Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2020

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-39620

PRAXIS PRECISION MEDICINES, INC.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-5195942**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
One Broadway, 16th Floor Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: 617-300-8460

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	**PRAX**	**The Nasdaq Global Select Market**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 13, 2020, the registrant had 38,261,893 shares of common stock, $0.0001 par value per share, outstanding.

SUMMARY OF THE MATERIAL AND OTHER RISKS ASSOCIATED WITH OUR BUSINESS

- 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.
- 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.
- The markets for PRAX-114 for major depressive disorder and perimenopausal disorder, PRAX-944 for essential tremor, PRAX-562 for multiple rare neurological conditions and any other product candidates we may develop may be smaller than we expect.
- 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.
- 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.
- Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.
- 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.
- Business interruptions resulting from COVID-19 or a similar pandemic, epidemic or outbreak of an infectious disease in the United States or worldwide may adversely affect our business.
- The price of our stock may be volatile, and you could lose all or part of your investment.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q 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 contained in this Quarterly Report on Form 10-Q 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, or a smaller reporting company as defined in the Securities Exchange Act of 1934, as amended;
- 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, you can identify forward-looking statements 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 the section titled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. 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 Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed with the Securities and Exchange Commission as exhibits hereto 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 Quarterly Report on Form 10-Q represent our views as of the date of this Quarterly Report on Form 10-Q. 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 Quarterly Report on Form 10-Q.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for our product candidates. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from our own internal estimates and research as well as from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. While we are not aware of any misstatements regarding any third-party information presented in this Quarterly Report on Form 10-Q, 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 titled “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q.

Table of Contents

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

(Amounts in thousands, except share and per share data)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 114,772	$ 44,815
Prepaid expenses and other current assets	1,079	681
Total current assets	115,851	45,496
Property and equipment, net	98	128
Restricted cash	600	600
Operating lease right-of-use assets	933	1,450
Other assets	2,066	20
Total assets	$ 119,548	$ 47,694
Liabilities, redeemable convertible preferred stock and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 3,452	$ 2,667
Accrued expenses	6,186	3,455
Operating lease liabilities	746	696
Total current liabilities	10,384	6,818
Long-term liabilities:		
Non-current portion of operating lease liabilities	198	763
Total liabilities	10,582	7,581
Commitments and contingencies (Note 5)		
Series A redeemable convertible preferred stock, $0.0001 par value; 8,075,799 shares authorized as of September 30, 2020 and December 31, 2019; 8,075,799 shares issued and outstanding as of September 30, 2020 and December 31, 2019; liquidation value as of September 30, 2020 and December 31, 2019 of $10,416 and $9,932, respectively	10,416	9,932
Series B redeemable convertible preferred stock, $0.0001 par value; 14,913,704 shares authorized as of September 30, 2020 and December 31, 2019; 14,913,704 shares issued and outstanding as of September 30, 2020 and December 31, 2019; liquidation value as of September 30, 2020 and December 31, 2019 of $52,648 and $49,969, respectively	52,648	49,969
Series B-1 redeemable convertible preferred stock, $0.0001 par value; 2,666,666 shares authorized as of September 30, 2020 and December 31, 2019; 2,666,666 shares issued and outstanding as of September 30, 2020 and December 31, 2019; liquidation value as of September 30, 2020 and December 31, 2019 of $11,029 and $10,431, respectively	11,029	10,431
Series C redeemable convertible preferred stock, $0.0001 par value; 8,543,692 shares authorized as of September 30, 2020 and 11,067,963 shares authorized as of December 31, 2019; 8,543,692 shares issued and outstanding as of September 30, 2020 and 9,805,827 shares issued and outstanding as of December 31, 2019; liquidation value as of September 30, 2020 and December 31, 2019 of $46,244 and $50,789, respectively	46,244	50,789
Series C-1 redeemable convertible preferred stock, $0.0001 par value; 19,444,453 shares authorized as of September 30, 2020 and no shares authorized as of December 31, 2019; 19,444,453 shares issued and outstanding as of September 30, 2020 and no shares issued and outstanding as of December 31, 2019; liquidation value as of September 30, 2020 and December 31, 2019 of $111,892 and $0, respectively	111,892	—
Stockholders' deficit:		
Common stock, $0.0001 par value; 70,500,000 shares authorized as of September 30, 2020 and 46,000,000 shares authorized as of December 31, 2019; 1,681,698 shares issued and 1,672,935 shares outstanding as of September 30, 2020 and 1,670,070 shares issued and 1,621,880 shares outstanding as of December 31, 2019	1	1
Accumulated deficit	(123,264)	(81,009)
Total stockholders' deficit	(123,263)	(81,008)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	$ 119,548	$ 47,694

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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 12,786	$ 9,346	$ 28,704	$ 23,858
General and administrative	3,431	1,308	7,552	4,437
Total operating expenses	16,217	10,654	36,256	28,295
Loss from operations	(16,217)	(10,654)	(36,256)	(28,295)
Other income:				
Interest income	1	48	134	171
Total other income	1	48	134	171
Loss before benefit from income taxes	(16,216)	(10,606)	(36,122)	(28,124)
Benefit from income taxes	—	—	(8)	—
Net loss and comprehensive loss	$ (16,216)	$ (10,606)	$ (36,114)	$ (28,124)
Accretion and cumulative dividends on redeemable convertible preferred stock	(3,943)	(1,267)	(8,046)	(3,450)
Gain on repurchase of redeemable convertible preferred stock	—	—	493	—
Net loss attributable to common stockholders	$ (20,159)	$ (11,873)	$ (43,667)	$ (31,574)
Net loss per share attributable to common stockholders, basic and diluted	$ (12.10)	$ (7.61)	$ (26.53)	$ (21.02)
Weighted average common shares outstanding, basic and diluted	1,665,902	1,559,800	1,645,982	1,501,908

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

(Unaudited)

(Amounts in thousands, except share data)

	Three Months Ended														
	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		Series C-1 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
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)
Issuance of Series C-1 redeemable convertible preferred stock, net of issuance costs of $154	—	—	—	—	—	—	—	—	19,444,453	110,096	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	953	—	953
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	—	—	11,628	—	27	—	27
Accretion of redeemable convertible preferred stock to redemption value	—	162	—	899	—	201	—	885	—	1,796	—	—	(980)	(2,963)	(3,943)
Vesting of restricted stock awards	—	—	—	—	—	—	—	—	—	—	13,142	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	(16,216)	(16,216)
Balance at September 30, 2020	8,075,799	$10,416	14,913,704	$52,648	2,666,666	$11,029	8,543,692	$46,244	19,444,453	$111,892	1,672,935	$ 1	$ —	$ (123,264)	$ (123,263)
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)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	276	—	276
Accretion of redeemable convertible preferred stock to redemption value	—	163	—	902	—	202	—	—	—	—	—	—	(276)	(991)	(1,267)
Vesting of restricted stock awards	—	—	—	—	—	—	—	—	—	—	58,410	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	(10,606)	(10,606)
Balance at September 30, 2019	8,075,799	$ 9,769	14,913,704	$49,066	2,666,666	$10,229	—	$ —	—	$ —	1,583,910	$ 1	$ —	$ (72,058)	$ (72,057)

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

(Unaudited)

(Amounts in thousands, except share data)

	Nine Months Ended														
	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		Series C-1 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
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	—	—	—	—	—	—	—
Issuance of Series C-1 redeemable convertible preferred stock, net of issuance costs of $154	—	—	—	—	—	—	—	—	19,444,453	110,096	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	1,385	—	1,385
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	—	—	11,628	—	27	—	27
Accretion of redeemable convertible preferred stock to redemption value	—	484	—	2,679	—	598	—	2,489	—	1,796	—	—	(1,412)	(6,634)	(8,046)
Vesting of restricted stock awards	—	—	—	—	—	—	—	—	—	—	39,427	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	(36,114)	(36,114)
Balance at September 30, 2020	8,075,799	$10,416	14,913,704	$52,648	2,666,666	$11,029	8,543,692	$ 46,244	19,444,453	$111,892	1,672,935	$ 1	$ —	$ (123,264)	$ (123,263)
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	—	—	—	—	—	—	—	—	—	—	—	—	510	—	510
Accretion of redeemable convertible preferred stock to redemption value	—	485	—	2,630	—	290	—	—	—	—	—	—	(836)	(2,569)	(3,405)
Vesting of restricted stock awards	—	—	—	—	—	—	—	—	—	—	175,233	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	(28,124)	(28,124)
Balance at September 30, 2019	8,075,799	$ 9,769	14,913,704	$49,066	2,666,666	$10,229	—	$ —	—	$ —	1,583,910	$ 1	$ —	$ (72,058)	$ (72,057)

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)

	Nine Months Ended September 30,	
	2020	2019
Cash flows from operating activities:		
Net loss	$ (36,114)	$ (28,124)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	30	27
Stock-based compensation expense	1,385	510
Non-cash operating lease expense	517	477
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(398)	829
Accounts payable	544	(356)
Accrued expenses	2,160	3,664
Operating lease liabilities	(515)	(468)
Other	(4)	(25)
Net cash used in operating activities	(32,395)	(23,466)
Cash flows from investing activities:		
Purchases of property and equipment	—	(103)
Net cash used in investing activities	—	(103)
Cash flows from financing activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	133,442	9,939
Repurchase of Series C redeemable convertible preferred stock	(30,000)	—
Payment of deferred offering costs	(1,117)	—
Proceeds from exercise of options to purchase common stock	27	—
Net cash provided by financing activities	102,352	9,939
Increase (decrease) in cash, cash equivalents and restricted cash	69,957	(13,630)
Cash, cash equivalents and restricted cash, beginning of period	45,415	18,550
Cash, cash equivalents and restricted cash, end of period	$115,372	$ 4,920
Supplemental disclosures of non-cash activities:		
Accretion of redeemable convertible preferred stock to redemption value	$ 8,046	$ 3,405
Operating lease liabilities recorded upon adoption of ASC 842	$ —	$ 2,092
Deferred offering costs included in accounts payable and accrued expenses	$ 925	$ —

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. The Company plans to continue to discover and develop additional product candidates and expand into additional therapeutic areas.

Praxis was incorporated in 2015 and commenced operations in 2016. 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"), Series C redeemable convertible preferred stock (the "Series C Preferred Stock") and Series C-1 redeemable convertible preferred stock (the "Series C-1 Preferred Stock") (the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock, Series C Preferred Stock and Series C-1 Preferred Stock are collectively referred to as the "Preferred Stock"). From inception through September 30, 2020, the Company raised $210.7 million in aggregate cash proceeds from these transactions, net of issuance costs.

On October 8, 2020, the board of directors 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, aggregate par value 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 per share par value of common stock were not adjusted as a result of the reverse stock split.

On October 20, 2020, the Company completed its initial public offering ("IPO"), in which the Company issued and sold 11,500,000 shares of its common stock at a public offering price of $19.00 per share, including 1,500,000 shares of common stock sold pursuant to the underwriters' exercise of their option to purchase additional shares of common stock, for aggregate gross proceeds of $218.5 million. The Company raised approximately $199.9 million in net proceeds after deducting underwriting discounts and commissions and offering expenses payable by the Company. Upon the closing of the IPO, all of the outstanding shares of the Company's Preferred Stock automatically converted into 25,067,977 shares of common stock. On October 20, 2020, in connection with the closing of the IPO, the Company filed its Amended and Restated Certificate of Incorporation which provides that the authorized capital stock of the Company consists of 150,000,000 shares of common stock and 10,000,000 shares of undesignated preferred stock, both with a par value of $0.0001 per share.

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.

Liquidity

In accordance with the Financial Accounting Standards Board ("FASB") Accounting Standards Update ("ASU") 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 $36.1 million and $28.1 million for the nine months ended September 30, 2020 and 2019, respectively. In addition, as of September 30, 2020, the Company had an accumulated deficit of $123.3 million. The Company expects to continue to generate operating losses for the foreseeable future.

The Company previously identified conditions and events that raised substantial doubt about its ability to continue as a going concern. As a result of the completion of its IPO, the Company expects that its cash and cash equivalents as of September 30, 2020 of $114.8 million, in addition to the net proceeds of $199.9 million from its IPO, will be sufficient to fund the operating expenses and capital expenditure requirements necessary to advance its research efforts and clinical trials for at least one year from the date of issuance of these condensed consolidated financial statements. 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.

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 ASUs of the FASB.

The Company's significant accounting policies are disclosed in the audited consolidated financial statements included in the Company's final prospectus filed with the Securities and Exchange Commission ("SEC") pursuant to Rule 424(b)(4) on October 16, 2020. Since the date of those audited consolidated financial statements, there have been no changes to the Company's significant accounting policies except as noted below.

Unaudited Interim Condensed Consolidated Financial Information

The accompanying condensed consolidated balance sheet as of September 30, 2020, the condensed consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2020 and 2019, the condensed consolidated statements of cash flows for the nine months ended September 30, 2020 and 2019 and the condensed consolidated statements of redeemable convertible preferred stock and stockholders' deficit for the three and nine months ended September 30, 2020 and 2019 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 September 30, 2020, the results of its operations for the three and nine months ended September 30, 2020 and 2019 and its cash flows for the nine months ended September 30, 2020 and 2019. Financial statement disclosures for the three and nine months ended September 30, 2020 and 2019 are condensed and do not include all disclosures required for an annual set of financial statements in accordance with GAAP.

The results for the three and nine months ended September 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.

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, and the valuation of equity awards. 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):

	September 30,	
	2020	2019
Cash and cash equivalents	$114,772	$4,320
Restricted cash	600	600
Total cash, cash equivalents and restricted cash as shown in the condensed consolidated statement of cash flows	$115,372	$4,920

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 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 September 30, 2020 were $2.0 million. Such costs are classified in other assets in the accompanying condensed consolidated balance sheet. No deferred offering costs were capitalized as of December 31, 2019.

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 nine months ended September 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 and Series C-1 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 included in the Company's final prospectus filed pursuant to Rule 424(b)(4) on October 16, 2020.

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 three and nine months ended September 30, 2020 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) 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 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 September 30, 2020			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$114,386	$ —	$ —	$114,386
	$114,386	$ —	$ —	$114,386

	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

4. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Accrued external research and development expenses	$ 3,370	$ 1,552
Accrued personnel-related expenses	1,606	1,059
Accrued license fees	—	363
Accrued professional services	1,135	110
Accrued other	75	371
Total accrued expenses	$ 6,186	$ 3,455

5. Commitments and Contingencies

In October 2018, the Company entered into a sublease agreement for office space located in Cambridge, Massachusetts that 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 reflected as a non-current asset 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 September 30, 2020, the authorized capital stock of the Company included 53,644,314 shares of $0.0001 par value Preferred Stock, of which 8,075,799 shares were designated as Series A Preferred Stock, 14,913,704 shares were designated as Series B Preferred Stock, 2,666,666 shares were designated as Series B-1 Preferred Stock, 8,543,692 shares were designated as Series C Preferred Stock and 19,444,453 shares were designated as Series C-1 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 were designated as Series A Preferred Stock, 14,913,704 shares were designated as Series B Preferred Stock, 2,666,666 shares were designated as Series B-1 Preferred Stock and 11,067,963 shares were designated as Series C Preferred Stock. The rights, preferences and privileges of the Series A Preferred Stock, the Series B Preferred Stock, the Series B-1 Preferred Stock and the Series C Preferred Stock are detailed in the final prospectus filed with the SEC pursuant to Rule 424(b)(4) on October 16, 2020.

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 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. 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.

On July 24, 2020, the Company entered into the Series C-1 Preferred Stock Purchase Agreement, which authorized the sale and issuance of up to 19,444,453 shares of its Series C-1 Preferred Stock at a purchase price of $5.67 per share. During the nine months ended September 30, 2020, the Company issued all 19,444,453 shares of Series C-1 Preferred Stock for gross cash proceeds of $110.3 million, and incurred issuance costs of approximately $0.2 million. Although there were multiple closings of the Series C-1 Preferred Stock, there was no obligation under the initial closing for investors to purchase, or for the Company to sell to such investors, additional shares of Series C-1 Preferred Stock. The holders of the Series C-1 Preferred Stock are entitled to accrue dividends at an annual rate of $0.4536 per share. Except for the original issuance price and cumulative dividend accrual rate, the terms of the Series C-1 Preferred Stock are substantially the same as the terms of the Series C Preferred Stock. The issuance of the Series C-1 Preferred Stock resulted in changes to certain rights, preferences and privileges of the Series A Preferred Stock, the Series B Preferred Stock, the Series B-1 Preferred Stock and the Series C Preferred Stock. The Company concluded that such changes were not significant and resulted in a modification, rather than an extinguishment, of the previously outstanding Preferred Stock. The changes to the terms of the Series A Preferred Stock, the Series B Preferred Stock, the Series B-1 Preferred Stock and the Series C Preferred Stock did not result in incremental value to the shareholders, and therefore there was no impact to the accounting for the previously outstanding Preferred Stock.

The Preferred Stock consisted of the following (in thousands, except share amounts):

	As of September 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	$ 10,416	$ 10,416	$ 10,416	3,773,820
Series B Preferred Stock	14,913,704	14,913,704	52,648	52,648	52,648	6,969,173
Series B-1 Preferred Stock	2,666,666	2,666,666	11,029	11,029	11,029	1,246,133
Series C Preferred Stock	8,543,692	8,543,692	46,244	46,244	46,244	3,992,463
Series C-1 Preferred Stock	19,444,453	19,444,453	111,892	111,892	111,892	9,086,388
	53,644,314	53,644,314	$232,229	$ 232,229	$ 232,229	25,067,977

	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. As of September 30, 2020, the applicable conversion price for the Series A Preferred Stock, Series B Preferred Stock, Series B-1 Preferred Stock, Series C Preferred Stock and Series C-1 Preferred Stock was equal to $2.14, $6.42, $8.03, $11.02 and $12.13, respectively, as adjusted for the Company's reverse stock split.

The Company's cumulative dividends on its Preferred Stock were as follows (in thousands):

	As of September 30, 2020	As of December 31, 2019
Series A Preferred Stock	$ 2,340	$ 1,857
Series B Preferred Stock	7,907	5,228
Series B-1 Preferred Stock	1,029	431
Series C Preferred Stock	2,244	289
Series C-1 Preferred Stock	1,642	—
	$ 15,162	$ 7,805

7. Common Stock

As of September 30, 2020 and December 31, 2019, the authorized capital stock of the Company included 70,500,000 and 46,000,000 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:

	September 30, 2020	December 31, 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	1,246,133
Series C Preferred Stock	3,992,463	4,582,257
Series C-1 Preferred Stock	9,086,388	—
Shares reserved for vesting of restricted common stock	8,763	48,190
Shares reserved for exercise of outstanding stock options	5,814,944	1,634,686
Shares reserved for future awards under the 2017 Stock Incentive Plan	6,051	617,101
Total shares of authorized common stock reserved for future issuance	30,897,735	18,871,360

8. Stock-Based Compensation

2017 Stock Incentive Plan

The total number of shares of common stock authorized for issuance under the 2017 Stock Incentive Plan ("the 2017 Plan") as of September 30, 2020 and December 31, 2019 was 5,937,763 shares and 2,356,927 shares, respectively. As of September 30, 2020, the Company did not hold any treasury shares.

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	(39,427)	0.05
Repurchased	—	—
Unvested as of September 30, 2020	8,763	$ 0.08

The total fair value of restricted common stock that vested during the three months ended September 30, 2020 and 2019 was $0.1 million and $0.2 million, respectively. The total fair value of restricted common stock that vested during the nine months ended September 30, 2020 and 2019 was $0.2 million and $0.5 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	4,295,618	7.54		
Exercised	(11,628)	2.32		$ 40
Cancelled or Forfeited	(103,732)	3.26		
Outstanding as of September 30, 2020	5,814,944	$ 6.20	9.41	$ 15,761
Exercisable as of September 30, 2020	959,256	$ 2.29	8.10	$ 6,350
Vested and expected to vest as of September 30, 2020	5,814,944	$ 6.20	9.41	$ 15,761

The aggregate intrinsic value of stock options outstanding, exercisable, and vested and expected to vest 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 September 30, 2020. The aggregate intrinsic value of stock options exercised 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 on the date of exercise for those stock options that had exercise prices lower than the estimated fair value of the Company's common stock on the exercise date.

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 three and nine months ended September 30, 2020 and 2019:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Risk-free interest rate	0.37 – 0.68%	1.55%	0.37 – 0.91%	1.55%
Expected term (in years)	6.00 – 10.00	6.00	6.00 – 10.00	6.00
Expected volatility	85.74 – 87.30%	79.09%	85.74 – 88.11%	79.09%
Expected dividend yield	0.00%	0.00%	0.00%	0.00%
Fair value per share of common stock	8.27 – $ 8.91	$ 3.30	5.59 – $ 8.91	$ 3.30

The weighted-average grant-date fair value of the Company's stock options granted during the three months ended September 30, 2020 and 2019 was $6.30 per share and $2.25 per share, respectively. The weighted-average grant-date fair value of the Company's stock options granted during the nine months ended September 30, 2020 and 2019 was $5.41 per share and $2.25 per share, respectively.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 384	$ 175	$ 639	$ 316
General and administrative	569	101	746	194
Total stock-based compensation expense	$ 953	$ 276	$ 1,385	$ 510

As of September 30, 2020, total unrecognized compensation cost related to unvested stock-based awards was $23.3 million, which is expected to be recognized over a weighted-average period of 3.74 years.

9. 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):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Numerator:				
Net loss	$ (16,216)	$ (10,606)	$ (36,114)	$ (28,124)
Accretion and cumulative dividends on redeemable convertible preferred stock	(3,943)	(1,267)	(8,046)	(3,450)
Gain on repurchase of redeemable convertible preferred stock	—	—	493	—
Net loss attributable to common stockholders	$ (20,159)	$ (11,873)	$ (43,667)	$ (31,574)
Denominator:				
Weighted average common shares outstanding, basic and diluted	1,665,902	1,559,800	1,645,982	1,501,908
Net loss per share attributable to common stockholders, basic and diluted	$ (12.10)	$ (7.61)	$ (26.53)	$ (21.02)

The following potential shares of common stock, 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:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Series A Preferred Stock	3,773,820	3,773,820	3,773,820	3,773,820
Series B Preferred Stock	6,969,173	6,969,173	6,969,173	6,969,173
Series B-1 Preferred Stock	1,246,133	1,246,133	1,246,133	1,246,133
Series C Preferred Stock	3,992,463	—	3,992,463	—
Series C-1 Preferred Stock	9,086,388	—	9,086,388	—
Outstanding stock options	5,814,944	1,634,686	5,814,944	1,634,686
Unvested restricted common stock	8,763	86,160	8,763	86,160
	30,891,684	13,709,972	30,891,684	13,709,972

The shares of common stock issuable upon conversion of the Preferred Stock assume automatic conversion in the event of a qualified public offering.

10. Related Party Transactions

On September 11, 2019, the Company entered into a Cooperation and License Agreement (the "License Agreement") with RogCon Inc. ("RogCon"). 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. Pursuant to the terms of the License Agreement, the Company will conduct, at its own cost and expense, the research and development activities assigned to it under the associated 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. 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. During the three months ended September 30, 2020, the Company expensed less than $0.1 million for the reimbursement of RogCon's out-of-pocket costs. During the nine months ended September 30, 2020, the Company expensed $0.1 million for the reimbursement of RogCon's out-of-pocket costs. As of September 30, 2020, the Company had accrued expenses of $0.3 million due to RogCon under the License Agreement.

11. Subsequent Events

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. In addition to those subsequent events disclosed previously, the Company has identified the following subsequent events:

2020 Stock Option and Incentive Plan and 2020 Employee Stock Purchase Plan

The Company's 2020 Stock Option and Incentive Plan (the "2020 Plan") and 2020 Employee Stock Purchase Plan (the "2020 ESPP") were adopted by the board of directors on September 9, 2020 and approved by the Company's stockholders on October 8, 2020. Both the 2020 Plan and the 2020 ESPP became effective upon the date immediately preceding the date on which the Company's registration statement filed with the SEC pursuant to Rule 424(b)(4) was declared effective by the SEC, which was October 15, 2020. The 2020 Plan replaced the 2017 Plan. However, the 2017 Plan will continue to govern outstanding equity awards granted thereunder. Under the 2020 Plan, the Company may make equity-based and cash-based incentive awards to its officers, employees, directors and consultants. The Company has initially reserved 3,271,028 shares and 327,102 shares of common stock for issuance under the 2020 Plan and the 2020 ESPP, respectively.

Item 2. 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 our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the final prospectus for our initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or SEC, on October 16, 2020. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q, 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 expect multiple topline clinical trial readouts from all three programs within the next 18 months 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 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, 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 and the closing of our initial public offering, or IPO.

On October 20, 2020, we completed our IPO in which we issued and sold 11,500,000 shares of our common stock at a public offering price of $19.00 per share, including 1,500,000 shares of common stock sold pursuant to the underwriters' exercise of their option to purchase additional shares of common stock, for aggregate gross proceeds of $218.5 million. We raised approximately $199.9 million in net proceeds after deducting underwriting discounts and commissions and offering expenses payable by us. Upon the closing of the IPO, all of the outstanding shares 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 automatically converted into 25,067,977 shares of common stock. Subsequent to the closing of the IPO, there were no shares of preferred stock outstanding. In connection with the closing of the IPO, we filed an Amended and Restated Certificate of Incorporation to change the authorized capital stock to 150,000,000 shares of common stock and 10,000,000 shares of undesignated 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 a net loss of $36.1 million for the nine months ended September 30, 2020. As of September 30, 2020, we had an accumulated deficit of $123.3 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;
- increase our headcount to support our development efforts and to expand our clinical development team; and
- incur additional costs and headcount associated with our transition to becoming a public company.

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 September 30, 2020, we had cash and cash equivalents of $114.8 million. We believe that our existing cash and cash equivalents, combined with the net proceeds from our IPO, will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022. 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."

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 possible 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):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
PRAX-114	$ 4,077	$1,241	$ 9,137	$ 6,122
PRAX-944	1,707	862	3,284	3,641
PRAX-562	885	873	2,527	3,132
Discovery-stage programs	2,154	4,294	4,050	5,024
Personnel-related (including stock-based compensation)	3,098	1,405	7,443	3,946
Other indirect research and development expenses	865	671	2,263	1,993
Total research and development expenses	$12,786	$9,346	$28,704	$23,858

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 U.S. Food and Drug Administration, or 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 intellectual property, or 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.

Other Income

Interest Income

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

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. 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 benefit recognized for the nine months ended September 30, 2020 related to income tax associated with our operations in Australia.

Results of Operations

Comparison of the Three Months Ended September 30, 2020 and 2019

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

	Three Months Ended September 30,		Change
	2020	2019	
Operating expenses:			
Research and development	$ 12,786	$ 9,346	$ 3,440
General and administrative	3,431	1,308	2,123
Total operating expenses	16,217	10,654	5,563
Loss from operations	(16,217)	(10,654)	(5,563)
Other income:			
Interest income	1	48	(47)
Total other income	1	48	(47)
Net loss	$(16,216)	$(10,606)	$(5,610)

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):

	Three Months Ended September 30,		Change
	2020	2019	
PRAX-114	$ 4,077	$1,241	$ 2,836
PRAX-944	1,707	862	845
PRAX-562	885	873	12
Discovery-stage programs	2,154	4,294	(2,140)
Personnel-related (including stock-based compensation)	3,098	1,405	1,693
Other indirect research and development expenses	865	671	194
Total research and development expenses	$12,786	$9,346	$ 3,440

Research and development expenses increased approximately $3.4 million from $9.3 million for the three months ended September 30, 2019 to $12.8 million for the three months ended September 30, 2020. The increase in research and development expenses was primarily attributable to the following:

- $2.8 million increase in expense related to our PRAX-114 program, driven by a $2.4 million increase in clinical-related spend and a $0.4 million increase in manufacturing spend both related to our ongoing Phase 2a clinical trial for this program;
- $1.7 million increase in personnel-related costs due to increased headcount;
- $0.8 million increase in expense related to our PRAX-944 program, driven primarily by a $0.9 million increase in clinical-related spend for our ongoing Phase 2a clinical trial;
- $0.2 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; and
- $2.1 million offsetting decrease in expense related to our discovery-stage programs, primarily driven by a $2.0 million decrease in research and development spend for discovery program assets acquired in the prior year.

General and Administrative Expense

General and administrative expenses increased $2.1 million from $1.3 million for the three months ended September 30, 2019 to $3.4 million for the three months ended September 30, 2020. The increase in general and administrative expenses was primarily attributable to the following:

- $1.1 million increase in professional fees including legal and consulting services, driven by a $0.5 million increase in accounting costs as we prepared for our IPO, a $0.4 million increase in legal fees, primarily related to intellectual property filings as we expand our research and development activities, and a $0.2 million increase in corporate development-related work; and
- $1.0 million increase in personnel-related costs driven by increased headcount.

Other Income

Other income for the three months ended September 30, 2020 and 2019, comprised of interest income on our cash and cash equivalents, was not material.

Comparison of the Nine Months Ended September 30, 2020 and 2019

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

	Nine Months Ended September 30,		Change
	2020	2019	
Operating expenses:			
Research and development	$ 28,704	$ 23,858	$ 4,846
General and administrative	7,552	4,437	3,115
Total operating expenses	36,256	28,295	7,961
Loss from operations	(36,256)	(28,295)	(7,961)
Other income:			
Interest income	134	171	(37)
Total other income	134	171	(37)
Loss before benefit from income taxes	(36,122)	(28,124)	(7,998)
Benefit from income taxes	(8)	—	(8)
Net loss	$(36,114)	$(28,124)	$(7,990)

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):

	Nine Months Ended September 30,		Change
	2020	2019	
PRAX-114	$ 9,137	$ 6,122	$3,015
PRAX-944	3,284	3,641	(357)
PRAX-562	2,527	3,132	(605)
Discovery-stage programs	4,050	5,024	(974)
Personnel-related (including stock-based compensation)	7,443	3,946	3,497
Other indirect research and development expenses	2,263	1,993	270
Total research and development expenses	$28,704	$23,858	$4,846

Research and development expenses increased $4.8 million from $23.9 million for the nine months ended September 30, 2019, to $28.7 million for the nine months ended September 30, 2020. The increase in research and development expenses was primarily attributable to the following:

- $3.5 million increase in personnel-related costs due to increased headcount;
- $3.0 million increase expense related to our PRAX-114 program, driven primarily by a $3.6 million increase in clinical-related spend for our ongoing Phase 2a clinical trial for this program, partially offset by a $0.5 million decrease related to pre-clinical expenses incurred during the nine months ended September 30, 2019;
- $0.3 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;
- $1.0 million offsetting decrease in discovery-stage program expense, driven by a $0.7 million decrease in expense for discovery program assets acquired in the prior year and a $0.3 million decrease in spend for our other discovery-stage programs;
- $0.6 million offsetting decrease in our PRAX-562 program, driven by a $1.7 million decrease related to pre-clinical expenses incurred during the nine months ended September 30, 2019 to identify our clinical candidate, offset by a $0.7 million increase in clinical-related spend and a $0.4 million manufacturing spend related to our ongoing Phase 1 clinical trial for this program; and
- $0.4 million offsetting decrease in expense related to our PRAX-944 program, driven by a $1.1 million decrease for pre-clinical work performed during the nine months ended September 30, 2019 to prepare for our Phase 2a clinical trial and a $0.6 million decrease related to a drug product manufacturing campaign that was executed during the nine months ended September 30, 2019, offset by a $1.3 million increase in clinical-related spend for our ongoing Phase 2 clinical trial for this program.

General and Administrative Expense

General and administrative expenses increased approximately $3.1 million from $4.4 million for the nine months ended September 30, 2019 to $7.6 million for the nine months ended September 30, 2020. The increase in general and administrative expenses was primarily attributable to the following:

- $1.6 million increase in professional fees including legal and consulting services, driven by a $1.2 million increase in consulting costs, including $0.8 million of increased accounting costs as we prepared for the IPO and $0.3 million of increased corporate development spend to support commercial assessments of our clinical-stage programs, and a $0.4 million increase in legal fees primarily related to intellectual property filings as we expand our research and development activities;
- $1.4 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 the nine months ended September 30, 2020 and 2019 was $0.1 million and $0.2 million, respectively, 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, the issuance of convertible debt and the closing of our IPO.

Prior to our IPO, we raised an aggregate of $206.7 million in proceeds from the sale of our redeemable convertible preferred stock and $4.0 million in proceeds from the issuance of convertible promissory notes. As of September 30, 2020, we had cash and cash equivalents of $114.8 million. On October 20, 2020, we completed our IPO for approximately $199.9 million in net proceeds after deducting underwriting discounts and commissions and offering expenses payable by us.

Historical Cash Flows

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

	Nine Months Ended September 30,	
	2020	2019
Net cash provided by (used in):		
Operating activities	$ (32,395)	$(23,466)
Investing activities	—	(103)
Financing activities	102,352	9,939
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 69,957	$(13,630)

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 have 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 nine months ended September 30, 2020, net cash used in operating activities of $32.4 million was primarily due to our $36.1 million net loss, partially offset by $1.9 million of non-cash charges and $1.8 million in changes in operating assets and liabilities.

During the nine months ended September 30, 2019, net cash used in operating activities of $23.5 million was primarily due to our $28.1 million net loss, partially offset by $1.0 million of non-cash charges and $3.6 million in changes in operating assets and liabilities.

Investing Activities

There were no cash flows from investing activities during the nine months ended September 30, 2020. During the nine months ended September 30, 2019, net cash used in investing activities related to the purchase of property and equipment.

Financing Activities

During the nine months ended September 30, 2020, net cash provided by financing activities of $102.4 million consisted of net proceeds from the issuance of our Series C redeemable convertible preferred stock and Series C-1 redeemable convertible preferred stock and exercise of stock options, offset by the cash paid for the repurchase of our Series C redeemable convertible preferred stock and cash paid for initial public offering costs incurred.

During the nine months ended September 30, 2019, net cash provided by financing activities of $9.9 million consisted of net proceeds from the issuance of our Series B-1 redeemable convertible preferred stock.

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, 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.

We are unable to estimate the exact amount of our working capital requirements, but based on our current operating plan, we believe that our existing cash and cash equivalents, combined with the cash proceeds from our IPO, will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022. 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.

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, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our common stock. 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 result in dilution to the holders of our common stock.

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

There have been no changes to our contractual obligations from those described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations – Contractual Obligations and Commitments" in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on October 16, 2020.

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 condensed consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of these condensed 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 condensed 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.

There have been no changes to our critical accounting policies from those described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations – Critical Accounting Policies and Significant Judgments and Estimates" included in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on October 16, 2020.

JOBS Act and Emerging Growth Company and Smaller Reporting 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 our IPO, (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.

We are also a "smaller reporting company" as defined in the Securities Exchange Act of 1934, as amended. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies until the fiscal year following the determination that our voting and non-voting common stock held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are less than $100 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter.

Recently Issued Accounting Pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q, such standards will not have a material impact on our condensed consolidated financial statements or do not otherwise apply to our current operations.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

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 three and nine months ended September 30, 2020 or 2019.

Item 4. Controls and Procedures.

Management's Evaluation of Our Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2020. Based on the evaluation of our disclosure controls and procedures as of September 30, 2020, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

As of the date of this Quarterly Report on Form 10-Q, 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.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Quarterly Report on Form 10-Q, including our condensed consolidated financial statements and the related notes appearing elsewhere in this Quarterly Report on Form 10-Q and in our other filings with the Securities and Exchange Commission, or SEC, before deciding to invest in our common stock. The risks described below are not the only risks facing our company. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could cause our business, prospects, operating results and financial condition to suffer materially. In such event, the trading price of our common stock could decline, and you might lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

Risks Related to Past Financial Condition

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 $36.1 million and $28.1 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020, we had an accumulated deficit of $123.3 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.

Risks Related to Future Financial Condition

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 our cash and cash equivalents as of September 30, 2020, together with the $199.9 million net cash proceeds from our initial public offering, or IPO, will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022. 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

Risks Related to Preclinical and Clinical Development

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 submitted an Investigational New Drug Application, or IND, for PRAX-114 with the FDA. In November 2020, the IND for PRAX-114 was placed on clinical hold by the FDA pending the resolution of certain non-clinical pharmacology and toxicology matters. 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 United States 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 are pursuing 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.

Risks Related to 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

Risks Related to Post-Marketing Regulatory Requirements

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.

Risks Related to Sales, Marketing and Competition

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

Risks Related to Healthcare and Related Laws

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, U.S. 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 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, 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.

Risks Related to International Regulations

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

Risks Related to Licensed 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.

Risks Related to License and Collaboration Agreements

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.

Risks Related to Potential Third-Party Claims

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.

Risks Related to Patent Laws and Protection

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

Risks Related to Third Parties Generally

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.

Risks Related to Third-Party Manufacturers

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.

Risks Related to Third-Party Suppliers

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

Risks Related to Business Operations

Business interruptions resulting from COVID-19 or a similar 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 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.

While we have taken and are continuing to take steps to mitigate against possible 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. 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.

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.

Risks Related to Employees

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.

Risks Related to Data Privacy

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

Risks Related to Tax Laws

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 subsequent shifts in our stock ownership, some of which are outside our control.

Risks Related to Our Common Stock

Risks Related to Investment in Securities

An active trading market for our common stock may not be sustained.

In October 2020, we closed our initial public offering. Prior to this offering, there was no public market for our common stock. Although we have completed our initial public offering and shares of our common stock are listed and trading on the Nasdaq Global Select Market, an active trading market for our shares may not be sustained. If an active market for our common stock does not continue, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares or sell their shares at or above the prices at which they acquired their shares or sell their shares at the time they would like to sell. Any inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares 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 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, 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. 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.

A significant portion of our total outstanding shares are eligible to be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Following the closing of our IPO, we had outstanding 36,749,675 shares of common stock, of which 26,737,873 shares were subject to restrictions on transfer under 180-day lock-up arrangements with the underwriters of our IPO. These restrictions are due to expire in April 2021, resulting in the majority of these shares then being eligible for public sale if they are registered under the Securities Act of 1933, as amended (the "Securities Act"), or if they qualify for an exemption from registration under the Securities Act including under Rules 144 or 701.

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 depends in part on the research and reports that securities or industry analysts publish about us or our business. We may never obtain research coverage by industry or financial analysts. 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.

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 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.

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 October 20, 2020, our executive officers, directors and their affiliates and our principal stockholders beneficially hold, in the aggregate, approximately 47.5% of our outstanding voting stock. These stockholders, acting together, would be able to significantly influence all matters requiring stockholder approval. 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.

Risks Related to Our Controls and Reporting Requirements

We will continue to 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 continue to incur significant legal, accounting and other expenses that we did not incur as a private company. We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which 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 IPO. 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.

The rules and regulations applicable to public companies substantially increase our legal and financial compliance costs and 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.

We are an emerging growth company and a smaller reporting company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies and smaller reporting 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 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 completed our IPO, 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 our IPO, (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 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.

Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company," which would allow us to continue to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on 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.

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.

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.

We have broad discretion over the use of our cash and cash equivalents and may not use them effectively.

Our management has broad discretion to use our cash and cash equivalents to fund our operations and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending our use to fund operations, we may invest our cash and cash equivalents in a manner that does not produce income or that loses value.

Risks Related to Charter and Bylaws

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 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.

Our amended and restated bylaws 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 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 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 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Equity Securities

From July to August 2020, we issued and sold an aggregate of 19,444,453 shares of our Series C-1 preferred stock at a per share purchase price of $5.67 for aggregate gross consideration of $110.3 million. We have relied on the exemption from the registration requirements of the Securities Act by virtue of Section 4(a)(2) thereof and the rules and regulations promulgated thereunder relating to transactions not involving any public offering.

During the period between July 1, 2020 to September 30, 2020, we issued to employees, directors and consultants, options to purchase an aggregate of 2,612,353 shares of our common stock at a weighted-average exercise price of $8.80 per share. We deemed these issuances to be exempt from registration under the Securities Act either in reliance on Rule 701 of the Securities Act as sales and offers under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701, or in reliance on Section 4(a)(2), as a transaction by an issuer not involving a public offering. On October 16, 2020, we filed a registration statement on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options and all shares of our common stock otherwise issuable pursuant to our equity compensation plans.

Use of Proceeds from Initial Public Offering

On October 20, 2020, we completed our initial public offering, or IPO, pursuant to which we issued and sold all 11,500,000 shares of our registered common stock at the public offering price of $19.00 per share, including 1,500,000 shares of common stock sold pursuant to the underwriters' exercise of their option to purchase additional shares of common stock. The aggregate gross proceeds of our IPO, inclusive of the underwriters' option to purchase additional shares, were $218.5 million. The offering commenced on October 15, 2020 and did not terminate until the sale of all of the shares offered.

The offer and sale of all of the shares of our common stock in our IPO were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-249074), which was declared effective by the Securities and Exchange Commission, or SEC, on October 15, 2020. Cowen and Company, LLC, Evercore Group L.L.C. and Piper Sandler & Co. acted as joint book-running managers for our IPO.

We received aggregate net proceeds from the IPO, inclusive of the underwriters' option to purchase additional shares, of approximately $199.9 million, after deducting $15.3 million of underwriting discounts and commissions and $3.3 million of other offering expenses payable by us. None of the underwriting discounts and commissions or offering expenses were paid directly or indirectly to any directors or officers of ours or their associates or to persons owning 10% or more of any class of equity securities or to any affiliates of ours. We had not used any of the net proceeds from the IPO as of September 30, 2020, as we have continued to fund operations from proceeds received through our preferred stock financings. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on October 16, 2020.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None

Item 6. Exhibits.

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 3.1 to the Registrant's Form 8-K (File No. 001-39620) filed on October 20, 2020).
3.2	Amended and Restated Bylaws of Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 3.2 to the Registrant's Form 8-K (File No. 001-39620) filed on October 20, 2020).
4.1	Specimen stock certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
4.2	Fourth Amended and Restated Investors' Rights Agreement among Praxis Precision Medicines, Inc. and certain of its stockholders, effective as of July 24, 2020 (incorporated by reference to Exhibit 4.2 to our Registration Statement on Form S-1 (File No. 333-249074) filed with the SEC on September 25, 2020).
10.1	Form of Director Indemnification Agreement, as amended (incorporated by reference to Exhibit 10.1 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.2	Form of Officer Indemnification Agreement (incorporated by reference to Exhibit 10.2 to our Registration Statement on Form S-1 (File No. 333-249074) filed with the SEC on September 25, 2020).
10.3#	Praxis Precision Medicines, Inc. 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.4 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.4#	Form of Incentive Stock Option Agreement under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.5 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.5#	Form of Non-Qualified Stock Option Agreement for Company Employees under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.6 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.6#	Form of Non-Qualified Stock Option Agreement for Non-Employee Directors under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.7 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.7#	Form of Restricted Stock Award Agreement under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.8 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.8#	Form of Restricted Stock Award Agreement for Company Employees under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.9 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.9#	Form of Restricted Stock Award Agreement for Non-Employee Directors under 2020 Stock Option and Incentive Plan (incorporated by reference to Exhibit 10.10 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
10.10#	Praxis Precision Medicines, Inc. 2020 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.11 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).

10.11*#	Form of Amended and Restated Employment Agreement.
10.12#	Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.13 to our Registration Statement on Form S-1/A (File No. 333-249074) filed with the SEC on October 9, 2020).
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

* Filed herewith.

** The certifications furnished in Exhibit 32.1 hereto are deemed to accompany this Quarterly Report on Form 10-Q and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended. Such certifications will not be deemed to be incorporated by reference into any filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

# Indicates a management contract or any compensatory plan, contract or arrangement.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Company Name

Date: November 23, 2020

By: /s/ Marcio Souza

Marcio Souza
Chief Executive Officer and Director (Principal Executive Officer)

Date: November 23, 2020

By: /s/ Stuart Chaffee

Stuart Chaffee, Ph.D.
Chief Financial Officer (Principal Financial Officer)

PRAXIS PRECISION MEDICINES, INC.

AMENDED AND RESTATED EMPLOYMENT AGREEMENT
for
[_____]

This Amended and Restated Executive Employment Agreement (the "**Agreement**") is made between Praxis Precision Medicines, Inc. (the "**Company**") and _____ ("**Executive**") (collectively, the "**Parties**") and is effective as of the closing of the Company's first underwritten public offering of its equity securities pursuant to an effective registration statement under the Securities Act of 1933, as amended (the "**Effective Date**"). This Agreement supersedes in all respects all prior agreements between Executive and the Company regarding the subject matter herein, including without limitation, the Employment Agreement between Executive and the Company dated _____ (the "**Prior Agreement**").

WHEREAS, the Company desires Executive to continue to provide services to the Company, and wishes to provide Executive with certain compensation and benefits in return for such employment services; and

WHEREAS, Executive wishes to continue to be employed by the Company and to provide personal services to the Company in return for certain compensation and benefits;

NOW, THEREFORE, in consideration of the mutual promises and covenants contained herein and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereto agree as follows:

1. Employment by the Company.

1.1 Position and Duties. Executive shall continue to serve as the Company's _____ (the "**Position**"). During the term of Executive's employment with the Company, Executive shall devote one hundred percent of Executive's business time on behalf of the Company and, on a full time basis, use Executive's skills and render services to the best of Executive's abilities on behalf of the Company, and shall comply fully with the policies and procedures of the Company. Executive shall report directly to the Company's **[Chief Executive Officer (the "CEO") or to the CEO's designee|[Board of Directors (the "Board")]**[1]. Executive shall perform those duties typically associated with the Position and such other duties consistent with the Position as may be assigned by the **[CEO]|[Board]**[2], including but not limited to executive responsibility for _____. Notwithstanding the foregoing, nothing herein shall preclude Executive from (i) serving, with the prior written consent of the **[CEO]|[Board]**[3], as a member of the boards of directors or advisory boards of non-competitive businesses and charitable organizations, (ii) engaging in charitable activities and community affairs, and (iii) managing Executive's personal investments and affairs; *provided*, *however*, that the activities set out in clauses (i), (ii), and (iii) shall be limited by Executive so as not to materially interfere, individually or in the aggregate, with the performance of Executive's duties and responsibilities hereunder and shall not, in the judgment of the **[CEO]|[Board]**[4] pose a conflict of interest with Executive's duties to the Company or its affiliates.

1 **NTD**: Board for the CEO and CEO for other executives.
2 **NTD**: Board for the CEO and CEO for other executives.
3 **NTD**: Board for the CEO and CEO for other executives.
4 **NTD**: Board for the CEO and CEO for other executives.

1.2 Location of Work. Executive shall work **[remotely]** in _____, USA. The Company reserves the right to reasonably require Executive to perform Executive's duties at places other than Executive's primary office location from time to time, and to require reasonable business travel.

1.3 Policies and Procedures. The employment relationship between the Parties shall be governed by the general employment policies and practices of the Company, except that when the terms of this Agreement differ from or are in conflict with the Company's general employment policies or practices, this Agreement shall control.

2. Compensation.

2.1 Salary. For services to be rendered hereunder, Executive shall receive an initial base salary at the rate of $_____ per year, subject to standard payroll deductions and withholdings and payable in accordance with the Company's regular payroll schedule (the "**Base Salary**"). Executive's Base Salary will be reviewed annually by the Board or the Compensation Committee of the Board (the "**Compensation Committee**").

2.2 Annual Cash Bonus. Executive will be eligible for an annual cash bonus with a target amount of _____ percent (__%) of Executive's Base Salary (the "**Annual Bonus**"). Whether Executive receives an Annual Bonus for any given year, and the amount of any such Annual Bonus, will be determined by the Board or the Compensation Committee based upon the Company's and Executive's achievement of objectives and milestones to be determined by the Board or the Compensation Committee on an annual basis. Except as otherwise provided herein or in applicable incentive compensation plan that may be in effect from time to time, Executive will not be eligible for, and will not earn, any Annual Bonus if Executive is not employed by the Company on the payment date (regardless of the reason for the separation from employment).

2.3 Equity. The stock options and other stock-based awards held by Executive shall continue to be governed by the terms and conditions of the Company's applicable equity incentive plan(s) and the applicable award agreement(s) governing the terms of such stock options and other stock-based awards (collectively, the "**Equity Documents**"); *provided*, *however*, and notwithstanding anything to the contrary in the Equity Documents, Section 5.3(ii)(b) of this Agreement shall apply in the event of a termination of Executive's employment by the Company without Cause or by Executive for Good Reason, in either case within the Change of Control Period (as such terms are defined below).

3. Standard Company Benefits. Executive shall be entitled to participate in all employee benefit programs for which Executive is eligible under the terms and conditions of the benefit plans that may be in effect from time to time and provided by the Company to its employees. The Company reserves the right to cancel or change the benefit plans or programs it offers to its employees at any time.

4. Expenses. The Company will reimburse Executive for reasonable travel, entertainment or other expenses incurred by Executive in furtherance or in connection with the performance of Executive's duties hereunder, in accordance with the Company's expense reimbursement policy as in effect from time to time.

5. Termination of Employment; Severance.

5.1 At-Will Employment. Executive's employment relationship is at-will. Either Executive or the Company may terminate the employment relationship at any time, with or without cause subject to the terms of this Agreement.

5.2 Severance Pay and Benefits Upon a Termination Without Cause or Resignation for Good Reason Outside the Change of Control Period.

(i) The Company may terminate Executive's employment with the Company at any time without Cause. Executive may terminate Executive's employment with the Company at any time for any reason, including for Good Reason.

(ii) In the event that Executive's employment with the Company is terminated by the Company without Cause or by Executive for Good Reason, in either case outside of the Change of Control Period, then, provided that Executive remains in compliance with the terms of this Agreement, the Company shall provide Executive with the following severance benefits:

(a) The Company shall pay Executive, as severance, ____ (__)[5] months of Executive's Base Salary in effect as of the date of Executive's employment termination, subject to standard payroll deductions and withholdings (the "**Severance**"). Subject to Section 5.2(iii) below, the Severance will be paid in equal installments on the Company's regular payroll schedule over the ____ (__)[6] month period following Executive's termination of employment.

(b) Provided Executive timely elects continued coverage under COBRA, the Company shall pay the Company's portion of Executive's COBRA premiums, equal to the percentage of health premiums paid by the Company prior to Executive's termination, to continue Executive's coverage (including coverage for eligible dependents, if applicable) (**"COBRA Premiums"**) through the period (the **"COBRA Premium Period**") starting on Executive's date of termination and ending on the earliest to occur of: (i) the _____ (__)[7] month anniversary of Executive's date of termination; (ii) the date Executive becomes eligible for group health insurance coverage through a new employer; or (iii) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In the event Executive becomes covered under another employer's group health plan or otherwise ceases to be eligible for COBRA during the COBRA Premium Period, Executive must immediately notify the Company of such event. Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot pay the COBRA Premiums without potentially violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company instead shall pay to Executive, on the first day of each calendar month, a fully taxable cash payment equal to the applicable COBRA premiums for that month (including premiums for Executive and Executive's eligible dependents who have elected and remain enrolled in such COBRA coverage), subject to applicable tax withholdings (such amount, the "**Special Cash Payment**"), for the remainder of the COBRA Premium Period. Executive may, but is not obligated to, use such Special Cash Payments toward the cost of COBRA premiums.

---

5 **NTD**: 12 months for the CEO and nine months for other executives.

6 **NTD**: 12 months for the CEO and nine months for other executives.

7 **NTD**: 12 months for the CEO and nine months for other executives.

(iii) The amounts payable under this Section 5.2, to the extent taxable, shall be paid or commence to be paid within 60 days after the date of termination; *provided*, *however*, that if the 60-day period begins in one calendar year and ends in a second calendar year, such payments to the extent they qualify as "non-qualified deferred compensation" within the meaning of Section 409A of the Internal Revenue Code of 1986, as amended (the "**Code**"), shall be paid or commence to be paid in the second calendar year by the last day of such 60-day period.

5.3 Severance Pay and Benefits Upon Termination Without Cause or Resignation for Good Reason Within the Change of Control Period.

(i) The provisions of this Section 5.3 shall apply in lieu of, and expressly supersede, the provisions of Section 5.2 regarding severance pay and benefits upon a termination of employment by the Company without Cause or by Executive for Good Reason if such termination of employment occurs on or within 12 months after the occurrence of the first event constituting a Change of Control (such period, the "**Change of Control Period**"). These provisions shall terminate and be of no further force or effect after the Change of Control Period

(ii) In the event that Executive's employment with the Company is terminated by the Company without Cause or by Executive for Good Reason, in either case during the Change of Control Period, then, provided Executive remains in compliance with the terms of this Agreement, the Company shall provide Executive with the following severance benefits:

(a) The Company shall pay Executive, as severance, a lump sum in cash in an amount equal to ___ (__)[8] times the sum of (A) Executive's then current Base Salary (or Executive's Base Salary in effect immediately prior to the Change of Control, if higher) plus (B) Executive's Annual Bonus for the then-current year (or Executive's Annual Bonus in effect immediately prior to the Change of Control, if higher) (the "**Change of Control Severance**").

(b) Notwithstanding anything to the contrary in any applicable option agreement or other stock-based award agreement, all time-based stock options and other stock-based awards subject to time-based vesting held by Executive (the "**Time-Based Equity Awards**") shall immediately accelerate and become fully exercisable or nonforfeitable as of the later of (i) the date of termination or (ii) the effective date of the Separation Agreement (as defined below) (the "**Accelerated Vesting Date**"); *provided* that any termination or forfeiture of the unvested portion of such Time-Based Equity Awards that would otherwise occur on the date of termination in the absence of this Agreement will be delayed until the effective date of the Separation Agreement and will only occur if the vesting pursuant to this subsection does not occur due to the absence of the Separation Agreement becoming fully effective within the time period set forth therein. Notwithstanding the foregoing, no additional vesting of the Time-Based Equity Awards shall occur during the period between Executive's date of termination and the Accelerated Vesting Date.

(c) Provided Executive timely elects continued coverage under COBRA, the Company shall pay the Company's portion of Executive's COBRA premiums, equal to the percentage of health premiums paid by the Company prior to Executive's termination, to continue Executive's coverage (including coverage for eligible dependents, if applicable) ("**COC COBRA Premiums**") through the period (the "**COC COBRA Premium Period**") starting on Executive's date of termination and ending on the earliest to occur of: (i) the _____ (__)[9] month anniversary of Executive's date of termination; (ii) the date Executive becomes eligible for group health insurance coverage through a new employer; or (iii) the date Executive ceases to be eligible for COBRA continuation coverage for any reason, including plan termination. In the event Executive becomes covered under another employer's group health plan or otherwise ceases to be eligible for COBRA during the COC COBRA Premium Period, Executive must immediately notify the Company of such event. Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot pay the COC COBRA Premiums without potentially violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company instead shall pay to Executive, on the first day of each calendar month, a fully taxable cash payment equal to the applicable COBRA premiums for that month (including premiums for Executive and Executive's eligible dependents who have elected and remain enrolled in such COBRA coverage), subject to applicable tax withholdings (such amount, the "**COC Special Cash Payment**"), for the remainder of the COC COBRA Premium Period. Executive may, but is not obligated to, use such COC Special Cash Payments toward the cost of COBRA premiums.

---

8 **NTD**: 1.5x for the CEO and 1x for other executives.

9 **NTD**: 18 months for the CEO and 12 months for other executives.

(iii) The amounts payable under this Section 5.3, to the extent taxable, shall be paid or commence to be paid within 60 days after the date of termination; *provided*, *however*, that if the 60-day period begins in one calendar year and ends in a second calendar year, such payments to the extent they qualify as "non-qualified deferred compensation" within the meaning of Section 409A of the Code, shall be paid or commence to be paid in the second calendar year by the last day of such 60-day period.

(iv) Additional Limitation.

(a) Anything in this Agreement to the contrary notwithstanding, in the event that the amount of any compensation, payment or distribution by the Company to or for the benefit of Executive, whether paid or payable or distributed or distributable pursuant to the terms of this Agreement or otherwise, calculated in a manner consistent with Section 280G of the Code, and the applicable regulations thereunder (the "**Aggregate Payments**"), would be subject to the excise tax imposed by Section 4999 of the Code, then the Aggregate Payments shall be reduced (but not below zero) so that the sum of all of the Aggregate Payments shall be $1.00 less than the amount at which Executive becomes subject to the excise tax imposed by Section 4999 of the Code; *provided* that such reduction shall only occur if it would result in Executive receiving a higher After Tax Amount (as defined below) than Executive would receive if the Aggregate Payments were not subject to such reduction. In such event, the Aggregate Payments shall be reduced in the following order, in each case, in reverse chronological order beginning with the Aggregate Payments that are to be paid the furthest in time from consummation of the transaction that is subject to Section 280G of the Code: (1) cash payments not subject to Section 409A of the Code; (2) cash payments subject to Section 409A of the Code; (3) equity-based payments and acceleration; and (4) non-cash forms of benefits; *provided* that in the case of all the foregoing Aggregate Payments, all amounts or payments that are not subject to calculation under Treas. Reg. §1.280G-1, Q&A-24(b) or (c) shall be reduced before any amounts that are subject to calculation under Treas. Reg. §1.280G-1, Q&A-24(b) or (c).

(b) For purposes of this Section 5.3(iv), the "**After Tax Amount**" means the amount of the Aggregate Payments less all federal, state, and local income, excise and employment taxes imposed on Executive as a result of Executive's receipt of the Aggregate Payments. For purposes of determining the After Tax Amount, Executive shall be deemed to pay federal income taxes at the highest marginal rate of federal income taxation applicable to individuals for the calendar year in which the determination is to be made, and state and local income taxes at the highest marginal rates of individual taxation in each applicable state and locality, net of the maximum reduction in federal income taxes which could be obtained from deduction of such state and local taxes.

(c) The determination as to whether a reduction in the Aggregate Payments shall be made pursuant to Section 5.3(iv)(a) shall be made by a nationally recognized accounting firm selected by the Company (the "**Accounting Firm**"), which shall provide detailed supporting calculations both to the Company and Executive within 15 business days of the date of termination, if applicable, or at such earlier time as is reasonably requested by the Company or Executive. Any determination by the Accounting Firm shall be binding upon the Company and Executive.

5.4 Termination for Cause; Resignation without Good Reason; Death or Disability.

(i) The Company may terminate Executive's employment with the Company at any time for Cause. Further, Executive may resign at any time for any reason other than Good Reason. Executive's employment with the Company may also be terminated due to Executive's death or Disability (as defined below).

(ii) If Executive resigns without Good Reason, or the Company terminates Executive's employment for Cause, or upon Executive's death or Disability, then (i) all payments of compensation by the Company to Executive hereunder will terminate immediately (except as to amounts already earned), and (ii) Executive will not be entitled to any severance benefits, including (without limitation) the Severance, COBRA Premiums, the Change of Control Severance or the COC COBRA Premiums.

6. Conditions to Receipt of Severance, COBRA Premiums, Special Cash Payments and Vesting Acceleration. The receipt of the payments and benefits described in Section 5.2 and Section 5.3 will be subject to subject to (i) Executive signing a separation agreement and release in a form and manner satisfactory to the Company, which shall include, without limitation, a general release of claims against the Company and all related persons and entities and, in the Company's sole discretion, a one-year post-employment noncompetition agreement (the "**Separation Agreement"**) and (ii) the Separation Agreement becoming irrevocable, all within 60 days after the date of termination (or such shorter period as set forth in the Separation Agreement), which shall include a seven business day revocation period. No amounts will be paid or provided under Section 5.2 or Section 5.3 until the Separation Agreement becomes effective. Executive shall be deemed to have resigned from all officer and board member positions that the Executive holds with the Company or any of its respective subsidiaries and affiliates upon the termination of Executive's employment for any reason. Executive shall execute any documents in reasonable form as may be requested to confirm or effectuate any such resignations.

7. Section 409A.

7.1 Anything in this Agreement to the contrary notwithstanding, if at the time of Executive's separation from service within the meaning of Section 409A of the Code, the Company determines that Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that Executive becomes entitled to under this Agreement or otherwise on account of Executive's separation from service would be considered deferred compensation otherwise subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (A) six months and one day after Executive's separation from service, or (B) Executive's death. If any such delayed cash payment is otherwise payable on an installment basis, the first payment shall include a catch-up payment covering amounts that would otherwise have been paid during the six-month period but for the application of this provision, and the balance of the installments shall be payable in accordance with their original schedule.

7.2 All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by Executive during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year (except for any lifetime or other aggregate limitation applicable to medical expenses). Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

7.3 To the extent that any payment or benefit described in this Agreement constitutes "non-qualified deferred compensation" under Section 409A of the Code, and to the extent that such payment or benefit is payable upon Executive's termination of employment, then such payments or benefits shall be payable only upon Executive's "separation from service." The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A-1(h).

7.4 The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. Each payment pursuant to this Agreement is intended to constitute a separate payment for purposes of Treasury Regulation Section 1.409A-2(b)(2). The parties agree that this Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

7.5 The Company makes no representation or warranty and shall have no liability to Executive or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Section 409A of the Code but do not satisfy an exemption from, or the conditions of, such Section.

8. Definitions.

8.1 Cause. For purposes of this Agreement, "**Cause**" for termination will mean: (a) conviction of or please of guilty or *nolo contendere* to any felony or any crime involving dishonesty; (b) participation in any fraud against the Company; (c) material breach of any Company's policy or procedure after written notice from the Company and a reasonable period of not less than twenty-one (21) calendar days in which to cure such breach (if deemed curable); (d) persistent failure or refusal to perform Executive's job duties after written notice from the Company and a reasonable period of not less than twenty-one (21) calendar days in which to cure such performance issues (if deemed curable); (e) intentional damage to any property of the Company; (f) willful misconduct, or other violation of Company policy that causes harm; (g) breach of any written agreement by and between Executive and the Company; and (h) conduct by Executive which in the good faith and reasonable determination of the Company demonstrates gross unfitness to serve.

8.2 Change of Control. For purposes of this Agreement, "**Change of Control**" shall mean: a "Sale Event," as defined in the Company's 2020 Stock Option and Incentive Plan.

8.3 Disability. For purposes of this Agreement, "**Disability**" shall mean Executive's physical or mental condition that renders Executive unable to substantially perform for a period of ninety (90) aggregate days (regardless of whether or not continuous) during any three hundred sixty (360) day period, Executive's regular responsibilities hereunder, with or without a reasonable accommodation.

8.4 Good Reason. For purposes of this Agreement, "**Good Reason**" shall mean: (a) a material reduction in Executive's Base Salary (unless pursuant to a salary reduction applicable generally to the Company's similarly situated employees); (b) the relocation of Executive's place of work for the Company that is more than 40 miles from Executive's primary place of work, unless mutually agreed upon; or (c) a material diminution in Executive's responsibilities, authority, or duties. Notwithstanding the foregoing, no act or omission described in subclauses (a), (b) or (c) above shall constitute "Good Reason" unless: (1) Executive first gives the Company written notice of such act or omission within forty-five (45) days of the later of the occurrence of such act or omission or Executive's first becoming aware thereof, (2) the Company fails to cure such act or omission within twenty-one (21) days after receiving such written notice from Executive, and (3) Executive resigns from employment (and all other positions, including as a member of the Board) within ten (10) days after the end of the cure period.

9. Other Obligations.

9.1 Restrictive Covenants. In connection with Executive's employment with the Company, Executive will continue to receive access to Company confidential information and trade secrets and develop valuable goodwill with the Company's customers, partners and vendors. To protect the Company's legitimate business interests, Executive executed the Employee Confidentiality, Assignment and Nonsolicitation Agreement on _____ (the "**Confidentiality Agreement**"). Executive acknowledges and agrees that (i) the Confidentiality Agreement shall continue in full force and effect in accordance with its terms, and (ii) Executive will abide by the terms of the Confidentiality Agreement at all times.

9.2 Third-Party Agreements and Information. Executive represents and warrants that Executive's employment by the Company does not conflict with any prior employment or consulting agreement or other agreement with any third party, and that Executive will perform Executive's duties to the Company without violating any such agreement. Executive represents and warrants that Executive will not use or disclose confidential information arising out of prior employment, consulting, or other third party relationships, in connection with Executive's employment by the Company, except as expressly authorized by that third party. During Executive's employment by the Company, Executive will use in the performance of Executive's duties only information which is generally known and used by persons with training and experience comparable to Executive's own, common knowledge in the industry, otherwise legally in the public domain, or obtained or developed by the Company or by Executive in the course of Executive's work for the Company.

10. Outside Activities During Employment.

10.1 Non-Company Business. Except with the prior written consent of the Board, Executive will not during the term of Executive's employment with the Company undertake or engage in any other employment, occupation or business enterprise, other than ones in which Executive is a passive investor. In any event, Executive may engage in civic and not-for-profit activities so long as such activities do not materially interfere with the performance of Executive's duties hereunder.

10.2 No Adverse Interests. Executive agrees not to acquire, assume or participate in, directly or indirectly, any position, investment or interest known to be adverse or antagonistic to the Company, its business or prospects, financial or otherwise.

11. General Provisions.

11.1 Notices. Any notices provided must be in writing and will be deemed effective upon the earlier of personal delivery (including personal delivery by fax) or the next day after sending by overnight carrier, to the Company at its primary office location and to Executive at the address as listed on the Company payroll.

11.2 Severability. Whenever possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement is held to be invalid, illegal or unenforceable in any respect under any applicable law or rule in any jurisdiction, such invalidity, illegality or unenforceability will not affect any other provision or any other jurisdiction, but this Agreement will be reformed, construed and enforced in such jurisdiction to the extent possible in keeping with the intent of the parties.

11.3 Waiver. Any waiver of any breach of any provisions of this Agreement must be in writing to be effective, and it shall not thereby be deemed to have waived any preceding or succeeding breach of the same or any other provision of this Agreement.

11.4 Complete Agreement. This Agreement, together with the Confidentiality Agreement, constitutes the entire agreement between Executive and the Company and is the complete, final, and exclusive embodiment of the Parties' agreement and supersedes all prior agreements between the parties concerning such subject matter, including the Prior Agreement. This Agreement is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. It is entered into without reliance on any promise or representation other than those expressly contained herein, and it cannot be modified or amended except in a writing signed by a duly authorized officer of the Company.

11.5 Counterparts. This Agreement may be executed in separate counterparts, any one of which need not contain signatures of more than one party, but all of which taken together will constitute one and the same Agreement.

11.6 Headings. The headings of the paragraphs hereof are inserted for convenience only and shall not be deemed to constitute a part hereof nor to affect the meaning thereof.

11.7 Successors and Assigns. This Agreement shall be binding upon and inure to the benefit of and be enforceable by Executive and the Company, and their respective successors, assigns, heirs, executors and administrators, except that Executive may not assign any of his duties hereunder and he may not assign any of his rights hereunder without the written consent of the Company, which shall not be withheld unreasonably.

11.8 Tax Withholding and Indemnification. All payments and awards contemplated or made pursuant to this Agreement will be subject to withholdings of applicable taxes in compliance with all relevant laws and regulations of all appropriate government authorities. Executive acknowledges and agrees that the Company has neither made any assurances nor any guarantees concerning the tax treatment of any payments or awards contemplated by or made pursuant to this Agreement. Executive has had the opportunity to retain a tax and financial advisor and fully understands the tax and economic consequences of all payments and awards made pursuant to the Agreement.

11.9 Choice of Law. All questions concerning the construction, validity and interpretation of this Agreement will be governed by the laws of the Commonwealth of Massachusetts.

IN WITNESS WHEREOF, the Parties have executed this Agreement on the day and year first above written.

PRAXIS PRECISION MEDICINES, INC.

By: _____

Name: [Marcio Souza]
Title: [CEO][10]

Date: _____

EXECUTIVE

_____

Name:

Date: _____

10 **NTD**: A representative of the Company other than the CEO (such as the Chair of the Board) should sign on behalf of the Company in the case of the Employment Agreement with the CEO.

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Marcio Souza, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Praxis Precision Medicines, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 23, 2020

By: /s/ MARCIO SOUZA

Marcio Souza
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Stuart Chaffee, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Praxis Precision Medicines, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 23, 2020

By: /s/ STUART CHAFFEE

Stuart Chaffee
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATIONS OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Praxis Precision Medicines, Inc. (the "Company") for the quarter ended September 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of their knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 23, 2020 By: /s/ MARCIO SOUZA

Marcio Souza
Chief Executive Officer
(Principal Executive Officer)

Date: November 23, 2020 By: /s/ STUART CHAFFEE

Stuart Chaffee
Chief Financial Officer
(Principal Financial Officer)