Encephalopathies, or DEEs. DEEs are rare neurologic diseases characterized by early onset (< 2 years of age), frequent seizures, abnormal epileptiform electroencephalographic activity, developmental impairment and resistance to available antiepileptic drugs. Additionally, DEEs are associated with a high mortality rate and comorbidities such as developmental delay in addition to psychiatric and mood disorders, movement disorders, pain and sensory dysfunction and sleep disruptions.

The etiology of DEEs has been revolutionized by recent whole-exome sequencing initiatives that showed over 60 genetic causes of epilepsy. An underlying pathologic feature of many DEEs is the dysregulated neuronal activity leading to hyperexcitability, seizures and associated comorbidities. This phenomenon is observed in many pediatric DEEs with an identified genetic cause, such as SCN8A, SCN2A, KCNT1, KCNQ2, KCNQ1 and STXBP1 epilepsy, as well as epilepsies in which a genetic cause remains unclear, such as Lennox Gastaut Syndrome, or Doose Syndrome. Up to 40% of DEEs are caused by single gene mutations, enabling precision medicine approaches.

Given the overlapping biology, phenotypic presentation and clinical execution considerations, we believe that developing a portfolio of drugs to treat DEEs creates a differentiated body of knowledge and operational synergies across our rare disease portfolio, positioning Praxis as a leader in developing meaningful therapies for this group of patients with devastating clinical needs.

PRAX-562

Standard sodium channel blockers, such as Tegretol (carbamazepine), Lidoderm (lidocaine), Lamictal (lamotrigine), Dilantin (phenytoin) and many others are an important class of medicines in neurology and psychiatry. All standard sodium channel blockers modulate neuronal activity by targeting peak sodium current, which can reverse the pathological neuronal hyperexcitability that underlies many CNS conditions, but simultaneously affects the physiological cellular action potential firing required for a functioning nervous and cardiovascular system. Hence, this class is widely used for the treatment of epilepsy, pain, migraine and bipolar disorder, but its efficacy is generally limited by side effects.

PRAX-562 is designed as the first selective, persistent sodium current blocker that has the potential of reducing pathological neuronal hyperexcitability with an improved tolerability profile. PRAX-562 is in development for the treatment of a broad range of rare, devastating CNS disorders, such as severe pediatric epilepsy and adult cephalgia. We intend to pursue development in a subset of rare diseases, initially Short-lasting Unilateral Neuralgiform headache attacks with Conjunctival injection and Tearing, or SUNCT, and Short-lasting Unilateral Neuralgiform headache with Autonomic symptoms, or SUNA, two rare types of cephalgia and pediatric epilepsies for which there are currently no FDA-approved treatments.

In *in-vitro* studies, PRAX-562 selectively blocks persistent sodium currents across all subtypes of sodium channels with minimal effects on the peak sodium current that is critical for the normal physiological function of these channels. In line with this selectivity for persistent current, PRAX-562 has been shown in *ex-vivo* studies to reduce neuronal hyperexcitability without impairing normal neuronal function. This is in contrast to marketed sodium channel blockers which significantly impact normal neuronal function, leading to a narrow therapeutic index.

To date, PRAX-562 has demonstrated robust pharmacological activity in an *in-vivo* seizure model with significantly improved tolerability compared to other sodium channel blockers, suggesting a potentially improved therapeutic index. The characteristics of PRAX-562 are expected to make it a versatile molecule that we believe can be broadly applied in diseases of hyperexcitability where sodium channel blockers have demonstrated efficacy but poor tolerability.

We have initiated a Phase 1 trial of PRAX-562 in Australia to evaluate the safety, tolerability, PK and effects on an EEG biomarker in up to 129 adult healthy volunteers. We anticipate Phase 1 SAD topline safety data in the fourth quarter of 2020. The clinical development plan for PRAX-562 encompasses exploring the broad potential for the mechanism of action in rare diseases through proof-of-concept trials in SUNCT and SUNA patients and then expanding into a range of rare pediatric DEEs.

Voltage-gated sodium channels, persistent sodium current and neuronal excitability

Voltage-gated sodium channels, or VGSCs, are transmembrane proteins that are required for electrical signaling and therefore communication in neurons. VGSCs respond to changes in the

membrane potential and are tightly regulated by their biophysical properties. Upon opening of VGSCs, sodium ions can move into the cell leading to a depolarization and therefore excitation of the neuron. This sodium current is the initiator and driver of neuronal action potentials, or APs, the primary means of electrical signal propagation along the neuron's axon.

The family of VGSCs consists of nine highly related isoforms (Nav1.1 – Nav1.9) with differential tissue distributions and functions. Nav1.1, 1.2 and 1.6 are the major sodium channels expressed in the central nervous system.

Isoform	Gene	Expression
Nav1.1	SCN1A	CNS
Nav1.2	SCN2A	CNS
Nav1.3	SCN3A	CNS/Pancreas
Nav1.4	SCN4A	Muscle
Nav1.5	SCN5A	Heart
Nav1.6	SCN8A	CNS/PNS
Nav1.7	SCN9A	PNS
Nav1.8	SCN10A	PNS
Nav1.9	SCN11A	PNS

CNS: Central Nervous System, PNS: Peripheral Nervous System

Table 21. Sodium Channel Isoforms and tissue distribution.

VGSCs undergo conformational changes that alter their ability to conduct sodium ions (Table 21) and are triggered to open upon excitation, or depolarization, of the cell membrane allowing sodium ions to enter the neuron. Sodium influx further excites, or depolarizes, the neuron, leading to the opening of even more sodium channels. This series of events can lead to a large peak sodium current underlying the initiation and propagation of neuronal action potentials, or APs, the primary means by which neurons propagate information in the nervous system. To prevent overexcitation of neurons, or hyperexcitability in the form of excessive high frequency AP firing, the majority of sodium channels only open very briefly after activation (1-2 ms), followed by a refractory period of inactivation or non-responsiveness.

However, at membrane potentials below the AP firing threshold, a small subset of sodium channels can remain open for hundreds of milliseconds, carrying the so-called persistent sodium current. Persistent sodium current is present under physiologic conditions where it modulates excitability of neurons and can be significantly increased in pathologic states (Figure 22).

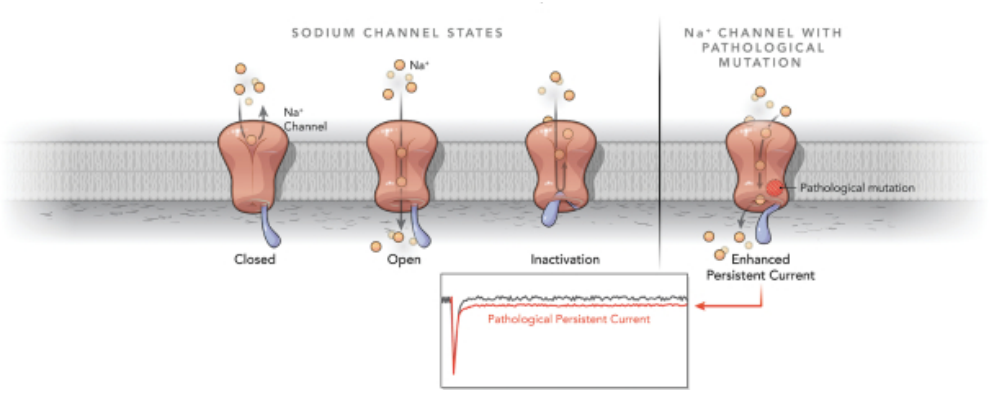


Figure 22. Impact of a pathological mutation on sodium channels.

There are currently more than 15 sodium channel blockers in the market commonly used to treat diseases such as epilepsy, bipolar disorder and pain. While standard of care sodium channel blockers, such as carbamazepine, lamotrigine and phenytoin, inhibit persistent sodium current, they likely also block peak sodium current at therapeutic concentrations, which can cause significant adverse events such as ataxia, drowsiness and dizziness, and therefore have a very narrow Therapeutic Index, or TI.

Genetics of persistent sodium current

In published whole-exome sequencing studies of diverse patient populations, mutations in all voltage gated sodium channel subtypes have been reported as a likely cause of disease. Furthermore, gain-of-function mutations that are associated with disease can cause an increase of persistent sodium current, raising the idea that this might be a critical driver of hyperexcitability in neurologic disorders.

The specific disease that a patient develops depends on both the sodium channel subtype and where the affected sodium channel is expressed. Gain-of-function mutations in SCN2A, or Nav1.2, and SCN8A, or Nav1.6, two of the major sodium channels in the brain, cause early onset epileptic encephalopathies with frequent seizures and developmental delay. Gain-of-function mutations in Nav1.1, Nav1.4, Nav1.5 and Nav1.7 cause familial hemiplegic migraine, myotonia, cardiac arrythmia and severe pain disorders, based on their primary expression in the CNS, muscle, heart and pain pathways, respectively. These channelopathies demonstrate the important role persistent sodium current plays as a modulator of cellular excitability.

Our initial indications for PRAX-562

Developmental and epileptic encephalopathy

Approximately 100,000 children suffer from DEEs in the United States alone, with over two hundred thousand children affected world-wide. An underlying pathologic feature of many DEEs with both known and unknown genetic causes, is the dysregulated neuronal activity leading to hyperexcitability and subsequently to seizure.

Sodium channel blockers have been a critical component of the pharmacological management of seizure related conditions, including epilepsy, for decades. However, current standard of care sodium channel blockers are limited by a narrow therapeutic window and inadequate efficacy. We believe these limitations are largely due to blockage of peak sodium current and disruption of normal neuronal function at or near therapeutic doses and significant off-target activity.

Given the role of persistent current in modulating excitability, we believe that PRAX-562 has the potential to be a broadly efficacious and generally well-tolerated antiepileptic drug for the treatment of DEEs of both genetic and unknown etiology.

SUNCT and SUNA cephalgias

SUNCT and SUNA cephalgias are devastating primary headache disorders that are part of a specific class of cephalgias known as Short Lasting Unilateral Neuralgiform headaches. These headaches are characterized by severe burning, stabbing and electrical unilateral head pain that is typically 9 to 10 in the Visual Analogue Scale, or VAS, for pain. These headache attacks lasts between one second and ten minutes in duration and can occur up to 600 times per day. SUNCT and SUNA headaches are rare diseases with a prevalence estimated to be 6.6 per 100,000 based on a recent Australian study.

Increased activation of the posterior hypothalamus during SUNCT and SUNA headache attacks indicates that hyperexcitability of central neurons is core to the conditions. Moreover, deep brain stimulation of the posterior inferior hypothalamus effectively controls headache attacks.

SUNCT and SUNA are often refractory to standard migraine and headache treatments, but are highly responsive to intravenous, or IV, infusion of the sodium channel blocker lidocaine. Response under IV lidocaine requires continuous infusion in an inpatient setting and is associated with side effects such as nausea, vomiting and cardiovascular effects, with headache attacks returning in majority of patients within days of IV lidocaine withdrawal. Preventative treatment of SUNCT and SUNA often includes oral sodium channel blocker lamotrigine, but this is limited by partial efficacy, tolerability concerns and the requirement of several weeks of dose-titration to reach therapeutic doses.

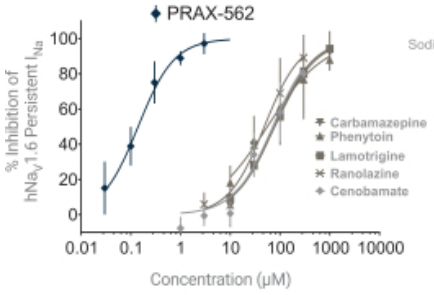
The absence of FDA-approved treatments specific to SUNCT and SUNA, combined with high comorbidity and healthcare utilization, substantiates the need for an efficacious, generally well-tolerated and orally bioavailable sodium channel blocker to treat headache attacks in acute and preventative settings.

PRAX-562 preclinical data

PRAX-562 is a highly differentiated, potent and selective inhibitor of persistent sodium current designed to overcome the limitations of currently available sodium channel blockers. PRAX-562 preclinical studies were designed to test our belief that the block of persistent sodium current is sufficient to demonstrate robust activity in animal models of hyperexcitation and that the selective block of persistent sodium current over physiological peak current leads to an improved therapeutic index.

Selective inhibition of persistent sodium channels

In preclinical studies, PRAX-562 is a highly potent inhibitor of persistent sodium current as measured in cell-based assays, in which sodium channel isoforms are heterologously expressed and channel activity is measured via patch clamp electrophysiology. Using electrophysiological voltage protocols, the effect of compounds on a specific channel state (e.g., peak current vs persistent current) can be measured. When compared to other approved sodium channel inhibitors for various neurological indications, PRAX-562 was hundreds of times more potent at inhibiting persistent sodium current. PRAX-562 had an IC50 of 141 nM compared to SOC sodium channel blockers lamotrigine and carbamazepine which had an IC50 of 78,530 nM and 77,520 nM, respectively – a potency difference of over 500-fold. PRAX-562 was ~60 fold selective for inhibiting persistent current over peak current.



		Persistent INa* IC50 (nM)	Ratio of persistent to peak inhibition (Tonic Block)
Praxis selective INaP blockers	PRAX-562	141	60
Sodium Channel Blockers with recent clinical experience	Vixotrigene (BIIB074)	3,676	14
Standard Sodium Channel Blockers	Cenobamate	73,263	23 Project 1,719,000 uM
	Carbamazepine	77,520	30
	Ranolazine	48,600	14
	Mexiletine	55,570	7
	Phenytoin	55,770	n/a**
	Lidocaine	68,230	19
	Lamotrigine	78,530	16
	Lacosamide	833,100	n/a**
	Valproic Acid	<10% @ 1 mM	No inhibition

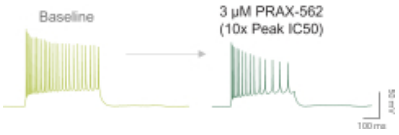
** solubility concerns

Figure 23. PRAX-562 is approximately 150-fold more potent and two to nine-fold more selective for persistent sodium current than standard sodium channel blockers.

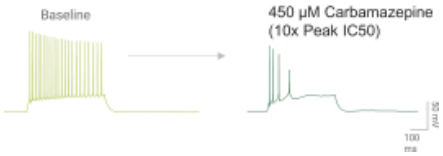
The selective block of persistent sodium current reduces neuronal hyperexcitability without affecting the action potential, or AP, amplitude, which is required for normal neuron function. In mouse

brain slice experiments, a hyperexcitable state can be mimicked by artificially depolarizing the neuron using the patch clamp method, which elicits high frequency AP firing. PRAX-562 reduced the neuronal AP firing frequency, an indicator of neuronal excitability, without a significant effect on AP amplitude, an indicator of normal neuronal function, suggesting reduction of hyperexcitability without impacting the ability of the neuron to respond to physiologic stimuli. In comparison, carbamazepine, a SOC sodium channel blocker, at comparable concentrations (relative to the potency in cells heterologously expressing Nav1.6), excessively decreased AP firing almost completely and reduced the amplitude of APs, indicating impairment of normal function.

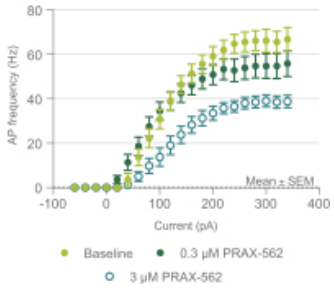
PRAX-562 Representative AP Traces



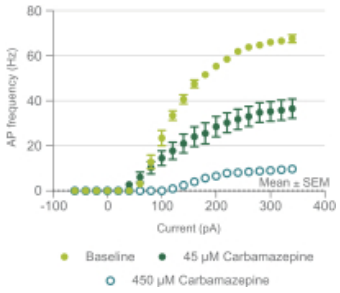
Carbamazepine Representative AP Traces



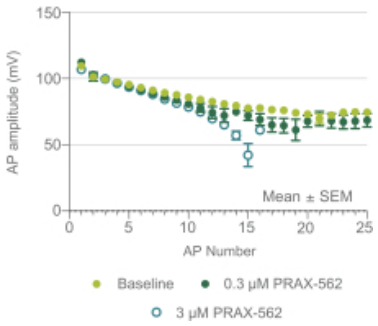
PRAX-562 Average AP Frequency During Increasing Current Steps



Carbamazepine Average AP Frequency During Increase Current Steps



PRAX-562 Average AP Amplitude During Current Step



Carbamazepine Average AP Amplitude During Current Step

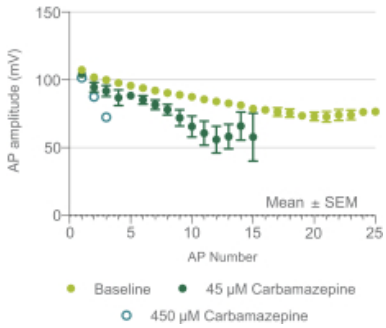


Figure 24. PRAX-562 reduced neuronal hyperexcitability (AP frequency) without impairing normal function (minimal effect on AP amplitude). In contrast, carbamazepine significantly reduced the AP amplitude suggesting impairment of normal function.

Preclinical in vivo pharmacological activity, tolerability and EEG pharmacodynamic biomarker

We investigated the preclinical activity of PRAX-562 in a maximal electroshock model of epilepsy, or MES model, that has shown good predictive validity for clinical anti-convulsant activity, and compared it to the effects of SOC sodium channel blockers carbamazepine and lamotrigine. To determine how well PRAX-562 is tolerated, we compared its effects on spontaneous locomotor activity, or sLMA, to the effects of carbamazepine and lamotrigine.

PRAX-562 was able to block seizures completely in mice at a dose that does not impair locomotor function (10mg/kg). In contrast, carbamazepine and lamotrigine only achieve full block of seizures in this model at doses that also show impairment of locomotion. PRAX-562 at a dose of 2mg/kg, inhibited the epilepsy response to half of its maximum value, or ED_{50}. Inhibition of sLMA required an estimated dose of 44mg/kg to obtain 50 percent inhibition, or TD_{50}.

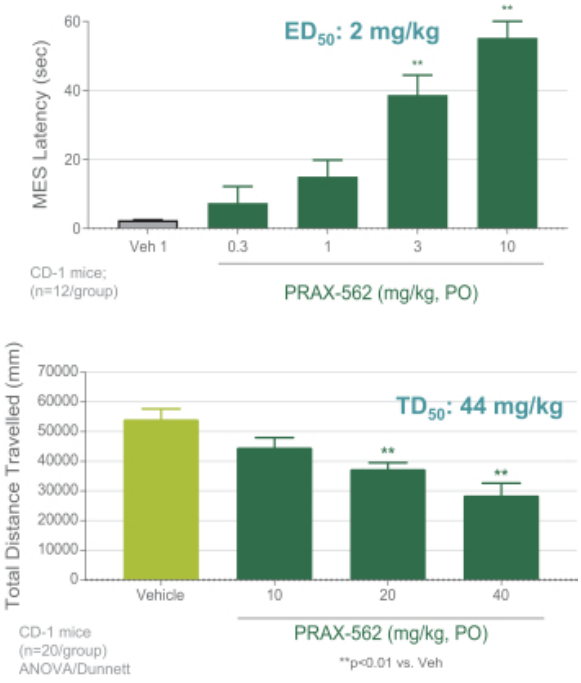


Figure 25. Doses of PRAX-562 resulting in potent anticonvulsant activity were associated with minimal effects on general locomotor activity.

We calculated the therapeutic index, or TI, of each molecule as the preclinical tolerability/pharmacological activity ratio. This ratio is calculated by dividing the plasma and brain concentrations at the dose that reduces locomotion by 50% by the concentrations that reduce seizures by 50%. We found that PRAX-562 had a significantly improved therapeutic index of ~16 fold (based on brain concentrations) and ~17 fold (based on plasma concentrations) compared to the currently prescribed sodium channel blockers carbamazepine and lamotrigine, which had a much lower protective index of three to six-fold. Notably, standard sodium channel blockers, such as carbamazepine and phenytoin,

show severe toxicity in humans at exposures that are only about 1.5 to 3 times the target therapeutic exposures, underscoring the need for modulators of sodium channels with an improved tolerability.

Molecule	Plasma Therapeutic Index	Brain Therapeutic Index
PRAX-562	**17.2x**	**16.4x**
Carbamazepine	3.4 x	5.9 x
Lamotrigine	6.4 x	4.6 x

Therapeutic Index (TI) = TC_{50}/ EC_{50}

Table 26. Compared to lamotrigine and carbamazepine, PRAX-562 had an increased ratio between drug levels that demonstrated preclinical pharmacological activity versus those that caused toxicity.

The auditory steady state response, or ASSR, is a non-invasive EEG measure of excitatory/inhibitory balance in the brain. This response is elicited with short lasting (2sec) auditory stimuli that lead to brain activity changes that are measured as a 40Hz EEG signature and depend on network activity between excitatory and inhibitory cortical neurons. We believe that persistent current block has the potential to lead to reduced excitability of the network and will be measurable with this endpoint.

Consistent with this hypothesis, dosing normal mice with PRAX-562 led to a dose-dependent decrease in the ASSR amplitude (40Hz power). This effect was maximal at doses that have robust anticonvulsant effects in the maximal electroshock model.

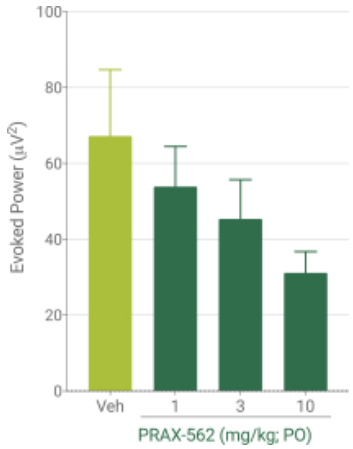


Figure 27: PRAX-562 dose-dependently reduced the 40Hz EEG power of the auditory steady state response in mice.

Together, our data suggest that the selective effects of PRAX-562 on hyperexcitable states without affecting normal neuronal function led to the robust preclinical reduction of seizures and improved tolerability seen in animal models. As shown below, exposures of PRAX-562 that led to biomarker change (ASSR amplitude reduction shown in top row) also demonstrated robust anticonvulsant activity (shown in middle row). Moreover, PRAX-562 has a ~16.4 fold protective index based on the spontaneous locomotor activity (shown in bottom row), which is a significant improvement over reported effects of approved sodium channel blockers. In the figure below, the lower bound of the preclinical pharmacological activity range, EEG and tolerability bars is determined by the brain EC_{50}

(preclinical seizure and ASSR assays) or TC_{50} (tolerability assay) in a given assay and the upper bound represents the mean brain concentration at the highest dose tested in a given assay.

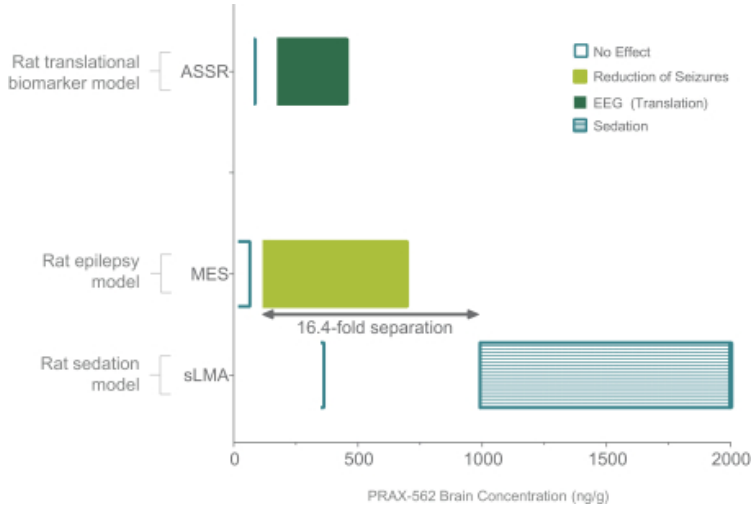


Figure 28. Summary of PRAX-562 preclinical data.

We believe that the profile of PRAX-562 may translate into therapies with the potential for clinical efficacy and tolerability across several indications caused by underlying hyperexcitability where standard sodium channel blockers have shown efficacy, albeit with limited tolerability, such as rare pediatric epilepsy and cephalgias like SUNCT/SUNA.

PRAX-562 clinical development in SUNCT and SUNA cephalgias and DEEs

We have initiated a randomized, double-blinded Phase 1 trial in Australia to evaluate the safety, tolerability and pharmacokinetics of single and multiple ascending doses of PRAX-562 in up to 129 adult healthy volunteers between the ages of 18 and 55. In addition, we plan to use ASSR as a pharmacodynamic biomarker in this trial to determine the doses required to achieve pharmacological blockade of persistent sodium current, which we believe is a potential indicator of efficacy in patients. Preliminary analysis of the first three of six planned cohorts of the Phase 1 SAD trial indicate PRAX-562 appears to be well tolerated at the doses tested. Safety data reviewed included adverse events, vital signs, ECG, C-SSRS, physical examination and safety laboratory data. In the first three cohorts tested there have been no reported SAEs, severe AEs or any AEs leading to study withdrawal or discontinuation. Dosing has started in the fourth of six planned dose cohorts. We anticipate Phase 1 SAD topline safety data in the fourth quarter of 2020.

The clinical development plan for PRAX-562 encompasses exploring the broad potential for the mechanism of action in rare diseases through proof-of-concept trials in SUNCT and SUNA patients and then expanding into a range of rare pediatric DEEs.

Along with our PRAX-562 program, we are developing a portfolio of sodium channel blockers that we plan to advance in other rare neuropsychiatric disorders.

PRAX-222

PRAX-222 is an antisense oligonucleotide, or ASO, that is designed to lower the expression levels of the protein encoded by the gene SCN2A, in patients with gain-of-function, or GOF, SCN2A epilepsy.

This program is ongoing under a three-way collaboration with Ionis Pharmaceuticals, Inc., or Ionis, and RogCon Inc., or RogCon. Under the terms of the collaboration agreement, Ionis is responsible for preclinical and IND-enabling toxicology studies and Praxis is responsible for clinical development and commercialization.

SCN2A is the gene that encodes the voltage-gated sodium channel Nav1.2 that is primarily found in excitatory neurons throughout the brain and which plays a critical role in action potential generation and signaling between neurons. Individuals with gain-of-function mutations in SCN2A develop early-onset epileptic encephalopathy with severe seizures that begin within the first month of life that are often refractory to standard of care antiepileptic medications. SCN2A GOF patients also suffer from significant intellectual disability, movement disorders and in some cases early death due to sudden unexpected death in epilepsy, or SUDEP. It is estimated that there are thousands of patients worldwide with gain-of-function changes in SCN2A leading to epileptic encephalopathy.

PRAX-222 directly targets the cause of disease by down-regulating Nav1.2 expression, an effect that has demonstrated disease-modifying activity in animal models of SCN2A epileptic encephalopathy. In transgenic mice carrying a human GOF SCN2A mutation, we observed a significant, dose-dependent reduction in seizures and increased survival of mice treated with a mouse ASO that down-regulates SCN2A. The survival benefit from the ASO was maintained with repeat dosing. We also observed survival benefits following administration of a mouse ASO to a group of mice after onset of disease and around the time of onset of mortality. This observation suggests that PRAX-222 may have the potential to provide clinical benefits for children after disease onset. The ASO-treated disease model animals demonstrated similar behavior and locomotor activity as wild type animals, suggesting SCN2A knockdown is generally well-tolerated and that the benefits extend beyond seizure control alone.

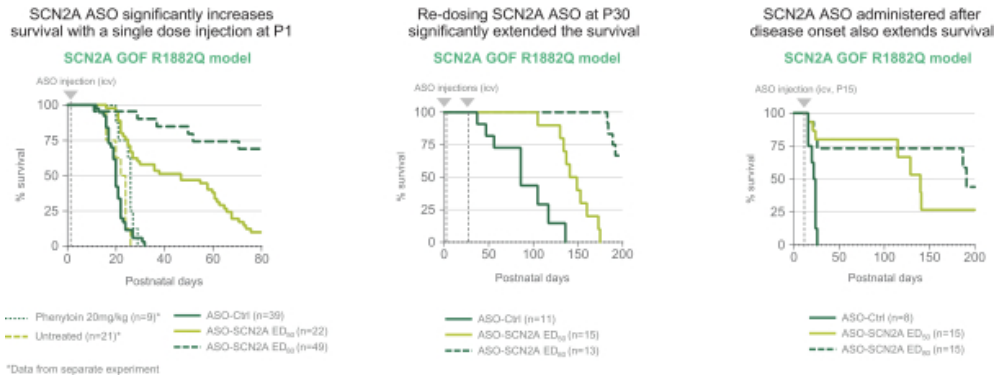


Figure 29. An SCN2A ASO increased survival in a SCN2A gain-of-function mouse model.

PRAX-222 is currently undergoing evaluation in IND-enabling toxicology studies. We intend to file an IND in the second half of 2021 to pursue clinical development in SCN2A GOF epilepsy.

KCNT1 Program

We are currently identifying small molecule inhibitors of the sodium-activated potassium channel encoded by the gene KCNT1 for the treatment of KCNT1 GOF epilepsy. Potassium channels encoded by the KCNT1 gene play a key role in regulating neuronal AP firing. Gain-of-function KCNT1 mutations promote neuronal hyperexcitability, resulting in severe early onset epilepsy with continuous seizures

and severe developmental delay, affecting thousands of patients worldwide. KCNT1 GOF epilepsy is often refractory to conventional treatment approaches. Anticonvulsants, such as stiripentol, benzodiazepines, levetiracetam and ketogenic diet, have all demonstrated limited efficacy.

Genetically lowering KCNT1 expression in transgenic mice carrying a KCNT1 human GOF mutation has been reported to result in disease modifying preclinical activity including seizure reduction, improved cognitive function and survival benefit. Through chemical optimization of the potency and pharmacokinetic properties of hits from a high-throughput screen, we have identified novel small molecule inhibitors of KCNT1. These inhibitors restored normal action potential firing *in-vitro* in KCNT1 GOF mutant neurons and reduce seizure and abnormal interictal spikes *in-vivo* in transgenic mice carrying a KCNT1 human GOF mutation, recapitulating the reported disease modifying preclinical activity demonstrated by genetic tools.

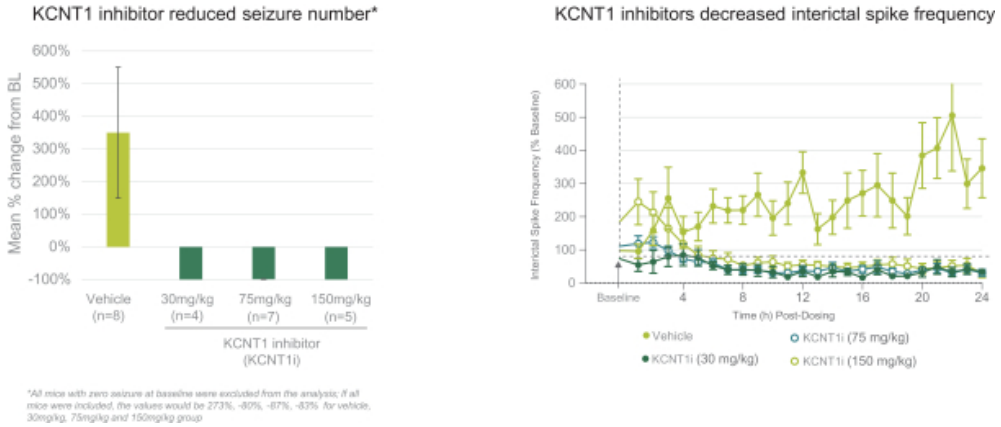


Figure 30. A KCNT1 inhibitor eliminated the occurrence of seizures in a KCNT1 transgenic mouse model and suppressed interictal spikes (or abnormal electrographic discharges observed between seizures) as detected by EEG

We are continuing to optimize the chemical structures of our molecules targeting KCNT1 channels and expect to select a development candidate in 2021.

Competition

The biopharmaceutical industry is characterized by rapidly advancing technologies, strong competition and an emphasis on proprietary products. While we believe that our technology, knowledge, experience and scientific personnel provide us with competitive advantages, we face substantial competition from many different sources, including large and small pharmaceutical and biotechnology companies, academic research institutions, governmental agencies and public and private institutions.

Any product candidates that we successfully develop and commercialize will compete with currently approved therapies and new therapies that may become available in the future. Key product features that would affect our ability to effectively compete with other therapeutics include the efficacy, safety, convenience, cost, effectiveness of promotional support and intellectual property protection of our products. Our competitors fall primarily into the following groups of treatment:

- $GABA_A$ receptor modulator programs in development targeting MDD, including that of SAGE Therapeutics, as well as other programs in clinical development targeting other mechanisms of action and approved therapies such as SSRIs.

- T-type calcium channel inhibitor programs in development targeting ET, including that of Jazz Pharmaceuticals, as well as other programs in clinical development targeting other mechanisms of action and approved therapies, such as propranolol, and off-label therapies, such as primidone.
- Sodium channel blocker programs in development for DEEs, including those of SK-Pharma and Xenon Pharmaceuticals, as well as other programs in clinical development targeting other mechanisms of action and approved therapies including other existing ion channel blockers.
- We are not aware of any development programs targeting SUNCT and SUNA, but we may face competition from off-label therapies such as intravenous lidocaine.

Many of our competitors have substantially greater financial resources, expertise and capabilities in research and development, the regulatory approval process, manufacturing and marketing than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through M&A activity and sizeable collaborative arrangements with established companies.

Intellectual Property

We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business, including seeking, maintaining and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets relating to our proprietary technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of neuroscience that may be important for the development of our business. We additionally may rely on regulatory protection afforded through data exclusivity, market exclusivity and patent term extensions, where available.

Our commercial success may depend in part on our ability to: obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and proprietary rights of third parties. Our ability to stop third parties from making, using, selling, offering to sell, or importing our products may depend on the extent to which we have rights under valid and enforceable licenses, patents, or trade secrets that cover these activities. In some cases, enforcement of these rights may depend on third party licensors. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of manufacturing the same.

$GABA_A$ receptor positive allosteric modulators

We own seven patent families directed to $GABA_A$ receptor positive allosteric modulators. One family discloses and claims salts and polymorphs of PRAX-114, including the lead clinical candidate citrate salt of PRAX-114. A U.S. patent covering the clinical candidate salt has granted in the U.S. (U.S. 10562930) and PCT, EP, JP and TW applications are pending. Patents issuing from applications in this family would expire in the United States on July 19, 2039 and on August 30, 2039 in other countries, not taking into account any potential patent term adjustment or extension that may be available in the future. A second family covers various methods of use, including treatment of the current lead clinical indication, major depressive disorder, with PRAX-114. This patent family has one pending PCT application and two pending U.S. utility applications and two pending U.S. provisional applications. The expected statutory expiration date of patents issuing from applications in this family is December 16, 2039. A third family is directed to methods of treating various perimenopausal symptoms with PRAX-114. This family has one pending PCT application and an expected statutory expiration date of March 4, 2040. Four additional

U.S. provisional applications have been filed covering combinations of GABA-PAMs (including PRAX-114) with NMDA antagonists, NMDA Negative Allosteric Modulators or NMDA partial agonists (filed Oct. 2, 2019); deuterated forms of PRAX-114 (filed Feb. 18, 2020); methods of treating adjustment disorder with PRAX-114 (filed July 2, 2020); and improved methods of treating depression with an evening dose of PRAX-114 (filed September 4, 2020).

T-type Calcium channel blockers

We own four patent families directed to T-type Calcium channel blockers. One family discloses and claims certain T-type calcium channel modulators, including PRAX-944. This patent family is issued in U.S., AU, CA, CN, EP, HK, IL, JP, KR and ZA and is pending in BR, HK and IL. The statutory expiration for patents issuing from this family in the United States is April 8, 2029 and June 2, 2029 in foreign jurisdictions. A second family is directed to methods of use of certain T-type calcium channel modulators, including PRAX-944, in treating disease such as epilepsy. This patent family is pending in the United States and the statutory expiration for any patent issuing from this family is December 21, 2037. A third patent family is directed to certain pharmaceutical formulations of PRAX-944 and methods of use in treating disorders such as essential tremor. This patent family is pending as a PCT, having a 30-month national phase deadline of January 11, 2022 and is also pending in AR, GC and TW. A fourth family is directed to methods of use of PRAX-944. This family is composed of two provisional applications filed respectively on April 29, 2020 and July 10, 2020.

Persistent sodium current blockers

We own twenty patent families directed to persistent sodium current blockers including five patent families that relate to our PRAX-562 program and fifteen families related to other persistent sodium current blockers. Additionally, we have in-licensed one patent family.

Regarding the five families directed to our PRAX-562 program, one family discloses and claims certain persistent sodium current blockers, including PRAX-562, and methods of use in treating diseases such as pediatric epilepsy. This family is pending as a PCT, having a 30-month national phase deadline of November 30, 2020 and is also pending in AR and TW. The statutory expiration for any patent issuing from this family is May 30, 2039. A second family discloses other persistent sodium current blockers and generically claims PRAX-562. This family also claims methods of use of the claimed compounds in treating diseases such as pediatric epilepsy. This patent family is pending in multiple jurisdictions, including US, AU, BR, CA, CN, EA, EP, HK and JP. The statutory expiration for any patent issuing from this family is November 28, 2037. A third family is directed to pharmaceutical formulations of PRAX-562 and methods of use in treating diseases such as pediatric epilepsy. This family has three provisional applications filed respectively on November 27, 2019, March 30, 2020 and May 21, 2020. A fourth family is directed to methods of making PRAX-562. This family is a provisional application filed November 27, 2019. A fifth family is directed to methods of use of persistent sodium current blockers, including PRAX-562, in treating diseases such as cephalgia, SUNCT and SUNA. This family is a provisional application filed March 30, 2020.

The remaining fifteen patent families are directed to a portfolio of sodium channel blockers that we plan to advance in other neuropsychiatric disorders. Fifteen patent families disclose and claim persistent sodium current blockers of various core structures and methods of use in treating diseases such as pediatric epilepsy, which include eight families pending in the United States (the statutory expiration for any patent issuing from these families range from November 28, 2037 to May 29, 2040), one family pending as a PCT, having a 30-month national phase deadline of March 28, 2021 (the statutory expiration for any patent issuing from this family is September 27, 2039) and six families pending as provisional applications filed between November 26, 2019 and March 30, 2020. One family is directed to methods of use of certain persistent sodium current blockers in treating diseases such as cephalgia, SUNCT and SUNA. This family is composed of two provisional applications filed respectively on March 30, 2020 and June 12, 2020.

We have exclusively in-licensed one patent family directed to additional persistent sodium current blockers. This family is owned by Gilead. This family has claims directed to certain persistent sodium current blockers and methods of use. This patent family is issued in US, AU, BO, CA, CN, EA, EP, HK, MO, NZ and TW and is pending in PK and VE. The statutory expiration for any patent issuing from this family is between July 22, 2030 and July 27, 2030, not taking into account any potential patent term adjustment or extension that may be available in the future.

KCNT1 blockers

We own 13 patent families directed to KCNT1 blockers including twelve families related to our KCNT1 program and one family related to antisense oligonucleotides.

Twelve patent families are directed to our KCNT1 program and disclose and claims small molecule KCNT1 blockers and methods of use in treating diseases such as epilepsy, including epilepsy having certain KCNT1 mutations. Two families are pending as a PCT, having a 30-month national phase deadline of November 3, 2021. The statutory expiration for any patent issuing from these two families is May 1, 2040. Ten families are provisional applications filed between February 28, 2020 to March 23, 2020.

One family is directed to certain antisense oligonucleotides and methods of use in treating diseases such as epilepsy, including epilepsy having certain KCNT1 mutations. This family is pending as a PCT, having a 30-month national phase deadline of June 20, 2021. The statutory expiration for any patent issuing from this family is December 20, 2039.

SCN2A downregulation

We have exclusively in-licensed two patent families directed to our PRAX-222 program. These patent families are owned by RogCon. These families disclose and claim certain antisense oligonucleotides targeting SCN2A and methods of use in treating diseases such as epilepsy, including epilepsy having certain SCN2A mutations. One family is pending in the United States and the statutory expiration for any patent issuing from this family is August 6, 2039. A second family is pending as a PCT, having a 30-month national phase deadline of February 20, 2021. The statutory expiration for any patent issuing from this family is August 20, 2039.

License Agreements

License Agreement with RogCon

In September 2019, we and RogCon entered into a Cooperation and License Agreement, or the RogCon Agreement, to collaborate to develop antisense oligonucleotides for the treatment of epilepsy caused by mutations of the SCN2A gene. RogCon had an existing collaboration arrangement with Ionis and as a result, we and Ionis negotiated a Research Collaboration, Option and License Agreement, or the Ionis Agreement (described below) in order to complete the license agreement with RogCon. In December 2018, we entered into an agreement with RogCon to advance to them a fully refundable deposit of up to $1.0 million while the RogCon Agreement was being negotiated. Under the RogCon Agreement, RogCon granted us, subject to a concurrent license grant of certain rights to Ionis, an exclusive, worldwide license under RogCon's intellectual property to research, develop and commercialize products for the treatment of all forms of epilepsy and/or neurodevelopmental disorders in each case caused by any mutation of the SCN2A gene. Under the RogCon Agreement, we will conduct, at our own cost and expense, the research and development activities assigned to us under the research plan set out in the Research Collaboration, Option and License Agreement with Ionis. Under the terms of the RogCon Agreement, RogCon is eligible to receive a one-time milestone payment of $3.0 million as well as profit share payments as a percentage of net profits in the mid-teens. Profit share payments will be calculated and due quarterly on any net profits generated from a product commercialized under the RogCon Agreement. The $3.0 million milestone payment will become due when (i) the first profit share payment has become due and payable and (ii) the Additional

Milestone, the Initial Interest Amount and the Second Interest Amount (each as defined within the Ionis Agreement as described below) have all become due and payable to Ionis under our collaboration agreement with Ionis. As part of the RogCon Agreement, we agreed to provide up-front consideration of $2.1 million, consisting of a $1.0 million deposit, $0.7 million in cash reimbursements for certain historical costs previously incurred by RogCon and $0.4 million for the retirement of existing loan balances as of September 11, 2019.

Subsequent to September 11, 2019, we will reimburse RogCon for its out-of-pocket costs incurred for activities performed under the RogCon Agreement. We expense these costs as incurred as research and development. Since the acquisition date, we expensed $0.1 million for the reimbursement of RogCon's out-of-pocket costs in the year ended December 31, 2019.

Additionally, RogCon has agreed to certain defined exclusivity obligations. The RogCon Agreement, unless earlier terminated, will continue until the latest of: (i) the expiration of all patent rights within RogCon patents, (ii) we certify we have abandoned the research, development and commercialization of product with no intention to re-establish such activities and (iii) no third party is obligated to pay any amounts that comprise net sublicense revenue. Either party may terminate the RogCon Agreement for material breach or insolvency of the other party. Additionally, we may terminate for convenience with prior written notice to RogCon. Upon termination by either party, all rights and licenses granted by RogCon to us will revert back to RogCon.

License Agreement with Purdue

In December 2017, we and Purdue Neuroscience Company, or Purdue, entered into a license agreement, or the Purdue Agreement, pursuant to which we were granted exclusive rights under certain Purdue know-how to research, develop and commercialize pharmaceutical products concerning a $GABA_A$ positive allosteric modulator. We are obligated to make future milestone payments based on the achievement of specified development and sales milestones up to $33.0 million. Additionally, under the Purdue Agreement, we were obligated to sell to Purdue $0.6 million of our Series B Preferred Stock in connection with our Series B financing. In addition, as consideration for the license obtained, we issued Purdue the anti-dilution obligation to ensure Purdue's ownership remained at a specified percentage throughout the Series B financing. Further, we are obligated to pay to Purdue a royalty percentage in the low single-digits of net sales of each licensed product for 12 years from the date of the first commercial sale of such product.

The Purdue Agreement will remain in effect until the expiration of our royalty obligation for all licensed products. Either us or Purdue may terminate the agreement in the event of a material breach by the other party and such party fails to cure such breach within a certain period of time. Either party may voluntarily terminate the agreement with prior notice. If the agreement is voluntarily terminated by Purdue, our license rights will survive such termination and such rights will become fully paid, perpetual and irrevocable. Purdue may also terminate in the event of our insolvency.

Ionis Collaboration Agreement

In September 2019, we and Ionis entered into the Ionis Agreement to discover and develop antisense oligonucleotides to treat forms of epilepsy caused by mutations of the SCN2A gene. Pursuant to the Ionis Agreement, we and Ionis will each conduct certain research activities and Ionis will be responsible for identifying a development candidate and conducting an IND-enabling toxicology study. The design of the IND-enabling toxicology study will be prepared and mutually agreed to by us and Ionis. We are obligated to reimburse any out-of-pocket costs incurred by Ionis related to research activities, identification of a development candidate and conducting IND-enabling studies. We hold an exclusive option, which we may exercise following the results of the IND-enabling toxicology study, to obtain the rights and license to further develop and commercialize a development candidate in the field of epilepsy and neurodevelopmental disorders, other than Dravet Syndrome. Upon the exercise of

such option, we will be required to pay Ionis a $2.0 million license fee. In addition, after option exercise, Ionis is eligible to receive certain contingent payments from us relating to development and other milestones, interest payments, royalties as a percentage of net product sales worldwide in the low-20s and any potential sublicense fees calculated as a percentage of sublicense revenue using a rate in the low-to-mid double digits.

Development milestones of $5.0 million for each product developed under the agreement are due upon the completion of the first clinical trial for each product, or the Development Milestone. Ionis will be entitled to receive an additional one-time milestone payment of $5.0 million, or the Additional Milestone, upon the earliest to occur of the following (each, a Payment Trigger): (i) the first acceptance of an NDA filing for a product by the regulatory authority in a major market, (ii) we have both (a) received, in the aggregate, $300.0 million in cash since September 11, 2019 and (b) initiated the first clinical study with respect to a product or (iii) the closing of a change of control event affecting Praxis. In addition, upon the occurrence of a Payment Trigger, Ionis is also entitled to certain interest payments equal to (i) 10% simple interest per annum calculated from the effective date of the agreement on the Additional Milestone, or the Initial Interest Amount, plus (ii) 10% simple interest per annum calculated from the date the Additional Milestone is paid on the initial Interest Amount, or the Second Interest Amount, until the earliest to occur of the following: (i) aggregate net sales of $100.0 million has been received, (ii) a change in control event affecting Praxis occurs or (iii) the Ionis Agreement has been terminated. Upon the occurrence of one of these three payment triggers, both the Initial Interest Amount and Second Interest Amount are due and payable to Ionis.

The Ionis Agreement will continue in full force and effect until the expiration of all payment obligations to Ionis, unless terminated earlier by either party. Either party may terminate the agreement upon material breach or insolvency of the other party or if Ionis is unable to identify a development candidate. Praxis is able to terminate the Ionis Agreement for convenience with prior written notice. Ionis may terminate if we fail to achieve certain performance milestones or Ionis' failure to identify a development candidate. Upon termination by us for convenience, we will stop selling all products, subject to certain wind-down provisions and all products will revert back to Ionis.

Manufacturing

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently source all of our nonclinical and clinical compound supply through third-party contract development and manufacturing organizations, or CDMOs.

For clinical supply, we use CDMOs who act in accordance with the FDA's current Good Manufacturing Practices, cGMP, for the manufacture of drug substance and product. We expect to rely on third parties for our manufacturing processes and the production of all clinical supply drug substance and drug product and currently expect to continue to do so for commercial supplies of our product candidates, if approved. We use additional contract manufacturers to fill, label, package, store and distribute our investigational drug products and currently expect to continue to do so for commercial supplies of our product candidates, if approved. It is our intent to identify and qualify additional manufacturers to provide active pharmaceutical ingredient and fill-and-finish services prior to submission of an NDA to the FDA for any product candidates that complete clinical development.

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of prescription drugs, such as those we are developing. These agencies regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export

and import of drug products and product candidates. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources.

U.S. government regulation of drug products

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The FDA also regulates biological products under the FDCA and the Public Health Service Act, or PHSA. If we advance clinical development of a biologic candidate in the future, these development activities will be subject to additional regulatory requirements specific to biologics. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties brought by the FDA and the Department of Justice, or DOJ, or other governmental entities.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- Submission to the FDA of an IND which must become effective before human clinical trials may begin;
- Approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;
- Performance of adequate and well-controlled human clinical trials in accordance with good clinical practice, or GCP, requirements to establish the safety and efficacy of the proposed drug product for each indication;
- Submission to the FDA of an NDA;
- Satisfactory completion of an FDA advisory committee review, if applicable;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practice, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- Satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- Payment of user fees and securing FDA approval of the NDA, including agreement to compliance with any post-approval requirements; and
- Compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical studies

Before testing any drug or biological product candidate, including our product candidates, in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies generally include laboratory evaluation of product chemistry, formulation and stability, as well as animal studies to assess potential toxicity, which support subsequent clinical testing. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any

available clinical data or literature and a proposed clinical protocol, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before human clinical trials may begin.

Clinical trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it initiates at that institution. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their www.clinicaltrials.gov website. Information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Sponsors are also obligated to disclose the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed in some cases for up to two years after the date of completion of the trial.

An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to initiate. Additionally, FDA will review any data from clinical trials conducted outside the United States when determining whether to allow an IND to proceed in the U.S. Specifically, FDA's acceptance of data from trials conducted outside of the U.S. is subject to certain conditions, including that the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with GCPs; the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful; and that the trials are conducted in compliance with all applicable U.S. laws and regulations. Clinical holds also may be imposed by the FDA at any time before or during studies due to safety concerns or non-compliance.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.
- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 trials may

not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution for a variety of reasons, including if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow up. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

Concurrent with clinical trials, companies usually complete additional animal studies and also must develop additional information about the chemistry and physical characteristics of the drug or biologic as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidates do not undergo unacceptable deterioration over their shelf life.

Marketing application submission and FDA review and approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has ten months from the date of "filing" of a standard NDA for a new molecular entity in which to complete its initial review and respond to the applicant, and six months from the filing date for priority applications. The FDA does not always meet its PDUFA goal dates, and the review process can be extended by FDA requests for additional information or clarification and a sponsor's process to respond to such inquiries. This FDA review typically takes twelve months from the date the NDA is submitted to FDA (for a standard review) because the FDA has approximately two months, or 60 days, after submission to make a "filing" decision on whether to accept an NDA for review.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review and informs the sponsor by the 74th day after the FDA's receipt of the submission whether an application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements.

Additionally, the FDA may refer any application to an advisory committee, including applications for novel drug candidates that present difficult questions of safety or efficacy. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. The FDA also may require submission of a risk evaluation and mitigation strategy, or REMS, plan, if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks and to assure the safe use of the drug product. The REMS plan could include medication guides, physician communication plans, assessment plans and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools. The FDA determines the requirement for a REMS, as well as the specific REMS provisions, on a case-by-case basis. If the FDA concludes a REMS plan is needed, the sponsor of the NDA must submit a proposed REMS. The FDA will not approve the NDA without a REMS, if required.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, as amended and reauthorized, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue either an approval letter or a complete response letter, or CRL. An approval letter authorizes commercial marketing of the drug with specific prescribing information and for specific indications. A CRL generally outlines the deficiencies in the submission and contains a statement of specific conditions that must be met in order to secure final approval of the NDA; it may require additional clinical or preclinical testing and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing in order for FDA to reconsider the application. If a CRL is issued, the applicant may choose to either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. If and when those deficiencies have been addressed to the FDA's satisfaction, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in response to an issued CRL in either two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan drug designation and exclusivity

Under the Orphan Drug Act, the FDA may designate a drug product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000

individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a drug product available in the United States for treatment of the disease or condition will be recovered from sales of the product). A company must request orphan product designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. If a drug or drug product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity. Orphan exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity.

Fast track, breakthrough therapy and priority review designations

The FDA is authorized to designate certain products for expedited development or review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs include fast track designation, breakthrough therapy designation and priority review designation.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life threatening disease or condition and demonstrates the potential to address unmet medical needs for the condition. Fast track designation provides opportunities for more frequent interactions with the FDA review team to expedite development and review of the product. The FDA may also review sections of the NDA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable and the sponsor pays any required user fees upon submission of the first section of the application. The sponsor can request the FDA to designate the product for fast track status any time before receiving NDA approval, but ideally no later than the pre-NDA meeting. Fast track designation may be withdrawn by the sponsor or rescinded by the FDA if the designation is no longer supported by data emerging in the clinical trial process.

In addition, with the enactment of FDASIA in 2012, Congress created a new regulatory program for product candidates designated by FDA as “breakthrough therapies” upon a request made by IND sponsors. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If the FDA designates a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy;

providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough therapy designation comes with all of the benefits of fast track designation, which means that the sponsor may file sections of the NDA for review on a rolling basis if certain conditions are satisfied, including an agreement with the FDA on the proposed schedule for submission of portions of the application and the payment of applicable user fees before the FDA may initiate a review. Finally, the FDA may designate a product for priority review if it is a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines at the time that the marketing application is submitted, on a case-by-case basis, whether the proposed drug represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting drug reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, or evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications and to shorten the FDA's goal for taking action on a marketing application from ten months to six months for an NDA for a new molecular entity from the date of filing. If criteria are not met for priority review, the application for a new molecular entity is subject to the standard FDA review period of ten months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or the time period for FDA review or approval may not be shortened. Furthermore, fast track designation, priority review and breakthrough therapy designation do not change the standards for approval and may not ultimately expedite the development or approval process.

Accelerated approval pathway

A drug product may also be eligible for accelerated approval if it is designed to treat a serious or life-threatening disease or condition and provides a meaningful therapeutic benefit over existing treatments. Such products can be approved on the basis of adequate and well-controlled clinical trials establishing an effect on either a surrogate endpoint that is reasonably likely to predict clinical benefit or on an intermediate clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity, or prevalence of the disease or condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials to verify and describe the predicted effect on IMM or other clinical endpoint. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

In addition, all promotional materials for products approved under the accelerated approval program are subject to prior review by the FDA, a requirement that could adversely impact the timing of the commercial launch of the product. FDA may withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product.

U.S. marketing and data exclusivity

Market exclusivity provisions under the FDCA can delay the submission or the approval of certain follow-on drug applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an Abbreviated New Drug Application, or ANDA, or a 505(b)(2) NDA submitted by another company for a generic or follow-on version of the protected drug product, respectively, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement.

The FDCA also provides three years of data exclusivity when an NDA or a supplement to an existing NDA, includes new clinical investigations, other than bioavailability studies, conducted or sponsored by the applicant that are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs or follow-on 505(b)(2) NDAs if the protected clinical data are not referenced. Five-year and three-year exclusivity will not delay the submission or approval of a stand-alone NDA submitted under section 505(b)(1) of the FDCA. However, an applicant submitting a stand-alone NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness, if applicable.

Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods. This six-month exclusivity may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued “Written Request” for such a trial.

Patent term restoration

Depending upon the timing, duration and specifics of FDA approval of our drug product candidates, some of our U.S. patents may be eligible for limited patent term extension. These patent term extensions permit a patent restoration term of up to five years as compensation for any patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the drug product’s approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the extension must be applied for prior to expiration of the patent. The United States Patent and Trademark Office, or the USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Post-approval requirements

Following approval of a new prescription drug product, the manufacturer and the approved drug are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to monitoring and recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs must be promoted by a manufacturer and any third parties acting on behalf of a manufacturer only for the approved indications and in a manner consistent with the approved label for the product. The FDA and other agencies actively enforce the laws and regulations prohibiting the

promotion of off-label uses and a company that is found to have improperly promoted off-label uses may be subject to significant liability. After approval, most changes to the approved product, such as adding new indications or other labeling claims or changes to the manufacturing processes or facilities, are subject to prior FDA review and approval. There are continuing, annual user fee requirements for any marketed products and the establishments where such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- Restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- Fines, warning letters or other enforcement-related letters or clinical holds on post-approval clinical trials;
- Refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or suspension or revocation of product approvals;
- Product seizure or detention, or refusal to permit the import or export of products; and
- Injunctions or the imposition of civil or criminal penalties.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution. From time to time, new legislation and regulations may be implemented that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. It is impossible to predict whether further legislative or regulatory changes will be enacted, or FDA regulations, guidance or interpretations changed or what the impact of such changes, if any, may be.

Other healthcare laws and regulations

Healthcare providers, physicians and third party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug products for which we obtain marketing approval. Our current and future arrangements with third party payors, customers, healthcare providers, physicians and others, in connection with the clinical research, sales, marketing and promotion of products, once approved, and related activities, may expose a pharmaceutical

manufacturer to broadly applicable fraud and abuse and other healthcare laws and regulations. In particular, the research of our product candidates, as well as the promotion, sales and marketing of healthcare items and services and certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business or financial arrangements.

The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include, but are not limited to:

- the U.S. federal Anti-Kickback Statute, which makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual, or the purchase, lease, order, arrangement, or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. The term remuneration has been interpreted broadly to include anything of value. Further, courts have found that if “one purpose” of renumeration is to induce referrals, the federal Anti-Kickback statute is violated. Violations are subject to significant civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment and exclusion from government healthcare programs. In addition, a claim submitted for payment to any federal healthcare program that includes items or services that were made as a result of a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act, or FCA. The Anti-Kickback Statute has been interpreted to apply to arrangements between biopharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers, among others, on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;
- the U.S. federal civil and criminal false claims laws, including the U.S. False Claims Act, or FCA, which can be enforced through “qui tam” or “whistleblower” actions, and civil monetary penalty laws, which impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false, fictitious or fraudulent; knowingly making, using, or causing to be made or used, a false record or statement material to a false, fictitious or fraudulent claim or an obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing or concealing such an obligation. to pay money to the federal government. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation. A claim that includes items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim under the FCA. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. When an entity is determined to have violated the FCA, the government may impose civil fines and penalties for each false claim, plus treble damages, and exclude the entity from participation in Medicare, Medicaid and other federal healthcare programs;
- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or

attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statement or representation, or making or using any false writing or document knowing the same to contain any materially false fictitious or fraudulent statement or entry in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA fraud provisions without actual knowledge of the statute or specific intent to violate it;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements relating to the privacy, security and transmission of individually identifiable health information on certain covered healthcare providers, health plans and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the creation, use, receipt, maintenance or disclosure of individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, there may be additional federal, state and non-U.S. laws which govern the privacy and security of health and other personal information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts;
- the U.S. federal Physician Payments Sunshine Act, created under Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the Centers for Medicare and Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS, under the Open Payments Program, information related to direct or indirect payments and other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made during the previous year to certain non-physician providers such as physician assistants and nurse practitioners;
- U.S. federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous U.S. state, local and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and other relevant compliance guidance promulgated by the federal government that otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts,

compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information, some of which may be more stringent than those in the United States (such as the European Union, which adopted the General Data Protection Regulation, which became effective in May 2018) in certain circumstances, and may differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. Efforts to ensure that business arrangements comply with applicable healthcare laws involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting its rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, imprisonment, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, reporting obligations and oversight if we become subject to integrity and oversight agreements to resolve allegations of non-compliance, contractual damages, reputational harm, diminished profits and future earnings and curtailment of operations, any of which could adversely affect our ability to operate our business and the results of operations. In addition, commercialization of any drug product outside the United States will also likely be subject to foreign equivalents of the healthcare laws mentioned above, among other foreign laws. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business with are found to be not in compliance with applicable laws, they may be subject to similar penalties.

Other data privacy and security laws

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws and federal and state consumer protection laws, govern the collection, use, disclosure and protection of health-related and other personal information outside of HIPAA and its implementing regulations. For example, in June 2018, the State of California enacted the California Consumer Privacy Act of 2018, or the CCPA, which came into effect on January 1, 2020 and provides new data privacy rights for consumers and new operational requirements for companies, which may increase our compliance costs and potential liability. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. While there is currently an exception for protected health information that is subject to HIPAA and clinical trial regulations, as currently written, the CCPA may impact certain of our business activities. The CCPA could mark the beginning of a trend toward more stringent state privacy legislation in the United States, which could increase our potential liability and adversely affect our business.

In the event we decide to conduct clinical trials or continue to enroll subjects in our ongoing or future clinical trials, we may be subject to additional privacy restrictions imposed by other countries and jurisdictions. The collection, use, storage, disclosure, transfer, or other processing of personal data regarding individuals in the European Economic Area, or EEA, including personal health data, is subject to the EU General Data Protection Regulation, or GDPR, which became effective in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals

regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR includes restrictions on cross-border data transfers. The GDPR may increase our responsibility and liability in relation to personal data that we process where such processing is subject to the GDPR, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, including as implemented by individual countries. Compliance with the GDPR will be a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with our European activities. Further, the United Kingdom's decision to leave the EU, often referred to as Brexit, has created uncertainty with regard to data protection regulation in the United Kingdom. In particular, it is unclear how data transfers to and from the United Kingdom will be regulated now that the United Kingdom has left the EU.

Current and future healthcare reform legislation

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and devices and to spur innovation, but its ultimate implementation is uncertain. More recently, in August 2017, the FDA Reauthorization Act was signed into law to reauthorize the FDA's user fee programs and included additional drug and device provisions that build on the Cures Act.

In addition, in both the United States and certain foreign jurisdictions, there have been, and likely will continue to be, a number of legislative and regulatory changes regarding the health care system directed at broadening the availability of healthcare, improving the quality of healthcare and containing or lowering the cost of healthcare. For example, in March 2010 the ACA was enacted, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacted the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, expands the types of entities eligible for the 340B drug discount program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations; established annual fees and taxes on manufacturers of certain branded prescription drugs; and created a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (increased to 70% pursuant to the Bipartisan Budget Act of 2018, or BBA, effective as of January 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since its enactment, there remain judicial, administrative, executive and legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Various portions of the ACA are currently undergoing legal and constitutional challenges in the United States Supreme Court; the Trump Administration has issued various Executive Orders which eliminated cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare

providers, health insurers, or manufacturers of pharmaceuticals or medical devices; and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the ACA. Also, in December 2018, CMS issued a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program. Since then, the ACA risk adjustment program payment parameters have been updated annually. It is unclear whether the ACA will be overturned, repealed, replaced, or further amended.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs, including aggregate reductions of Medicare payments to providers of up to 2% per fiscal year. These reductions went into effect on April 1, 2013, and, due to subsequent legislative amendments, will remain in effect through 2030 unless additional Congressional action is taken. However, pursuant to the Coronavirus Aid, Relief and Economic Security Act, or CARES Act, these Medicare sequester reductions under the Budget Control Act of 2011 will be suspended from May 1, 2020 through December 31, 2020 due to the COVID-19 pandemic. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, the BBA, among other things, also amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount (from 50% under the ACA to 70%) that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole".

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. There also has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. For example, at the federal level, on March 10, 2020, the Trump administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses and place limits on pharmaceutical price increases. Moreover, the U.S. Presidential administration previously released a "Blueprint" to lower drug prices and reduce out-of-pocket costs of drugs that contained proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. HHS has solicited feedback on some of these measures and has implemented others under its existing authority. For example, in May 2019, CMS issued a final rule that would allow Medicare Advantage Plans the option of using step therapy, a type of prior authorization, for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019. Although a number of these and other measures may require additional authorization to become effective, Congress and the U.S. Presidential administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers.

At the state level, legislatures in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

In the European Union and in other foreign jurisdictions, similar political, economic and regulatory developments may affect our ability to profitably commercialize our products. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or EU member state level may result in significant additional requirements or obstacles that may increase our operating costs.

Legislative and regulatory proposals, and enactment of laws, at the foreign, federal and state levels, directed at containing or lowering the cost of healthcare, will likely continue into the future.

Rest of world regulation

For other countries outside of the European Union and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing product development, the conduct of clinical trials, manufacturing, distribution, marketing approval, advertising and promotion, product licensing, pricing and reimbursement vary from country to country. Additionally, clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Additionally, to the extent that any of our product candidates, once approved, are sold in a foreign country, we may be subject to applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws and implementation of corporate compliance programs and reporting of payments or other transfers of value to healthcare professionals.

Additional laws and regulations governing international operations

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered by U.S. authorities that enforce the FCPA, including the Department of Justice, to be foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Coverage, pricing and reimbursement

Significant uncertainty exists as to the coverage, pricing and reimbursement status of any products seeking regulatory approval. Successful commercialization of new drug products depends in part on the extent to which coverage and reimbursement for those drug products will be available from government health administration authorities, private health insurers and other organizations. The availability and extent of reimbursement depend substantially, both domestically and abroad, on the extent to which the costs of drugs products are paid for by third-party payors, such as government health care programs (e.g., Medicare, Medicaid), health maintenance organizations, managed care providers, pharmacy benefit and similar healthcare management organizations, private health coverage insurers and other third-party payors. These third-party payors decide which medications they will pay for and will establish reimbursement levels.

In the United States, the principal decisions about reimbursement for new drug products are typically made by CMS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. Factors payors consider in determining reimbursement are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products can differ significantly from payor to payor. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the reimbursement rate that the payor will pay for the product. One payor's determination to provide coverage for a product does not assure that other payors will also provide coverage and reimbursement for the product. Third-party payors may also limit coverage to specific products on an approved list, or formulary, which might not include all of the FDA-approved products for a particular indication.

Various federal laws may impact the extent of coverage and reimbursement status provided by government health care programs. For instance, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. While all Medicare drug plans must give at least a standard level of coverage set by

Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each Part D prescription drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for drugs for which we obtain marketing approval. Any negotiated prices for any of our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the average manufacturer price, or AMP, and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although under the current state of the law these newly eligible entities (with the exception of children's hospitals) will not be eligible to receive discounted 340B pricing on orphan drugs. As 340B drug pricing is determined based on average manufacturer price, or AMP, and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase. The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health and periodic reports on the status of the research and related expenditures are made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidates, if any such drug or the condition that they are intended to treat are the subject of a trial.

It is also possible that comparative effectiveness research, whether conducted by government or private entities, demonstrating benefits in a competitor's drug could adversely affect the sales of our product candidate. If third-party payors do not consider our drugs to be cost-effective compared to other available therapies, they may not cover our drugs after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis.

In addition to the above-mentioned laws, increasing efforts by third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates, if approved, may nonetheless not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third-party payors in connection with the potential sale of any of our product candidates

Outside of the United States, the pricing of pharmaceutical products and medical devices is subject to governmental control in many countries. For example, in the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products and medical devices will likely continue as countries attempt to manage healthcare expenditures. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower.

Employees

As of September 30, 2020, we had 50 full-time employees and one part-time employee. Of our 51 employees, 24 have Ph.D. or M.D. degrees and 37 are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Facilities

We sublease a facility containing 6,374 square feet of office space, which is located at One Broadway, Cambridge, Massachusetts 02142. Our sublease expires on December 31, 2021. We believe that our current facilities are sufficient to meet our current and near-term needs and that, should it be needed, suitable additional space will be available.

Legal Proceedings

As of the date of this prospectus, we are not party to any material legal matters or claims. We may become party to legal matters and claims arising in the ordinary course of business. We cannot predict the outcome of any such legal matters or claims, and despite the potential outcomes, the existence thereof may have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

MANAGEMENT

Executive Officers and Directors

The following table sets forth the name, age, and position of each of our current executive officers and directors as of September 30, 2020:

Name	Age	Position
Executive Officers:		
Marcio Souza	41	President, Chief Executive Officer, Director
Bernard Ravina, M.D.	53	Chief Medical Officer
Stuart Chaffee, Ph.D.	47	Chief Financial Officer
Alex Nemiroff, J.D.	41	General Counsel, Secretary
Nicole Sweeny	45	Chief Commercial Officer
Non-Employee Directors:		
Dean Mitchell(2)	64	Chairman, Director
Nicholas Galakatos, Ph.D.(2)(3)	62	Director
Gregory Norden(1)	62	Director
Ari Brettman, M.D.(4)	38	Director
Thomas Dyrberg, M.D.(4)	65	Director
Kiran Reddy, M.D.	43	Director
Stefan Vitorovic(1)(3)	35	Director
William Young(1)(2)(3)	76	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

(3) Member of the nominating and corporate governance committee.

(4) Each of Dr. Brettman and Dr. Dyrberg resigned from our board of directors effective immediately prior to the effectiveness of the registration statement of which this prospectus forms a part.

Executive Officers

Marcio Souza has served as a director and our President and Chief Executive Officer since April 2020. Prior to joining us, Mr. Souza was at PTC Therapeutics, Inc., or PTC, where he served as its Chief Operating Officer from May 2017 to April 2020 and its Senior Vice President and Head of Product Strategy from July 2016 to May 2017. Prior to joining PTC, Mr. Souza served in positions of increasing responsibility at NPS Pharmaceuticals, Inc., Shire Human Genetic Therapies Inc. and Sanofi Genzyme Corporation. From May 2019 to May 2020, Mr. Souza also served on the board of directors of Clearpoint Neuro, Inc. (NASDAQ: CLPT) (previously MRI Interventions, Inc.). Mr. Souza received a degree in pharmacy and biochemistry with a specialization in toxicology and clinical analysis from the University of São Paulo and an M.B.A. from Fundação Dom Cabral. We believe Mr. Souza is qualified to serve on our board of directors because of his business and leadership experience in the life sciences industry and his scientific background.

Bernard Ravina, M.D., has served as our Chief Medical Officer since August 2018. Prior to joining us, Dr. Ravina was at Voyager Therapeutics, Inc. (NASDAQ: VYGR), where he served as Chief Medical Officer from February 2017 to August 2018 and as Vice President of Clinical Development

from March 2014 to January 2017. Dr. Ravina was also Medical Director in Clinical Development at Biogen Inc. (NASDAQ: BIIB), or Biogen, from 2010 to 2014, where he worked on both small molecule drugs and biologics for the treatment of neurological disorders. From 2005 to 2010, Dr. Ravina was an Associate Professor of Neurology, Director of the Movement and Inherited Neurological Disorders Unit, Associate Director of Clinical Trials Coordination Center and Vice Chair of Neurology at the University of Rochester School of Medicine. Dr. Ravina received a B.A. in psychology from Columbia University, an M.D. from Johns Hopkins University School of Medicine and an M.S.C.E. in clinical epidemiology from the University of Pennsylvania where he completed his residency and fellowship training in Neurology.

Stuart Chaffee, Ph.D., has served as our Chief Financial Officer since June 2020. Prior to his role as Chief Financial Officer, Dr. Chaffee served as our Chief Business Officer from November 2017 to June 2020. Dr. Chaffee has an extensive background in drug discovery and development, including as an Entrepreneur in Residence at Atlas Venture from November 2015 to November 2017 where he was a co-founder and the Head of Business Operations at Kymera Therapeutics, Inc. from June 2016 to November 2017. From 2014 to 2015, Dr. Chaffee served as Senior Director of Corporate Development at Biogen. Dr Chaffee received a B.S. in chemistry from The College of William and Mary, a Ph.D. in chemistry from Yale University and an M.B.A. in finance from the Wharton School of the University of Pennsylvania.

Alex Nemiroff, J.D., has served as our General Counsel since June 2020. Prior to his role as General Counsel, Mr. Nemiroff served as our VP of Legal from January 2020 to June 2020. Mr. Nemiroff was also a co-founder of RogCon, Inc. and RogCon U.R., Inc., and he has served as both entities' Chief Executive Officer since inception in November 2015. Mr. Nemiroff has experience working in commercial and securities litigation while at Greenberg Traurig LLP, and served as law clerk to the Honorable Paul C. Huck of the United States District Court for the Southern District of Florida. Mr. Nemiroff received a B.B.A from the University of Michigan's Ross School of Business, and a J.D. from Northwestern University School of Law.

Nicole Sweeny has served as our Chief Commercial Officer since August 2020. Prior to joining us, Ms. Sweeny was at Takeda Pharmaceuticals (NYSE: TAK) where she served as a Vice President, Franchise Head, Rare Diseases from February 2019 to July 2020. Prior to Takeda, Ms. Sweeny served in several roles at Shire Pharmaceuticals plc (later acquired by Takeda Pharmaceuticals Company Limited) from August 2010 to January 2019, including Vice President, Head of US Marketing from September 2017 to January 2019 and Vice President, Global Product Strategy Lead from December 2016 to August 2017. Prior to joining Shire, Ms. Sweeny served in commercial positions of increasing responsibility at AMAG Pharmaceuticals and Sanofi Genzyme Corporation. Ms. Sweeny received her B.S. from Boston College.

Non-Employee Directors

Dean Mitchell has served as chairman of our board of directors since September 2020. He served as executive chairman of the board of directors of Covis Pharma Holdings, a specialty pharmaceutical company, from August 2013 until its sale in March 2020 and was chairman of PaxVax Corporation from January 2016 until its sale in October 2018. Mr. Mitchell served as President and Chief Executive Officer of Lux Biosciences, Inc., a biotechnology company focusing on the treatment of ophthalmic diseases, from July 2010 to August 2013. Prior to Lux Biosciences, he served as President and Chief Executive Officer of both Alpharma, Inc., a publicly traded specialty pharmaceutical company, from 2006 until its acquisition by King Pharmaceuticals, Inc. in 2008, and Guilford Pharmaceuticals, Inc., a publicly traded pharmaceutical company focused in oncology and acute care, from 2004 until its acquisition by MGI Pharma Inc. in 2005. From 2001 to 2004, he served in various senior executive capacities in the worldwide medicines group of Bristol-Myers Squibb Company, a pharmaceutical company. Prior to the Bristol-Myers Squibb Company, he spent 14 years at GlaxoSmithKline plc, in

assignments of increasing responsibility spanning sales, marketing, general management, commercial strategy and clinical development and product strategy. Mr. Mitchell currently serves on the board of directors of Theravance Biopharma, Inc. (NASDAQ: TBPH), ImmunoGen Inc. (NASDAQ: IMGN) and Precigen Inc. (formerly Intrexon Inc.) (NASDAQ: PGEN). Mr. Mitchell holds an M.B.A. from City University London and a B.Sc. in biology from Coventry University. We believe Mr. Mitchell is qualified to serve on our board of directors because of his management experience in the pharmaceutical and biotherapeutics industries and his experience as a President, Chief Executive Officer and board member of multiple biotechnology companies.

Nicholas Galakatos, Ph.D., has served as a member our board of directors since September 2015. Dr. Galakatos is the Global Head of Life Sciences of The Blackstone Group Inc., or Blackstone. Prior to joining Blackstone, Dr. Galakatos was a co-Founder and Managing Director of Clarus Ventures, LLC (acquired by Blackstone in 2018), or Clarus, since the firm's inception in 2005. Dr. Galakatos is currently the chairman of the board of directors of Anthos Therapeutics, Inc., or Anthos, a private, clinical-stage cardiovascular biotech founded in 2019, and a member of the board of directors of Talaris, Inc. He is a member of the Director's Council of the Koch Institute at MIT and a member of the Board of Trustees at Reed College. Dr. Galakatos received a B.A. in chemistry from Reed College and a Ph.D. in organic chemistry from the Massachusetts Institute of Technology. We believe Dr. Galakatos is qualified to serve on our board of directors because of his business and leadership experience in the life sciences industry and his scientific background.

Gregory Norden is the former Chief Financial Officer of Wyeth and has served as a member of our board of directors since March 2019. Mr. Norden currently serves as the Managing Director of G9 Capital Group LLC, which invests in early stage ventures and provides corporate finance advisory services, since 2010. Mr. Norden currently serves on the boards of directors of Zoetis (NYSE: ZTS), the leading animal health company, NanoString Technologies (NASDAQ: NSTG), a leading provider of life science tools for translational research, Royalty Pharma (NASDAQ: RPRX), a leading funder of innovation across the biopharmaceutical industry, and Univision Communications, the leading multimedia company serving Hispanic America. Mr. Norden is a former director of Human Genome Sciences, Welch Allyn and Entasis Therapeutics. Mr. Norden received a B.S. in management and economics from the State University of New York at Plattsburgh and an M.S. in accounting from Long Island University—C.W. Post. We believe Mr. Norden is qualified to serve on our board of directors because of his background in finance and experience as a senior executive in the global healthcare and pharmaceutical industries, as well as his public company board experience.

Ari Brettman, M.D., one of our co-founders, has served as a member of our board of directors since March 2018. Dr. Brettman is also currently a Managing Director at Blackstone, a position he has held since January 2020. Previously, Dr. Brettman served as a Principal at Blackstone from January 2017 to December 2019, and as an Associate from September 2014 to December 2016. Additionally, Dr. Brettman is a co-founder of Anthos Therapeutics, Inc., or Anthos, and served as Anthos's Chief Medical Officer until July 2019. Dr. Brettman received an A.B. in history and science from Harvard College and an M.D. from Duke University. Dr. Brettman resigned from our board of directors effective immediately prior to the effectiveness of the registration statement of which this prospectus forms a part.

Thomas Dyrberg, M.D., has served as a member of our board of directors since March 2018. In December 2000, Dr. Dyrberg joined Novo Holdings A/S, or Novo, then known as Novo A/S, a Danish limited liability company that manages investments and financial assets, where he was employed as Senior Partner. Since August 2015, Dr. Dyrberg has been employed as a Managing Partner of Novo. From August 2007 to May 2019, Dr. Dyrberg was a member of the board of directors of Ophthotech Corp. (now IVERIC bio, Inc. (NASDAQ: ISEE)). Dr. Dyrberg previously served on the board of directors of Veloxis A/S, a publicly traded specialty pharmaceutical company. Dr. Dyrberg has held research

positions at the Hagedorn Research Institute in Denmark and at the Scripps Research Institute in California. Dr. Dyrberg received a D.M.Sc. and an M.D. from the University of Copenhagen. Dr. Dyrberg resigned from our board of directors effective immediately prior to the effectiveness of the registration statement of which this prospectus forms a part.

Kiran Reddy, M.D., has served as a member of our board of directors since September 2015. Prior to his role as our director, Dr. Reddy served as our President and Chief Executive Officer from November 2015 to April 2020. Dr. Reddy is also currently a Managing Director at Blackstone, a position he has served in since May 2020. Dr. Reddy was a venture partner at Clarus from November 2015 to November 2019 prior to its acquisition by Blackstone. From 2014 to 2015, Dr. Reddy served as part of Biogen's Corporate Strategy leadership team, where he focused on sourcing new technologies and product opportunities to support the Company's growth. Dr. Reddy was previously a Howard Hughes science fellow and has authored several peer-reviewed scientific papers in the field of epilepsy, neuroimmunology and neurodegenerative diseases. Dr. Reddy received a B.S. in economics, an M.D. and an M.B.A. from Georgetown University. We believe Dr. Reddy is qualified to serve on our board of directors because of his corporate leadership experience, business background, and perspective and experience as one of Praxis' former executive officers.

Stefan Vitorovic has served as a member of our board of directors since March 2018 . Mr. Vitorovic is the co-founder and Managing Director of Vida Ventures, LLC, or Vida Ventures, a role he has served in since January 2017. Prior to Vida Ventures, Mr. Vitorovic was a Principal at Third Rock Ventures, where he was employed from July 2014 to January 2017. Prior to Third Rock Ventures, Mr. Vitorovic was a healthcare private equity investor at TPG Capital from August 2012 to June 2014. Mr. Vitorovic received a B.S. with honors in molecular & cellular biology and an M.S. in molecular & cellular biology from Stanford University as well as an M.B.A from Harvard University. We believe Mr. Vitorovic is qualified to serve on our board of directors because of his scientific background and business experience.

William Young has served as a member of our board of directors since December 2016. Mr. Young is a Senior Advisor with Blackstone. Prior to its acquisition by Blackstone, Mr. Young joined Clarus in March 2010 and held various roles, including Venture Partner, Senior Advisor and portfolio company board member. Mr. Young currently serves as the chairman of the board of directors of Annexon, Inc. (NASDAQ: ANNX) and NanoString, and as a member of the board of directors of Theravance BioPharma Inc. (NASDAQ: TBPH). Mr. Young also served on the boards of directors of Vertex Pharmaceuticals Inc. (NASDAQ: VRTX) from May 2015 to June 2020 and BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) from September 2010 to November 2015. Mr. Young was elected to the National Academy of Engineering in 1993 for his contributions to biotechnology. Mr. Young received a B.S. in chemical engineering from Purdue University and an M.B.A. from Indiana University in marketing and finance and holds an honorary doctorate in engineering from Purdue University. We believe Mr. Young is qualified to serve on our board of directors because of his scientific background, business experience and his service on the board of directors of other life sciences companies

Family Relationships

There are no family relationships among any of our executive officers or directors.

Composition of Our Board of Directors

Our board of directors currently consists of nine members, each of whom are members pursuant to the board composition provisions of our amended and restated certificate of incorporation and agreements with our stockholders, which agreements are described under "Certain Relationships and Related Party Transactions." These board composition provisions will terminate upon the closing of this offering. Upon the termination of these provisions, there will be no further contractual obligations

regarding the election of our directors. Our nominating and corporate governance committee and our board of directors may therefore consider a broad range of factors relating to the qualifications and background of nominees, which may include diversity, which is not only limited to race, gender or national origin. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is identification of persons who will further the interests of our stockholders through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape and professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least 66.67% of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Director Independence

The Nasdaq Stock Market LLC, or Nasdaq, Marketplace Rules, or the Nasdaq Listing Rules, require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq Listing Rules require that, subject to specified exceptions and phase in periods following the initial public offering, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under the Nasdaq Listing Rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee: (1) accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries; or (2) otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting, advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In September 2020, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of , is an "independent director" as defined under the Nasdaq Listing Rules. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our

board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director.

Staggered Board

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering, the number of our directors shall be fixed from time to time by a resolution of the majority of our board of directors and our board of directors will be divided into three staggered classes of directors and each director will be assigned to one of the three classes. At each annual meeting of the stockholders, a class of directors will be elected for a three-year term to succeed the directors of the same class whose terms are then expiring. The terms of the directors will expire upon the election and qualification of successor directors at the annual meeting of stockholders to be held during the years 2021 for Class I directors, 2022 for Class II directors and 2023 for Class III directors.

- Our Class I director will be Dean Mitchell;
- Our Class II directors will be Nicholas Galakatos, Ph.D., Kiran Reddy, M.D., and Stefan Vitorovic; and
- Our Class III directors will be Gregory Norden, Marcio Souza and William Young.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control. See the “Description of Capital Stock—Anti-Takeover Effects of Delaware Law, Our Amended and Restated Certificate of Incorporation and Our Amended and Restated By-Laws” section of this prospectus for a discussion of these and other anti-takeover provisions found in our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the closing of this offering. We expect that additional directorships resulting from an increase in the number of directors, if any, will be distributed among the three classes so that, as nearly as possible, each class shall consist of one third of the board of directors.

Board Leadership Structure and the Role of the Board in Risk Oversight

Board Leadership Structure

The positions of our chairman of the board and chief executive officer are separated, with Mr. Souza serving as our Chief Executive Officer and Mr. Mitchell serving as the chairman of our board of directors. Separating these positions allows Mr. Souza, as our Chief Executive Officer, to focus on our day-to-day business, while allowing the chairman of the board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that Mr. Souza, as our Chief Executive Officer, must devote to his position in the current business environment, as well as the commitment required to serve as our chairman, particularly as the board of directors’ oversight responsibilities continue to grow. Our board of directors also believes that this structure ensures a greater role for the independent directors in the oversight of our company and active participation of the independent directors in setting agendas and establishing priorities and procedures for the work of our board of directors. Our board of directors believes its administration of its risk oversight function has not affected its leadership structure. Our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

Role of the Board in Risk Oversight

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including those described under the section titled

"Risk Factors" in this prospectus. Our board of directors is actively involved in oversight of risks that could affect us. This oversight is conducted primarily by our full board of directors, which has responsibility for general oversight of risks.

Following the closing of this offering, our board of directors will satisfy this responsibility through full reports by each committee chair regarding the committee's considerations and actions, as well as through regular reports directly from officers responsible for oversight of particular risks within our company. Our board of directors believes that full and open communication between management and the board of directors is essential for effective risk management and oversight.

Committees of Our Board of Directors

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee, each of which will operate pursuant to a charter adopted by our board of directors and that became effective upon the effectiveness of the registration statement of which this prospectus is a part. Our board of directors may establish other committees to facilitate the management of our business. The composition and functioning of all of our committees comply with all applicable requirements of the Sarbanes-Oxley Act of 2002, Nasdaq and U.S. Securities and Exchange Commission rules and regulations. Members will serve on these committees until their resignation or until otherwise determined by our board of directors. In addition, from time to time, special committees may be established under the direction of our board of directors when necessary to address specific issues.

Audit Committee

Effective upon the completion of this offering, Gregory Norden, Stefan Vitorovic and William Young will serve on the audit committee, which will be chaired by Gregory Norden. Our board of directors has determined that Gregory Norden, Stefan Vitorovic and William Young are "independent" for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and each has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated Gregory Norden as an "audit committee financial expert," as defined under the applicable rules of the SEC. The audit committee's responsibilities upon the closing of this offering include:

- appointing, approving the compensation of and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee's review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;

- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

Compensation Committee

Effective upon the completion of this offering, Nicholas Galakatos, Dean Mitchell and William Young will serve on the compensation committee, which will be chaired by William Young. Our board of directors has determined that Nicholas Galakatos, Dean Mitchell and William Young are independent" as defined in the applicable Nasdaq rules. The compensation committee's responsibilities upon the closing of this offering include:

- annually reviewing and approving corporate goals and objectives relevant to the compensation of our chief executive officer;
- evaluating the performance of our chief executive officer in light of such corporate goals and objectives and determining the compensation of our chief executive officer;
- reviewing and approving the compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- retaining and approving the compensation of any compensation advisors;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- evaluating director compensation and making recommendations on director compensation to the Board;
- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention or termination of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

Nominating and Corporate Governance Committee

Effective upon the completion of this offering, Nicholas Galakatos, Stefan Vitorovic and William Young will serve on the nominating and corporate governance committee, which will be chaired by Nicholas Galakatos. Our board of directors has determined that Nicholas Galakatos, Stefan Vitorovic and William Young are "independent" as defined in the applicable Nasdaq rules. The nominating and corporate governance committee's responsibilities upon the closing of this offering include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the size and composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;

- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines; and
- overseeing the evaluation of our board of directors and management.

Our board of directors may from time to time establish other committees.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee. For a description of transactions between us and members of our compensation committee and affiliates of such members, please see "Certain Relationships and Related Party Transactions."

Code of Business Conduct and Ethics

We plan to adopt a written code of business conduct and ethics that applies to all of our employees, officers and directors, including those officers responsible for financial reporting, which will become effective upon the closing of this offering. Upon the closing of this offering, our code of business conduct and ethics will be available on the Corporate Governance section of our website at https://praxismedicines.com. We intend to disclose any amendments to the code, or any waivers of its requirements, on our website or in a Current Report on Form 8-K as may be required by SEC or Nasdaq rules. We do not incorporate the information on or accessible through our website into this prospectus, and you should not consider any information on, or that can be accessed through, our website as part of this prospectus.

EXECUTIVE COMPENSATION

Executive Compensation Overview

The following discussion contains forward looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. The actual amount and form of compensation and the compensation policies and practices that we adopt in the future may differ materially from currently planned programs as summarized in this discussion.

As an emerging growth company, we have opted to comply with the executive compensation disclosure rules applicable to "smaller reporting companies," as such term is defined in the rules promulgated under the Securities Act of 1933, as amended, or the Securities Act. The compensation provided to our named executive officers for the fiscal year ended December 31, 2019 is detailed in the 2019 Summary Compensation Table and accompanying footnotes and narrative that follow. Our named executive officers for the fiscal year ended December 31, 2019 were:

- Kiran Reddy, M.D., our former President and Chief Executive Officer;
- Stuart Chaffee, Ph.D., our current Chief Financial Officer and former Chief Business Officer; and
- Bernard Ravina, M.D., our Chief Medical Officer.

In April 2020, Dr. Reddy resigned as our President and Chief Executive Officer and Marcio Souza became our President and Chief Executive Officer.

To date, the compensation of our named executive officers has consisted of a combination of base salary, bonuses and long-term incentive compensation in the form of stock options. Our named executive officers, like all full-time employees, are eligible to participate in our health and welfare benefit plans. As we transition from a private company to a publicly traded company, we intend to evaluate our compensation values and philosophy and compensation plans and arrangements as circumstances require.

2019 Summary Compensation Table

The following table sets forth information regarding compensation awarded to, earned by or paid to our named executive officers for services rendered to us in all capacities during the fiscal year ended December 31, 2019.

Name and Principal Position	Year	Salary ($)	Bonus ($)(1)	Stock Awards ($)	Option Awards ($)	Non-Equity Incentive Plan Compensation ($)(2)	All Other Compensation ($)(3)	Total ($)
Kiran Reddy, M.D. *Former President and Chief Executive Officer(4)*	2019	385,500	–	–	–	190,823	269	576,592
Stuart Chaffee, Ph.D. *Chief Financial Officer and former Chief Business Officer(5)*	2019	308,250	–	–	–	83,228	297	391,775
Bernard Ravina, M.D. *Chief Medical Officer*	2019	350,000	75,000	–	–	126,000	271	551,271

(1) Amount represents a $75,000 bonus paid to Dr. Ravina pursuant to the terms of his offer letter with us.

(2) Amounts represent annual cash bonuses paid based on achievement of pre-determined corporate performance metrics in 2019, which were paid in March 2020. The corporate performance metrics were achieved at 90% of target.
(3) Amounts represent tax gross-ups on taxable long-term disability and commuter benefits in the following amounts: Dr. Reddy—$65 for long-term disability benefits and $204 for commuter benefits; Dr. Chaffee—$92 for long-term disability benefits and $205 for commuter benefits; and Dr. Ravina—$66 for long-term disability benefits and $205 for commuter benefits.
(4) Dr. Reddy resigned as our President and Chief Executive Officer effective April 20, 2020.
(5) Effective as of May 28, 2020, Dr. Chaffee was appointed Chief Financial Officer.

Narrative to the 2019 Summary Compensation Table

Base Salaries

Each named executive officer's base salary is a fixed component of annual compensation for performing specific duties and functions, and has been established by our board of directors taking into account each individual's role, responsibilities, skills and expertise. Base salaries are reviewed annually, typically in connection with our annual performance review process, approved by our board of directors and adjusted from time to time to realign salaries with market levels after taking into account individual responsibilities, performance and experience. During 2019, the annual base salaries for Dr. Reddy, Dr. Chaffee, and Dr. Ravina, were $385,500, $308,250 and $350,000, respectively.

Annual Bonus

For the fiscal year ended December 31, 2019, each of our named executive officers was eligible to earn an annual bonus based on the achievement of certain pre-determined corporate performance objectives. During 2019, the target annual bonuses for Dr. Reddy, Dr. Chaffee, and Dr. Ravina, were 55%, 30% and 40% of their base salary, respectively. The annual bonus earned by each named executive officer with respect to the fiscal year ended December 31, 2019 is reported under the "Non-Equity Incentive Plan Compensation" column in the "2019 Summary Compensation Table" above and was determined based upon achievement of the corporate performance objectives at 90% of target.

Equity Compensation

Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants promote executive retention because they incentivize our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and may grant equity incentive awards to them from time to time. Our named executive officers have been granted certain options to purchase shares of our common stock, as described in more detail in the "Outstanding Equity Awards at 2019 Fiscal Year-end" table below.

Employment Arrangements with our Named Executive Officers

We initially entered into an offer letter with each of the named executive officers in connection with his employment with us, which set forth the terms and conditions of his employment, including base salary, target annual bonus opportunity and initial equity awards. In April 2020, we entered into employment agreements with Dr. Chaffee and Dr. Ravina that replaced the offer letters and provided for specified payments and benefits in connection with a termination of employment in certain circumstances.

We intend to enter into amended and restated employment agreements with Dr. Chaffee and Dr. Ravina that will be effective as of the closing of this offering, or the New Employment Agreements.

The New Employment Agreements will replace the employment agreements entered into in April 2020 and will provide for specified payments and benefits in connection with a termination of employment in certain circumstances. Our goal in providing these specified payments is to offer sufficient cash continuity protection such that the named executive officers will focus their full time and attention on the requirements of the business rather than the potential implications for their respective positions. We prefer to have certainty regarding the potential severance amounts payable to the named executive officers, rather than negotiating severance at the time that a named executive officer's employment terminates. We have also determined that accelerated vesting provisions with respect to outstanding equity awards in connection with a qualifying termination of employment in certain circumstances are appropriate because they encourage our named executive officers to stay focused on the business in those circumstances, rather than focusing on the potential implications for them personally. The material terms of the offer letter with Dr. Reddy and the New Employment agreements with Dr. Chaffee and Dr. Ravina are summarized below.

Kiran Reddy, M.D. Dr. Reddy resigned as our President and Chief Executive Officer effective April 20, 2020. In connection with the commencement of his employment with us, Clarus Ventures entered into an offer letter with Dr. Reddy to serve as Chief Executive Officer of our company, which set forth his initial annual base salary, target bonus and initial equity award. In addition, the offer letter provided that, if he transitioned from the position of Chief Executive Officer of our company for good reason, he would have the opportunity to join Clarus Ventures as a venture partner at his current compensation for a period of one year.

Stuart Chaffee, Ph.D. Under the New Employment Agreement with Dr. Chaffee, Dr. Chaffee will continue to serve as our Chief Financial Officer on an at-will basis. Dr. Chaffee's current annual base salary is $330,000, which is subject to annual review, and he is eligible to earn an annual bonus with a target amount equal to 30% of his base salary. Dr. Chaffee is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Bernard Ravina, M.D. Under the New Employment Agreement with Dr. Ravina, Dr. Ravina will continue to serve as our Chief Medical Officer on an at-will basis. Dr. Ravina's current annual base salary is $425,000, which is subject to annual review, and he is eligible to earn an annual bonus with a target amount equal to 40% of his base salary. Dr. Ravina is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Pursuant to the New Employment Agreements, in the event that Dr. Chaffee or Dr. Ravina's employment is terminated by us without "cause" or Dr. Chaffee or Dr. Ravina resigns for "good reason" (as each term shall be defined in the New Employment Agreements), subject to the execution and effectiveness of a separation agreement, including a general release of claims in our favor, Dr. Chaffee or Dr. Ravina, as applicable, (i) will be entitled to receive base salary continuation for nine months following termination, and (ii) subject to the executive's copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, we will cover the portion of the premium amount equal to the amount that we would have paid to provide health insurance to the executive had he remained employed with us until the earliest of (A) nine months following termination, (B) the executive's eligibility for group medical plan benefits under any other employer's group medical plan or (C) the end of the executive's COBRA health continuation period.

In lieu of the payments and benefits described in the preceding sentence, in the event Dr. Chaffee or Dr. Ravina's employment is terminated by us without cause or Dr. Chaffee or Dr. Ravina resigns for good reason, in either case on or within 12 months following a "change of control" (as such term shall be defined in the New Employment Agreements), subject to the execution and effectiveness of a separation agreement, including a general release of claims in our favor, (i) the executive will be entitled to receive a lump sum in cash equal to one times the sum of (A) the executive's then-current

annual base salary (or the executive's annual base salary in effect immediately prior to the change of control, if higher) plus (B) the executive's target annual cash incentive compensation for the year of termination (or the executive's target annual cash incentive compensation in effect immediately prior to the change of control, if higher), (ii) subject to the executive's copayment of premium amounts at the applicable active employees' rate and proper election to continue COBRA health coverage, we will cover the portion of the premium amount equal to the amount that we would have paid to provide health insurance to the executive had such executive remained employed with us until the earliest of (A) 12 months following termination, (B) the executive's eligibility for group medical plan benefits under any other employer's group medical plan or (C) the end of the executive's COBRA health continuation period, and (iii) the vesting of 100% of all stock options and other stock-based awards subject solely to time-based vesting held by the executive shall be accelerated.

The payments and benefits provided to each of the executives in connection with a change of control may not be eligible for a federal income tax deduction for the company pursuant to Section 280G of the Code and may subject the executive to an excise tax under Section 4999 of the Code. If the payments or benefits payable to the executive in connection with a change of control would be subject to the excise tax on golden parachutes imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to the executive. We initially entered into an offer letter with each of the named executive officers in connection with his employment with us, which set forth the terms and conditions of his employment, including base salary, target annual bonus opportunity and initial equity awards. In April 2020, we entered into employment agreements with Dr. Chaffee and Dr. Ravina that replaced the offer letters and provided for specified payments and benefits in connection with a termination of employment in certain circumstances.

Outstanding Equity Awards at 2019 Fiscal Year-End

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2019.

			Option Awards			
Name	Grant Date	Vesting Commencement Date	Number of Securities Underlying Unexercised Options (#) Exercisable (1)	Number of Securities Underlying Unexercised Options (#) Unexercisable (1)	Option Exercise Price ($)	Option Expiration Date
Kiran Reddy, M.D.	10/19/2018	10/6/2015	230,928	–	2.27	10/18/2028
Stuart Chaffee, Ph.D.	10/19/2018	11/20/2017	90,913	83,641	2.27	10/18/2028
Bernard Ravina, M.D.	10/19/2018	8/21/2018	65,166	130,334	2.27	10/18/2028

(1) The stock options vest over four years, with 25% of the total shares vesting on the first anniversary of the vesting commencement date and the remainder vesting in 36 approximately equal monthly installments.

Compensation Risk Assessment

We believe that although a portion of the compensation provided to our executive officers and other employees is performance-based, our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

Employee Benefit and Equity Compensation Plans

2017 Stock Incentive Plan

The Praxis Precision Medicines, Inc. 2017 Stock Incentive Plan, or our 2017 Plan, was approved by our board of directors and our stockholders in May 2017 and was most recently amended in September 2020. Under the 2017 Plan, as amended through the date hereof, we have reserved for issuance an aggregate of 5,937,763 shares of our common stock. The number of shares of common stock reserved for issuance is subject to adjustment in the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event or any dividend or distribution to holders of common stock other than an ordinary cash dividend.

The shares of common stock underlying awards that are expired, lapsed, terminated, surrendered, canceled without having been fully exercised or forfeited in whole or in part (including as a result of shares of common stock subject to such award being repurchased by us at or below the original issuance price), and shares of common stock that are withheld upon exercise of an option or settlement of an award to cover the exercise price or tax withholding are added back to the shares of common stock available for issuance under the 2017 Plan. Following this offering, such shares will be added to the shares of common stock available under the 2020 Stock Option and Incentive Plan, or the 2020 Plan.

Our board of directors has acted as administrator of the 2017 Plan. The administrator has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, and to determine the specific terms and conditions of each award, subject to the provisions of the 2017 Plan. Persons eligible to participate in the 2017 Plan are those employees, officers and directors of, and consultants and advisors to, our company as selected from time to time by the administrator in its discretion.

The 2017 Plan permits the granting of (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, or the Code, and (2) options that do not so qualify. The per share option exercise price of each option is determined by the administrator but may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each option is fixed by the administrator but may not exceed 10 years from the date of grant. The administrator determines at what time or times each option may be exercised. In addition, the 2017 Plan permits the granting of restricted shares of common stock, restricted stock units and other stock-based awards, including but not limited to stock appreciation rights and awards entitling recipients to receive shares of common stock to be delivered in the future. Other stock-based awards may be paid in shares of common stock or in cash, as determined by our board of directors.

The 2017 Plan provides that upon the occurrence of a “reorganization event,” as defined in the 2017 Plan, our board of directors may take one or more of the following actions as to all or any (or any portion of) awards outstanding under the 2017 Plan other than restricted stock awards: (i) provide that awards will be assumed or substituted by the acquiring or successor corporation, (ii) upon written notice to participants, provide that all unexercised awards will terminate immediately prior to the consummation of the reorganization event unless exercised by the participant within a specified period following the date of such notice, (iii) provide that outstanding awards shall become exercisable, realizable or deliverable, or that all restrictions applicable to such awards shall lapse, in whole or in part, prior to or upon such reorganization event, (iv) make or provide for a cash payment to the award holder equal to the excess, if any, of the per share cash consideration in the reorganization event times the number of shares subject to the participant’s award over any aggregate exercise price of such outstanding award and any applicable tax withholdings in exchange for the termination of such awards, (v) provide that, in connection with a liquidation or dissolution of our company, awards shall convert

into the right to receive liquidation proceeds (if applicable, net of the exercise price thereof and any applicable tax withholdings) and (vi) any combination of the foregoing. Upon the occurrence of a reorganization event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company under each outstanding restricted stock award shall inure to the benefit of our successor and shall, unless our board of directors determines otherwise, apply to the cash, securities or other property which the common stock was converted into or exchanged for pursuant to such reorganization event in the same manner and to the same extent as they applied to the common stock subject to such restricted stock award. Upon the occurrence of a reorganization event involving the liquidation or dissolution of our company (except as otherwise provided for in the award agreement), all restrictions and conditions on all outstanding restricted stock awards will be automatically deemed terminated or satisfied.

The administrator may amend, suspend or terminate the 2017 Plan at any time, subject to stockholder approval where such approval is required by applicable law. The administrator of the 2017 Plan may also amend or cancel any outstanding award, provided that no amendment to an award may adversely affect a participant's rights without his or her consent. The administrator of the 2017 Plan is specifically authorized to exercise its discretion to reduce the exercise price of outstanding awards under the 2017 Plan or effect the repricing of such awards through cancellation and re-grants without stockholder approval.

The 2017 Plan will terminate automatically upon the earlier of 10 years from the date on which the 2017 Plan was adopted by our board of directors or 10 years from the date the 2017 Plan was approved by our stockholders. As of September 30, 2020, options to purchase 5,814,944 shares of common stock were outstanding under the 2017 Plan. Our board of directors has determined not to make any further awards under the 2017 Plan following the closing of this offering.

2020 Stock Option and Incentive Plan

Our 2020 Plan was adopted by our board of directors on September 9, 2020, approved by our stockholders on October 8, 2020 and became effective upon the date immediately preceding the date on which the registration statement of which this prospectus is part was declared effective by the SEC. The 2020 Plan will replace the 2017 Plan as our board of directors has determined not to make additional awards under the 2017 Plan following the closing of our initial public offering. However, the 2017 Plan will continue to govern outstanding equity awards granted thereunder. The 2020 Plan allows us to make equity-based and cash-based incentive awards to our officers, employees, directors and consultants.

We have initially reserved 3,271,028 shares of our common stock, or the Initial Limit, for the issuance of awards under the 2020 Plan. The 2020 Plan provides that the number of shares reserved and available for issuance under the 2020 Plan will automatically increase each January 1, beginning on January 1, 2021, by 5% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee, or the Annual Increase. These limits are subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2020 Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying awards under the 2020 Plan or the 2017 Plan that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without the issuance of stock, expire or are otherwise terminated (other than by exercise) will be added back to the shares of common stock available for issuance under the 2020 Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options shall not exceed the Initial Limit cumulatively increased on January 1, 2021 and on each January 1 thereafter by the lesser of the Annual Increase for such year or 1,635,514 shares of common stock.

The grant date fair value of all awards made under our 2020 Plan and all other cash compensation paid by us to any non-employee director in any calendar year for services as a non-employee director shall not exceed $750,000; provided, however, that such amount shall be $1,000,000 for the calendar year in which the applicable non-employee director is initially elected or appointed to the board of directors and $1,500,000 for the non-executive chair of our board of directors. Notwithstanding the foregoing, the independent members of the board of directors may make exceptions to such limits in extraordinary circumstances.

The 2020 Plan will be administered by our compensation committee. Our compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted and the number of shares subject to such awards, to make any combination of awards to participants, to accelerate at any time the exercisability or vesting of any award and to determine the specific terms and conditions of each award, subject to the provisions of the 2020 Plan. Persons eligible to participate in the 2020 Plan will be those full or part-time officers, employees, non-employee directors and consultants as selected from time to time by our compensation committee in its discretion.

The 2020 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code, and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant unless the option is granted (i) pursuant to a transaction described in, and in a manner consistent with, Section 424(a) of the Code or (ii) to individuals who are not subject to U.S. income tax. The term of each option will be fixed by our compensation committee and may not exceed 10 years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right may not be less than 100% of the fair market value of our common stock on the date of grant unless the stock appreciation right is granted (i) pursuant to a transaction described in, and in a manner consistent with, Section 424(a) of the Code or (ii) to individuals who are not subject to U.S. income tax. The term of each stock appreciation right will be fixed by our compensation committee and may not exceed 10 years from the date of grant. Our compensation committee will determine at what time or times each stock appreciation right may be exercised.

Our compensation committee may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our compensation committee may also grant shares of common stock that are free from any restrictions under the 2020 Plan. Unrestricted stock may be granted to participants in recognition of past services or other valid consideration and may be issued in lieu of cash compensation due to such participant.

Our compensation committee may grant dividend equivalent rights to participants that entitle the recipient to receive credits for dividends that would be paid if the recipient had held a specified number of shares of our common stock.

Our compensation committee may grant cash bonuses under the 2020 Plan to participants, subject to the achievement of certain performance goals.

The 2020 Plan provides that upon the effectiveness of a “sale event,” as defined in the 2020 Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2020

Plan. To the extent that awards granted under the 2020 Plan are not assumed or continued or substituted by the successor entity, upon the effective time of the sale event, such awards shall terminate. In such case, except as may be otherwise provided in the relevant award certificate, all awards with time-based vesting, conditions or restrictions shall become fully vested and nonforfeitable as of the effective time of the sale event, and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in connection with a sale event in the administrator's discretion or to the extent specified in the relevant award certificate. In the event of such termination, individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) within a specified period of time prior to the sale event. In addition, in connection with the termination of the 2020 Plan upon a sale event, we may make or provide for a payment, in cash or in kind, to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights and we may make or provide for a payment, in cash or in kind, to participants holding other vested awards.

Our board of directors may amend or discontinue the 2020 Plan and our compensation committee may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose but no such action may adversely affect rights under an award without the holder's consent. Certain amendments to the 2020 Plan require the approval of our stockholders. No awards may be granted under the 2020 Plan after the date that is 10 years from the effective date of the 2020 Plan.

Our board of directors has approved the issuance under the 2020 Plan of stock options to acquire an aggregate of 37,615 shares of common stock on the effective date of the registration statement of which this prospectus is a part. These stock options will have an exercise price equal to the public offering price. No other awards under the 2020 Plan have been made prior to the date of this prospectus.

2020 Employee Stock Purchase Plan

Our 2020 Employee Stock Purchase Plan, or the 2020 ESPP, was adopted by our board of directors on September 9, 2020, approved by our stockholders on October 8, 2020 and became effective on the date immediately preceding the date on which the registration statement of which this prospectus is a part was declared effective by the SEC. The 2020 ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code. The 2020 ESPP initially reserves and authorizes the issuance of up to a total of 327,102 shares of common stock to participating employees. The 2020 ESPP provides that the number of shares reserved and available for issuance will automatically increase on January 1, 2021 and each January 1 thereafter through January 1, 2030, by the least of (i) 327,102 shares of common stock, (ii) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31 or (iii) such lesser number of shares of common stock as determined by the administrator of the 2020 ESPP. The number of shares reserved under the 2020 ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees whose customary employment is for more than 20 hours per week are eligible to participate in the ESPP. However, any employee who owns 5% or more of the total combined voting power or value of all classes of stock will not be eligible to purchase shares under the 2020 ESPP.

We may make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will usually begin on each May 1 and November 1 and will continue for six-month periods, referred to as offering periods. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the 2020 ESPP may purchase shares by authorizing payroll deductions of up to 15% of his or her eligible compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase shares of common stock on the last business day of the offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower, provided that no more than $25,000 worth of shares of common stock may be purchased by any one employee during any offering period. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of common stock, valued at the start of the purchase period, under the 2020 ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under the 2020 ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The 2020 ESPP may be terminated or amended by our board of directors at any time. An amendment that increases the number of shares of common stock authorized under the 2020 ESPP and certain other amendments require the approval of our stockholders.

401(k) Plan

We participate in a retirement savings plan, or 401(k) plan, that is intended to qualify for favorable tax treatment under Section 401(a) of the Code, and contains a cash or deferred feature that is intended to meet the requirements of Section 401(k) of the Code. U.S. employees who are at least 18 years of age are generally eligible to participate in the 401(k) plan, subject to certain criteria. Participants may make pre-tax and certain after-tax (Roth) salary deferral contributions to the plan from their eligible earnings up to the statutorily prescribed annual limit under the Code. Participants who are 50 years of age or older may contribute additional amounts based on the statutory limits for catch-up contributions. Participant contributions are held in trust as required by law. An employee's interest in his or her salary deferral contributions is 100% vested when contributed. We have the ability to make matching and discretionary contributions under the plan but did not make any contributions to the 401(k) plan in 2019.

Rule 10b5-1 Sales Plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. The director or officer may amend or terminate the plan in limited circumstances. Our directors and executive officers may also buy or sell additional shares of our common stock outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

DIRECTOR COMPENSATION

The following table presents the total compensation for each person who served as a non-employee member of our board of directors during the year ended December 31, 2019. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any equity awards or non-equity awards to or pay any other compensation to any of the non-employee members of our board of directors in 2019 for their services as members of the board of directors. Amounts paid to Dr. Reddy, our former President and Chief Executive Officer, for his service as employees during 2019 are presented above in the "2019 Summary Compensation Table" above. Dr. Reddy did not receive any compensation for his services as director for the fiscal year ended December 31, 2019.

2019 Director Compensation Table

Name	Fees Earned or Paid in Cash ($)	Stock Awards ($)	Option Awards ($)(1)(2)	All Other Compensation ($)	Total ($)
Nicholas Galakatos, Ph.D.	–	–	–	–	–
Thomas Dyrberg, M.D.	–	–	–	–	–
Stefan Vitorovic	–	–	–	–	–
Ari Brettman, M.D.	–	–	–	–	–
Paul Medeiros(3)	–	–	–	–	–
Gregory Norden	–	–	62,815	–	62,815
Alfred Sandrock(4)	–	–	–	–	–
William Young	–	–	–	–	–

(1) The amount reported represents the aggregate grant date fair value of the stock option awarded to Mr. Norden during fiscal year 2019, calculated in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718. Such grant date fair values do not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in Note 11 of our consolidated financial statements included elsewhere in this prospectus. The amounts reported in this column reflect the accounting cost for these stock options and do not correspond to the actual economic value that may be received by our directors upon the exercise of the stock options or any sale of the underlying shares of common stock.

(2) Each option grant is subject to the terms of our 2017 Plan. Mr. Norden received an option to purchase 27,928 shares of our common stock, which vested as to 25% of the total shares on December 20, 2019, with the remainder vesting in 36 approximately equal monthly installments over the following three years. As of December 31, 2019, Mr. Norden held options to purchase 27,928 shares of our common stock and Mr. Sandrock and Mr. Young each held options to purchase 27,980 shares of our common stock.

(3) Mr. Medeiros resigned as a director in September 2020.

(4) Mr. Sandrock resigned as a director in March 2020 and continues to serve in an advisory capacity.

Non-Employee Director Compensation Policy

In connection with this offering, we intend to adopt a non-employee director compensation policy that will become effective upon the completion of this offering and will be designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering, as set forth below:

	Annual Retainer
Board of Directors:	
Members	$40,000
Additional retainer for non-executive chair	$30,000
Audit Committee:	
Members (other than chair)	$ 8,000
Retainer for chair	$16,000
Compensation Committee:	
Members (other than chair)	$ 6,000
Retainer for chair	$12,000
Nominating and Corporate Governance Committee:	
Members (other than chair)	$ 4,000
Retainer for chair	$ 8,000

In addition, the non-employee director compensation policy will provide that, upon initial election to our board of directors, each non-employee director will be granted an option to purchase a number of shares equal to 0.1% of the total number of shares of our common stock issued and outstanding on the grant date, or the Initial Grant. The Initial Grant will vest in equal monthly installments over three years from the grant date, subject to continued service as a director through the applicable vesting date. Furthermore, on the date of each annual meeting of stockholders following the completion of this offering, each non-employee director who continues as a non-employee director following such meeting will be granted an annual option to purchase a number of shares equal to 0.05% of the total number of shares of our common stock issued and outstanding on the grant date, or the Annual Grant. The Annual Grant will vest in twelve equal monthly installments, subject to continued service as a director through the applicable vesting date. Such awards are subject to full accelerated vesting upon the sale of the company.

We will reimburse all reasonable out-of-pocket expenses incurred by non-employee directors in attending meetings of the board of directors and committees.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under “Executive Compensation” and “Director Compensation” in this prospectus and the transactions described below, since January 1, 2017, there has not been, and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of five percent or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

Sales and Purchases of Securities

Series A Preferred Stock Financing

From October 2016 to July 2017, we issued and sold to investors in private placements an aggregate of 6,100,000 shares of our Series A preferred stock at a price of $1.00 per share, for aggregate consideration of $6.1 million. In October 2016, we also issued and sold an aggregate of 1,375,799 shares of Series A preferred stock upon the conversion of a series of convertible promissory notes previously issued to Clarus Lifesciences III, L.P., or Clarus, or the 2016 Notes, at a conversion price of $0.75 per share. We refer to these transactions collectively as our Series A Preferred Stock Financing. The following table sets forth the aggregate number and purchase price of shares of our Series A preferred stock purchased by related persons:

Purchaser	Shares of Series A Preferred Stock Purchased	Aggregate Purchase Price ($)
Entities affiliated with Blackstone(1)	7,375,799	7,031,849
Total	7,375,799	7,031,849

(1) Nicholas Galakatos, Ph.D., a member of our board of directors, was a managing director of Clarus, prior to its acquisition by The Blackstone Group Inc., or Blackstone, and is a senior managing director of Blackstone. Ari Brettman, M.D. a member of our board of directors, was a principal at Clarus, prior to its acquisition by Blackstone, and is a managing director of Blackstone.

Convertible Promissory Note Financings

In December 2017 and January 2018, we issued convertible promissory notes to Clarus, collectively the Convertible Notes, for $2.0 million and $1.0 million, respectively. The Convertible Notes were settled in March 2018 upon their automatic conversion into shares of our Series B preferred stock, as described below.

Nicholas Galakatos, Ph.D., a member of our board of directors, was a managing director of Clarus, prior to its acquisition by Blackstone, and is a senior managing director of Blackstone. Ari Brettman, M.D. a member of our board of directors, was a principal at Clarus, prior to its acquisition by Blackstone, and is a managing director of Blackstone.

Series B Preferred Stock Financing

During the year ended December 31, 2018, we issued a total of 12,333,333 shares of Series B preferred stock in two separate closings at a purchase price of $3.00 per share for gross cash proceeds of $37.0 million, and incurred issuance costs of $0.2 million. We also issued an aggregate of 1,286,185 shares of Series B preferred stock upon the conversion of the Convertible Notes at an average conversion price of $2.40 per share. We also issued 1,294,186 shares of Series B preferred stock in connection with the anti-dilutive provision within the Purdue License Agreement. The following

table sets forth the aggregate number and purchase price of shares of our Series B preferred stock purchased by related persons:

Purchaser	Shares of Series B Preferred Stock Purchased	Aggregate Purchase Price ($)
Entities affiliated with Blackstone(1)	5,052,852	14,337,643
Novo Holdings A/S(2)	4,150,000	12,450,000
Vida Ventures, LLC(3)	4,150,000	12,450,000
Total	13,352,852	39,237,643

(1) Nicholas Galakatos, Ph.D., a member of our board of directors, was a managing director of Clarus, prior to its acquisition by Blackstone, and is a senior managing director of Blackstone. Ari Brettman, M.D. a member of our board of directors, was a principal at Clarus, prior to its acquisition by Blackstone, and is a managing director of Blackstone.
(2) Thomas Dyrberg, M.D., a member of our board of directors, is employed as a Managing Partner of Novo Holdings A/S, or Novo. Dr. Dyrberg is not deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Novo.
(3) Stefan Vitorovic, a member of our board of directors, is a managing director of Vida Ventures, LLC.

Series B-1 Preferred Stock Financing

In June 2019, we issued and sold to investors in a private placement an aggregate of 2,666,666 shares of our Series B-1 preferred stock at a price of $3.75 per share, for aggregate consideration of approximately $10.0 million, or our Series B-1 Preferred Stock Financing. The following table sets forth the aggregate number and purchase price of shares of our Series B-1 preferred stock purchased by related persons:

Purchaser	Shares of Series B-1 Preferred Stock Purchased	Aggregate Purchase Price ($)
Entities affiliated with Blackstone(1)	1,410,477	5,289,288
Novo Holdings A/S(2)	551,794	2,069,228
Vida Ventures, LLC(3)	551,794	2,069,228
Purdue Neuroscience Company(4)	133,334	500,003
Total	2,647,399	9,927,747

(1) Nicholas Galakatos, Ph.D., a member of our board of directors, was a managing director of Clarus, prior to its acquisition by Blackstone, and is a senior managing director of Blackstone. Ari Brettman, M.D. a member of our board of directors, was a principal at Clarus, prior to its acquisition by Blackstone, and is a managing director of Blackstone.
(2) Thomas Dyrberg, M.D., a member of our board of directors, is employed as a Managing Partner of Novo. Dr. Dyrberg is not deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Novo.
(3) Stefan Vitorovic, a member of our board of directors, is a managing director of Vida Ventures, LLC.
(4) Purdue Neuroscience Company became a holder of five percent or more of our capital stock pursuant to our Series B-1 Preferred Financing, but is no longer a holder of five percent or more of our capital stock as of December 2019. Paul Medeiros, a member of our board of directors at the time of this financing who resigned in September 2020, is a senior vice president of Purdue Pharma L.P., an affiliate of Purdue Neuroscience Company.

Series C Preferred Stock Financing

From November 2019 through May 2020, we issued and sold to investors in a private placement an aggregate of 14,368,935 shares of our Series C preferred stock at a price of $5.15 per share, for

aggregate consideration of approximately $74.0 million, or our Series C Preferred Stock Financing. The following table sets forth the aggregate number and purchase price of shares of our Series C preferred stock purchased by related persons:

Purchaser	Shares of Series C Preferred Stock Purchased	Aggregate Purchase Price ($)
Entities affiliated with Blackstone (1)	2,500,956	12,879,923
Novo Holdings A/S(2)	171,410	882,762
Vida Ventures, LLC(3)	171,410	882,762
Entities affiliated with Eventide(4)	3,883,496	20,000,004
Purdue Neuroscience Company(5)	59,333	305,565
Total	6,786,605	34,951,016

(1) Ari Brettman, M.D., and Nicholas Galakatos, Ph.D., members of our board of directors, are a managing director and senior managing director, respectively, of Blackstone, an affiliate of Clarus and BSOF Parallel Master Fund L.P., or BSOF. Kiran Reddy, M.D. a member of our board of directors and our former president and chief executive officer, is a managing director of Blackstone, an affiliate of Clarus and BSOF.
(2) Thomas Dyrberg, M.D., a member of our board of directors, is employed as a Managing Partner of Novo. Dr. Dyrberg is not deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Novo.
(3) Stefan Vitorovic, a member of our board of directors, is a managing director of Vida Ventures, LLC.
(4) Mutual Fund Series Trust, On Behalf of Eventide Healthcare & Life Sciences Fund, or Eventide Healthcare, and Mutual Fund Series Trust, On Behalf of Eventide Gilead Fund, or Eventide Gilead, together became a holder of five percent or more of our capital stock pursuant to our Series C Preferred Stock Financing.
(5) Purdue Neuroscience Company is no longer a holder of five percent or more of our capital stock as of December 2019. Paul Medeiros, a member of our board of directors at the time of this financing who resigned in September 2020, is a senior vice president of Purdue Pharma L.P., an affiliate of Purdue Neuroscience Company.

Series C Repurchase

On February 2020 and March 2020, we repurchased from certain holders of five percent or more of our capital stock an aggregate of 5,825,243 shares of our Series C preferred stock at a price of $5.15 per share, for an aggregate consideration of approximately $30.0 million, or the Series C Repurchase. The following table sets forth the aggregate number and purchase price of shares of our Series C preferred stock repurchased by us from related persons:

Name of Holder	Shares of Series C Preferred Stock Repurchased	Aggregate Purchase Price ($)
Entities affiliated with RTW	2,912,622	15,000,003
Entities affiliated with Venrock	2,912,621	14,999,998
Total	5,825,243	30,000,001

Series C-1 Preferred Stock Financing

From July to August 2020, we issued and sold to investors in a private placement an aggregate of 19,444,453 shares of our Series C-1 preferred stock at a price of $5.67 per share, for aggregate consideration of approximately $110.3 million, or our Series C-1 Preferred Stock Financing. The following table sets forth the aggregate number and purchase price of shares of our Series C-1 preferred stock purchased by related persons:

Purchaser	Shares of Series C-1 Preferred Stock Purchased	Aggregate Purchase Price ($)
Entities affiliated with Blackstone (1)	352,734	2,000,002
Novo Holdings A/S (2)	352,734	2,000,002
Vida Ventures, LLC (3)	881,835	5,000,004
Entities affiliated with Eventide (4)	3,527,337	20,000,001
Marcio Souza (5)	44,092	250,002
Total	5,158,732	29,250,011

(1) Ari Brettman, M.D., and Nicholas Galakatos, Ph.D., members of our board of directors, are a managing partner and senior managing director, respectively, of Blackstone, an affiliate of Clarus and BSOF Parallel Master Fund L.P., or BSOF. Kiran Reddy, M.D., a member of our board of directors and our former president and chief executive officer, is a managing director of Blackstone, an affiliate of Clarus and BSOF.
(2) Thomas Dyrberg, M.D., a member of our board of directors, is employed as a Managing Partner of Novo. Dr. Dyrberg is not deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Novo.
(3) Stefan Vitorovic, a member of our board of directors, is a managing director of Vida Ventures, LLC.
(4) Mutual Fund Series Trust, On Behalf of Eventide Healthcare & Life Sciences Fund, or Eventide Healthcare, and Mutual Fund Series Trust, On Behalf of Eventide Gilead Fund, or Eventide Gilead, together hold five percent or more of our capital stock pursuant to our Series C-1 Preferred Stock Financing.
(5) Marcio Souza serves on our board of directors and is our President and Chief Executive Officer.

Commercial Agreements with Related Parties

Purdue

In December 2017, we and Purdue Neuroscience Company, or Purdue, a former holder of five percent or more of our capital stock, entered into a license agreement, described in the section of this prospectus captioned “Business—Third-Party Licenses.” Paul Medeiros, a former member of our board of directors, serves as a Senior Vice President of Purdue Pharma L.P.

RogCon

In December 2018, we entered into an agreement with RogCon Inc., or RogCon, pursuant to which we agreed to advance RogCon a deposit of up to $1.0 million related to the cooperation and license agreement described below. The amounts funded to RogCon under this agreement were applied towards the purchase price of the license agreement with RogCon described below.

In September 2019, we entered into a cooperation and license agreement with RogCon, described in the section of this prospectus captioned “Business—Third-Party Licenses.”

Alex Nemiroff, our General Counsel and Secretary, is a co-founder and chief executive officer of RogCon.

Underwriting Arrangements

Blackstone Securities Partners L.P., an affiliate of Blackstone, is an underwriter in the initial public offering of our common stock and shall be entitled to commissions and fees on substantially similar terms as our other underwriters. Ari Brettman, M.D., and Nicholas Galakatos, Ph.D., members of our board of directors, are a managing director and senior managing director, respectively, of Blackstone. For more information regarding our agreement with the underwriters for this offering, see the section titled “Underwriting.”

Other Arrangements

In March 2020, we reimbursed an affiliate of Blackstone approximately $164,000 in third-party expenses related to the recruitment of our chief executive officer.

Indemnification Agreements

In connection with this offering, we entered into agreements to indemnify our directors and executive officers. These agreements, among other things, require us to indemnify these individuals for certain expenses (including attorneys’ fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person’s status as a member of our board of directors to the maximum extent allowed under Delaware law.

Agreements with Stockholders

In connection with our Series A preferred stock financing, our Series B preferred stock financing, our Series B-1 preferred stock financing, our Series C preferred stock financing and our Series C-1 preferred stock financing, we entered into investors’ rights, voting and right of first refusal and co-sale agreements containing registration rights, information rights, voting rights and rights of first refusal, among other things, with certain holders of our preferred stock and certain holders of our common stock. These stockholder agreements will terminate upon the closing of this offering, except for the registration rights granted under our investors’ rights agreement, as more fully described in “Description of Capital Stock—Registration Rights.”

Directed Share Program

The underwriters have reserved for sale, at the public offering price, up to 5% of the shares of our common stock being offered hereby to individuals, which may include certain of our officers, directors and employees, as part of a directed share program. The sales will be made by the directed share program administrator. The directed share program will not limit the ability of our officers, directors and employees to purchase more than $120,000 in value of our common stock. We do not currently know the extent to which these related persons will participate in our directed share program, if at all, or the extent to which they will purchase more than $120,000 in value of our common stock.

Policies for Approval of Related Party Transactions

Our board of directors reviews and approves transactions with directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party’s relationship or interest in the transaction were disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party’s relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

In connection with this offering, we adopted a formal written policy that our executive officers, directors, holders of more than five percent of any class of our voting securities, and any member of the immediate family of and any entity affiliated with any of the foregoing persons, are not permitted to enter into a related

party transaction with us without the prior consent of our audit committee, or other independent members of our board of directors in the event it is inappropriate for our audit committee to review such transaction due to a conflict of interest. Any request for us to enter into a transaction with an executive officer, director, holders of more than 5% of any class of our voting securities, or any of their immediate family members or affiliates, in which the amount involved exceeds $120,000 must first be presented to our audit committee for review, consideration and approval. In approving or rejecting any such proposal, our audit committee will consider the relevant facts and circumstances available and deemed relevant to our audit committee, including, but not limited to, whether the transaction will be on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related party's interest in the transaction. All of the transactions described in this section were entered into prior to the adoption of this policy.

PRINCIPAL STOCKHOLDERS

The following table sets forth certain information known to us regarding beneficial ownership of our capital stock as of September 30, 2020, as adjusted to reflect the sale of common stock offered by us in this offering, for:

- each person or group of affiliated persons known by us to be the beneficial owner of more than five percent of our capital stock;
- each of our named executive officers;
- each of our directors; and
- all of our executive officers and directors as a group.

To the extent that the underwriters sell more than 10,000,000 shares in this offering, the underwriters have the option to purchase up to an additional 1,500,000 shares at the initial public offering price less the underwriting discount.

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Under those rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power. Except as noted by footnote, and subject to community property laws where applicable, we believe, based on the information provided to us, that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

The percentage of beneficial ownership prior to this offering in the table below is based on 26,749,675 shares of common stock deemed to be outstanding as of September 30, 2020, including 8,763 shares of unvested restricted common stock as of September 30, 2020 and assuming the conversion of all outstanding shares of our preferred stock upon the closing of this offering, and the percentage of beneficial ownership after this offering in the table below is based on 36,749,675 shares of common stock assumed to be outstanding after the closing of the offering. The information in the table below assumes no exercise of the underwriters' option to purchase additional shares. Options to purchase shares of common stock that are exercisable within 60 days of September 30, 2020 are deemed to be beneficially owned by the persons holding these options for the purpose of computing percentage ownership of that person, but are not treated as outstanding for the purpose of computing any other person's ownership percentage.

Name and Address of Beneficial Owner (1)	Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% Stockholders:			
Entities affiliated with Blackstone(2)	8,501,487	31.8%	23.1%
Novo Holdings A/S(3)	2,442,080	9.1%	6.6%
Vida Ventures, LLC(4)	2,689,329	10.1%	7.3%
Entities affiliated with Eventide(5)	3,463,081	12.9%	9.4%
Named Executive Officers and Directors:			
Nicholas Galakatos, Ph.D.(2)	—	—	—
Ari Brettman, M.D.(2)	—	—	—
Thomas Dyrberg, M.D.(3)	—	—	—
Stefan Vitorovic(4)	2,689,329	10.1%	7.3%
William Young(6)	27,397	*	*
Gregory Norden(7)	13,382	*	*
Kiran Reddy, M.D.(2)(8)	698,217	2.6%	1.9%
Stuart Chaffee, Ph.D.(9)	130,915	*	*
Bernard Ravina(10)	109,968	*	*
Marcio Souza(11)	20,604	*	*
Dean Mitchell	—	—	—%
All executive officers and directors as a group (13 persons)(12)	**3,689,812**	**13.5%**	**9.9%**

* Represents beneficial ownership of less than one percent.

(1) Unless otherwise indicated, the address for each beneficial owner is c/o Praxis Precision Medicines, Inc., One Broadway, 16th Floor, Cambridge, MA 02142.

(2) Consists of (i) 3,446,711 shares of common stock issuable upon conversion of shares of Series A preferred stock held by Clarus Lifesciences III, L.P. ("Clarus"); (ii) 2,361,198 shares of common stock issuable upon conversion of shares of Series B preferred stock held by Clarus; (iii) 659,116 shares of common stock issuable upon conversion of shares of Series B-1 preferred stock held by Clarus; (iv) 261,318 shares of common stock issuable upon conversion of shares of Series C preferred stock held by Clarus; (v) 164,832 shares of common stock issuable upon conversion of shares of Series C-1 preferred stock held by Clarus; (vi) 700,934 shares of common stock held by Clarus, and (vii) 907,378 shares of common stock issuable upon conversion of shares of Series C preferred stock held by BSOF Parallel Master Fund L.P. (together with Clarus, the "Blackstone Funds"). Clarus Ventures III GP, L.P. is the general partner of Clarus. Blackstone Clarus III L.L.C. is the general partner of Clarus GP. The sole member of Blackstone Clarus III L.L.C. is Blackstone Holdings II L.P. Blackstone Strategic Opportunity Associates L.L.C. is the general partner of BSOF Parallel Master Fund L.P. Blackstone Holdings II L.P. is the sole member of Blackstone Strategic Opportunity Associates L.L.C. Blackstone Alternative Solutions L.L.C. is the investment manager of BSOF Parallel Master Fund L.P. Blackstone Holdings I L.P. is the sole member of Blackstone Alternative Solutions L.L.C. The general partner of Blackstone Holdings I L.P. and Blackstone Holdings II L.P. is Blackstone Holdings I/II GP L.L.C. The sole member of Blackstone Holdings I/II GP L.L.C. is The Blackstone Group Inc. The sole holder of the Class C common stock of The Blackstone Group Inc. is Blackstone Group Management L.L.C. Blackstone Group Management L.L.C. is wholly-owned by Blackstone's senior managing directors and controlled by its founder, Stephen A. Schwarzman. Each of such entities and Mr. Schwarzman may be deemed to

beneficially own the shares beneficially owned by the Blackstone Funds controlled by it or him, but each (other than the Blackstone Funds to the extent of their direct ownership) disclaims beneficial ownership of such shares. Each of Ari Brettman, M.D. Nicholas Galakatos, Ph.D., and Kiran Reddy, M.D. members of our board of directors, is an employee of an entity affiliated with the Blackstone Funds and each disclaims beneficial ownership of the shares beneficially owned by the Blackstone Funds. The address for each of Clarus and Clarus Ventures III GP, L.P. is c/o Clarus Ventures LLC, 101 Main Street, Suite 1210, Cambridge, MA 02142. The address for each of the other Blackstone entities and Mr. Schwarzman is c/o The Blackstone Group Inc., 345 Park Avenue, New York, NY 10154.

(3) Consists of (i) 1,939,295 shares of common stock issuable upon conversion of shares of Series B preferred stock; (ii) 257,853 shares of common stock issuable upon conversion of shares of Series B-1 preferred stock; (iii) 80,100 shares of common stock issuable upon the conversion of Series C preferred stock, and (iv) 164,832 shares of common stock issuable upon the conversion of Series C-1 preferred stock. All shares are held directly by Novo Holdings A/S, a Danish limited liability company that manages investments and financial assets. Thomas Dyrberg M.D. is employed as a managing partner at Novo Holdings A/S and is also a member of our board of directors. Dr. Dyrberg is not deemed to hold any beneficial ownership or reportable pecuniary interest in the shares held by Novo Holdings A/S. The address for Novo Holdings A/S is Tuborg Havnevej 19, DK-2900 Hellerup, Denmark.

(4) Consists of (i) 1,939,295 shares of common stock issuable upon conversion of shares of Series B preferred stock; (ii) 257,853 shares of common stock issuable upon conversion of shares of Series B-1 preferred stock; (iii) 80,100 shares of common stock issuable upon conversion of shares of Series C preferred stock, and (iv) 412,081 shares of common stock issuable upon the conversion of Series C-1 preferred stock. All shares are held directly by Vida Ventures, LLC, a United States limited liability company. Stefan Vitorovic is the Co-Founder and Managing Director of Vida Ventures, LLC and is also a member of our board of directors. VV Manager, LLC, or VV Manager, is the managing member of Vida. Stefan Vitorovic, Arjun Goyal, Fred Cohen, Arie Belldegrun and Leonard Potter are managers of VV Manager, and may be deemed to share voting and dispositive power over the shares held by Vida. The address of Vida is 40 Broad Street, Suite 201, Boston, Massachusetts 02109.

(5) Consists of (i) 907,379 shares of common stock issuable upon conversion of shares of Series C preferred stock held by Mutual Fund Series Trust, On Behalf Of Eventide Healthcare & Life Sciences Fund, or Eventide Healthcare; (ii) 988,994 shares of common stock issuable upon conversion of shares of Series C-1 preferred stock held by Eventide Healthcare; (iii) 907,378 shares of common stock issuable upon conversion of shares of Series C preferred stock held by Mutual Fund Series Trust, On Behalf Of Eventide Gilead Fund, or Eventide Gilead, and (iv) 659,330 shares of common stock issuable upon conversion of shares of Series C-1 preferred stock held by Eventide Gilead. The address for both Eventide Healthcare and Eventide Gilead is One International Place, Suite 4210, Boston, Massachusetts 02110.

(6) Consists of options to purchase 27,397 shares of common stock that are exercisable within 60 days of September 30, 2020.

(7) Consists of options to purchase 13,382 shares of common stock that are exercisable within 60 days of September 30, 2020.

(8) Consists of (i) 467,289 shares of common stock and (ii) options to purchase 230,928 shares of common stock that are exercisable within 60 days of September 30, 2020.

(9) Consists of options to purchase 130,915 shares of common stock that are exercisable within 60 days of September 30, 2020.

(10) Consists of (i) 11,021 shares of common stock and (ii) options to purchase 98,947 shares of common stock that are exercisable within 60 days of September 30, 2020.

(11) Consists of 20,604 shares of common stock issuable upon the conversion of Series C-1 preferred stock.

(12) Consists of (i) 478,310 shares of common stock; (ii) 1,939,295 shares of common stock issuable upon conversion of shares of Series B preferred stock; (iii) 257,853 shares of common stock issuable upon conversion of shares of Series B-1 preferred stock; (iv) 80,100 shares of common stock issuable upon conversion of shares of Series C preferred stock; (v) 432,685 shares of common stock issuable upon the conversion of Series C-1 preferred stock and (vi) options to purchase 501,569 shares of common stock that are exercisable within 60 days of September 30, 2020.

DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our amended and restated certificate of incorporation and amended and restated bylaws which will become effective immediately upon the closing of this offering. The descriptions of the common stock and preferred stock give effect to changes to our capital structure that will occur immediately upon the closing of this offering. We refer in this section to our amended and restated certificate of incorporation as our certificate of incorporation, and we refer to our amended and restated bylaws as our bylaws.

General

Upon the closing of this offering, our authorized capital stock will consist of 150,000,000 shares of common stock, par value $0.0001 per share and 10,000,000 shares of preferred stock, par value $0.0001 per share, all of which shares of preferred stock will be undesignated.

As of September 30, 2020, 26,749,675 shares of our common stock were outstanding and held by stockholders of record. This amount assumes the conversion of all outstanding shares of our preferred stock into common stock upon the closing of this offering. In addition, as of September 30, 2020, we had outstanding options to purchase 5,814,944 shares of our common stock under our 2017 Stock Incentive Plan, at a weighted average exercise price of $6.20 per share, 957,214 of which were exercisable.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred stock

Immediately prior to the completion of this offering, all outstanding shares of our preferred stock will be converted into shares of our common stock. Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our Company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Registration rights

Upon the completion of this offering, the holders of 25,067,977 shares of our common stock, including those issuable upon the conversion of preferred stock, will be entitled to rights with respect to

the registration of these securities under the Securities Act. These rights are provided under the terms of a third amended and restated investors' rights agreement between us and the holders of our preferred stock, or the investors' rights agreement. The investors' rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under this agreement will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand registration rights

Beginning 180 days after the completion of this offering, the holders of 25,067,977 shares of our common stock, including those issuable upon the conversion of shares of our preferred stock upon the closing of this offering, will be entitled to demand registration rights. Under the terms of the investors' rights agreement, we will be required, upon the written request of holders of at least a majority of the securities eligible for registration then outstanding, and if anticipated aggregate offering price, net of related fees and expenses, would exceed $5 million, we will be required to file a registration statement covering all securities eligible for registration that our stockholders request to be included in such registration. We are required to effect only two registrations pursuant to this provision of the investors' rights agreement in any twelve-month period.

Short-form registration rights

Pursuant to the investors' rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of stockholders holding at least a majority of the securities eligible for registration then outstanding, we will be required to file a Form S-3 registration restatement with respect to outstanding securities of such stockholders having an anticipated aggregate offering, net of related fees and expenses, of at least $3 million. We are required to effect only two registrations in any twelve-month period pursuant to this provision of the amended and restated investors' rights agreement. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Piggyback registration rights

Upon the completion of this offering, the holders of 25,067,977 shares of our common stock, including those issuable upon the conversion of shares of our preferred stock upon the closing of this offering, are entitled to piggyback registration rights. If we register any of our securities either for our own account or for the account of other security holders, the holders of these shares are entitled to include their shares in the registration. Subject to certain exceptions contained in the investor rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering.

Indemnification

Our investors' rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

Expiration of registration rights

The demand registration rights and short-form registration rights granted under the investors' rights agreement will terminate on the earliest to occur of: (i) on the fifth anniversary of the completion of this offering or (ii) a merger, sale or liquidation of our company.

Anti-Takeover Effects of our Certificate of Incorporation and Bylaws and Delaware Law

Our amended and restated certificate of incorporation and amended and restated bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Undesignated Preferred Stock

Our amended and restated certificate of incorporation provides for 10,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control.

Exclusive Jurisdiction for Certain Actions

Our amended and restated bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers and employees to us or our stockholders, (iii) any action asserting a claim against us or any of our current or former directors, officers, or other employees or stockholders arising pursuant to any provision of the Delaware General Corporation Law, or the DGCL, our certificate of incorporation or our bylaws, (iv) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws or (v) any action asserting a claim against us or any of our current or former directors or officers or other employees that is governed by the internal affairs doctrine.

Section 203 of the Delaware General Corporation Law

Upon the closing of this offering, we will be subject to the provisions of Section 203 of the DGCL. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a “business combination” with an “interested stockholder” for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or

- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

In general, Section 203 defines a business combination to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an “interested stockholder” as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Provisions of our amended and restated certificate of incorporation and our amended and restated bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our amended and restated certificate of incorporation and amended and restated bylaws:

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- provide that the authorized number of directors may be changed only by resolution adopted by a majority of the board of directors;
- provide that the board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66.67% of the voting power of all of our then outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law or subject to the rights of holders of preferred stock as designated from time to time, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide our board of directors into three classes;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent or electronic transmission;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder’s notice;

- do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);
- provide that special meetings of our stockholders may be called only by our board of directors acting pursuant to a resolution approved by the affirmative vote of a majority of the directors then in office; and
- provide that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers and employees to us or our stockholders, (iii) any action asserting a claim against us or any of our current or former directors, officers, or other employees or stockholders arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws, (iv) any action to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws or (v) any action asserting a claim against us or any of our current or former directors or officers or other employees that is governed by the internal affairs doctrine.

The amendment of any of these provisions included in our amended and restated certificate of incorporation, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require the affirmative vote of the majority of all of our then outstanding common stock. The amendment of any of these provisions included in our amended and restated bylaws would require the affirmative vote of the holders of at least 66.67% of the voting power of our then outstanding common stock.

Nasdaq Global Select Market Listing

Our common stock has been approved for listing on The Nasdaq Global Select Market under the trading symbol “PRAX.”

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our shares. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of September 30, 2020 upon the completion of this offering, 36,749,675 shares of our common stock will be outstanding, assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options. Of the outstanding shares, all of the shares sold in this offering will be freely tradable, except that any shares held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below, and shares of our common stock are restricted shares of common stock subject to service-based vesting terms. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

As a result of the lock-up agreements referred to below and the provisions of Rule 144 and Rule 701 under the Securities Act, the shares of our common stock (excluding the shares sold in this offering) that will be available for sale in the public market are as follows:

- beginning on the date of this prospectus, the 10,000,000 shares of common stock sold in this offering will be immediately available for sale in the public market;
- beginning 181 days after the date of this prospectus, 26,737,873 additional shares of common stock will become eligible for sale in the public market, of which 18,096,460 shares will be held by affiliates and subject to the volume and other restrictions of Rule 144, as described below; and
- the remainder of the shares of common stock will be eligible for sale in the public market from time to time thereafter, subject in some cases to the volume and other restrictions of Rule 144, as described below.

Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Securities Exchange Act of 1934, as amended, or the Exchange Act, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares, based on the number of shares outstanding as of September 30, 2020; or
- the average weekly trading volume of our common stock on The Nasdaq Global Select Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under “Underwriting” included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-Up Agreements

We, all of our directors and executive officers, and the holders of substantially all of our capital stock and securities convertible into or exchangeable for our capital stock have entered into lock-up agreements with the underwriters and/or are subject to market standoff agreements or other agreements with us, which prevents them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of the representatives, subject to certain exceptions. See the section entitled “Underwriting” appearing elsewhere in this prospectus for more information.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled “Description of Capital Stock—Registration rights” appearing elsewhere in this prospectus for more information.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above. As of September 30, 2020, we estimate that such registration statement on Form S-8 will cover approximately 9,413,074 shares.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following discussion is a summary of certain material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a foreign corporation or any other foreign organization taxable as a corporation for U.S. federal income tax purposes; or
- a foreign estate or trust, the income of which is not subject to U.S. federal income tax on a net income basis.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset within the meaning of Section 1221 of the Code, which is generally property held for investment.

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any U.S. state, local or non-U.S. taxes, the alternative minimum tax, the Medicare tax on net investment income, the rules regarding qualified small business stock within the meaning of Section 1202 of the Code or "Section 1244 stock" within the meaning of Section 1244 of the Code, or any other aspect of any U.S. federal tax other than the income tax. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- "controlled foreign corporations," "passive foreign investment companies" and corporations that accumulate earnings to avoid U.S. federal income tax;

- "qualified foreign pension funds," or entities wholly owned by a "qualified foreign pension fund";
- persons that elect to apply Section 1400Z-2 of the Code to gains recognized with respect to shares of our common stock;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on Our Common Stock

As indicated in the "Dividend Policy" section of this prospectus, we have never declared or paid cash dividends on any of our capital stock and currently intend to retain all available funds and any future earnings to fund the development and growth of our business.

In the event that we do make distributions, subject to the discussions below under the sections titled "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA", distributions paid on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "Gain on Sale or Other Taxable Disposition of Our Common Stock."

Subject to the discussion in the following two paragraphs in this section, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) to the applicable withholding agent and satisfy applicable certification and other requirements. Non-U.S. holders are urged to

consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for such lower rate of U.S. withholding tax as may be specified under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Sale or Other Taxable Disposition of Our Common Stock

Subject to the discussions below under "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA," a non-U.S. holder generally will not be subject to any U.S. federal income or withholding tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on Our Common Stock" also may apply;
- the non-U.S. holder is a nonresident alien individual who is present in the United States for a period or periods aggregating 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been, at any time during the five-year period preceding such sale or other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market, as defined by applicable U.S. Treasury Regulations, and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above. Non-U.S. holders should consult their own tax advisors about the consequences that could result if we are, or become, a U.S. real property holding corporation.

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on Our Common Stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker.

Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

Withholding and Information Reporting Requirements—FATCA

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally impose a U.S. federal withholding tax at a rate of 30% on payments of dividends on our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any or (iii) the foreign entity is otherwise exempt under FATCA. Such withholding may also apply to payments of gross proceeds of sales or other dispositions of our common stock, although under recently proposed U.S. Treasury Regulations (the preamble to which specifies that taxpayers are permitted to rely on such proposed U.S. Treasury Regulations pending finalization), no withholding will apply to such payments of gross proceeds. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

UNDERWRITING (CONFLICTS OF INTEREST)

We and the underwriters for the offering named below, have entered into an underwriting agreement with respect to the common stock being offered. Subject to the terms and conditions of the underwriting agreement, each underwriter has severally agreed to purchase from us the number of shares of our common stock set forth opposite its name below. Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co. are the representatives of the underwriters.

Underwriter	Number of Shares
Cowen and Company, LLC	3,300,000
Evercore Group L.L.C.	2,600,000
Piper Sandler & Co.	2,600,000
Wedbush Securities Inc.	1,000,000
Blackstone Securities Partners L.P.	500,000
Total	10,000,000

The underwriting agreement provides that the obligations of the underwriters are subject to certain conditions precedent and that the underwriters have agreed, severally and not jointly, to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased, other than those shares covered by the overallotment option described below. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the non-defaulting underwriters may be increased or the underwriting agreement may be terminated.

We have agreed to indemnify the underwriters against specified liabilities, including liabilities under the Securities Act of 1933, and to contribute to payments the underwriters may be required to make in respect thereof.

The underwriters are offering the shares, subject to prior sale, when, as and if issued to and accepted by them, subject to approval of legal matters by their counsel and other conditions specified in the underwriting agreement. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Overallotment Option to Purchase Additional Shares. We have granted to the underwriters an option to purchase up to 1,500,000 additional shares of common stock at the public offering price, less the underwriting discount. This option is exercisable for a period of 30 days. The underwriters may exercise this option solely for the purpose of covering overallotments, if any, made in connection with the sale of common stock offered hereby. To the extent that the underwriters exercise this option, the underwriters will purchase additional shares from us in approximately the same proportion as shown in the table above.

Directed share program. At our request, the underwriters have reserved for sale at the public offering price up to 5% of the shares of common stock for sale to individuals, including our officers, directors and employees, as well as friends and family members of our officers and directors, who have expressed an interest in purchasing shares in this offering. The sales will be made by the directed share program administrator. If purchased by persons who are not officers or directors, the shares will not be subject to a lock-up restriction. If purchased by any officer or director, the shares will be subject to a 180-day lock-up restriction. The underwriters will receive the same underwriting discount on any shares purchased by these persons as they will on any other shares sold to the public in this offering.

The number of shares of common stock available for sale to the general public in this offering, referred to as the general public shares, will be reduced to the extent these persons purchase the directed shares in the program. Any directed shares not so purchased will be offered by the underwriters to the general public on the same terms as the other shares. Likewise, to the extent demand by these persons exceeds the number of directed shares reserved for sale in the program, and there are remaining shares available for sale to these persons after the general public shares have first been offered for sale to the general public, then such remaining shares may be sold to these persons at the discretion of the underwriters. We have agreed to indemnify the underwriters against certain liabilities and expenses, including liabilities under the Securities Act, in connection with sales of the directed shares.

Discounts and Commissions. The following table shows the public offering price, underwriting discount and commissions and proceeds, before expenses to us. These amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

We estimate that the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $3.3 million and are payable by us. We also have agreed to reimburse the underwriters for up to $30,000 for their FINRA counsel fee. In accordance with FINRA Rule 5110, this reimbursed fee is deemed underwriting compensation for this offering.

		Total	
	Per Share	Without Over-Allotment	With Over Allotment
Public offering price	$ 19.00	$ 190,000,000	$ 218,500,000
Underwriting discount	1.33	13,300,000	15,295,000
Proceeds, before expenses, to Praxis Precision Medicines, Inc.	$ 17.67	$ 176,700,000	$ 203,205,000

The underwriters propose to offer the shares of common stock to the public at the public offering price set forth on the cover of this prospectus. The underwriters may offer the shares of common stock to securities dealers at the public offering price less a concession not in excess of $0.798 per share. If all of the shares are not sold at the public offering price, the underwriters may change the offering price and other selling terms.

Discretionary Accounts. The underwriters do not intend to confirm sales of the shares to any accounts over which they have discretionary authority.

Market Information. Prior to this offering, there has been no public market for shares of our common stock. The initial public offering price has been determined by negotiations between us and the representatives of the underwriters. In addition to prevailing market conditions, the factors considered in these negotiations included:

- the history of, and prospects for, our company and the industry in which we compete;
- our past and present financial information;
- an assessment of our management; its past and present operations, and the prospects for, and timing of, our future revenues;
- the present state of our development; and
- the above factors in relation to market values and various valuation measures of other companies engaged in activities similar to ours.

An active trading market for the shares may not develop. It is also possible that after the offering the shares will not trade in the public market at or above the initial public offering price.

Our common stock has been approved for listing on The Nasdaq Global Select Market under the symbol "PRAX".

Stabilization. In connection with this offering, the underwriters may engage in stabilizing transactions, overallotment transactions, syndicate covering transactions, penalty bids and purchases to cover positions created by short sales.

- Stabilizing transactions permit bids to purchase shares of common stock so long as the stabilizing bids do not exceed a specified maximum, and are engaged in for the purpose of preventing or retarding a decline in the market price of the common stock while the offering is in progress.
- Overallotment transactions involve sales by the underwriters of shares of common stock in excess of the number of shares the underwriters are obligated to purchase. This creates a syndicate short position which may be either a covered short position or a naked short position. In a covered short position, the number of shares over-allotted by the underwriters is not greater than the number of shares that they may purchase in the overallotment option. In a naked short position, the number of shares involved is greater than the number of shares in the overallotment option. The underwriters may close out any short position by exercising their overallotment option and/or purchasing shares in the open market.
- Syndicate covering transactions involve purchases of common stock in the open market after the distribution has been completed in order to cover syndicate short positions. In determining the source of shares to close out the short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared with the price at which they may purchase shares through exercise of the overallotment option. If the underwriters sell more shares than could be covered by exercise of the overallotment option and, therefore, have a naked short position, the position can be closed out only by buying shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that after pricing there could be downward pressure on the price of the shares in the open market that could adversely affect investors who purchase in the offering.
- Penalty bids permit the representatives to reclaim a selling concession from a syndicate member when the common stock originally sold by that syndicate member is purchased in stabilizing or syndicate covering transactions to cover syndicate short positions.

These stabilizing transactions, syndicate covering transactions and penalty bids may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock in the open market may be higher than it would otherwise be in the absence of these transactions. Neither we nor the underwriters make any representation or prediction as to the effect that the transactions described above may have on the price of our common stock. These transactions may be effected on the Nasdaq Stock Market, in the over-the-counter market or otherwise and, if commenced, may be discontinued at any time.

Passive Market Making. In connection with this offering, underwriters and selling group members may engage in passive market making transactions in our common stock on the Nasdaq Stock Market in accordance with Rule 103 of Regulation M under the Securities Exchange Act of 1934, as amended, during a period before the commencement of offers or sales of common stock and extending through the completion of the distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, such bid must then be lowered when specified purchase limits are exceeded.

Lock-Up Agreements. Pursuant to certain "lock-up" agreements, we and our executive officers, directors and the holders of substantially all of our capital stock and securities convertible into or exchangeable for our capital stock, have agreed, subject to certain exceptions, not to offer, sell, assign, transfer, pledge, contract to sell, or otherwise dispose of or announce the intention to otherwise dispose of, or enter into any swap, hedge or similar agreement or arrangement that transfers, in whole or in part, the economic consequence of ownership of, directly or indirectly, or make any demand or request or exercise any right with respect to the registration of, or file with the SEC a registration statement under the Securities Act relating to, any common stock or securities convertible into or exchangeable or exercisable for any common stock without the prior written consent of Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co. for a period of 180 days after the date of the pricing of the offering.

This lock-up provision applies to common stock and to securities convertible into or exchangeable or exercisable for common stock. It also applies to common stock owned now or acquired later by the person executing the agreement or for which the person executing the agreement later acquires the power of disposition. The exceptions permit us, among other things and subject to restrictions, to: (a) issue common stock or options pursuant to employee benefit plans, (b) issue common stock upon exercise of outstanding options or warrants or (c) file registration statements on Form S-8. The exceptions permit parties to the "lock-up" agreements, among other things and subject to restrictions, to: (a) make certain gifts, (b) if the party is a corporation, partnership, limited liability company or other business entity, make transfers to any shareholders, partners, members of or owners of similar equity interests in, the party, or to an affiliate of the party, if such transfer is not for value, (c) if the party is a corporation, partnership, limited liability company or other business entity, make transfers in connection with the sale or transfer of all of the party's capital stock, partnership interests, membership interests or other similar equity interests, as the case may be, or all or substantially all of the party's assets, in any such case not undertaken for the purpose of avoiding the restrictions imposed by the "lock-up" agreement and (d) participate in tenders involving the acquisition of 75% or more of our stock. In addition, the lock-up provision will not restrict broker-dealers from engaging in market making and similar activities conducted in the ordinary course of their business.

Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co., in their sole discretion, may release our common stock and other securities subject to the lock-up agreements described above in whole or in part at any time. In certain circumstances, the release of shares of common stock from the lock-up restrictions described above will trigger a pro rata release of shares of common stock held by certain other holders. When determining whether or not to release our common stock and other securities from lock-up agreements, Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co. will consider, among other factors, the holder's reasons for requesting the release, the number of shares for which the release is being requested and market conditions at the time of the request. In the event of such a release or waiver for one of our directors or officers, Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co. shall provide us with notice of the impending release or waiver at least three business days before the effective date of such release or waiver and we will announce the impending release or waiver by issuing a press release at least two business days before the effective date of the release or waiver.

Other Relationships. Certain of the underwriters and their affiliates have provided, and may in the future provide, various investment banking, commercial banking and other financial services for us and our affiliates for which they have received, and may in the future receive, customary fees.

Clarus Life Sciences III, L.P., or Clarus, an entity affiliated with Blackstone Securities Partners L.P., purchased (i) an aggregate of 7,375,799 shares of our Series A preferred stock from October 2016 to July 2017, including shares received upon conversion of convertible promissory notes previously issued, (ii) an aggregate of 5,052,852 shares of our Series B preferred stock from March

2018 to October 2018, including shares received upon conversion of convertible promissory notes previously issued, (iii) 1,410,477 shares of our Series B-1 preferred stock in June 2019; (iv) 559,208 shares of our Series C preferred stock in November 2019; and (v) 352,734 shares of our Series C-1 preferred stock in July 2020. In addition, BSOF Parallel Master Fund L.P., or BSOF, an entity affiliated with Blackstone Securities Partners L.P. purchased 1,941,748 shares of our Series C preferred stock in April 2020. Clarus and BSOF, together the Blackstone Entities, have agreed, pursuant to Rule 5110(g) of the Financial Industry Regulatory Authority, Inc., or FINRA Rule 5110(g), that such shares of Series A preferred stock, Series B preferred stock, Series B-1 preferred stock, Series C preferred stock and Series C-1 preferred stock, or together the Preferred Stock, and the shares of our common stock to be issued to the Blackstone Entities upon conversion of the Preferred Stock in connection with this offering will not be sold during this offering, or sold, transferred, assigned, pledged, or hypothecated, or be the subject of any hedging, short sale, derivative, put or call transaction that would result in the effective economic disposition of such shares of the Preferred Stock or common stock by any person for a period of 180 days immediately following the date of effectiveness of the registration statement of which this prospectus is a part or commencement of sales of common stock in this offering, except as permitted by FINRA Rule 5110(g)(2).

Conflicts of Interest. The Blackstone Entities, which beneficially own more than 10% of our outstanding preferred equity prior to the consummation of this offering, are affiliates of Blackstone Securities Partners L.P., an underwriter in this offering. As a result, Blackstone Securities Partners L.P. is deemed to have a "conflict of interest" within the meaning of FINRA Rule 5121. Accordingly, this offering is being made in compliance with the applicable requirements of FINRA Rule 5121. A qualified independent underwriter is not necessary for this offering pursuant to FINRA Rule 5121(a)(1)(A). Pursuant to FINRA Rule 5121, Blackstone Securities Partners L.P. will not confirm any sales to any account over which it exercises discretionary authority without the specific prior written approval of the account holder.

Canada. The common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration Requirements, Exemptions and Ongoing Registrant Obligations*. Any resale of the common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 *Underwriting Conflicts* (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Switzerland. The securities will not be offered, directly or indirectly, to the public in Switzerland and this prospectus does not constitute a public offering prospectus as that term is understood pursuant to article 652a or 1156 of the Swiss Federal Code of Obligations.

European Economic Area and the United Kingdom. In relation to each Member State of the European Economic Area and the United Kingdom (each, a "Member State"), no shares have been

offered or will be offered pursuant to the offering to the public in that Member State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Member State or, where appropriate, approved in another Member State and notified to the competent authority in that Member State, all in accordance with the Prospectus Regulation, except that offers of shares may be made to the public in that Member State at any time under the following exemptions under the Prospectus Regulation:

A. to any legal entity which is a qualified investor as defined under the Prospectus Regulation;

B. to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation), subject to obtaining the prior consent of the underwriters; or

C. in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of shares shall require the Company or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the underwriters and the Company that it is a "qualified investor" within the meaning of Article 2(e) of the Prospectus Regulation.

In the case of any shares being offered to a financial intermediary as that term is used in Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Member State to qualified investors as so defined or in circumstances in which the prior consent of the underwriters have been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an "offer to the public" in relation to shares in any Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression "Prospectus Regulation" means Regulation (EU) 2017/1129.

References to the Prospectus Regulation includes, in relation to the United Kingdom, the Prospectus Regulation as it forms part of the United Kingdom domestic law by virtue of the European Union (Withdrawal) Act of 2018.

United Kingdom. In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Regulation) (i) who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the "Order") and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order and/or (iii) to whom it may otherwise be lawfully communicated (all such persons together being referred to as "relevant persons") in circumstances which have not resulted and will not result in an offer to the public of the shares in the United Kingdom within the meaning of the Financial Services and Markets Act 2000.

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons.

Hong Kong. The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) (the "SFO") of Hong Kong and any rules made thereunder; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong) (the "CO"), or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the SFO and any rules made thereunder.

Singapore. Each underwriter has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each underwriter has represented and agreed that it has not offered or sold any shares or caused the shares to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares or cause the shares to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares, whether directly or indirectly, to any person in Singapore other than:

A. to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time (the "SFA")) pursuant to Section 274 of the SFA;

B. to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or

C. otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

A. a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

B. a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities or securities-based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries' rights and interest (however described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

(i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

(ii) where no consideration is or will be given for the transfer;

(iii) where the transfer is by operation of law;

(iv) as specified in Section 276(7) of the SFA; or

(v) as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

Singapore SFA Product Classification—In connection with Section 309B of the SFA and the CMP Regulations 2018, unless otherwise specified before an offer of shares, we have determined, and hereby notify all relevant persons (as defined in Section 309A(1) of the SFA), that the shares are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Israel. In the State of Israel this prospectus shall not be regarded as an offer to the public to purchase shares of common stock under the Israeli Securities Law, 5728 – 1968, which requires a prospectus to be published and authorized by the Israel Securities Authority, if it complies with certain provisions of Section 15 of the Israeli Securities Law, 5728 – 1968, including, inter alia, if: (i) the offer is made, distributed or directed to not more than 35 investors, subject to certain conditions (the "Addressed Investors"); or (ii) the offer is made, distributed or directed to certain qualified investors defined in the First Addendum of the Israeli Securities Law, 5728 – 1968, subject to certain conditions (the "Qualified Investors"). The Qualified Investors shall not be taken into account in the count of the Addressed Investors and may be offered to purchase securities in addition to the 35 Addressed Investors. The company has not and will not take any action that would require it to publish a prospectus in accordance with and subject to the Israeli Securities Law, 5728 – 1968. We have not and will not distribute this prospectus or make, distribute or direct an offer to subscribe for our common stock to any person within the State of Israel, other than to Qualified Investors and up to 35 Addressed Investors.

Qualified Investors may have to submit written evidence that they meet the definitions set out in of the First Addendum to the Israeli Securities Law, 5728 – 1968. In particular, we may request, as a condition to be offered common stock, that Qualified Investors will each represent, warrant and certify to us and/or to anyone acting on our behalf: (i) that it is an investor falling within one of the categories listed in the First Addendum to the Israeli Securities Law, 5728 – 1968; (ii) which of the categories listed in the First Addendum to the Israeli Securities Law, 5728 – 1968 regarding Qualified Investors is applicable to it; (iii) that it will abide by all provisions set forth in the Israeli Securities Law, 5728 – 1968 and the regulations promulgated thereunder in connection with the offer to be issued common stock; (iv) that the shares of common stock that it will be issued are, subject to exemptions available under the Israeli Securities Law, 5728 – 1968: (a) for its own account; (b) for investment purposes only; and (c) not issued with a view to resale within the State of Israel, other than in accordance with the provisions of the Israeli Securities Law, 5728 – 1968; and (v) that it is willing to provide further evidence of its Qualified Investor status. Addressed Investors may have to submit written evidence in respect of their identity and may have to sign and submit a declaration containing, inter alia, the Addressed Investor's name, address and passport number or Israeli identification number.

We have not authorized and do not authorize the making of any offer of securities through any financial intermediary on our behalf, other than offers made by the underwriters and their respective affiliates, with a view to the final placement of the securities as contemplated in this document. Accordingly, no purchaser of the shares, other than the underwriters, is authorized to make any further offer of shares on our behalf or on behalf of the underwriters.

Electronic Offer, Sale and Distribution of Shares. A prospectus in electronic format may be made available on the websites maintained by one or more of the underwriters or selling group members, if

any, participating in this offering and one or more of the underwriters participating in this offering may distribute prospectuses electronically. The representatives may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the underwriters and selling group members that will make internet distributions on the same basis as other allocations. Other than the prospectus in electronic format, the information on these websites is not part of this prospectus or the registration statement of which this prospectus forms a part, has not been approved or endorsed by us or any underwriter in its capacity as underwriter, and should not be relied upon by investors.

LEGAL MATTERS

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters related to this offering will be passed upon for the underwriters by Mintz, Levin, Cohn, Ferris, Glovsky & Popeo, P.C., Boston, Massachusetts.

EXPERTS

The consolidated financial statements of Praxis Precision Medicines, Inc. at December 31, 2019 and 2018, and for each of the two years in the period ended December 31, 2019, appearing in this Prospectus and Registration Statement have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon (which contains an explanatory paragraph describing conditions that raise substantial doubt about the Company's ability to continue as a going concern as described in Note 1 to the consolidated financial statements) appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 (File Number 333-249074) under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon the completion of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. We also maintain a website at www.praxismedicines.com. Upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendment to those reported filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Index to Consolidated Financial Statements as of and for the Years Ended December 31, 2018 and 2019

Index to Unaudited Interim Condensed Consolidated Financial Statements as of June 30, 2020 and for the Six Months Ended June 30, 2019 and 2020

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Praxis Precision Medicines, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Praxis Precision Medicines, Inc. (the Company) as of December 31, 2019 and 2018, the related consolidated statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' (deficit) equity and cash flows for each of the two years in the period ended December 31, 2019, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2019 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, in conformity with U.S. generally accepted accounting principles.

Adoption of ASU No. 2016-02

As discussed in Note 2 to the consolidated financial statements, the Company changed its method of accounting for leases in 2019 due to the adoption of Accounting Standards Update (ASU) No.2016-02 Leases (Topic 842) and the related amendments.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations, has limited financial resources, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2019.
Boston, Massachusetts
July 22, 2020, except for Note 17, as to which the date is October 9, 2020

PRAXIS PRECISION MEDICINES, INC.

CONSOLIDATED BALANCE SHEETS

(Amounts in thousands, except share and per share data)

	December 31, 2018	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 17,950	$ 44,815
Prepaid expenses and other current assets	1,176	681
Total current assets	19,126	45,496
Property and equipment, net	103	128
Restricted cash	600	600
Operating lease right-of-use assets	–	1,450
Other non-current assets	–	20
Total assets	$ 19,829	$ 47,694
Liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity		
Current liabilities:		
Accounts payable	$ 3,391	$ 2,667
Accrued expenses	1,754	3,455
Operating lease liabilities	–	696
Total current liabilities	5,145	6,818
Long-term liabilities:		
Non-current portion of operating lease liabilities	–	763
Other long-term liabilities	2	–
Total liabilities	5,147	7,581
Commitments and contingencies (Note 8)		
Series A redeemable convertible preferred stock, $0.0001 par value; 8,075,799 shares authorized; 8,075,799 shares issued and outstanding as of December 31, 2018 and 2019; liquidation value as of December 31, 2018 and 2019 of $9,284 and $9,932, respectively	9,284	9,932
Series B redeemable convertible preferred stock, $0.0001 par value; 14,913,704 shares authorized; 14,913,704 shares issued and outstanding as of December 31, 2018 and 2019; liquidation value as of December 31, 2018 and 2019 of $46,381 and $49,969, respectively	46,436	49,969
Series B-1 redeemable convertible preferred stock, $0.0001 par value; 2,666,666 shares authorized; no shares issued or outstanding as of December 31, 2018, and 2,666,666 shares issued and outstanding as of December 31, 2019; liquidation value as of December 31, 2019 of $10,431	–	10,431
Series C redeemable convertible preferred stock, $0.0001 par value; 11,067,963 shares authorized; no shares issued or outstanding as of December 31, 2018, and 9,805,827 shares issued and outstanding as of December 31, 2019; liquidation value as of December 31, 2019 of $50,789	–	50,789
Stockholders' (deficit) equity:		
Common stock, $0.0001 par value; 46,000,000 shares authorized; 1,670,070 shares issued and 1,408,677 shares outstanding as of December 31, 2018, and 1,670,070 shares issued and 1,621,880 shares outstanding as of December 31, 2019	1	1
Additional paid-in capital	326	–
Accumulated deficit	(41,365)	(81,009)
Total stockholders' (deficit) equity	(41,038)	(81,008)
Total liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity	$ 19,829	$ 47,694

The accompanying notes are an integral part of these consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(Amounts in thousands, except share and per share data)

	Year Ended December 31,	
	2018	2019
Operating expenses:		
Research and development	$ 18,820	$ 29,557
General and administrative	3,899	6,232
Total operating expenses	22,719	35,789
Loss from operations	(22,719)	(35,789)
Total other income (expense):		
Interest income	92	193
Interest expense	(127)	–
Other expense	(3,648)	–
Total other income (expense), net	(3,683)	193
Loss before provision for (benefit from) income taxes	(26,402)	(35,596)
Provision for (benefit from) income taxes	133	(84)
Net loss and comprehensive loss	$ (26,535)	$ (35,512)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,296)	(5,170)
Loss on conversion of convertible notes	(392)	–
Net loss attributable to common stockholders	$ (29,223)	$ (40,682)
Net loss per share attributable to common stockholders, basic and diluted	$ (22.52)	$ (26.60)
Weighted average common shares outstanding, basic and diluted	1,297,633	1,529,629
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)		$ (2.68)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)		13,270,761

The accompanying notes are an integral part of these consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' (DEFICIT) EQUITY

(Amounts in thousands, except share data)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series B-1 Redeemable Convertible Preferred Stock		Series C Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at December 31, 2017	8,075,799	$ 9,031	–	$ –	–	$ –	–	$ –	1,174,547	$ 1	$ –	$ (14,438)	$ (14,437)
Conversion of convertible notes to Series B redeemable convertible preferred stock	–	–	1,286,185	3,074	–	–	–	–	–	–	–	(392)	(392)
Issuance of Series B redeemable convertible preferred stock, net of issuance costs of $197	–	–	13,627,519	43,362	–	–	–	–	–	–	–	–	–
Stock-based compensation expense	–	–	–	–	–	–	–	–	–	–	579	–	579
Accretion of redeemable convertible preferred stock to redemption value	–	253	–	–	–	–	–	–	–	–	(253)	–	(253)
Vesting of restricted stock awards	–	–	–	–	–	–	–	–	234,130	–	–	–	–
Net loss	–	–	–	–	–	–	–	–	–	–	–	(26,535)	(26,535)
Balance at December 31, 2018	8,075,799	$ 9,284	14,913,704	$ 46,436	–	$ –	–	$ –	1,408,677	$ 1	$ 326	$ (41,365)	$ (41,038)
Issuance of Series B-1 redeemable convertible preferred stock, net of issuance costs of $61	–	–	–	–	2,666,666	9,939	–	–	–	–	–	–	–
Issuance of Series C redeemable convertible preferred stock, net of issuance costs of $165	–	–	–	–	–	–	9,805,827	50,336	–	–	–	–	–
Stock-based compensation expense	–	–	–	–	–	–	–	–	–	–	668	–	668
Accretion of redeemable convertible preferred stock to redemption value	–	648	–	3,533	–	492	–	453	–	–	(994)	(4,132)	(5,126)
Vesting of restricted stock awards	–	–	–	–	–	–	–	–	213,203	–	–	–	–
Net loss	–	–	–	–	–	–	–	–	–	–	–	(35,512)	(35,512)
Balance at December 31, 2019	8,075,799	$9,932	14,913,704	$49,969	2,666,666	$10,431	9,805,827	$50,789	1,621,880	$ 1	$ –	$ (81,009)	$ (81,008)

The accompanying notes are an integral part of these consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(Amounts in thousands)

	Year Ended December 31, 2018	2019
Cash flows from operating activities:		
Net loss	$ (26,535)	$ (35,512)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	1	37
Stock-based compensation expense	579	668
Change in fair value of financial instruments	3,648	–
Non-cash operating lease expense	–	642
Non-cash interest expense	127	–
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(867)	495
Accounts payable	1,667	(837)
Accrued expenses	659	1,742
Operating lease liabilities	–	(633)
Other	–	(22)
Net cash used in operating activities	(20,721)	(33,420)
Cash flows from investing activities:		
Purchases of property and equipment	(63)	(103)
Net cash used in investing activities	(63)	(103)
Cash flows from financing activities:		
Proceeds from issuance of convertible note	1,000	–
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	36,804	60,388
Net cash provided by financing activities	37,804	60,388
Increase in cash, cash equivalents and restricted cash	17,020	26,865
Cash, cash equivalents and restricted cash, beginning of period	1,530	18,550
Cash, cash equivalents and restricted cash, end of period	$ 18,550	$ 45,415
Supplemental disclosures of non-cash activities:		
Issuance of Series B redeemable convertible preferred stock upon settlement of convertible notes	$ 3,074	$ –
Settlement of derivative liabilities upon issuance of Series B redeemable convertible preferred stock	$ 5,406	$ –
Issuance of Series B redeemable convertible preferred stock to acquire Purdue license	$ 3,738	$ –
Accretion of redeemable convertible preferred stock to redemption value	$ 253	$ 5,126
Operating lease liabilities recorded upon adoption of ASC 842	$ –	$ 2,092
Interest expense converted into Series B redeemable convertible preferred stock	$ 127	$ –
Purchases of property and equipment included in accrued expenses	$ 41	$ –
Redeemable convertible preferred stock issuance costs included in accounts payable	$ –	$ 113

The accompanying notes are an integral part of these consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of the Business

Praxis Precision Medicines, Inc. ("Praxis" or the "Company") is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system ("CNS") disorders characterized by neuronal imbalance. The Company has established a broad portfolio, including five disclosed programs across multiple CNS disorders, including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. The Company's most advanced programs, PRAX-114 and PRAX-944, are currently in Phase 2 development. PRAX-114 is being developed for the treatment of major depressive disorder and perimenopausal depression, and PRAX-944 is a selective small molecule inhibitor of T-type calcium channels for the treatment of Essential Tremor.

Praxis was incorporated in 2015. The Company has funded its operations primarily with proceeds from the issuance of convertible debt, Series A redeemable convertible preferred stock (the "Series A Preferred Stock"), Series B redeemable convertible preferred stock (the "Series B Preferred Stock"), Series B-1 redeemable convertible preferred stock (the "Series B-1 Preferred Stock") and Series C redeemable convertible preferred stock (the "Series C Preferred Stock") (the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock and Series C Preferred Stock are collectively referred to as the "Preferred Stock"). From inception through December 31, 2019, the Company raised $107.1 million in aggregate cash proceeds from these transactions, net of issuance costs. On February 19, 2020 and March 3, 2020, the Company repurchased shares of Series C Preferred Stock for an aggregate cash repurchase price of $30.0 million. On April 15, 2020 and May 8, 2020, the Company sold and issued additional shares of Series C Preferred Stock for aggregate cash proceeds of $23.5 million.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Programs currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Going Concern

In accordance with the Financial Accounting Standards Board Accounting Standards Update 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the consolidated financial statements are issued.

The Company has incurred recurring losses since its inception, including net losses of $26.5 million and $35.5 million for the years ended December 31, 2018 and 2019, respectively. In addition, as of December 31, 2019, the Company had an accumulated deficit of $81.0 million. The Company expects to continue to generate operating losses for the foreseeable future. The future viability of the Company is dependent on its ability to raise additional capital to finance its operations.

The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurance that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

The Company expects that its cash and cash equivalents as of December 31, 2019 of $44.8 million, together with the $23.5 million aggregate cash proceeds from the sale and issuance of additional shares of Series C Preferred Stock on April 15, 2020 and May 8, 2020, offset by the repurchase of shares of Series C Preferred Stock for an aggregate cash repurchase price of $30.0 million on February 19, 2020 and March 3, 2020, will not be sufficient to fund the operating expenses and capital expenditure requirements necessary to advance its research efforts and clinical trials for at least the next twelve months from the date of issuance of these consolidated financial statements, and the Company will need to obtain additional funding. The future viability of the Company beyond one year from the date of issuance of these consolidated financial statements is dependent on its ability to raise additional capital to finance its operations. If the Company is unable to obtain additional funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion, or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations. The Company expects to seek additional funding through private or public equity transactions, debt financings, or other capital sources, including collaborations with other companies or other strategic transactions.

Although management plans to pursue additional funding, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

Based on its recurring losses and negative cash flows from operations since inception, expectation of continuing operating losses and negative cash flows from operations for the foreseeable future, and the need to raise additional capital to finance its future operations, management concluded that there is substantial doubt about the Company's ability to continue as a going concern for one year after the date that these consolidated financial statements are issued.

The accompanying consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Principles of Consolidation

The consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries, Praxis Security Corporation and Praxis Precision Medicines Australia Pty Ltd. All intercompany transactions have been eliminated in consolidation.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual for research and development expenses, stock-based compensation expense, the valuation of equity and derivative instruments and the recoverability of the Company's net deferred tax assets and related valuation allowance. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from those estimates.

Unaudited Pro Forma Information

Upon the closing of a qualified public offering (as defined in the Company's Amended and Restated Certificate of Incorporation), all of the Company's outstanding shares of redeemable convertible preferred stock will automatically convert into shares of common stock. The unaudited pro forma basic and diluted net loss per share attributable to common stockholders in the accompanying consolidated statement of operations and comprehensive loss for the year ended December 31, 2019 was computed using the weighted average number of shares of common stock outstanding, including the pro forma effect of the automatic conversion of all outstanding shares of redeemable convertible preferred stock into shares of common stock as if the Company's proposed initial public offering ("IPO") had occurred on the later of: (i) January 1, 2019 or (ii) the date the equity instruments were issued. The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019: (i) excludes the effects of cumulative dividends accrued for redeemable convertible preferred stock from the net loss attributable to common stockholders and (ii) excludes the effects of other accretion recorded for redeemable convertible preferred stock from the net loss attributable to common stockholders. The unaudited pro forma basic and diluted net loss per share attributable to common stockholders does not include the shares expected to be sold or related proceeds to be received in the proposed IPO.

Segments

The Company has one operating segment. The Company's chief operating decision maker, its Chief Executive Officer, manages the Company's operations on a consolidated basis for the purposes of assessing performance and allocating resources. The majority of the Company's long-lived assets are held in the United States.

Cash, Cash Equivalents and Restricted Cash

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks

and amounts held in interest-bearing money market funds. Cash equivalents are carried at cost, which approximates their fair market value.

Restricted cash comprises a letter of credit for the benefit of the landlord in connection with the Company's lease facility. Restricted cash is classified as either current or non-current based on the terms of the underlying lease arrangement.

The following table presents cash, cash equivalents and restricted cash as reported on the consolidated balance sheets that equal the total amounts on the consolidated statements of cash flows (in thousands):

	December 31,	
	2018	2019
Cash and cash equivalents	$17,950	$44,815
Restricted cash	600	600
Total cash, cash equivalents and restricted cash as shown on the consolidated statement of cash flows	$18,550	$45,415

Concentrations of Credit Risk and Significant Suppliers and License Agreements

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and restricted cash. The Company maintains deposits in accredited financial institutions in excess of federally insured limits. Bank accounts in the United States are insured by the Federal Deposit Insurance Corporation (the "FDIC") up to $250,000. As of December 31, 2018 and 2019, the Company's primary operating accounts significantly exceeded the FDIC limits. The Company deposits its cash in financial institutions that it believes have high credit quality, and has not experienced any losses on such accounts and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply materials for research and development activities of its programs, including preclinical and clinical testing. In particular, the Company relies, and expects to continue to rely, on a small number of third-party manufacturers to produce and process its current and potential product candidates and to manufacture supply of its current and potential product candidates for preclinical and clinical activities. These programs could be adversely affected by a significant interruption in the supply of the necessary materials. The Company is also dependent on third parties who provide license rights used in the development of certain programs. The Company could experience delays in the development of its programs if any of these license agreements are terminated, if the Company fails to meet the obligations required under its arrangements, or if the Company is unable to successfully secure new strategic alliances or licensing agreements.

Off-Balance Sheet Risk

As of December 31, 2018 and 2019, the Company had no off-balance sheet risk such as foreign exchange contracts, option contracts, or other hedging arrangements.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Fair Value Measurements

Certain assets and liabilities of the Company are carried at fair value under GAAP. The fair values of the Company's financial assets and liabilities reflects the Company's estimate of the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

An entity may choose to measure many financial instruments and certain other items at fair value at specified election dates. Subsequent unrealized gains and losses on items for which the fair value option has been elected will be reported in earnings.

The carrying amounts reflected in the consolidated balance sheets for cash, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Property and Equipment

Property and equipment are recorded at cost. Depreciation is calculated using the straight-line method over the following estimated useful lives of the assets:

	Estimated Useful Life
Office furniture and equipment	5 years
Laboratory equipment	3 years
Computer equipment	3 years

Upon disposal, retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the results of

operations. Expenditures for repairs and maintenance that do not improve or extend the lives of the respective assets are charged to expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. The Company did not record any impairment losses on long-lived assets in either year ended December 31, 2018 or 2019.

Leases

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*. This standard established a right-of-use model that requires all lessees to recognize right-of-use assets and lease liabilities on their balance sheet that arise from leases as well as provide disclosures with respect to certain qualitative and quantitative information related to a company's leasing arrangements to meet the objective of allowing users of financial statements to assess the amount, timing and uncertainty of cash flows arising from leases. The FASB subsequently issued the following amendments to ASU 2016-02 that have the same effective date and transition date: ASU No. 2018-01, *Leases (Topic 842): Land Easement Practical Expedient for Transition to Topic 842*, ASU No. 2018-10, *Codification Improvements to Topic 842, Leases*, ASU No. 2018-11, *Leases (Topic 842): Targeted Improvements*, ASU No. 2018-20, *Narrow-Scope Improvement for Lessors*, and ASU No. 2019-01, *Leases (Topic 842): Codification Improvements*. The Company adopted these amendments with ASU 2016-02 (collectively, the "new leasing standards"), effective January 1, 2019.

The Company adopted the new leasing standards using the modified retrospective transition approach, with no restatement of prior periods and there was no cumulative adjustment to retained earnings. Upon adoption, the Company elected the package of transition practical expedients, which allowed the Company to not reassess the following: (i) whether any expired or existing contracts are or contain leases, (ii) the lease classification for any expired or existing leases and (iii) the treatment of initial direct costs for existing leases. The Company made an accounting policy election to not recognize short-term leases with an initial term of twelve months or less within its consolidated balance sheets and to recognize those lease payments on a straight-line basis in its consolidated statements of operations over the lease term. Upon adopting the new leasing standards, the Company recognized an operating lease right-of-use asset of $2.1 million and a corresponding operating lease liability of $2.1 million, which are included in its consolidated balance sheets. The adoption of the new leasing standards did not have a material impact on the Company's consolidated statements of operations.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

The Company determines if an arrangement is a lease at contract inception. Operating lease right-of-use assets represent the Company's right to use an underlying asset for the lease term and operating lease liabilities represent its obligation to make lease payments arising from the lease. Operating right-of-use assets and liabilities are recognized at the commencement date of the lease based upon the present value of lease payments over the lease term. When determining the lease term, the Company includes options to extend or terminate the lease when it is reasonably certain that the option will be exercised.

The Company uses the implicit rate when readily determinable and uses its incremental borrowing rate when the implicit rate is not readily determinable, based upon the information available at the commencement date in determining the present value of the lease payments. The incremental borrowing rate is determined using a secured borrowing rate for the same currency and term as the associated lease.

The lease payments used to determine operating lease right-of-use assets may include lease incentives and stated rent increases. The Company's lease agreements may include both lease and non-lease components, which the Company accounts for as a single lease component when the payments are fixed, for all classes of underlying assets. Variable payments included in the lease agreement are expensed as incurred.

The Company's operating leases are reflected in operating lease right-of-use assets and in current operating lease liabilities and long-term operating lease liabilities in its consolidated balance sheets. The Company's operating lease right-of-use asset as of December 31, 2019 did not include any lease incentives. Lease expense for future lease payments is recognized on a straight-line basis over the lease term.

Prior to the adoption of the new leasing standards, the Company recognized lease costs on a straight-line basis once it gained control of the space, without regard to deferred payment terms, such as rent holidays, that would defer the commencement date of required payments or escalating payment amounts. Any lease incentives received were treated as a reduction of costs over the term of the lease agreement, as they were considered an inseparable part of the lease agreement. The difference between required lease payments and rent expense was recorded as deferred rent, which was included in other non-current liabilities in the December 31, 2018 consolidated balance sheet.

Redeemable Convertible Preferred Stock

The Company records all redeemable convertible preferred stock upon issuance at its respective fair value or original issuance price, less issuance costs and any associated discounts. The Company classifies its redeemable convertible preferred stock outside of stockholders' (deficit) equity as the redemption of such shares is outside the Company's control. The Company adjusts the carrying values of the redeemable convertible preferred stock to redemption value when the redemption value exceeds the carrying value.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including salaries, stock-based compensation and benefits, facilities costs, depreciation, third-party license fees, and external

costs of outside vendors engaged to conduct preclinical development activities and clinical trials as well as to manufacture research and development materials. Non-refundable prepayments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as an expense as the goods are delivered or the related services are performed or until it is no longer expected that the goods will be delivered or the services rendered. Costs incurred to obtain licenses are recognized as research and development expense as incurred if the technology licensed has no alternative future uses.

The Company has entered into various research and development related contracts with parties both inside and outside of the United States. These agreements are cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accrued liabilities for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent-Related Costs

Patent-related costs incurred in connection with patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses in the accompanying consolidated statement of operations and comprehensive loss.

Stock-Based Compensation

The Company accounts for all stock-based awards granted to employees and non-employees as stock-based compensation expense at fair value in accordance with FASB ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). For stock-based awards issued to employees, non-employees and members of the Company's board of directors (the "Board") for their services on the Board, the Company measures the estimated fair value of the stock-based award on the date of the grant. The Company recognizes compensation expense for those awards granted to employees and members of the Board over the requisite service period, which is generally the vesting period of the respective award. For non-employee awards, compensation expense is recognized as the services are provided, which is generally ratably over the vesting period. The Company determines the fair value of restricted stock awards in reference to the fair value of its common stock less any applicable purchase price. The Company issues stock-based awards with service-based vesting conditions and records the expense for these awards on a straight-line basis over the vesting period. To date, the Company has not issued any stock-based awards with performance or market-based vesting conditions. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in its consolidated statement of operations and comprehensive loss in the same manner in which the award recipient's salary or service payments are classified.

Given the absence of an active market for the Company's common stock, the fair value of shares of common stock underlying the Company's stock-based awards is determined on each grant date.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

The Company determines the estimated fair value of its equity instruments based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector. The Company utilizes various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its common stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors in determining the value of the Company's common stock at each grant date, such as the following: (i) prices paid for the Company's redeemable convertible preferred stock, and the rights, preferences, and privileges of the Company's redeemable convertible preferred stock and common stock; (ii) the Company's stage of development; (iii) the fact that the grants of stock-based awards related to illiquid securities in a private company; and (iv) the likelihood of achieving a liquidity event for the common stock underlying the stock-based awards, such as an IPO or sale of the Company, given prevailing market conditions. The methodology utilized to estimate the fair value of the Company's common stock was the option-pricing method ("OPM") to back-solve the estimated value of the Company's equity and corresponding value of the Company's common stock.

The fair value of each stock option award is estimated on the date of grant using the Black-Scholes option pricing model, which requires inputs of subjective assumptions, including: (i) the expected volatility of the Company's stock; (ii) the expected term of the award; (iii) the risk-free interest rate; (iv) expected dividends; and (v) the fair value of common stock. Due to the lack of Company-specific historical and implied volatility data, the Company determines the volatility for awards granted based on an analysis of reported data for a group of guideline publicly-traded companies that issued options with substantially similar terms. For this analysis, the Company selects companies with comparable characteristics including enterprise value, risk profiles, and with historical share price information sufficient to meet the expected life of the stock-based awards. The Company determines expected volatility using a weighted average of the historical volatilities of the guideline group of companies. The Company expects to continue to apply this process until such time as it has adequate historical data regarding the volatility of its own traded stock price. As permitted under ASC 718, the Company has elected to use the contractual term as the expected term for certain non-employee awards, on an award-by-award basis. For all other awards, the expected term of the Company's stock options has been determined utilizing the "simplified" method whereby the expected term equals the average of the vesting term and the original contractual term of the option, on a weighted basis based on the vesting of each tranche. The Company utilizes this method as it has insufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for instruments with a term commensurate with the expected term assumption. The expected dividend yield is assumed to be zero as the Company has never paid dividends, and does not have current plans to pay any dividends on its common stock.

Foreign Currency

The functional currency of the Company's wholly owned foreign subsidiary in Australia is the U.S. dollar. Monetary assets and liabilities resulting from transactions denominated in currencies other than the functional currency are remeasured into the functional currency at exchange rates prevailing at the balance sheet date. Non-monetary assets and liabilities denominated in foreign currencies are measured using historical exchange rates prevailing at the date of the transaction and are not subsequently remeasured. Exchange gains or losses arising from foreign currency transactions are

included in the determination of net loss. The Company recorded a foreign currency loss of $0.3 million for the year ended December 31, 2018, which is included in research and development expense in the consolidated statement of operations and comprehensive loss. There were no material foreign currency gains or losses for the year ended December 31, 2019.

Income Taxes

Deferred tax assets and liabilities are determined on the basis of the differences between the consolidated financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established.

The Company accounts for uncertain tax positions recognized in the consolidated financial statements by prescribing a more-likely-than-not threshold for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Comprehensive Loss

Comprehensive loss includes net loss and certain changes in stockholders' deficit that are excluded from net loss. The Company's comprehensive loss was equal to net loss for the years ended December 31, 2018 and 2019.

Net Loss per Share

The Company follows the two-class method to compute net loss per share attributable to common stockholders as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (losses) for the period to be allocated between common and participating securities based upon their respective rights to share in the earnings as if all income (losses) for the period had been distributed. During periods of loss, there is no allocation required under the two-class method since the participating securities do not have a contractual obligation to fund the losses of the Company.

Net loss attributable to common stockholders is equal to the net loss for the period, as adjusted for: (i) cumulative dividends accrued for redeemable convertible preferred stock, whether or not declared, (ii) increases in carrying value recorded for redeemable convertible preferred stock, including accretion on redeemable convertible preferred stock to redemption value for amounts other than cumulative dividends and (iii) losses resulting from conversions of convertible notes recorded as capital transactions.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, which excludes shares of restricted common stock that are not vested. Diluted net loss

per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, after giving consideration to the dilutive effect of potentially dilutive common shares. For purposes of this calculation, outstanding options to purchase shares of common stock, unvested shares of restricted common stock and shares of redeemable convertible preferred stock are considered potentially dilutive common shares. The Company has generated a net loss in all periods presented so the basic and diluted net loss per share attributable to common stockholders are the same as the inclusion of the potentially dilutive securities would be anti-dilutive.

Emerging Growth Company Status

The Company is an "emerging growth company" ("EGC"), as defined in the Jumpstart Our Business Startups Act ("JOBS Act"), and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not EGCs. The Company may take advantage of these exemptions until it is no longer an EGC under Section 107 of the JOBS Act, which provides that an EGC can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. The Company has elected to avail itself of the extended transition period and, therefore, while the Company is an EGC it will not be subject to new or revised accounting standards the same time that they become applicable to other public companies that are not EGCs, unless it chooses to early adopt a new or revised accounting standard.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments—Overall (Subtopic 825-10)—Recognition and Measurement of Financial Assets and Financial Liabilities*, which has been subsequently amended by ASU No. 2018-03, ASU No. 2019-04, ASU No. 2020-01 and ASU No. 2020-03 ("ASU 2016-01"). The provisions of ASU 2016-01 make targeted improvements to enhance the reporting model for financial instruments to provide users of financial statements with more decision-useful information, including certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. The Company early adopted ASU 2016-01 effective January 1, 2019. The implementation of this standard had no impact on the Company's financial position or results of operations.

In February 2016, the FASB issued the new leasing standards to increase transparency and comparability among organizations related to their leasing activities. The Company early adopted the new leasing standards effective January 1, 2019. For additional information on the adoption of the new leasing standards, please read the Company's policy above entitled *Leases*, and Note 8, *Commitments and Contingencies*, to these consolidated financial statements.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments* ("ASU 2016-15"), to address diversity in practice in how certain cash receipts and cash payments are presented and classified in the consolidated statement of cash flows. The Company early adopted ASU 2016-15 effective January 1, 2018. The adoption of ASU 2016-15 had no impact on the Company's financial position or results of operations.

In January 2017, the FASB issued ASU No. 2017-01, *Business Combinations (Topic 805): Clarifying the Definition of a Business* ("ASU 2017-01"). The standard clarifies the framework for

determining whether an integrated set of assets and activities meets the definition of a business. The revised framework establishes a screen for determining whether an integrated set of assets and activities is a business and narrows the definition of a business. The screen requires that when substantially all of the fair value of the gross assets acquired (or disposed of) is concentrated in a single identifiable asset or a group of similar identifiable assets, the set is not a business. This screen reduces the number of transactions that need to be further evaluated. The Company early adopted ASU 2017-01 effective January 1, 2018. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. The standard is effective for annual periods beginning after December 15, 2017. The Company adopted ASU 2017-09 effective January 1, 2018. The adoption of ASU 2017-09 had no impact on the Company's financial position or results of operations.

In July 2017, the FASB issued ASU No. 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) I. Accounting for Certain Financial Instruments with Down Round Features II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* ("ASU 2017-11"). Part I applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. The Company early adopted ASU 2017-11 effective January 1, 2019. The adoption of ASU 2017-11 had no impact on the Company's financial position or results of operations.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"). The standard eliminates, adds and modifies certain disclosure requirements for fair value measurements. The Company early adopted ASU 2018-13 as of January 1, 2018. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326)—Measurement of Credit Losses on Financial Instruments,* which has been subsequently amended by ASU No. 2018-19, ASU No. 2019-04, ASU No. 2019-05, ASU No. 2019-10, ASU No. 2019-11 and ASU No. 2020-03 ("ASU 2016-13"). The provisions of ASU 2016-13 modify the impairment model to utilize an expected loss methodology in place of the currently used incurred loss methodology and require a consideration of a broader range of reasonable and supportable information to inform credit loss estimates. ASU 2016-13 is effective for the Company on January 1, 2023, with early adoption permitted. The Company is currently evaluating the potential impact that this

standard may have on its financial position and results of operations, as well as the timing of its adoption of this standard.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740)—Simplifying the Accounting for Income Taxes* ("ASU 2019-12"), as part of its initiative to reduce complexity in the accounting standards. The amendments in ASU 2019-12 eliminate certain exceptions related to the approach for intraperiod tax allocation, the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. ASU 2019-12 also clarifies and simplifies other aspects of the accounting for income taxes. ASU 2019-12 is effective for the Company on January 1, 2022, with early adoption permitted. The Company is currently evaluating the potential impact that this standard may have on its financial position and results of operations, as well as the timing of its adoption of this standard.

3. Restricted Cash

As of December 31, 2018 and 2019, the Company had restricted cash of $0.6 million, held as a letter of credit for the benefit of the landlord in connection with the Company's lease in Cambridge, Massachusetts. Restricted cash was classified as a non-current asset on the consolidated balance sheets as the associated lease term expires more than twelve months from each respective consolidated balance sheet date.

4. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	As of December 31, 2018			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$15,739	$ –	$ –	$15,739
	$15,739	$–	$–	$15,739

	As of December 31, 2019			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$44,429	$ –	$ –	$44,429
	$44,429	$–	$–	$44,429

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

The following table sets forth a summary of changes in fair value of the Company's derivative liabilities for which fair value was determined by Level 3 inputs (in thousands):

	Series B Preferred Stock Tranche Obligation	Anti-Dilution Obligation	Conversion Features	Total
Balance as of December 31, 2017	$ –	$ 2,392	$ 614	$ 3,006
Fair value on measurement date	3,185	–	304	3,489
Change in fair value	2,221	1,346	81	3,648
Settlement of liability	(5,406)	(3,738)	(999)	(10,143)
Balance as of December 31, 2018	$ –	$ –	$ –	$ –

During the years ended December 31, 2018 and 2019, there were no transfers into or out of Level 3.

Series B Preferred Stock Tranche Obligation

The Series B Preferred Stock purchase agreement provided for an initial closing on March 13, 2018 and a subsequent closing upon the occurrence of a specified clinical milestone event (the "Milestone Closing"). The Milestone Closing required the Company to sell, and certain investors to purchase, a total of 7,880,000 additional shares of Series B Preferred Stock at $3.00 per share on the same terms and conditions as the initial closing (the "Preferred Stock Tranche Obligation"). The Board and Preferred Stock investors determined that the related clinical milestone event was achieved on June 25, 2018.

The Company concluded that the Preferred Stock Tranche Obligation represented a freestanding financial instrument as the underlying shares could be transferred separately from the tranche right. The freestanding financial instrument was classified as a liability on the Company's consolidated balance sheet and initially recorded at fair value. The initial fair value of the Preferred Stock Tranche Obligation recognized in connection with the Company's issuance of the Series B Preferred Stock in March 2018 was determined using a binomial model with significant inputs not observable in the market, including the estimated future value of the Company's Series B Preferred Stock, the discount rate, estimated time from the initial closing to the tranche closing, and probability of the tranche closing. Therefore, the derivative liability represented a Level 3 measurement within the fair value hierarchy.

A change in the assumptions related to the valuation of the Preferred Stock Tranche Obligation could have a significant impact on the value of the obligation. The following reflects the significant quantitative inputs used in the valuation of the Preferred Stock Tranche Obligation upon the issuance of the associated shares of Series B Preferred Stock on March 13, 2018:

Future value of Series B Preferred Stock	$ 3.69
Discount rate	1.90%
Time from initial closing to tranche closing (in years)	0.72
Probability of tranche closing	59.10%

The Company determined the per-share future value of the Series B Preferred Stock by back-solving from the initial proceeds of the Series B Preferred Stock closing. The discount rate was estimated using the capital asset pricing model. The time to tranche closing and probability of tranche closing was determined using industry data on clinical trial timing and success.

The obligation was fully satisfied in October 2018 upon the second closing of the Series B Preferred Stock. Upon settlement of the Preferred Stock Tranche Obligation, the Company remeasured the fair value using current assumptions. Upon settlement, the time from the measurement date to the settlement date was zero, and the probability of closing was 100%. The fair value of the tranche obligation at settlement was determined using a retrospective binomial valuation, driven by the difference between the fair value of the Series B Preferred Stock upon settlement of $3.69 and the exercise price of the forward contract of $3.00.

Anti-Dilution Obligation

Under a license agreement entered into with Purdue Neuroscience Company (“Purdue”) in December 2017, as partial consideration for the exclusive license provided to the Company under the license agreement, the Company issued Purdue a right to receive additional shares of Preferred Stock, for no additional consideration from Purdue, to ensure Purdue’s ownership remained at a specified percentage throughout future issuances of the Series B Preferred Stock (the “Anti-Dilution Obligation”).

The Company determined that the Anti-Dilution Obligation represented a derivative liability as it was a freestanding instrument representing a conditional obligation to issue additional shares of the Company’s optionally redeemable equity securities in exchange for no additional consideration. The fair value of the Anti-Dilution Obligation was determined using a discounted cash flow model under the income approach, based on significant inputs not observable in the market including the estimated future value of the Series B Preferred Stock, discount rates, estimated time to liquidity, and probability of each tranche closing. Therefore, the derivative liability represented a Level 3 measurement within the fair value hierarchy. A change in the assumptions related to the valuation of the Anti-Dilution Obligation could have a significant impact on the value of the obligation. The initial fair value of the derivative liability of $2.4 million was recorded as research and development expense in December 2017.

The Company issued shares of Series B Preferred Stock to Purdue under the Anti-Dilution Obligation upon the initial and second tranche financing of the Series B Preferred Stock. The Anti-Dilution Obligation was settled in October 2018 upon the final tranche closing of the Series B Preferred Stock. Upon settlement of the Anti-Dilution Obligation, the Company remeasured the fair value using current assumptions, resulting in an increase in fair value of $1.3 million, which was recorded in other expense in the consolidated statement of operations and comprehensive loss for the year ended December 31, 2018. The primary assumption used to determine the fair value upon settlement was the fair value of the Series B Preferred Stock, which was estimated to be $2.39 and $3.69 for shares issued pursuant to the March 31, 2018 and October 16, 2018 closings, respectively.

Conversion Features

On December 1, 2017 and January 29, 2018, the Company issued two separate unsecured convertible promissory notes (the “Convertible Notes”) of $2.0 million and $1.0 million, respectively, to an investor. The Convertible Notes would automatically convert, upon the Company’s next equity

financing of at least $2.0 million in gross proceeds following the issuance of the Convertible Notes, into the type of equity securities issued in such financing (the "Automatic Conversion"). Pursuant to the terms of the Convertible Notes, the shares received upon conversion would be determined based on the outstanding principal, plus accrued interest, divided by 75.0% of the price paid by investors in the Company's next equity financing.

The Company determined that the conversion features represented a derivative instrument as they were based on a variable number of shares resulting in a fixed dollar amount of value being provided to the lender, and therefore were in substance a redemption feature. Furthermore, the conversion features were not determined to be clearly and closely related to the debt host contracts, and therefore were required to be separately accounted for. The initial fair value of the derivative was determined by calculating the fair values of the Convertible Notes with and without the conversion features. The difference between the fair values of the Convertible Notes in the "with" and "without" scenarios was then concluded as initial fair value of the conversion features. The valuation used significant inputs which were not observable in the market, including the probability of various exit scenarios and discount rates. Therefore, the conversion features represented a Level 3 measurement within the fair value hierarchy. The initial fair value of the conversion features for the $2.0 million and $1.0 million Convertible Notes was $0.6 million and $0.3 million, respectively.

A change in the assumptions related to the valuation of the conversion features could have a significant impact on their determined fair value. The following reflects the significant quantitative inputs used in the valuation of the conversion features upon issuance of the 2018 Convertible Note:

	January 29, 2018 Convertible Note
Probability of next equity financing scenario	75.60%
Probability of contractual maturity scenario	24.40%
Time until equity financing scenario (in years)	0.09
Discount rate	15.00%

The Company estimated the probability of each settlement scenario and time until equity financing using information obtained from discussions with investors and the Board. The discount rate was calculated based on an average of market rates of return for similar preferred stock financings.

The conversion features were settled upon the first closing of the Series B Preferred Stock in March 2018, which triggered the Automatic Conversion and resulted in the conversion of both Convertible Notes into shares of Series B Preferred Stock. The redemption terms of the 2018 Convertible Note were adjusted such that the discount provided on the effective price of the conversion was decreased from 25.0% to 12.5%. The fair value of the conversion features upon settlement was determined by calculating the fair values of the Convertible Notes with and without the conversion features, which was equivalent to the discount provided to the holders of the Convertible Notes.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

5. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	December 31, 2018	December 31, 2019
Office furniture and equipment	$ 84	$ 113
Laboratory equipment	15	48
Computer equipment	5	5
Total property and equipment	104	166
Less: Accumulated depreciation	(1)	(38)
Property and equipment, net	$ 103	$ 128

Depreciation expense was not material to the years ended December 31, 2018 and 2019.

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31, 2018	December 31, 2019
Accrued external research and development expenses	$ 645	$ 1,552
Accrued personnel-related expenses	732	1,059
Accrued license fees	–	363
Accrued other	377	481
Total accrued expenses	$ 1,754	$ 3,455

7. Convertible Promissory Notes

On December 1, 2017 and January 29, 2018, the Company issued the Convertible Notes for $2.0 million and $1.0 million, respectively, to an investor. The Convertible Notes bore an annual interest rate of 6%.

Upon issuance, the proceeds were allocated to the Convertible Notes and the derivative instrument resulting from the conversion features (Note 4). The Company remeasured the conversion features to fair value at each reporting date, and recognized a loss of $0.1 million in other expense in the consolidated statement of operations and comprehensive loss for the year ended December 31, 2018. Additionally, the Company incurred interest expense on the Convertible Notes of $0.1 million during the year ended December 31, 2018, which was reflected in interest expense in the consolidated statement of operations and comprehensive loss.

In March 2018, both Convertible Notes were settled in full upon the first closing of the initial tranche of the Series B Preferred Stock, which triggered the Automatic Conversion and resulted in the conversion of both Convertible Notes into 1,286,185 shares of Series B Preferred Stock at a specified discount. The transaction was accounted for as an extinguishment of debt under ASC 470-50. As the lender was a related party, the settlement was in substance a capital transaction and therefore the impact upon extinguishment was recognized as an adjustment to accumulated deficit.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

8. Commitments and Contingencies

Leases

In October 2018, the Company entered into a sublease agreement for office space located in Cambridge, Massachusetts which expires on December 30, 2021, with no option to renew or terminate early. The base rent increases by approximately 1% annually. The Company issued a letter of credit to the landlord related to the security deposit, secured by restricted cash, which is recorded in other assets on the accompanying consolidated balance sheets. This lease qualifies as an operating lease. Prior to entering into this sublease arrangement, the Company rented office space from a related party.

In January 2019, the Company entered into an arrangement with a third party to sublease a portion of its Cambridge, Massachusetts office space. This sublease was terminated in November 2019.

The following table summarizes the presentation of the operating lease in the Company's consolidated balance sheets as of December 31, 2019 (in thousands):

Assets:	
Operating lease right-of-use assets	$1,450
Liabilities:	
Current operating lease liabilities	$ 696
Non-current portion of operating lease liabilities	763
Total lease liabilities	$1,459

The following table summarizes total lease costs recognized in the Company's consolidated statement of operations for the year ended December 31, 2019 (in thousands):

Operating lease cost	$ 782
Variable lease costs	3
Sublease income	(31)
Total lease costs	$ 754

Variable lease costs were primarily related to operating expenses, taxes and insurance associated with the operating lease, which were assessed based on the Company's proportionate share of such costs for the leased premises. As these costs are generally variable in nature, they are not included in the measurement of the operating lease right-of-use asset and related lease liability. Total lease costs are included as operating expenses in the Company's consolidated statement of operations and comprehensive loss. Total rent expense for the year ended December 31, 2018 was $0.3 million.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Future lease payments under non-cancelable lease agreements as of December 31, 2019 were as follows (in thousands):

Year Ended December 31,	Future Lease Payments
2020	$ 783
2021	791
Total future lease payments	$ 1,574
Less: interest	(115)
Present value of operating lease liabilities	$ 1,459

The weighted average remaining lease term and weighted average incremental borrowing rate of the Company's operating lease as of December 31, 2019 were as follows:

Weighted average remaining lease term (in years)	2.0
Weighted average incremental borrowing rate	8.0%

Under the prior lease accounting guidance, minimum rental commitments under non-cancelable leases as of December 31, 2018 were as follows (in thousands):

Year Ended December 31,	Minimum Lease Payments
2019	$ 774
2020	783
2021	791
	$ 2,348

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any legal proceedings during the years ended December 31, 2018 and 2019, and no material legal proceedings are currently pending or threatened.

Purchase Orders

The Company has agreements with third parties for various services, including services related to research, preclinical and clinical operations and support, for which the Company is not contractually able to terminate for convenience to avoid future obligations to the vendors. Certain agreements provide for termination rights subject to termination fees or wind down costs. Under such agreements, the Company is contractually obligated to make certain payments to vendors, primarily to reimburse them for their unrecoverable outlays incurred prior to cancelation. The actual amounts the Company could pay in the future to the vendors under such agreements may differ from the purchase order amounts due to cancellation provisions.

Indemnification Agreements

The Company enters into indemnification agreements and agreements containing indemnification provisions in the ordinary course of business. Pursuant to these agreements, the Company agrees to

indemnify, hold harmless, and to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is generally perpetual upon execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

9. Redeemable Convertible Preferred Stock

As of December 31, 2019, the authorized capital stock of the Company included 36,724,132 shares of $0.0001 par value Preferred Stock, of which 8,075,799 shares have been designated as Series A Preferred Stock, 14,913,704 shares have been designated as Series B Preferred Stock, 2,666,666 shares have been designated as Series B-1 Preferred Stock and 11,067,963 shares have been designated as Series C Preferred Stock.

During the year ended December 31, 2018, the Company issued a total of 12,333,333 shares of Series B Preferred Stock in two separate closings at a purchase price of $3.00 per share for gross cash proceeds of $37.0 million, and incurred issuance costs of $0.2 million. The Company also issued an aggregate of 1,286,185 shares of Series B Preferred Stock upon the conversion of the Convertible Notes at an average conversion price of $2.40 per share. The Company also issued 1,294,186 shares of Series B Preferred Stock in connection with the anti-dilutive provision within the Purdue License Agreement. The issuance of the Series B Preferred Stock resulted in changes to certain rights, preferences and privileges of the Series A Preferred Stock. The Company concluded such changes were not significant and resulted in a modification, rather than an extinguishment, of the Series A Preferred Stock. The changes to the terms of the Series A Preferred Stock did not result in incremental value to the shareholders, and therefore there was no impact to the accounting for the Series A Preferred Stock.

On June 18, 2019, the Company entered into the Series B-1 Preferred Stock Purchase Agreement which authorized the sale and issuance of up to 2,666,666 shares of its Series B-1 Preferred Stock at a purchase price of $3.75 per share. During the year ended December 31, 2019, the Company issued all 2,666,666 shares of Series B-1 Preferred Stock for gross cash proceeds of $10.0 million, and incurred an immaterial amount of issuance costs. The issuance of the Series B-1 Preferred Stock resulted in changes to certain rights, preferences and privileges of the Series A Preferred Stock and the Series B Preferred Stock. The Company concluded that such changes were not significant and resulted in a modification, rather than an extinguishment, of the Series A Preferred Stock and the Series B Preferred Stock. The changes to the terms of the Series A Preferred Stock and the Series B Preferred Stock did not result in incremental value to the shareholders, and therefore there was no impact to the accounting for the Series A Preferred Stock and the Series B Preferred Stock.

On November 18, 2019, the Company entered into the Series C Preferred Stock Purchase Agreement which authorized the sale and issuance of up to 5,825,243 shares at $5.15 per share. On December 10, 2019, the Company executed Amendment No. 1 and Joinder to the Series C Preferred Stock Purchase Agreement which authorized the sale and issuance of an additional 5,242,720 shares at $5.15 per share. During the year ended December 31, 2019, the Company issued 9,805,827 shares of Series C Preferred Stock for gross cash proceeds of $50.5 million, and incurred issuance costs of $0.2 million. Although there were multiple closings of the Series C Preferred Stock, there was no

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

obligation under the initial closing for investors to purchase, or for the Company to sell to such investors, additional shares of Series C Preferred Stock. The issuance of the Series C Preferred Stock resulted in changes to certain rights, preferences and privileges of the Series A Preferred Stock, the Series B Preferred Stock and the Series B-1 Preferred Stock. The Company concluded that such changes were not significant and resulted in a modification, rather than an extinguishment, of the Series A Preferred Stock, the Series B Preferred Stock and the Series B-1 Preferred Stock. The changes to the terms of the Series A Preferred Stock, the Series B Preferred Stock and the Series B-1 Preferred Stock did not result in incremental value to the shareholders, and therefore there was no impact to the accounting for the Series A Preferred Stock, the Series B Preferred Stock and the Series B-1 Preferred Stock.

The Preferred Stock consisted of the following (in thousands, except share amounts):

	As of December 31, 2018					
	Shares Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Redemption Value	Common Stock Issuable Upon Conversion
Series A Preferred Stock	8,075,799	8,075,799	$ 9,284	$ 9,284	$ 9,284	3,773,820
Series B Preferred Stock	14,927,584	14,913,704	46,436	46,381	46,381	6,969,173
	23,003,383	22,989,503	$ 55,720	$ 55,665	$ 55,665	10,742,993

	As of December 31, 2019					
	Shares Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Redemption Value	Common Stock Issuable Upon Conversion
Series A Preferred Stock	8,075,799	8,075,799	$ 9,932	$ 9,932	$ 9,932	3,773,820
Series B Preferred Stock	14,913,704	14,913,704	49,969	49,969	49,969	6,969,173
Series B-1 Preferred Stock	2,666,666	2,666,666	10,431	10,431	10,431	1,246,133
Series C Preferred Stock	11,067,963	9,805,827	50,789	50,789	50,789	4,582,257
	36,724,132	35,461,996	$ 121,121	$ 121,121	$ 121,121	16,571,383

Common stock issuable upon conversion in the tables above represents shares of common stock issuable upon an automatic conversion in the event of a qualified public offering, pursuant to the Company's Amended and Restated Certificate of Incorporation.

Rights, Preferences and Privileges

Pursuant to the Company's Amended and Restated Certificate of Incorporation, the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock and Series C Preferred Stock have the following rights, preferences and privileges:

Voting Rights

The holders of outstanding shares of the Preferred Stock are entitled to vote, together with the holders of common stock, on all matters submitted to the stockholders for a vote, and are entitled to

the number of votes equal to the number of whole shares of common stock into which such holders of the Preferred Stock could convert on the record date for determining stockholders entitled to vote. Except for the actions requiring the approval or consent of the majority of the holders of the Preferred Stock, the holders of the Preferred Stock will vote together with the holders of common stock and vote as a single class. The holders of the Series A Preferred Stock, exclusively and as a separate class, are entitled to elect two directors of the Company. The holders of the Series B Preferred Stock and Series B-1 Preferred Stock, exclusively and together as a separate class, are entitled to elect two directors of the Company. The holders of common stock and of any other class or series of voting stock (including the Preferred Stock), exclusively and voting as a single class, are entitled to elect the balance of total number of directors of the Company.

Dividends

The holders of the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock and Series C Preferred Stock are entitled to accrue cumulative dividends at an annual rate of $0.08, $0.24, $0.30 and $0.412 per share, respectively, subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock. Dividends accrue from day to day whether or not declared by the Board, and are payable only when, as, and if declared by the Board. As of December 31, 2019, no dividends have been declared or paid by the Company since its inception.

The Company's cumulative dividends on its Preferred Stock were as follows (in thousands):

	As of December 31,	
	2018	2019
Series A Preferred Stock	$ 1,209	$ 1,857
Series B Preferred Stock	1,639	5,228
Series B-1 Preferred Stock	–	431
Series C Preferred Stock	–	289
	$ 2,848	$ 7,805

No dividends may be declared, paid or set aside to any other class or series of capital stock (other than dividends on shares of common stock payable in common stock) unless, in addition to obtaining any consents otherwise required in the Company's certificate of incorporation, the holders of the Preferred Stock first receive a dividend on each outstanding share in an amount at least equal to the greater of: (i) all accrued and unpaid dividends and (ii) in the case of a dividend being distributed to common stock or any class or series of capital stock that is convertible into common stock, the equivalent dividend on an as-converted basis or (iii) in the case of a dividend being distributed on a series or class not convertible into common stock, an additional dividend equal to a dividend rate calculated based on the respective original issue price of the Preferred Stock. The original issue price per share is equal to $1.00 for the Series A Preferred Stock, $3.00 for the Series B Preferred Stock, $3.75 for the Series B-1 Preferred Stock and $5.15 for the Series C Preferred Stock. The holders of the Series C Preferred Stock, Series B Preferred Stock and Series B-1 Preferred Stock are entitled to receive dividends prior to any dividends on the Series A Preferred Stock.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Liquidation Rights

In the event of any voluntary or involuntary liquidation event, dissolution, winding up of the Company or upon the occurrence of certain events designated by a majority of the holders of the Preferred Stock, and at least two out of three specific holders, to be a deemed liquidation event, each holder of the then outstanding Series C Preferred Stock, Series B-1 Preferred Stock and Series B Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of Series A Preferred Stock and common stock, an amount equal to the greater of (i) original issuance price (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any cumulative accrued dividends, whether or not declared, with any other dividends declared but unpaid thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

After the payment of all preferential amounts to the holders of the Series C Preferred Stock, Series B-1 Preferred Stock and Series B Preferred Stock, each holder of the then outstanding Series A Preferred Stock will be entitled to receive, prior and in preference to any distributions to the holders of common stock, an amount equal to the greater of (i) original issuance price (adjusted in the event of any stock dividend, stock split, combination or other similar activity) plus any cumulative accrued dividends, whether or not declared, with any other dividends declared but unpaid thereon, or (ii) the amount such holder would have received if such holder had converted its shares into common stock immediately prior to such liquidation event.

After payments have been made in full to the holders of the Preferred Stock, then, to the extent available, the remaining amounts will be distributed among the holders of the shares of common stock, pro rata based on the number of shares held by each holder.

Conversion

Each share of the Preferred Stock is convertible, at any time, at the option of the holder, and without the payment of additional consideration, into such shares of non-assessable shares of common stock as is determined by dividing the original issue price by the applicable conversion price in effect at the time of conversion. The applicable conversion price for the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock and Series C Preferred Stock is initially equal to $1.00, $3.00, $3.75 and $5.15, respectively. Each share of the Preferred Stock will be automatically converted into common stock at the applicable conversion ratio then in effect for each series of the Preferred Stock upon either (i) the closing of the sale of shares of common stock at a price of at least $10.30 per share in a firm-commitment underwritten public offering pursuant to an effective registration statement resulting in at least $75.0 million of gross proceeds and the listing of the Company's common stock on the New York Stock Exchange, The Nasdaq Global Select Market, or The Nasdaq Global Market or (ii) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the outstanding Preferred Stock, voting together as a single class and at least two of three specific holders. As of December 31, 2019, each share of the Preferred Stock was convertible into one share of common stock and may be adjusted in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock.

The Company accounts for potential beneficial conversion features at the time of issuance. The Company's common stock into which each series of the Company's Preferred Stock is convertible had an estimated fair value less than the effective conversion prices of the Preferred Stock at the time of

each of the issuances of the Preferred Stock. Therefore, there was no intrinsic value on the respective commitment dates.

Redemption

Each series of the Preferred Stock is redeemable at a price equal to the applicable original issuance price per share (adjusted in the event of any stock dividend, stock split, combination or other similar activity), plus any cumulative accrued dividends, whether or not declared together with any other dividends declared but unpaid, in three annual installments commencing not more than 60 days on or after November 18, 2024 at the written election of at least a majority of the holders of the Preferred Stock voting together as a single class and at least two out of three specific parties.

10. Common Stock

As of December 31, 2018 and 2019, the authorized capital stock of the Company included 33,000,000 and 46,000,000 shares of common stock, $0.0001 par value, respectively.

Rights, Preferences and Privileges

The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock. The Company's common stock has the following rights, preferences and privileges:

Voting Rights

Each share of common stock entitles the holder to one vote, together with the holders of the Preferred Stock, on all matters submitted to the stockholders for a vote.

Dividends

The holders of shares of common stock are not entitled to receive dividends.

Liquidation Rights

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company, after the payment of all preferential amounts required to be paid to the holders of the Preferred Stock, the remaining assets of the Company available for distribution to its stockholders will be distributed to the holders of common stock on a pro rata basis based on the number of shares held by each such holder.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Shares Reserved for Future Issuance

The Company has reserved the following shares of common stock for future issuance:

	December 31,	
	2018	2019
Series A Preferred Stock	3,773,820	3,773,820
Series B Preferred Stock	6,969,173	6,969,173
Series B-1 Preferred Stock	–	1,246,133
Series C Preferred Stock	–	4,582,257
Shares reserved for vesting of restricted common stock	261,393	48,190
Shares reserved for exercise of outstanding stock options	1,192,454	1,634,686
Shares reserved for future awards under the 2017 Stock Incentive Plan	942,691	617,101
Total shares of authorized common stock reserved for future issuance	13,139,531	18,871,360

11. Stock-Based Compensation

2017 Stock Incentive Plan

On May 9, 2017, the Board adopted the 2017 Stock Incentive Plan (the "2017 Plan"). The 2017 Plan allows the Company to grant stock options, restricted stock, restricted stock units and other stock-based awards to employees, officers, directors, consultants and advisors of the Company. The 2017 Plan is administered by the Board, which has the authority to grant awards and determine the terms of awards under the 2017 Plan, provided that generally the exercise price per share of stock options granted may not be less than 100% of the fair market value of a share of the Company's common stock on the date of grant, and the term of stock options granted may not exceed ten years.

The total number of shares of common stock authorized for issuance under the 2017 Plan as of December 31, 2018 and 2019 was 2,240,285 shares and 2,356,927 shares, respectively.

As of December 31, 2019, the Company has only issued stock options and restricted stock under the 2017 Plan. Stock options issued comprise service-based awards granted to employees and non-employee consultants. Stock options and restricted stock issued under the 2017 Plan have vesting conditions in which 25% vests upon the first anniversary of a specified vesting commencement date, and the remaining 75% vests in 36 monthly installments over the remaining three years. Vesting of stock options is subject to the recipient's continued employment or service. The Company has the right to repurchase any unvested shares of restricted stock held by a recipient during the vesting period if the relationship between the recipient and the Company has terminated. Stock options issued under the 2017 Plan expire ten years from the date of grant.

Shares that expire, are terminated, surrendered or canceled under the 2017 Plan without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future awards. Upon stock option exercise, the Company issues new shares and delivers them to the participant.

As of December 31, 2019, the Company did not hold any treasury shares.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Restricted Common Stock

Prior to the adoption of the 2017 Plan, the Company granted restricted common stock in 2016 with time-based vesting conditions to certain employees and non-employee founders of the Company pursuant to individual award agreements. The restricted common stock granted pursuant to these agreements vests either: (i) 25% upon vesting commencement or the first anniversary of a specified vesting commencement date, and the remaining 75% monthly over 36 months thereafter, (ii) monthly over 48 months after a specified vesting commencement date, or (iii) monthly over 12 months from a specified vesting commencement date. The Company has the right to repurchase the unvested shares held by a recipient during the vesting period if the relationship between the recipient and the Company has terminated. Shares of restricted common stock are not accounted for as outstanding common stock until they have vested. Unvested shares of restricted common stock may not be sold or transferred by the holder. The Company did not grant any restricted common stock during the years ended December 31, 2018 or 2019.

The following table summarizes all of the Company's restricted common stock activity, including restricted common stock issued under the 2017 Plan and under individual award agreements prior to the adoption of the 2017 Plan:

	Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2018	261,393	$ 0.02
Issued	–	–
Vested	(213,203)	0.01
Repurchased	–	–
Unvested as of December 31, 2019	48,190	$ 0.06

The total fair value of restricted common stock that vested during the years ended December 31, 2018 and 2019 was $0.6 million and $0.6 million, respectively.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Stock Options

The following table summarizes the Company's stock option activity:

	Number of Shares	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding as of December 31, 2018	1,192,454	$ 2.15		
Granted	442,232	3.30		
Exercised	–	–		–
Cancelled or Forfeited	–	–		
Outstanding as of December 31, 2019	1,634,686	$ 2.46	9.00	$ 5,107
Exercisable as of December 31, 2019	695,505	$ 2.18	8.75	$ 2,372
Vested and expected to vest as of December 31, 2019	1,634,686	$ 2.46	9.00	$ 5,107

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the underlying stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the estimated fair value of the Company's common stock at December 31, 2019.

Stock Option Valuation

The assumptions that the Company used in the Black-Scholes option pricing model to determine the grant-date fair value of stock options granted to employees, members of the Board and non-employees on the date of grant were as follows:

	Year Ended December 31,	
	2018	2019
Risk-free interest rate	3.10 – 3.20%	1.55%
Expected term (in years)	6.00 – 10.00	6.00
Expected volatility	80.03%	79.09%
Expected dividend yield	0.00%	0.00%
Fair value per share of common stock	$ 2.27	$ 3.30

The weighted-average grant-date fair value of the Company's stock options granted during the years ended December 31, 2018 and 2019 was $1.65 and $2.25, respectively.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Stock-Based Compensation

Stock-based compensation expense was allocated as follows (in thousands):

	Year Ended December 31,	
	2018	2019
Research and development	$ 181	$ 430
General and administrative	398	238
Total stock-based compensation expense	$ 579	$ 668

As of December 31, 2019, total unrecognized compensation cost related to unvested stock-based awards was $1.6 million, which is expected to be recognized over a weighted-average period of 2.69 years.

12. Significant Agreements

Purdue License Agreement

On December 31, 2017, the Company entered into a License Agreement with Purdue (the "Purdue License Agreement"), pursuant to which Purdue granted the Company exclusive rights under certain Purdue know-how to research, develop and commercialize pharmaceutical products concerning a $GABA_A$ positive allosteric modulator. The Company is obligated to make future milestone payments based on the achievement of specified development and sales milestones up to $33.0 million. Furthermore, the Company is required to pay Purdue royalties of a low-single-digit percentage of annual net sales of licensed products.

Under the Purdue License Agreement, Purdue agreed to purchase $0.6 million of shares of Series B Preferred Stock. In addition, as consideration for the license obtained, the Company issued Purdue the Anti-Dilution Obligation to ensure Purdue's ownership remained at a specified percentage throughout the Series B Preferred Stock financing (Note 4). The Company concluded that the Purdue License Agreement represented the acquisition of in-process research and development assets with no alternative future use. Therefore, the initial fair value of the Anti-Dilution Obligation of $2.4 million was expensed as research and development in December 2017.

The Purdue License Agreement will remain in effect until the expiration of the Company's royalty obligation for all licensed products. Either the Company or Purdue may terminate the agreement in the event of a material breach by the other party and fails to cure such breach within a certain period of time. Either party may voluntarily terminate the agreement with prior notice. If the agreement is voluntarily terminated by Purdue, the Company's license rights will survive such termination and such rights will become fully paid, perpetual and irrevocable.

The Anti-Dilution Obligation was settled in October 2018. As of December 31, 2019, none of the developmental or sales milestones under the Purdue License Agreement were achieved.

RogCon and Ionis Agreements

During 2018, the Company began negotiating a license agreement with RogCon Inc. ("RogCon") for intellectual property related to treating SCN2A mutations in epilepsy, which is recognized as the

second most common genetic cause of epilepsy. RogCon had an existing collaboration with Ionis Pharmaceuticals, Inc. ("Ionis") and as a result the Company needed to negotiate an agreement with Ionis in order to complete the license agreement with RogCon. On December 21, 2018, the Company entered into an agreement with RogCon to advance RogCon a deposit of up to $1.0 million on the pending license agreement while the agreement with Ionis was being negotiated. The deposit was fully refundable to the Company. As of December 31, 2018, the outstanding balance was $0.6 million and is included within prepaid expenses and other current assets on the Company's accompanying consolidated balance sheet. On September 11, 2019, the Company entered into both a Cooperation and License Agreement (the "License Agreement") with RogCon, and a Research, Collaboration, Option and License Agreement (the "Collaboration Agreement") with Ionis. The agreements were entered into contemporaneously to enable the parties to advance their collective efforts related to SCN2A. Upon execution of the License Agreement, the $1.0 million outstanding balance of the deposit was applied toward the purchase price of the License Agreement.

RogCon Agreement

Under the License Agreement, RogCon granted to the Company an exclusive, worldwide license under RogCon's intellectual property to research, develop and commercialize products for the treatment of all forms of epilepsy and/or neurodevelopmental disorders in each case caused by any mutation of the SCN2A gene. Under the License Agreement, the Company will conduct, at its own cost and expense, the research and development activities assigned to it under the research plan. In addition, the Company is responsible for reimbursing RogCon for any costs associated with research and development activities RogCon performs at the request of the Company. As part of the agreement, the Company agreed to provide up-front consideration of $2.1 million, consisting of the $1.0 million deposit, $0.7 million in cash reimbursements for certain historical costs previously incurred by RogCon, and $0.4 million for the retirement of existing loan balances as of September 11, 2019.

The Company concluded that the License Agreement represented the acquisition of in-process research and development assets with no alternative future use. Therefore, the aggregate acquisition cost of $2.2 million, consisting of the $2.1 million of up-front consideration and $0.1 million of acquisition costs, was expensed as research and development on September 11, 2019.

Subsequent to September 11, 2019, the Company will reimburse RogCon for its out-of-pocket costs incurred for activities performed under the License Agreement. The Company expenses these costs as incurred as research and development. Since the acquisition date, the Company expensed $0.1 million for the reimbursement of RogCon's out-of-pocket costs in the year ended December 31, 2019.

Additionally, the Company may pay RogCon a milestone payment of $3.0 million and profit share payments. The $3.0 million milestone payment will become due when the first profit share payment has become due and certain contingent payments have become payable to Ionis under the Collaboration Agreement, which are subject to the Company exercising its option to obtain license rights to a development candidate, as well as other contingent events. The profit share payments will be based on a low-double-digit percentage of net profits, depending on sales volume.

The License Agreement, unless earlier terminated, will continue until the latest of: (i) expiration of all patent rights within RogCon patents, (ii) the Company and its affiliates certify they have abandoned the research, development and commercialization of product with no intention to re-establish such

activities, and (iii) no third party is obligated to pay the Company or its affiliates any amounts that comprise net sublicense revenue. Either party may terminate the License Agreement for material breach or insolvency of the other party. Additionally, the Company may terminate for convenience with prior written notice to RogCon. Upon termination by either party, all rights and licenses granted by RogCon to the Company will revert back to RogCon.

Ionis Collaboration Agreement

Under the Collaboration Agreement, both parties will participate in research activities related to the downregulation of SCN2A gene products associated with the treatment of any and all forms of epilepsy and/or neurodevelopmental disorders in each case caused by any mutation of the SCN2A gene, other than one severe type of epilepsy. Ionis will also be responsible for identifying a development candidate and conducting an IND-enabling toxicology study. The Company will reimburse Ionis for any out-of-pocket costs incurred related to the research activities, identification of a development candidate and conducting an IND-enabling toxicology study. Additionally, the Company agreed to reimburse $0.3 million of costs incurred by Ionis for the performance of research activities prior to the execution of the Collaboration Agreement, which the Company recognized as research and development expense. The reimbursement of out-of-pocket costs is recognized as research and development expense as incurred. Inclusive of the up-front payment of $0.3 million, the Company expensed a total of $0.6 million as research and development under the Collaboration Agreement for the year ended December 31, 2019.

Ionis granted the Company an exclusive option to obtain the rights and license related to the development candidate, which the Company may exercise following completion of the IND-enabling toxicology study. Upon option exercise, the Company will pay Ionis a $2.0 million license fee. After option exercise, the Company is responsible for clinical development and commercialization of the development candidate. If the option is not exercised, the Collaboration Agreement will expire, and the Company will have no further rights to the development candidate. Additionally, if the option is not exercised, at the request of Ionis, the Company will assign the RogCon License Agreement to Ionis. The Company concluded that there is no accounting recognition for the exclusive option unless and until such option is exercised because it is a unilateral right of the Company that is priced at an amount that approximates fair value.

If the Company exercises its exclusive option, Ionis may be entitled to development milestone payments, additional milestone payments, and sales royalties or sublicense fees.

The Collaboration Agreement will continue until the expiration of all payment obligations to Ionis, unless earlier terminated. Either party may terminate the Collaboration Agreement upon material breach or insolvency of the other party or if Ionis is unable to identify a development candidate. Ionis may terminate if the Company fails to achieve a performance milestone. The Company may terminate for convenience with prior written notice to Ionis. Upon termination by the Company for convenience, the Company will stop selling all products, subject to certain wind-down provisions, and all products will revert back to Ionis.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

13. Net Loss per Share and Unaudited Pro Forma Net Loss per Share

Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share data):

	Year Ended December 31,	
	2018	2019
Numerator:		
Net loss	$ (26,535)	$ (35,512)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,296)	(5,170)
Loss on conversion of convertible notes	(392)	–
Net loss attributable to common stockholders	$ (29,223)	$ (40,682)
Denominator:		
Weighted average common shares outstanding, basic and diluted	1,297,633	1,529,629
Net loss per share attributable to common stockholders, basic and diluted	$ (22.52)	$ (26.60)

The following potential common shares, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have been anti-dilutive:

	Year Ended December 31,	
	2018	2019
Series A Preferred Stock	3,773,820	3,773,820
Series B Preferred Stock	6,969,173	6,969,173
Series B-1 Preferred Stock	–	1,246,133
Series C Preferred Stock	–	4,582,257
Outstanding stock options	1,192,454	1,634,686
Unvested restricted common stock	261,393	48,190
	12,196,840	18,254,259

The shares of common stock issuable upon conversion of the Preferred Stock assume automatic conversion in the event of a qualified public offering.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Unaudited Pro Forma Net Loss per Share

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share data):

	Year Ended December 31, 2019
Numerator:	
Net loss attributable to common stockholders	$ (40,682)
Accretion and cumulative dividends on redeemable convertible preferred stock	5,170
Pro forma net loss attributable to common stockholders	$ (35,512)
Denominator:	
Weighted average common shares outstanding, basic and diluted	1,529,629
Pro forma adjustment to reflect the automatic conversion of redeemable convertible preferred stock into common stock upon the completion of the proposed initial public offering	11,741,132
Pro forma weighted average common shares outstanding, basic and diluted	13,270,761
Pro forma net loss per share attributable to common stockholders, basic and diluted	$ (2.68)

14. Income Taxes

The Company maintains a full valuation allowance on its U.S. net deferred tax assets due to the uncertainty of future taxable income. The Company did not recognize an income tax benefit in the years ended December 31, 2018 or 2019 related to its U.S. operations due to the uncertainty regarding future taxable income. In the years ended December 31, 2018 and 2019, the difference between the statutory tax rate in the U.S. and the Company's effective tax rate was due primarily to the valuation allowance recorded to offset any potential tax benefit. The income tax provision and benefit recognized for the years ended December 31, 2018 and 2019, respectively, related to income tax associated with the Company's operations in Australia.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

The reconciliation of the U.S. statutory income tax rate to the Company's effective tax rate is as follows:

	Year Ended December 31,	
	2018	2019
Federal statutory income tax rate	21.0%	21.0%
State taxes, net of federal benefit	5.1%	6.0%
Federal and state research and development credits	0.7%	2.9%
Non-deductible items	(3.6)%	(0.4)%
Foreign	(0.1)%	0.2%
Change in valuation allowance	(23.1)%	(29.6)%
Other	(0.5)%	0.1%
Effective income tax rate	(0.5)%	0.2%

Net deferred tax assets consisted of the following (in thousands):

	December 31,	
	2018	2019
Deferred tax assets:		
Net operating loss carryforwards	$ 6,931	$ 14,502
Amortization	1,776	3,512
Research and development credits	196	1,230
Accrued expenses	175	340
Foreign exchange loss	73	–
Leases	–	396
Stock-based compensation	25	60
Total gross deferred tax assets	$ 9,176	$ 20,040
Less: Valuation allowance	(9,176)	(19,647)
Net deferred tax assets	$ –	$ 393
Deferred tax liabilities:		
Operating lease right-of-use asset	–	(393)
Total gross deferred tax liabilities	–	(393)
Net deferred tax assets	$ –	$ –

As of December 31, 2018 and 2019, the Company had U.S. federal net operating loss carryforwards which may be able to offset future income tax liabilities of approximately $25.6 million and $53.4 million, respectively. Federal net operating loss carryforwards of $7.7 million will expire at various dates through 2037 and approximately $45.7 million may be carried forward indefinitely. As of December 31, 2018 and 2019, the Company also had state net operating loss carryforwards of

approximately $25.1 million and $52.0 million, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2039.

As of December 31, 2018 and 2019, the Company had federal research and development tax credit carryforwards of approximately $0.1 million and $0.9 million, respectively, available to reduce future tax liabilities which expire at various dates through 2039. As of December 31, 2018 and 2019, the Company had state research and development tax credit carryforwards of approximately $0.1 million and $0.4 million, respectively, available to reduce future tax liabilities which expire at various dates through 2034. The Company has generated research credits but has not conducted a study to document the qualified activity. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed, and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

Under the provisions of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards would be subject to an annual limitation under Section 382, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization. Further, until a study is completed by the Company and any limitation is known, no amounts are being presented as an uncertain tax position.

ASC 740 requires a valuation allowance to reduce the deferred tax assets reported if, based on the weight of the evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. After consideration of all of the evidence, both positive and negative, the Company has recorded a valuation allowance against its deferred tax assets at December 31, 2018 and 2019 because management has determined that it is more likely than not that the Company will not recognize the benefits of its federal and state deferred tax assets, primarily due to its history of cumulative net losses incurred since inception and its lack of commercialization of any products or generation of any revenue from product sales since inception. As a result, a valuation allowance of $9.2 million and $19.6 million has been established at December 31, 2018 and 2019, respectively. Management reevaluates the positive and negative evidence at each reporting period.

The valuation allowance increased by approximately $6.1 million and $10.5 million during the years ended December 31, 2018 and 2019, respectively, due primarily to the generation of net operating losses.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2018 and 2019. The Company's policy is to record interest and penalties related to uncertain tax positions as part of its income tax provision. As of December 31, 2018 and 2019, the Company had no accrued interest or penalties related to uncertain tax positions and no such amounts have been recognized in the Company's consolidated statement of operations and comprehensive loss for either year ended December 31, 2018 or 2019. In many cases, the Company's uncertain tax positions are related to years that remain subject to examination by relevant tax authorities. The statute of limitations for federal and state tax authorities is open for tax years ended December 31, 2016 through December 31, 2019. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state and local income tax authorities for all tax years in which a loss carryforward is available.

15. Related Party Transactions

During the year ended December 31, 2019, the Company reimbursed $0.2 million of third-party recruiting costs incurred by a significant shareholder on behalf of the Company. These amounts were recorded in general and administrative expenses during the year ended December 31, 2019 and were within accrued expenses as of December 31, 2019.

In December 2017 and January 2018, the Company issued the Convertible Notes to a significant shareholder with Board representation for $2.0 million and $1.0 million, respectively. The Convertible Notes were settled in March 2018 upon their automatic conversion into shares of the Company's Series B Preferred Stock.

A member of the Board is affiliated with Purdue. During the years ended December 31, 2018 and 2019, the Company continued to perform certain research and development activities pursuant to the Purdue License Agreement (Note 12).

During the years ended December 31, 2018 and 2019, related parties participated in each of the Company's offerings of the Preferred Stock (Note 9).

During the year ended December 31, 2018, the Company leased an office space from a significant shareholder. The Company moved out of the space in December 2018 and recognized $0.1 million of rent expense related to this lease during the year ended December 31, 2018.

16. Employee Benefit Plan

The Company did not have an employee benefit plan under Section 401(k) of the Internal Revenue Code during the year ended December 31, 2018. During the year ended December 31, 2019, the Company implemented a defined contribution savings plan for eligible employees. The plan covers substantially all employees who meet a minimum age requirement and allows participants to defer a portion of their annual compensation on a pre-tax basis. Under the plan, the Company is not obligated to match any participant contributions. The Company did not make any contributions to the plan during the year ended December 31, 2019.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

17. Subsequent Events

Subsequent Events Through July 22, 2020

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. The Company has evaluated subsequent events through July 22, 2020, the date these consolidated financial statements were issued, and identified the following subsequent events:

Series C Redeemable Convertible Preferred Stock Transactions

On January 20, 2020, the Company granted two investors holding 5,825,243 shares of Series C Preferred Stock that were purchased in December 2019 the option to put their shares back to the Company at the original issuance price. On February 19, 2020 and March 3, 2020, the investors exercised their put option in full via the execution of Stock Redemption and Release Agreements in order to effect the repurchase. Pursuant to the Stock Redemption and Release Agreements, the Company agreed to repurchase a total of 5,825,243 shares of Series C Preferred Stock at the original issuance price of $5.15 per share, for an aggregate cash repurchase price of $30.0 million. Under the terms of the Stock Redemption and Release Agreements, the investors waived their right to cumulative dividends that had accumulated from the original issuance date through the date of repurchase.

The Company determined that the additional put right that was granted to the investors represented a modification of the affected shares of Series C Preferred Stock, but that it did not result in incremental value to the shareholders. As there was no incremental value associated with the modification, there was no impact to the accounting for the Series C Preferred Stock. The Company also determined that the put right did not require bifurcation, as it does not contain the characteristics of a derivative instrument. Further, the Company determined that the shares of Series C Preferred Stock that were subject to repurchase did not become mandatorily redeemable until the execution of the Stock Redemption and Release Agreements because the parties did not have an unconditional legal obligation to complete the redemptions until the associated agreements were finalized. Such determination was made in consultation with legal counsel. Accordingly, the Company recorded each of the redemptions on the respective date of repurchase and recognized a gain on repurchase equal to the difference between the repurchase price and the carrying value of the Series C Preferred Stock on the respective date of repurchase. The aggregate gain of $0.5 million will be recorded as an adjustment to accumulated deficit in the statement of redeemable convertible preferred stock and stockholders' (deficit) equity. The gain related exclusively to the dividends accrued on the repurchased shares, which were waived by the investors as part of the Stock Redemption and Release Agreements.

On April 15, 2020 and May 8, 2020, the Company completed additional closings for the sale and issuance of its Series C Preferred Stock for a total of 4,563,108 shares at $5.15 per share for aggregate cash proceeds of $23.5 million, less an immaterial amount of issuance costs.

Common Stock Authorized for Issuance

On June 5, 2020, the Company amended the Company's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance to 50,000,000 shares.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Continued)

Amendment to 2017 Stock Incentive Plan

On June 5, 2020, the Company amended the 2017 Stock Incentive Plan to increase the total number of shares authorized for issuance to 3,909,725 shares.

Related Party Transactions

One of the founders of RogCon became the Company's General Counsel in June 2020. The Company continues to reimburse RogCon for its out-of-pocket costs incurred for activities performed under the License Agreement.

Subsequent Events Through October 9, 2020

In connection with the re-issuance of these consolidated financial statements, the Company has evaluated subsequent events through October 9, 2020, the date these consolidated financial statements were re-issued, and identified the following subsequent events:

Common Stock and Preferred Stock Authorized for Issuance

On July 24, 2020, the Board amended the Company's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance to 66,000,000 shares. In addition, the Board amended the Company's Amended and Restated Certificate of Incorporation to increase the number of shares of preferred stock authorized for issuance to 53,644,314 shares, of which 8,075,799 shares are designated as Series A Preferred Stock, 14,913,704 shares are designated as Series B Preferred Stock, 2,666,666 shares are designated as Series B-1 Preferred Stock, 8,543,692 shares are designated as Series C Preferred Stock and 19,444,453 shares are designated as Series C-1 redeemable convertible preferred stock.

Series C-1 Redeemable Convertible Preferred Stock Issuance

In the third quarter of 2020, the Company issued a total of 19,444,453 shares of Series C-1 redeemable convertible preferred stock at a purchase price of $5.67 per share for aggregate proceeds of approximately $110.1 million, net of issuance costs.

Common Stock Authorized for Issuance

On September 2, 2020, the Board amended the Company's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance to 70,500,000 shares.

Amendment to 2017 Stock Incentive Plan

On September 2, 2020, the Board amended the 2017 Stock Incentive Plan to increase the total number of shares authorized for issuance to 5,937,763 shares.

Reverse Stock Split

On October 8, 2020, the Board and the Company's stockholders approved a one-for-2.14 reverse stock split. Effective on October 8, 2020, the reverse stock split impacted the Company's issued and outstanding shares of common stock. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. All shares of common stock, per share amounts and additional paid-in capital amounts for all periods presented in

the accompanying consolidated financial statements and related notes have been retroactively adjusted, where applicable, to reflect the reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices were proportionately increased, as applicable, in accordance with the terms of the agreements governing such securities. The respective conversion prices of the Preferred Stock were proportionately increased. The number of shares of common stock authorized for issuance and the par value of common stock were not adjusted as a result of the reverse stock split.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

(Amounts in thousands, except share and per share data)

	December 31, 2019	June 30, 2020	Pro Forma June 30, 2020
Assets			
Current assets:			
Cash and cash equivalents	$ 44,815	$ 19,748	$ 19,748
Prepaid expenses and other current assets	681	692	692
Total current assets	45,496	20,440	20,440
Property and equipment, net	128	108	108
Restricted cash	600	600	600
Operating lease right-of-use assets	1,450	1,109	1,109
Other non-current assets	20	396	396
Total assets	$ 47,694	$ 22,653	$ 22,653
Liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity			
Current liabilities:			
Accounts payable	$ 2,667	$ 3,688	$ 3,688
Accrued expenses	3,455	3,740	3,740
Operating lease liabilities	696	729	729
Total current liabilities	6,818	8,157	8,157
Long-term liabilities:			
Non-current portion of operating lease liabilities	763	390	390
Total liabilities	7,581	8,547	8,547
Commitments and contingencies (Note 5)			
Series A redeemable convertible preferred stock, $0.0001 par value; 8,075,799 shares authorized as of December 31, 2019 and June 30, 2020; 8,075,799 shares issued and outstanding as of December 31, 2019 and June 30, 2020; liquidation value as of December 31, 2019 and June 30, 2020 of $9,932 and $10,254, respectively; no shares authorized, issued or outstanding, pro forma as of June 30, 2020	9,932	10,254	–
Series B redeemable convertible preferred stock, $0.0001 par value; 14,913,704 shares authorized as of December 31, 2019 and June 30, 2020; 14,913,704 shares issued and outstanding as of December 31, 2019 and June 30, 2020; liquidation value as of December 31, 2019 and June 30, 2020 of $49,969 and $51,749, respectively; no shares authorized, issued or outstanding, pro forma as of June 30, 2020	49,969	51,749	–
Series B-1 redeemable convertible preferred stock, $0.0001 par value; 2,666,666 shares authorized as of December 31, 2019 and June 30, 2020; 2,666,666 shares issued and outstanding as of December 31, 2019 and June 30, 2020; liquidation value as of December 31, 2019 and June 30, 2020 of $10,431 and $10,828, respectively; no shares authorized, issued or outstanding, pro forma as of June 30, 2020	10,431	10,828	–
Series C redeemable convertible preferred stock, $0.0001 par value; 11,067,963 shares authorized as of December 31, 2019 and June 30, 2020; 9,805,827 shares issued and outstanding as of December 31, 2019 and 8,543,692 shares issued and outstanding as of June 30, 2020; liquidation value as of December 31, 2019 and June 30, 2020 of $50,789 and $45,359, respectively; no shares authorized, issued or outstanding, pro forma as of June 30, 2020	50,789	45,359	–
Stockholders' (deficit) equity:			
Common stock, $0.0001 par value; 46,000,000 shares authorized as of December 31, 2019 and 50,000,000 shares authorized as of June 30, 2020; 1,670,070 shares issued and 1,621,880 shares outstanding as of December 31, 2019, and 1,670,070 shares issued and 1,648,165 shares outstanding as of June 30, 2020; 17,651,659 shares issued and 17,629,754 shares outstanding, pro forma as of June 30, 2020	1	1	2
Additional paid-in capital	–	–	118,189
Accumulated deficit	(81,009)	(104,085)	(104,085)
Total stockholders' (deficit) equity	(81,008)	(104,084)	14,106
Total liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity	$ 47,694	$ 22,653	$ 22,653

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(Unaudited)

(Amounts in thousands, except share and per share data)

	Six Months Ended June 30,	
	2019	2020
Operating expenses:		
Research and development	$ 14,512	$ 15,918
General and administrative	3,129	4,121
Total operating expenses	17,641	20,039
Loss from operations	(17,641)	(20,039)
Total other income:		
Interest income	123	133
Total other income	123	133
Loss before provision for (benefit from) income taxes	(17,518)	(19,906)
Benefit from income taxes	–	(8)
Net loss and comprehensive loss	$ (17,518)	$ (19,898)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,184)	(4,103)
Gain on repurchase of redeemable convertible preferred stock	–	493
Net loss attributable to common stockholders	$ (19,702)	$ (23,508)
Net loss per share attributable to common stockholders, basic and diluted	$ (13.38)	$ (14.37)
Weighted average common shares outstanding, basic and diluted	1,472,484	1,635,913
Pro forma net loss per share attributable to common stockholders, basic and diluted		$ (1.16)
Pro forma weighted average common shares outstanding, basic and diluted		17,177,274

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' (DEFICIT) EQUITY

(Unaudited)

(Amounts in thousands, except share data)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series B-1 Redeemable Convertible Preferred Stock		Series C Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at December 31, 2018	8,075,799	$ 9,284	14,913,704	$ 46,436	–	$ –	–	$ –	1,408,677	$ 1	$ 326	$ (41,365)	$ (41,038)
Issuance of Series B-1 redeemable convertible preferred stock, net of issuance costs of $61	–	–	–	–	2,666,666	9,939	–	–	–	–	–	–	–
Stock-based compensation expense	–	–	–	–	–	–	–	–	–	–	234	–	234
Accretion of redeemable convertible preferred stock to redemption value	–	322	–	1,728	–	88	–	–	–	–	(560)	(1,578)	(2,138)
Vesting of restricted stock awards	–	–	–	–	–	–	–	–	116,823	–	–	–	–
Net loss	–	–	–	–	–	–	–	–	–	–	–	(17,518)	(17,518)
Balance at June 30, 2019	8,075,799	$ 9,606	14,913,704	$ 48,164	2,666,666	$ 10,027	–	$ –	1,525,500	$ 1	$ –	$ (60,461)	$ (60,460)
Balance at December 31, 2019	8,075,799	$ 9,932	14,913,704	$ 49,969	2,666,666	$ 10,431	9,805,827	$ 50,789	1,621,880	$ 1	$ –	$ (81,009)	$ (81,008)
Repurchase of Series C redeemable convertible preferred stock	–	–	–	–	–	–	(5,825,243)	(30,493)	–	–	–	493	493
Issuance of Series C redeemable convertible preferred stock, net of issuance costs of $41	–	–	–	–	–	–	4,563,108	23,459	–	–	–	–	–
Stock-based compensation expense	–	–	–	–	–	–	–	–	–	–	432	–	432
Accretion of redeemable convertible preferred stock to redemption value	–	322	–	1,780	–	397	–	1,604	–	–	(432)	(3,671)	(4,103)
Vesting of restricted stock awards	–	–	–	–	–	–	–	–	26,285	–	–	–	–
Net loss	–	–	–	–	–	–	–	–	–	–	–	(19,898)	(19,898)
Balance at June 30, 2020	8,075,799	$ 10,254	14,913,704	$ 51,749	2,666,666	$ 10,828	8,543,692	$ 45,359	1,648,165	$ 1	$ –	$ (104,085)	$ (104,084)
Conversion of redeemable convertible preferred stock into common stock	(8,075,799)	(10,254)	(14,913,704)	(51,749)	(2,666,666)	(10,828)	(8,543,692)	(45,359)	15,981,589	1	118,189	–	118,190
Pro forma balance at June 30, 2020	–	$ –	–	$ –	–	$ –	–	$ –	17,629,754	$ 2	$ 118,189	$ (104,085)	$ 14,106

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)

(Amounts in thousands)

	Six Months Ended June 30,	
	2019	2020
Cash flows from operating activities:		
Net loss	$(17,518)	$(19,898)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	17	20
Stock-based compensation expense	234	432
Non-cash operating lease expense	315	341
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(520)	(11)
Accounts payable	553	912
Accrued expenses	1,492	22
Operating lease liabilities	(308)	(340)
Other	(31)	9
Net cash used in operating activities	(15,766)	(18,513)
Cash flows from investing activities:		
Purchases of property and equipment	(74)	–
Net cash used in investing activities	(74)	–
Cash flows from financing activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	9,997	23,459
Repurchase of Series C redeemable convertible preferred stock	–	(30,000)
Payment of deferred offering costs	–	(13)
Net cash provided by (used in) financing activities	9,997	(6,554)
Decrease in cash, cash equivalents and restricted cash	(5,843)	(25,067)
Cash, cash equivalents and restricted cash, beginning of period	18,550	45,415
Cash, cash equivalents and restricted cash, end of period	$ 12,707	$ 20,348
Supplemental disclosures of non-cash activities:		
Accretion of redeemable convertible preferred stock to redemption value	$ 2,138	$ 4,103
Operating lease liabilities recorded upon adoption of ASC 842	$ 2,092	$ –
Purchases of property and equipment included in accounts payable	$ 29	$ –
Deferred offering costs included in accounts payable and accrued expenses	$ 58	$ 372

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. Nature of the Business

Praxis Precision Medicines, Inc. ("Praxis" or the "Company") is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system ("CNS") disorders characterized by neuronal imbalance. The Company has established a broad portfolio, including five disclosed programs across multiple CNS disorders, including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. The Company's most advanced programs, PRAX-114 and PRAX-944, are currently in Phase 2 development. PRAX-114 is being developed for the treatment of major depressive disorder and perimenopausal depression, and PRAX-944 is a selective small molecule inhibitor of T-type calcium channels for the treatment of Essential Tremor.

Praxis was incorporated in 2015. The Company has funded its operations primarily with proceeds from the issuance of convertible debt, Series A redeemable convertible preferred stock (the "Series A Preferred Stock"), Series B redeemable convertible preferred stock (the "Series B Preferred Stock"), Series B-1 redeemable convertible preferred stock (the "Series B-1 Preferred Stock") and Series C redeemable convertible preferred stock (the "Series C Preferred Stock") (the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock and Series C Preferred Stock are collectively referred to as the "Preferred Stock"). From inception through June 30, 2020, the Company raised $100.6 million in aggregate cash proceeds from these transactions, net of issuance costs. In the third quarter of 2020, the Company sold and issued shares of Series C-1 redeemable convertible preferred stock for aggregate cash proceeds of approximately $110.1 million, net of issuance costs.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Programs currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Going Concern

In accordance with the Financial Accounting Standards Board Accounting Standards Update 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that these condensed consolidated financial statements are issued.

The Company has incurred recurring losses since its inception, including net losses of $17.5 million and $19.9 million for the six months ended June 30, 2019 and 2020, respectively. In addition, as of June 30, 2020, the Company had an accumulated deficit of $104.1 million. The Company expects to continue to generate operating losses for the foreseeable future. The future viability of the Company is dependent on its ability to raise additional capital to finance its operations.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurance that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

The Company expects that its cash and cash equivalents as of June 30, 2020 of $19.7 million, together with the $110.1 million net cash proceeds from the sale and issuance of shares of Series C-1 Preferred Stock in the third quarter of 2020, will be sufficient to fund the operating expenses and capital expenditure requirements necessary to advance its research efforts and clinical trials into the second half of 2021.

The future viability of the Company beyond one year from the date of issuance of these condensed consolidated financial statements is dependent on its ability to raise additional capital to finance its operations. If the Company is unable to obtain additional funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion, or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations. The Company expects to seek additional funding through private or public equity transactions, debt financings, or other capital sources, including collaborations with other companies or other strategic transactions.

Although management plans to pursue additional funding, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

Based on its recurring losses and negative cash flows from operations since inception, expectation of continuing operating losses and negative cash flows from operations for the foreseeable future, and the need to raise additional capital to finance its future operations, management concluded that there is substantial doubt about the Company's ability to continue as a going concern for one year after the date that these condensed consolidated financial statements are issued.

The accompanying condensed consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Accordingly, the condensed consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the years ended December 31, 2018 and 2019, included elsewhere in this prospectus. Since the date of those financial statements, there have been no changes to its significant accounting policies except as noted below.

Unaudited Interim Condensed Consolidated Financial Information

The accompanying condensed consolidated balance sheet as of June 30, 2020, the condensed consolidated statements of operations and comprehensive loss and statements of cash flows for the six months ended June 30, 2019 and 2020 and the condensed consolidated statements of redeemable convertible preferred stock and stockholders' (deficit) equity for the six months ended June 30, 2019 and 2020 are unaudited. The unaudited interim financial statements have been prepared on the same basis as the audited annual consolidated financial statements, and in the opinion of management reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of June 30, 2020 and the results of its operations and its cash flows for the six months ended June 30, 2019 and 2020. Financial statement disclosures for the six months ended June 30, 2019 and 2020 are condensed and do not include all disclosures required for an annual set of financial statements in accordance with GAAP.

The results for the six months ended June 30, 2020 are not necessarily indicative of results to be expected for the year ended December 31, 2020, any other interim periods, or any future year or period.

Unaudited Pro Forma Information

Upon the closing of a qualified public offering (as defined in the Company's Amended and Restated Certificate of Incorporation), all of the Company's outstanding shares of redeemable convertible preferred stock will automatically convert into shares of common stock. The accompanying unaudited pro forma condensed consolidated balance sheet and condensed consolidated statement of redeemable convertible preferred stock and stockholders' (deficit) equity as of June 30, 2020 have been prepared as if the Company's proposed initial public offering ("IPO") had occurred on June 30, 2020 to give effect to the automatic conversion of all outstanding shares of redeemable convertible preferred stock into an aggregate of 15,981,589 shares of common stock upon the consummation of the proposed IPO. The shares of common stock expected to be issued and the proceeds expected to be received in the proposed IPO are excluded from such pro forma financial information.

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders in the accompanying condensed consolidated statement of operations and comprehensive loss for the six months ended June 30, 2020 was computed using the weighted average number of shares of common stock outstanding, including the pro forma effect of the automatic conversion of all outstanding shares of redeemable convertible preferred stock into shares of common stock as if the proposed IPO had occurred on the later of: (i) January 1, 2019 or (ii) the date the equity instruments were issued. The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the six months ended June 30, 2020: (i) excludes the effects of cumulative dividends accrued for redeemable convertible preferred stock from the net loss attributable to common

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

stockholders, (ii) excludes the effects of other accretion recorded for redeemable convertible preferred stock from the net loss attributable to common stockholders and (iii) excludes the effects of the gains on the redemptions of redeemable convertible preferred stock from the net loss attributable to common stockholders. The unaudited pro forma basic and diluted net loss per share attributable to common stockholders does not include the shares expected to be sold or related proceeds to be received in the proposed IPO.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual for research and development expenses, stock-based compensation expense, the valuation of equity instruments and the recoverability of the Company's net deferred tax assets and related valuation allowance. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from those estimates.

Cash, Cash Equivalents and Restricted Cash

The following table reconciles cash, cash equivalents and restricted cash to the total amounts on the condensed consolidated statements of cash flows (in thousands):

	June 30,	
	2019	2020
Cash and cash equivalents	$12,107	$19,748
Restricted cash	600	600
Total cash, cash equivalents and restricted cash as shown in the condensed consolidated statement of cash flows	$12,707	$20,348

Deferred Offering Costs

The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After the consummation of the equity financing, these costs are recorded in stockholders' (deficit) equity as a reduction of additional paid-in capital or the associated preferred stock account, as applicable. In the event the offering is terminated, all capitalized deferred offering costs are expensed. Deferred offering costs as of June 30, 2020 were $0.4 million. Such costs are classified in other non-current assets in the accompanying condensed consolidated balance sheet. No deferred offering costs were capitalized as of December 31, 2019.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

Stock-Based Compensation

The Company utilizes significant estimates and assumptions in determining the fair value of its equity and equity-based awards. Beginning in the six months ended June 30, 2020, the Company determined the fair value of shares of its common stock underlying stock-based awards granted using a hybrid probability-weighted expected return method ("PWERM"). The fair value of the Company's common stock was calibrated to contemporaneous transactions in the Series C Preferred Stock. The hybrid PWERM determined the fair value of the Company's common stock using a probability-weighted present value of expected future investment returns considering various outcomes, as well as the rights of each class of stock, with one of the outcomes calculated using an option pricing model ("OPM"). The hybrid PWERM considered three scenarios: an "early" IPO completed in 2020, a "late" IPO completed in 2021, and a "remain private" scenario in which value is allocated using the OPM. An incremental discount for lack of marketability ("DLOM") was applied to the values of the common stock. The DLOM was estimated using a put option model which considered the expected time to liquidity and the volatility of the common shares. The hybrid PWERM used a risk-adjusted discount rate.

Other than as noted herein, there were no other changes to the Company's stock-based compensation policy since the date of the audited consolidated financial statements for the years ended December 31, 2018 and 2019, included elsewhere in this prospectus.

Comprehensive Loss

Comprehensive loss includes net loss and certain changes in stockholders' deficit that are excluded from net loss. The Company's comprehensive loss was equal to net loss for the six months ended June 30, 2019 and 2020.

Net Loss per Share

The Company follows the two-class method to compute net loss per share attributable to common stockholders as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (losses) for the period to be allocated between common and participating securities based upon their respective rights to share in the earnings as if all income (losses) for the period had been distributed. During periods of loss, there is no allocation required under the two-class method since the participating securities do not have a contractual obligation to fund the losses of the Company.

Net loss attributable to common stockholders is equal to the net loss for the period, as adjusted for: (i) cumulative dividends accrued for redeemable convertible preferred stock, whether or not declared, (ii) increases in carrying value recorded for redeemable convertible preferred stock, including accretion on redeemable convertible preferred stock to redemption value for amounts other than cumulative dividends and (iii) gains on the redemptions of redeemable convertible preferred stock.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, which excludes shares of restricted common stock that are not vested. Diluted net loss

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(Continued)

(Unaudited)

per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, after giving consideration to the dilutive effect of potentially dilutive common shares. For purposes of this calculation, outstanding options to purchase shares of common stock, unvested shares of restricted common stock and shares of redeemable convertible preferred stock are considered potentially dilutive common shares. The Company has generated a net loss in all periods presented so the basic and diluted net loss per share attributable to common stockholders are the same as the inclusion of the potentially dilutive securities would be anti-dilutive.

3. Fair Value of Financial Assets

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	As of December 31, 2019			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$44,429	$ –	$ –	$44,429
	$44,429	$ –	$ –	$44,429

	As of June 30, 2020			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$19,361	$ –	$ –	$19,361
	$19,361	$ –	$ –	$19,361

4. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31, 2019	June 30, 2020
Accrued external research and development expenses	$ 1,552	$ 2,125
Accrued personnel-related expenses	1,059	887
Accrued license fees	363	–
Accrued professional services	110	673
Accrued other	371	55
Total accrued expenses	$ 3,455	$ 3,740

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

5. Commitments and Contingencies

Leases

In October 2018, the Company entered into a sublease agreement for office space located in Cambridge, Massachusetts which expires on December 30, 2021, with no option to renew or terminate early. The base rent increases by approximately 1% annually. The Company issued a letter of credit to the landlord related to the security deposit, secured by restricted cash, which is recorded in other assets on the accompanying condensed consolidated balance sheets. This lease qualifies as an operating lease.

In January 2019, the Company entered into an arrangement with a third party to sublease a portion of its Cambridge, Massachusetts office space. This sublease was terminated in November 2019.

6. Redeemable Convertible Preferred Stock

As of December 31, 2019 and June 30, 2020, the authorized capital stock of the Company included 36,724,132 shares of $0.0001 par value Preferred Stock, of which 8,075,799 shares have been designated as Series A Preferred Stock, 14,913,704 shares have been designated as Series B Preferred Stock, 2,666,666 shares have been designated as Series B-1 Preferred Stock and 11,067,963 shares have been designated as Series C Preferred Stock.

On January 20, 2020, the Company granted two investors holding 5,825,243 shares of Series C Preferred Stock that were purchased in December 2019 the option to put their shares back to the Company at the original issuance price. On February 19, 2020 and March 3, 2020, the investors exercised their put option in full via the execution of Stock Redemption and Release Agreements in order to effect the repurchase. Pursuant to the Stock Redemption and Release Agreements, the Company agreed to repurchase a total of 5,825,243 shares of Series C Preferred Stock at the original issuance price of $5.15 per share, for an aggregate cash repurchase price of $30.0 million. Under the terms of the Stock Redemption and Release Agreements, the investors waived their right to cumulative dividends that had accumulated from the original issuance date through the date of repurchase. The 5,825,243 shares of Series C Preferred Stock were retired upon repurchase, and subsequently authorized for reissuance pursuant to a waiver to the Company's Amended and Restated Certificate of Incorporation entered into by the Company and the holders of the Preferred Stock.

The Company determined that the additional put right that was granted to the investors represented a modification of the affected shares of Series C Preferred Stock, but that it did not result in incremental value to the shareholders. As there was no incremental value associated with the modification, there was no impact to the accounting for the Series C Preferred Stock. The Company also determined that the put right did not require bifurcation, as it does not contain the characteristics of a derivative instrument. Further, the Company determined that the shares of Series C Preferred Stock that were subject to repurchase did not become mandatorily redeemable until the execution of the Stock Redemption and Release Agreements because the parties did not have an unconditional legal obligation to complete the redemptions until the associated agreements were finalized. Such determination was made in consultation with legal counsel. Accordingly, the Company recorded each of the redemptions on the respective date of repurchase and recognized a gain on repurchase equal to the difference between the repurchase price and the carrying value of the Series C Preferred Stock on

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

the respective date of repurchase. The aggregate gain of $0.5 million was recorded upon repurchase as an adjustment to accumulated deficit in the statement of redeemable convertible preferred stock and stockholders' (deficit) equity. The gain relates exclusively to the dividends accrued on the repurchased shares, which were waived by the investors as part of the Stock Redemption and Release Agreements.

On April 15, 2020 and May 8, 2020, the Company completed additional closings for the sale and issuance of its Series C Preferred Stock for a total of 4,563,108 shares at $5.15 per share for aggregate cash proceeds of $23.5 million, less an immaterial amount of issuance costs.

The Preferred Stock consisted of the following (in thousands, except share amounts):

	As of December 31, 2019					
	Shares Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Redemption Value	Common Stock Issuable Upon Conversion
Series A Preferred Stock	8,075,799	8,075,799	$ 9,932	$ 9,932	$ 9,932	3,773,820
Series B Preferred Stock	14,913,704	14,913,704	49,969	49,969	49,969	6,969,173
Series B-1 Preferred Stock	2,666,666	2,666,666	10,431	10,431	10,431	1,246,133
Series C Preferred Stock	11,067,963	9,805,827	50,789	50,789	50,789	4,582,257
	36,724,132	35,461,996	$ 121,121	$ 121,121	$ 121,121	16,571,383

	As of June 30, 2020					
	Shares Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Redemption Value	Common Stock Issuable Upon Conversion
Series A Preferred Stock	8,075,799	8,075,799	$ 10,254	$ 10,254	$ 10,254	3,773,820
Series B Preferred Stock	14,913,704	14,913,704	51,749	51,749	51,749	6,969,173
Series B-1 Preferred Stock	2,666,666	2,666,666	10,828	10,828	10,828	1,246,133
Series C Preferred Stock	11,067,963	8,543,692	45,359	45,359	45,359	3,992,463
	36,724,132	34,199,861	$ 118,190	$ 118,190	$ 118,190	15,981,589

Common stock issuable upon conversion in the tables above represents shares of common stock issuable upon an automatic conversion in the event of a qualified public offering, pursuant to the Company's Amended and Restated Certificate of Incorporation.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

The Company's cumulative dividends on its Preferred Stock were as follows (in thousands):

	As of December 31, 2019	As of June 30, 2020
Series A Preferred Stock	$ 1,857	$ 2,178
Series B Preferred Stock	5,228	7,008
Series B-1 Preferred Stock	431	828
Series C Preferred Stock	289	1,360
	$ 7,805	$ 11,374

As of June 30, 2020, each share of the Preferred Stock was convertible into one share of common stock and may be adjusted in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock.

7. Common Stock

As of December 31, 2019 and June 30, 2020, the authorized capital stock of the Company included 46,000,000 and 50,000,000 shares of common stock, $0.0001 par value, respectively.

Shares Reserved for Future Issuance

The Company has reserved the following shares of common stock for future issuance:

	December 31, 2019	June 30, 2020
Series A Preferred Stock	3,773,820	3,773,820
Series B Preferred Stock	6,969,173	6,969,173
Series B-1 Preferred Stock	1,246,133	1,246,133
Series C Preferred Stock	4,582,257	3,992,463
Shares reserved for vesting of restricted common stock	48,190	21,905
Shares reserved for exercise of outstanding stock options	1,634,686	3,220,201
Shares reserved for future awards under the 2017 Stock Incentive Plan	617,101	584,384
Total shares of authorized common stock reserved for future issuance	18,871,360	19,808,079

8. Stock-Based Compensation

2017 Stock Incentive Plan

The total number of shares of common stock authorized for issuance under the 2017 Plan as of December 31, 2019 and June 30, 2020 was 2,356,927 shares and 3,909,725 shares, respectively. As of June 30, 2020, the Company did not hold any treasury shares.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

Restricted Common Stock

The following table summarizes all of the Company's restricted common stock activity:

	Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2019	48,190	$ 0.06
Issued	–	–
Vested	(26,285)	0.05
Repurchased	–	–
Unvested as of June 30, 2020	21,905	$ 0.06

The total fair value of restricted common stock that vested during the six months ended June 30, 2019 and 2020 was $0.3 million and $0.1 million, respectively.

Stock Options

The following table summarizes the Company's stock option activity:

	Number of Shares	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding as of December 31, 2019	1,634,686	$ 2.46		
Granted	1,683,265	5.59		
Exercised	–	–		–
Cancelled or Forfeited	(97,750)	3.23		
Outstanding as of June 30, 2020	3,220,201	$ 4.07	9.23	$ 4,876
Exercisable as of June 30, 2020	890,030	$ 2.27	8.34	$ 2,950
Vested and expected to vest as of June 30, 2020	3,220,201	$ 4.07	9.23	$ 4,876

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the underlying stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the estimated fair value of the Company's common stock at June 30, 2020.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

Stock Option Valuation

The assumptions that the Company used in the Black-Scholes option pricing model to determine the grant-date fair value of stock options granted to employees, members of the board of directors and non-employees on the date of grant were as follows for the six months ended June 30, 2020:

Risk-free interest rate	0.59 – 0.91%
Expected term (in years)	6.00 – 10.00
Expected volatility	86.08 – 88.11%
Expected dividend yield	0.00%
Fair value per share of common stock	$ 5.59

The weighted-average grant-date fair value of the Company's stock options granted during the six months ended June 30, 2020 was $4.02. The Company did not grant any stock options during the six months ended June 30, 2019.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows (in thousands):

	Six Months Ended June 30,	
	2019	2020
Research and development	$ 141	$ 255
General and administrative	93	177
Total stock-based compensation expense	$ 234	$ 432

As of June 30, 2020, total unrecognized compensation cost related to unvested stock-based awards was $7.8 million, which is expected to be recognized over a weighted-average period of 3.56 years.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

9. Net Loss per Share and Unaudited Pro Forma Net Loss per Share

Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share data):

	Six Months Ended June 30,	
	2019	2020
Numerator:		
Net loss	$ (17,518)	$ (19,898)
Accretion and cumulative dividends on redeemable convertible preferred stock	(2,184)	(4,103)
Gain on repurchase of redeemable convertible preferred stock	–	493
Net loss attributable to common stockholders	$ (19,702)	$ (23,508)
Denominator:		
Weighted average common shares outstanding, basic and diluted	1,472,484	1,635,913
Net loss per share attributable to common stockholders, basic and diluted	$ (13.38)	$ (14.37)

The following potential common shares, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have been anti-dilutive:

	Six Months Ended June 30,	
	2019	2020
Series A Preferred Stock	3,773,820	3,773,820
Series B Preferred Stock	6,969,173	6,969,173
Series B-1 Preferred Stock	1,246,133	1,246,133
Series C Preferred Stock	–	3,992,463
Outstanding stock options	1,192,465	3,220,201
Unvested restricted common stock	144,570	21,905
	13,326,161	19,223,695

The shares of common stock issuable upon conversion of the Preferred Stock assume automatic conversion in the event of a qualified public offering.

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

Unaudited Pro Forma Net Loss per Share

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share data):

	Six Months Ended June 30, 2020
Numerator:	
Net loss attributable to common stockholders	$ (23,508)
Accretion and cumulative dividends on redeemable convertible preferred stock	4,103
Gain on repurchase of redeemable convertible preferred stock	(493)
Pro forma net loss attributable to common stockholders	$ (19,898)
Denominator:	
Weighted average common shares outstanding, basic and diluted	1,635,913
Pro forma adjustment to reflect the automatic conversion of redeemable convertible preferred stock into common stock upon the completion of the proposed initial public offering	15,541,361
Pro forma weighted average common shares outstanding, basic and diluted	17,177,274
Pro forma net loss per share attributable to common stockholders, basic and diluted	$ (1.16)

10. Related Party Transactions

One of the founders of RogCon Inc. (“RogCon”) became the Company’s General Counsel in June 2020. The Company continues to reimburse RogCon for its out-of-pocket costs incurred for activities performed under its license agreement with RogCon entered into in September 2019 (the “License Agreement”). During the six months ended June 30, 2020, the Company expensed $0.1 million for the reimbursement of RogCon’s out-of-pocket costs. As of June 30, 2020, the Company had accrued expenses of $0.3 million due to RogCon under the License Agreement.

11. Subsequent Events

Subsequent Events Through August 28, 2020

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the condensed consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. The Company has evaluated subsequent events through August 28, 2020, the date these condensed consolidated financial statements were issued and identified the following subsequent events:

Common Stock and Preferred Stock Authorized for Issuance

On July 24, 2020, the board of directors (the “Board”) amended the Company’s Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance to 66,000,000 shares. In addition, the Board amended the Company’s Amended and

PRAXIS PRECISION MEDICINES, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

(Continued)

Restated Certificate of Incorporation to increase the number of shares of preferred stock authorized for issuance to 53,644,314 shares, of which 8,075,799 shares are designated as Series A Preferred Stock, 14,913,704 shares are designated as Series B Preferred Stock, 2,666,666 shares are designated as Series B-1 Preferred Stock, 8,543,692 shares are designated as Series C Preferred Stock and 19,444,453 shares are designated as Series C-1 redeemable convertible preferred stock.

Series C-1 Redeemable Convertible Preferred Stock Issuance

In the third quarter of 2020, the Company issued a total of 19,444,453 shares of Series C-1 redeemable convertible preferred stock at a purchase price of $5.67 per share for aggregate proceeds of approximately $110.1 million, net of issuance costs.

Subsequent Events Through October 9, 2020

In connection with the re-issuance of these condensed consolidated financial statements, the Company has evaluated subsequent events through October 9, 2020, the date these condensed consolidated financial statements were re-issued, and identified the following subsequent events:

Common Stock Authorized for Issuance

On September 2, 2020, the Company amended the Company's Amended and Restated Certificate of Incorporation to increase the number of shares of common stock authorized for issuance to 70,500,000 shares.

Amendment to 2017 Stock Incentive Plan

On September 2, 2020, the Company amended the 2017 Stock Incentive Plan to increase the total number of shares authorized for issuance to 5,937,763 shares.

Reverse Stock Split

On October 8, 2020, the Board and the Company's stockholders approved a one-for-2.14 reverse stock split. Effective on October 8, 2020, the reverse stock split impacted the Company's issued and outstanding shares of common stock. Stockholders entitled to fractional shares as a result of the reverse stock split will receive a cash payment in lieu of receiving fractional shares. All shares of common stock, per share amounts and additional paid-in capital amounts for all periods presented in the accompanying condensed consolidated financial statements and related notes have been retroactively adjusted, where applicable, to reflect the reverse stock split. Shares of common stock underlying outstanding stock options and other equity instruments were proportionately reduced and the respective exercise prices were proportionately increased, as applicable, in accordance with the terms of the agreements governing such securities. The respective conversion prices of the Preferred Stock were proportionately increased. The number of shares of common stock authorized for issuance and the par value of common stock were not adjusted as a result of the reverse stock split.

10,000,000 Shares

Common Stock

P R O S P E C T U S

Book-running Managers

Cowen **Evercore ISI** **Piper Sandler**

Lead Manager
Wedbush PacGrow

Co-Manager
Blackstone Capital Markets

Until November 9, 2020, all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.