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 March 31, 2026

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.)**
99 High Street, 30th Floor Boston, MA	**02110**
(Address of principal executive offices)	**(Zip Code)**

617-300-8460

(Registrant's telephone number, including area code)

N/A

(Former name, former address and former fiscal year, if changed since last report)

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, a 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 May 4, 2026, the registrant had 27,878,774 shares of common stock, $0.0001 par value per share, outstanding.

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:

- our expectations surrounding potential regulatory submissions, progress or approvals and timing thereof for any of our product candidates;
- the success, cost and timing of our product candidate 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 product candidates, if approved, in light of the intellectual property rights of others;
- our ability to fund our operations and obtain additional funding, if necessary, to complete further development and, if approved, commercialization of our product candidates;
- the commercialization of our product candidates, if approved;
- our plans to research, develop and, if approved, commercialize our product candidates;
- future agreements with third parties in connection with the commercialization of our product candidates, if approved, 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, if approved;
- 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; and
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing.

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

sections titled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2025. 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” in our Annual Report on Form 10-K for the year ended December 31, 2025.

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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)

	March 31, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 536,333	$ 357,329
Marketable securities	250,532	242,002
Prepaid expenses and other current assets	10,909	11,580
Total current assets	797,774	610,911
Long-term marketable securities	660,938	326,757
Property and equipment, net	170	147
Operating lease right-of-use assets	1,320	92
Total assets	$ 1,460,202	$ 937,907
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 31,158	$ 24,628
Accrued expenses	17,648	35,033
Operating lease liabilities	1,435	110
Total current liabilities	50,241	59,771
Total liabilities	50,241	59,771
Commitments and contingencies (Note 6)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized and no shares issued or outstanding as of March 31, 2026 and December 31, 2025	—	—
Common stock, $0.0001 par value; 150,000,000 shares authorized; 27,859,514 shares issued and outstanding as of March 31, 2026, and 25,195,092 shares issued and outstanding as of December 31, 2025	15	15
Additional paid-in capital	2,644,109	2,017,566
Accumulated deficit	(1,232,569)	(1,140,008)
Accumulated other comprehensive (loss) gain	(1,594)	563
Total stockholders' equity	1,409,961	878,136
Total liabilities and stockholders' equity	$ 1,460,202	$ 937,907

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited)

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

	Three Months Ended March 31,	
	2026	**2025**
Operating expenses:		
Research and development	$ 77,987	$ 60,806
General and administrative	27,873	13,922
Total operating expenses	105,860	74,728
Loss from operations	(105,860)	(74,728)
Other income:		
Other income, net	13,299	5,432
Total other income	13,299	5,432
Net loss	$ (92,561)	$ (69,296)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.20)	$ (3.29)
Weighted average common shares outstanding, basic and diluted	28,883,596	21,055,834

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited)

(Amounts in thousands)

	Three Months Ended March 31,	
	2026	**2025**
Net loss	$ (92,561)	$ (69,296)
Change in unrealized (loss) gain on marketable securities, net of tax	(2,157)	5
Comprehensive loss	$ (94,718)	$ (69,291)

The accompanying notes are an integral part of these condensed consolidated financial statements.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(Unaudited)

(Amounts in thousands, except share data)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2025	25,195,092	$ 15	$ 2,017,566	$ (1,140,008)	$ 563	$ 878,136
Stock-based compensation expense	—	—	17,112	—	—	17,112
Issuance of common stock from follow-on public offering, net of underwriting discounts, commissions and offering costs of $40,153	2,543,800	—	621,235	—	—	621,235
Vesting of restricted stock units	134,225	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(45,548)	—	(12,923)	—	—	(12,923)
Issuance of common stock upon exercise of stock options	31,945	—	1,119	—	—	1,119
Change in unrealized gain on marketable securities, net of tax	—	—	—	—	(2,157)	(2,157)
Net loss	—	—	—	(92,561)	—	(92,561)
Balance at March 31, 2026	27,859,514	$ 15	$ 2,644,109	$ (1,232,569)	$ (1,594)	$ 1,409,961

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2024	19,422,358	$ 14	$ 1,281,522	$ (836,740)	$ 654	$ 445,450
Stock-based compensation expense	—	—	8,786	—	—	8,786
Issuance of common stock from at-the-market public offerings, net of commission costs of $1,120	694,212	—	54,904	—	—	54,904
Issuance of common stock from exercise of pre-funded warrants	173,840	—	—	—	—	—
Vesting of restricted stock units	55,850	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(18,765)	—	(1,195)	—	—	(1,195)
Issuance of common stock upon exercise of stock options	14,422	—	560	—	—	560
Change in unrealized gain on marketable securities, net of tax	—	—	—	—	5	5
Net loss	—	—	—	(69,296)	—	(69,296)
Balance at March 31, 2025	20,341,917	$ 14	$ 1,344,577	$ (906,036)	$ 659	$ 439,214

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)

	Three Months Ended March 31,	
	2026	**2025**
Cash flows from operating activities:		
Net loss	$ (92,561)	$ (69,296)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	39	47
Stock-based compensation expense	17,112	8,786
Non-cash operating lease expense	197	249
Amortization of premiums and discounts on marketable securities, net	(655)	(1,229)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	672	6,637
Accounts payable	6,530	10,381
Accrued expenses	(17,385)	(8,280)
Operating lease liabilities	(101)	(303)
Other	—	(2)
Net cash used in operating activities	(86,152)	(53,010)
Cash flows from investing activities:		
Purchases of property and equipment	(62)	—
Purchases of marketable securities	(486,782)	(117,057)
Maturities of marketable securities	142,569	65,993
Net cash used in investing activities	(344,275)	(51,064)
Cash flows from financing activities:		
Issuance of common stock from follow-on public offering, net of underwriting discounts, commissions and offering costs	621,235	—
Proceeds from at-the-market offerings, net of issuance and commission costs	—	54,904
Payments of tax withholdings related to vesting of restricted stock units	(12,923)	(1,195)
Proceeds from exercise of stock options	1,119	560
Net cash provided by financing activities	609,431	54,269
Increase (decrease) in cash, cash equivalents and restricted cash	179,004	(49,805)
Cash, cash equivalents and restricted cash, beginning of period	357,745	215,788
Cash, cash equivalents and restricted cash, end of period	$ 536,749	$ 165,983
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	536,333	165,567
Restricted cash	416	416
Total cash, cash equivalents and restricted cash	$ 536,749	$ 165,983
Supplemental disclosures of non-cash activities:		
Purchase of property and equipment included in accrued expenses	$ —	$ 64
Right-of-use assets obtained in exchange for lease obligations	$ 1,425	$ —
Removal of right-of-use assets in connection with lease termination	$ 4,086	$ —

The accompanying notes are an integral part of these condensed consolidated financial statements.

1. Nature of the Business

Praxis Precision Medicines, Inc. (“Praxis” or the “Company”) is a fully integrated, leading central nervous system ("CNS") precision neuroscience biopharmaceutical company translating insights from genetic epilepsies into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Normal brain function requires a delicate balance of excitation and inhibition in neuronal circuits, which, when dysregulated, can lead to abnormal function and both rare and more prevalent neurological disorders. The Company is applying genetic insights to the discovery and development of therapies for neurological disorders through two proprietary platforms, using its understanding of shared biological targets and circuits in the brain. Each platform has multiple programs currently, with significant potential for additional program and indication expansion:

- ***Cerebrum***™, the Company's small molecule platform, utilizes deep understanding of neuronal excitability and neuronal networks and applies a series of computational and experimental tools to develop orally available precision therapies
- ***Solidus***™, the Company's antisense oligonucleotide ("ASO"), platform, is an efficient, targeted precision medicine discovery and development engine anchored on a proprietary, computational methodology

The Company's platforms utilize a deliberate, pragmatic and patient-guided 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, the Company has established a diversified, multimodal CNS portfolio with four clinical-stage product candidates across movement disorders and epilepsy.

Praxis was incorporated in 2015 and commenced operations in 2016. The Company has funded its operations primarily with proceeds from the issuance of redeemable convertible preferred stock, from the sale of common stock through an initial public offering, at-the-market offerings under its shelf registration statement, and follow-on public offerings of common stock and pre-funded warrants to purchase common stock. From inception through March 31, 2026, the Company raised $2.4 billion in aggregate cash proceeds from these transactions, net of issuance costs.

The Company is subject to risks and uncertainties common to clinical-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 may require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of 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 a net loss of $92.6 million for the three months ended March 31, 2026. In addition, as of March 31, 2026, the Company had an accumulated deficit of $1.2 billion. The Company expects to continue to generate operating losses for the foreseeable future.

The Company expects that its cash, cash equivalents and marketable securities as of March 31, 2026 of $1.4 billion will be sufficient to fund its operating expenditures 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 achieve regulatory approvals of its new drug applications, which are subject to risk and timing uncertainty, and raising additional capital to finance its operations, if needed. 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 significant accounting policies used in preparation of these condensed consolidated financial statements for the three months ended March 31, 2026 are consistent with those discussed in Note 2 to the consolidated financial statements included in the Company's 2025 Annual Report on Form 10-K, other than as noted below.

Unaudited Interim Condensed Consolidated Financial Information

The accompanying condensed consolidated balance sheet as of March 31, 2026, the condensed consolidated statements of operations for the three months ended March 31, 2026 and 2025, the condensed consolidated statements of comprehensive loss for the three months ended March 31, 2026 and 2025, the condensed consolidated statements of cash flows for the three months ended March 31, 2026 and 2025 and the condensed consolidated statements of stockholders’ equity for the three months ended March 31, 2026 and 2025 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 March 31, 2026, the results of its operations for the three months ended March 31, 2026 and 2025 and its cash flows for the three months ended March 31, 2026 and 2025. Financial statement disclosures for the three months ended March 31, 2026 and 2025 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 months ended March 31, 2026 are not necessarily indicative of results to be expected for the year ended December 31, 2026, 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, accrued and prepaid research and development expense, collaboration revenue, stock-based compensation expense and the recoverability of the Company’s net deferred tax assets and related valuation allowance. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from those estimates.

Common Stock Warrants

The Company accounts for warrants to purchase shares of its common stock in accordance with the guidance in FASB ASC No. 480, *Distinguishing Liabilities from Equity* ("ASC 480") and ASC No. 815, *Derivatives and Hedging* ("ASC 815"). The Company classifies warrants issued for the purchase of shares of its common stock as either equity or liability instruments based on an assessment of the specific terms and conditions of each respective contract. Such assessment includes determining whether the warrants are freestanding financial instruments or embedded in a host instrument, whether the warrants are liabilities within the scope of ASC 480, whether the warrants meet the definition of a derivative in ASC 815 and whether the warrants meet the requirements for equity classification pursuant to the indexation and equity classification criteria in ASC 815. The Company determines the classification for its warrants at the time of issuance and updates its assessment, as necessary. Warrants that meet

all of the criteria for equity classification are recorded as a component of additional paid-in capital. Equity classified warrants are accounted for at fair value on the issuance date with no changes in fair value recognized after the issuance date.

Net Loss per Share

Basic net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding for the period. The calculation of weighted average number of common shares outstanding excludes shares of restricted common stock that are not vested but includes shares of common stock underlying pre-funded warrants. Diluted net loss per share is computed by dividing net loss 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 potential shares issuable under the 2020 ESPP 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 are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

Performance Stock Units

For equity awards that do not vest unless the performance condition is met, the Company recognizes expense if, and to the extent that, it is determined that achievement of the performance conditions are probable. If the Company concludes that vesting is probable, expense is recognized over the requisite service period.

Recent Accounting Pronouncements

Recently Issued Accounting Pronouncements

In November 2024, the FASB issued ASU No. 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU 2024-03"), requiring public entities to disclose additional information about specific expense categories in the notes to the financial statements on an interim and annual basis. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and for interim periods beginning after December 15, 2027, with early adoption permitted. The amendments in this update should be applied either prospectively to financial statements issued for reporting periods after the effective date of this update, or retrospectively to any or all prior periods presented in the financial statements. The Company is currently evaluating the impact of adopting ASU 2024-03 on its consolidated financial statements.

In September 2025, the FASB issued ASU No. 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software* ("ASU 2025-06"), which amends certain aspects of the accounting for and disclosure of software costs under ASC 350-40. The ASU makes targeted improvements to ASC 350-40 to modernize the guidance for internal-use software and better reflect current software development practices. ASU 2025-06 is effective for fiscal periods beginning after December 15, 2027 and interim periods within those fiscal years. The amendments in this update can be applied using a prospective, retrospective, or modified transition approach. The Company does not expect the adoption of ASU 2025-06 to have a material impact on its consolidated financial statements.

In December 2025, the FASB issued ASU No. 2025-11, *Interim Reporting (Topic 270): Narrow-Scope Improvements* ("ASU 2025-11"), which clarifies the guidance in Topic 270 to improve the consistency of interim financial reporting. The ASU provides a comprehensive list of required interim disclosures and introduces a disclosure principle requiring entities to disclose events since the end of the last annual reporting period that have material impact on the entity. ASU 2025-11 is effective for fiscal years beginning after December 15, 2027, including interim periods within those fiscal years, with early adoption permitted. The Company is currently evaluating the impact of adopting ASU 2025-11on its consolidated financial statements.

3. Cash Equivalents and Marketable Securities

The following is a summary of the Company's investment portfolio as of March 31, 2026 and December 31, 2025 (in thousands):

	As of March 31, 2026			
	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 245,659	$ —	$ —	$ 245,659
Available-for-sale securities:				
Debt securities issued by U.S. government agencies	257,475	—	(177)	257,298
Corporate debt securities	311,749	—	(643)	311,106
Asset backed securities	88,419		(31)	88,388
Commercial paper	6,552	—	(15)	6,537
Other debt securities	248,869	—	(728)	248,141
Total cash equivalents and marketable securities	$ 1,158,723	$ —	$ (1,594)	$ 1,157,129

	As of December 31, 2025			
	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 195,296	$ —	$ —	$ 195,296
Debt securities issued by U.S. government agencies	7,003	—	(1)	7,002
Available-for-sale securities:				
Corporate debt securities	198,036	282	—	198,318
Debt securities issued by U.S. government agencies	183,739	258	—	183,997
Asset backed securities	61,282	70	—	61,352
Commercial paper	7,714	4	—	7,718
Other debt securities	117,424	—	(50)	117,374
Total cash equivalents and marketable securities	$ 770,494	$ 614	$ (51)	$ 771,057

As of March 31, 2026, the Company had 178 securities with a total fair market value of $763.2 million in an unrealized loss position. The Company believes that any unrealized losses associated with the decline in value of its securities is temporary and primarily related to the change in market interest rates since purchase, and believes that it is more likely than not that it will be able to hold its debt securities to maturity. The Company anticipates a full recovery of the amortized cost basis of its debt securities at maturity and an allowance was not recognized.

Contractual maturities of the marketable securities at each balance sheet date are as follows (in thousands):

	March 31, 2026	December 31, 2025
Within one year	$ 250,532	$ 242,002
After one year through five years	660,938	326,757
Total	$ 911,470	$ 568,759

Securities are evaluated for impairment at the end of each reporting period. The Company did not record any impairment related to its available-for-sale securities during the three months ended March 31, 2026 and 2025.

4. Fair Value Measurements

Fair value is defined as the price that would be received to sell an asset in an orderly transaction between market participants at the measurement date. The Company categorizes financial assets measured at fair value based on a fair value hierarchy. The following fair value hierarchy is used to classify financial assets based on observable inputs and unobservable inputs used to value the financial assets:

- Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical assets;

- Level 2: Quoted prices for similar assets in active markets, quoted prices in markets that are not active, or inputs which are unobservable, either directly or indirectly, for substantially the full term of the asset; or
- Level 3: Prices or valuation techniques that require inputs that are both significant to the valuation of the asset and unobservable.

The following table presents 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 as of March 31, 2026 and December 31, 2025 (in thousands):

	As of March 31, 2026			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$ 245,659	$ —	$ —	$ 245,659
Debt securities issued by U.S. government agencies	257,298	—	—	257,298
Corporate debt securities	—	311,106	—	311,106
Asset backed securities	—	88,388	—	88,388
Commercial paper	—	6,537	—	6,537
Other debt securities	—	248,141	—	248,141
	$ 502,957	$ 654,172	$ —	$ 1,157,129

	As of December 31, 2025			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$ 195,296	$ —	$ —	$ 195,296
Debt securities issued by U.S. government agencies	190,999	—	—	190,999
Corporate debt securities	—	198,318	—	198,318
Asset backed securities	—	61,352	—	61,352
Commercial paper	—	7,718	—	7,718
Other debt securities	—	117,374	—	117,374
	$ 386,295	$ 384,762	$ —	$ 771,057

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	March 31, 2026	December 31, 2025
Accrued external research and development expenses	$ 12,944	$ 14,291
Accrued personnel-related expenses	2,989	18,713
Accrued other expenses	1,715	2,029
Total accrued expenses	$ 17,648	$ 35,033

6. Commitments and Contingencies

In May 2021, the Company entered into a sublease agreement for office space located in Boston, Massachusetts that expired on January 31, 2026. The Company issued a letter of credit to the landlord related to the security deposit, secured by restricted cash, which is reflected within prepaid expenses and other current assets on the accompanying condensed consolidated balance sheets as of March 31, 2026 and December 31, 2025. In September 2025, the Company entered into a license agreement for its existing office space which commenced on

March 1, 2026 and expires on March 31, 2027, with no option to renew or terminate early. At commencement, the Company recorded an operating lease right-of-use asset and operating lease liability of $1.4 million, which are reflected within the accompanying condensed consolidated balance sheet as of March 31, 2026. This license agreement qualifies as an operating lease.

7. Collaboration and License Agreements

UCB Option and License Agreement

In December 2022, the Company entered into an Option and License Agreement ("the Collaboration Agreement") with UCB Biopharma SRL ("UCB") for the discovery of small molecule therapeutics as potential treatments of KCNT1-related epilepsies. Under the terms of the Collaboration Agreement, the Company has agreed to perform general biology-related research services as part of a mutually agreed upon research plan in exchange for a $5.0 million upfront payment. In addition, the Company provided UCB an exclusive option to in-license global development and commercialization rights to any resulting KCNT1 small molecule development candidate identified as part of the research plan. If UCB exercises its option to in-license global development and commercialization rights, the Collaboration Agreement stipulates that UCB will assume research, development, manufacturing and commercialization responsibilities and costs. Under the terms of the Collaboration Agreement, the Company was eligible to receive an option fee and future success-based development and commercialization milestone payments, totaling up to $98.5 million, in addition to tiered royalties on net sales of any resulting products from the Collaboration Agreement.

The Company concluded that UCB is a customer, and as such, the arrangement falls within the scope of Topic 606. At the commencement of the Collaboration Agreement, the Company identified one performance obligation, which was to perform the research services for UCB. The Company determined the transaction price to be $5.0 million, comprised of the upfront payment it received. The option provided to UCB was determined not to be a material right.

The Company recognized revenue for its research services performance obligation over time using an input method over the duration of the research services. In December 2024, UCB exercised its option to in-license global development and commercialization rights for a KCNT1 development candidate as part of the Collaboration Agreement. Upon notice of the exercise, the Company recognized the $6.0 million option exercise fee and the $2.6 million of remaining collaboration revenue associated with the $5.0 million up front payment as collaboration revenue within the December 31, 2024 consolidated statement of operations, as the Company had no further research service obligations under the terms of the Collaboration Agreement. Accordingly, the Company did not recognize any collaboration revenue associated with this Collaboration Agreement during the three months ended March 31, 2026 or March 31, 2025. As of March 31, 2026, there was no deferred revenue recorded in the condensed consolidated balance sheet.

Tenacia Collaboration and License Agreement

On January 4, 2024, the Company entered into an exclusive collaboration and license Agreement ("the Tenacia License Agreement") with Tenacia Biotechnology (Shanghai) Co., Ltd. ("Tenacia"), a China-based portfolio company of Bain Capital, which provides Tenacia an exclusive license to use certain intellectual property for the development and commercialization of ulixacaltamide and products containing ulixacaltamide in China, Hong Kong, Macau and Taiwan. Tenacia is solely responsible for the development and commercialization under the arrangement, with the exception of the associated manufacturing. The Company also entered into a Stock Purchase Agreement ("the Stock Purchase Agreement") with BCPE Tenet Holdings Cayman, Ltd. ("BCPE"), a related party of Tenacia. Pursuant to the terms of the Tenacia License Agreement, the Company was entitled to an up-front, non-refundable and non-creditable cash payment of $5.0 million, net of certain tax withholdings. In addition, the Company is eligible to receive $264.0 million in success-based development and commercialization milestone payments as well as tiered royalties on net sales. Pursuant to the terms of the Stock Purchase Agreement, the Company issued and sold 443,253 shares of its common stock to BCPE at a price per share of $22.5605 for aggregate gross proceeds of $10.0 million. The per share price was based on a 20% premium over the 30-day volume-weighted average price.

Under the terms of the Tenacia License Agreement, the Company granted to Tenacia an exclusive license to use certain intellectual property for the development and commercialization of ulixacaltamide and products containing ulixacaltamide in China, Hong Kong, Macau and Taiwan. Tenacia is solely responsible for the development and commercialization under the arrangement, with the exception of the associated manufacturing.

The Company concluded that the Tenacia License Agreement and the Stock Purchase Agreement is a combined arrangement since they were executed at the same time and in contemplation of each other with the same counterparty or a related party thereof and the combined arrangement falls within the scope of Topic 606.

The Company's obligations under the arrangement comprise a single promise, or one performance obligation, related to the exclusive development and commercialization license granted to Tenacia. Total proceeds associated with the combined arrangement at inception were $14.8 million consisting of the following: (i) $10.0 million gross proceeds from the sale of common stock under the Stock Purchase Agreement and (ii) $4.8 million, net of tax, related to the up-front payment under the Tenacia License Agreement, both of which were received in January 2024. During the three months ended March 31, 2026, the Company had not achieved any development or sales milestones or earned any royalties under the Tenacia License Agreement.

The Company recorded the common stock sold to BCPE at its issuance date fair value of $38.95 per share, or $17.3 million in the aggregate, which exceeded the proceeds received which were calculated based on the 20% premium over the prior 30-day volume-weighted average price. Accordingly, there is no transaction price allocable to the performance obligation. The Company accounted for the excess of the fair value of the equity securities issued over the total proceeds received as consideration paid to a customer for which no distinct good or service was transferred in exchange. As a result, the transaction gives rise to negative revenue on a cumulative basis in the amount of $2.5 million, which was recorded at the inception of the Tenacia License Agreement in research and development expense in the accompanying consolidated statement of operations. For agreements where the licenses are considered separate performance obligations or represent the only performance obligation, the Company recognizes revenue at the point in time that the Company effectively grants the license, as the licenses or assignments represent functional intellectual property.

As of March 31, 2026, the Company did not have any receivables or deferred revenue related to the arrangement with Tenacia. The Company did not recognize any contract assets related to costs to obtain a contract with a customer or costs to fulfill a contract with a customer through March 31, 2026 because no qualifying costs were incurred. During the three months ended March 31, 2026 and March 31, 2025, the Company did not recognize any revenue associated with the combined arrangement.

8. Common Stock and Preferred Stock

Common Stock

As of March 31, 2026 and December 31, 2025, the authorized capital stock of the Company included 150,000,000 shares of common stock, $0.0001 par value.

As of March 31, 2026 and December 31, 2025, the Company did not hold any treasury shares.

Follow-On Public Offerings

January 2024 Public Offering

On January 16, 2024, the Company completed a public offering of: (i) an aggregate of 3,802,025 shares of its common stock at a public offering price of $35.50 per share, including the underwriters' full exercise of their option to purchase 633,750 additional shares of common stock, and (ii) pre-funded warrants to purchase 1,056,725 shares of common stock at a public offering price of $35.4999 per share of common stock underlying the warrants. The purchase price per share of each pre-funded warrant represents the per share offering price for the common stock, less the $0.0001 per share exercise price of each underlying share. Total net proceeds generated from the offering were approximately $161.6 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

The pre-funded warrants are exercisable at any time on or after the date of issuance at the option of the holder, subject to a beneficial ownership blocker that may limit exercisability. No holder may exercise any portion of the warrants that would cause the aggregate number of shares of common stock beneficially owned by such holder, together with its affiliates, to exceed 4.99% (or 9.99%) of the issued and outstanding common stock. A holder of a pre-funded warrant may increase or decrease this percentage up to 19.99% by providing at least 61 days' prior notice to the Company. The pre-funded warrants do not expire. The pre-funded warrants may be settled through either physical or net share settlement. Following the occurrence of certain fundamental transactions, the holders of the pre-funded warrants have the right to receive upon exercise of the warrants the same amount and kind of

securities, cash, or property as they would have been entitled to receive if they had been holders of the common shares issuable under the warrants immediately prior to such transaction. During the year ended December 31, 2025, 200,355 pre-funded warrants were exercised via cashless exercise, resulting in 200,349 shares of common stock issued. No cash proceeds associated with the exercise were received by the Company. As of December 31, 2025, a total of 704,225 pre-funded warrants associated with this offering remained outstanding.

During the three months ended March 31, 2026, no pre-funded warrants were exercised. As of March 31, 2026, a total of 704,225 pre-funded warrants associated with this offering remained outstanding.

April 2024 Public Offering

On April 2, 2024, the Company completed a public offering of: (i) an aggregate of 3,849,558 shares of its common stock at a public offering price of $56.50 per share, including the underwriters' full exercise of their option to purchase 530,973 additional shares of common stock, and (ii) pre-funded warrants to purchase 221,238 shares of common stock at a public offering price of $56.4999 per share of common stock underlying the warrants. The purchase price per share for each pre-funded warrant represents the per share offering price for the common stock, less the $0.0001 per share exercise price of each underlying share. Total net proceeds generated from the offering were approximately $216.0 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

The pre-funded warrants are exercisable at any time on or after the date of issuance at the option of the holder, subject to a beneficial ownership blocker that may limit exercisability. No holder may exercise any portion of the warrants that would cause the aggregate number of shares of common stock beneficially owned by such holder, together with its affiliates, to exceed 4.99% (or 9.99%) of the issued and outstanding common stock. A holder of a pre-funded warrant may increase or decrease this percentage up to 19.99% by providing at least 61 days' prior notice to the Company. The pre-funded warrants do not expire. The pre-funded warrants may be settled through either physical or net share settlement. Following the occurrence of certain fundamental transactions, the holders of the pre-funded warrants have the right to receive upon exercise of the warrants the same amount and kind of securities, cash or property as they would have been entitled to receive if they had been holders of the common shares issuable under the warrants immediately prior to such transaction. As of March 31, 2026, none of the pre-funded warrants had been exercised and all remained outstanding.

October 2025 Public Offering

On October 20, 2025, the Company completed a public offering of: (i) an aggregate of 3,527,072 shares of its common stock at a public offering price of $157.00 per share, including the underwriters' full exercise of their option to purchase 501,592 additional shares of common stock, and (ii) pre-funded warrants to purchase 318,470 shares of common stock at a public offering price of $156.9999 per share of common stock underlying the warrants. The purchase price per share of each pre-funded warrant represents the per share offering price for the common stock, less the $0.0001 per share exercise price of each underlying share. Total net proceeds generated from the offering were approximately $567.1 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

The pre-funded warrants are exercisable at any time on or after the date of issuance at the option of the holder, subject to a beneficial ownership blocker that may limit exercisability. No holder may exercise any portion of the warrants that would cause the aggregate number of shares of common stock beneficially owned by such holder, together with its affiliates, to exceed 4.99% (or 9.99%) of the issued and outstanding common stock. A holder of a pre-funded warrant may increase or decrease this percentage up to 19.99% by providing at least 61 days’ prior notice to the Company. The pre-funded warrants do not expire. The pre-funded warrants may be settled through either physical or net share settlement. Following the occurrence of certain fundamental transactions, the holders of the pre-funded warrants have the right to receive upon exercise of the warrants the same amount and kind of securities, cash, or property as they would have been entitled to receive if they had been holders of the common shares issuable under the warrants immediately prior to such transaction. As of March 31, 2026, none of the pre-funded warrants had been exercised and all remained outstanding.

The Company determined that the pre-funded warrants related to the January 2024, April 2024, and October 2025 public offerings are freestanding financial instruments because they are both legally detachable and separately exercisable from the common stock sold in the offering. As such, the Company evaluated the pre-funded warrants to determine whether they represent instruments that require liability classification pursuant to the guidance in ASC 480. However, the Company concluded that the pre-funded warrants are not a liability within the

scope of ASC 480 due to their characteristics. Further, the Company determined that the pre-funded warrants do not meet the definition of a derivative under ASC 815 because they do not meet the criteria regarding no or little initial net investment. Accordingly, the Company assessed the pre-funded warrants relative to the guidance in ASC No. 815-40, *Contracts in Entity's Own Equity*, to determine the appropriate treatment. The Company concluded that the pre-funded warrants are both indexed to its own stock and meet all other conditions for equity classification. Accordingly, the Company has classified the pre-funded warrants as permanent equity.

January 2026 Public Offering

On January 7, 2026, the Company completed a public offering of 2,543,800 shares of its common stock at a public offering price of $260.00 per share, including the underwriters' full exercise of their option to purchase 331,800 additional shares of common stock. Total net proceeds generated from the offering were approximately $621.2 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

Shares Reserved for Future Issuance

The Company has reserved the following shares of common stock for future issuance:

	March 31, 2026	December 31, 2025
Shares reserved for exercise of outstanding stock options	1,946,616	1,876,778
Shares reserved for exercise of pre-funded warrants related to the January 2024 Financing	704,225	704,225
Shares reserved for exercise of pre-funded warrants related to the April 2024 Financing	221,238	221,238
Shares reserved for exercise of pre-funded warrants related to October 2025 Financing	318,470	318,470
Shares reserved for future awards under the 2020 Stock Option and Incentive Plan	1,423,495	473,358
Shares reserved for future awards under the 2020 Employee Stock Purchase Plan	533,130	281,180
Shares reserved for future awards under the 2024 Inducement Plan	944,629	945,280
Shares reserved for vesting of restricted stock units and performance stock units	636,178	501,853
Total shares of authorized common stock reserved for future issuance	6,727,981	5,322,382

Preferred Stock

As of March 31, 2026 and December 31, 2025, the authorized capital stock of the Company included 10,000,000 shares of undesignated preferred stock, $0.0001 par value.

9. Stock-Based Compensation

2024 Inducement Plan

In January 2024, the Board of Directors ("the Board") adopted the 2024 Inducement Plan (the "Inducement Plan") without stockholder approval pursuant to Rule 5635(c)(4) of the Nasdaq Stock Market LLC listing rules ("Rule 5635(c)(4)"). In accordance with Rule 5635(c)(4), the Inducement Plan allows the Company to grant awards only to a newly hired employee who was not previously an employee or non-employee director or to an employee who is being rehired following a bona fide period of non-employment if such award is a material inducement to such employee entering into employment. In January 2025, the number of shares of common stock authorized for issuance under the Inducement Plan was increased by 870,000 shares. The total number of shares of common stock authorized for issuance under the Inducement Plan as of March 31, 2026 and December 31, 2025 was 1,000,000 shares.

2020 Stock Option and Incentive Plan

The total number of shares of common stock authorized for issuance under the 2020 Stock Option and Incentive Plan (the "2020 Plan") as of March 31, 2026 and December 31, 2025 was 4,201,704 shares and 2,941,950 shares, respectively.

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 March 31, 2026 and December 31, 2025 was 395,850 shares. Any authorization to issue new

options under the 2017 Plan was cancelled upon the effectiveness of the 2020 Plan and no further awards will be granted under the 2017 Plan.

2020 Employee Stock Purchase Plan

The total number of shares of common stock authorized for issuance under the 2020 Employee Stock Purchase Plan (the "2020 ESPP") as of March 31, 2026 and December 31, 2025 was 621,318 shares and 369,368 shares, respectively.

Restricted Stock Units

The following table summarizes the Company's restricted stock unit activity:

	Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2025	501,853	$ 64.12
Issued	145,016	295.79
Vested	(126,702)	66.81
Forfeited	(15,791)	109.24
Unvested as of March 31, 2026	504,376	$ 128.64

As of March 31, 2026, total unrecognized compensation cost related to unvested restricted stock units was $60.4 million, which is expected to be recognized over a weighted-average period of 3.43 years.

Performance Stock Units

In January 2026, the Company granted performance stock units ("PSUs") to its executive officers under the 2020 Plan. Vesting of the PSUs is subject to the achievement of specified performance conditions, which include achieving objectively measurable clinical, regulatory and commercial revenue targets. If these conditions are not met, the PSUs would be forfeited and returned to the plan. The actual number of common shares issued that could potentially vest is calculated by multiplying the target number of PSUs granted by a multiplier determined for each performance goal, resulting in an aggregate payout ranging from 0% to 250% of the total target PSUs granted. The performance conditions are required to be achieved during the performance period, which ends on December 31, 2027.

The following table summarizes the Company's performance stock unit activity:

	Shares	Grant Date Fair Value
Unvested as of December 31, 2025	—	$ —
Issued[(a)]	131,802	294.38
Vested	—	—
Forfeited	—	—
Unvested as of March 31, 2026	131,802	$ 294.38

(a) The number of shares issued during the period is the maximum amount of shares that could be payable under the terms of the PSU agreement.

As of March 31, 2026, total unrecognized compensation cost related to unvested PSUs that were determined to be probable of achievement was $24.1 million, which is expected to be recognized over a weighted-average period of 0.85 years.

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	Aggregate Intrinsic Value
			(In years)	(In thousands)
Outstanding as of December 31, 2025	1,876,778	$ 89.80		
Granted	102,063	294.49		
Exercised	(25,402)	50.26		$ 6,500
Cancelled or Forfeited	(6,823)	81.11		
Outstanding as of March 31, 2026	1,946,616	$ 101.08	7.59	$ 454,075
Exercisable as of March 31, 2026	1,236,855	$ 107.19	7.03	$ 289,586
Vested and expected to vest as of March 31, 2026	1,946,616	$ 101.08	7.59	$ 454,075

Valuation of Stock Options

The weighted-average 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 and non-employees on the date of grant were as follows for the three months ended March 31, 2026:

	Three Months Ended March 31,
	2026
Risk-free interest rate	3.85 %
Expected term (in years)	6.00
Expected volatility	80.22 %
Expected dividend yield	— %
Weighted average grant-date fair value per share	$ 209.19

As of March 31, 2026, total unrecognized compensation cost related to unvested stock options was $46.4 million, which is expected to be recognized over a weighted-average period of 2.52 years.

Stock-Based Compensation

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

	Three Months Ended March 31,	
	2026	2025
Research and development	$ 5,513	$ 3,780
General and administrative	11,599	5,006
Total stock-based compensation expense	$ 17,112	$ 8,786

10. Net Loss per Share

The following potential common shares, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have been anti-dilutive:

	Three Months Ended March 31,	
	2026	2025
Outstanding stock options	1,946,616	2,050,354
Unvested restricted stock units and performance stock units	636,178	522,257
Potential shares issuable under the 2020 ESPP	4,991	6,369
	2,587,785	2,578,980

Common shares issuable upon exercise of the pre-funded warrants that were sold in connection with the January 2024, April 2024, and October 2025 underwritten public offerings are included in the calculation of basic weighted average number of common shares outstanding for the three months ended March 31, 2026 and 2025. Consistent with the guidance in ASC 260-10-45-13, the underlying common shares are issuable for little to no consideration and there are no vesting conditions or contingencies associated with the warrants. Accordingly, the aggregate number of common shares underlying the pre-funded warrants have been considered outstanding for purposes of the calculation of basic net loss per share from the date of issuance.

11. 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. Expenses incurred during all periods presented were not material.

12. Segment Information

The Company has one operating segment. The Company's operating segment discovers and develops therapies for CNS disorders. The Company's chief operating decision maker ("CODM"), its Chief Executive Officer, manages the Company's operations on a consolidated basis for the purposes of assessing performance and allocating resources based on net loss that also is reported on the condensed consolidated statement of operations as consolidated net loss. Net loss is used by the CODM to make key strategic and operational decisions. The measure of segment assets is reported on the condensed consolidated balance sheet as total consolidated assets. The majority of the Company's long-lived assets are held in the United States.

The following table presents selected financial information about the Company's single operating segment for the three months ended March 31, 2026 and 2025 (in thousands):

	Three Months Ended March 31,	
	2026	2025
Program-specific expenses:		
Ulixacaltamide	$ 19,103	$ 23,513
Vormatrigine	20,494	13,733
Relutrigine	12,854	5,377
Elsunersen	3,197	761
Other early stage assets	3,242	1,105
Personnel-related expenses	17,730	13,030
Stock-based compensation expense	17,112	8,786
Depreciation expense	39	47
Other segment expenses[(a)]	12,089	8,376
Other income, net	13,299	5,432
Consolidated net loss	$ (92,561)	$ (69,296)

(a) Other segment expenses includes research and development and general and administrative costs not attributable to a specific program.

13. 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 identify matters that require additional disclosure. The Company has concluded that no subsequent events have occurred that require disclosure.

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 audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2025, which was filed with the Securities and Exchange Commission, or the SEC, on February 19, 2026. 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 our Annual Report on Form 10-K for the year ended December 31, 2025, 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 fully integrated, leading central nervous system, or CNS, precision neuroscience biopharmaceutical company translating insights from genetic epilepsies into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Normal brain function requires a delicate balance of excitation and inhibition in neuronal circuits, which, when dysregulated, can lead to abnormal function and both rare and more prevalent neurological disorders. We are applying genetic insights to the discovery and development of therapies for neurological disorders through two proprietary platforms, using our understanding of shared biological targets and circuits in the brain. Each platform currently has multiple programs, with significant potential for additional program and indication expansion:

- ***Cerebrum***™, our small molecule platform, utilizes deep understanding of neuronal excitability and neuronal networks and applies a series of computational and experimental tools to develop orally available precision therapies
- ***Solidus***™, our antisense oligonucleotide, or ASO, platform, is an efficient, targeted precision medicine discovery and development engine anchored on a proprietary, computational methodology

Our platforms utilize a deliberate, pragmatic and patient-guided 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 diversified, multimodal CNS portfolio with four clinical-stage product candidates across movement disorders and epilepsy.

For our ulixacaltamide program within the Cerebrum™ platform, we announced positive topline results for the two studies in the Phase 3 Essential3 program in essential tremor, or ET, in October 2025. We submitted a New Drug Application, or NDA, for ulixacaltamide HCl for the treatment of ET to the FDA, and it was accepted for review with a target action date under the Prescription Drug User Fee Act, or PDUFA, of January 29, 2027. In anticipation of potential approval, commercial preparations and pre-launch activities are ongoing.

For our relutrigine program within the Cerebrum™ platform, in December 2025, we announced positive results from the registrational cohort of the EMBOLD study in SCN2A and SCN8A developmental and epileptic encephalopathies, or DEEs, after receiving a recommendation from the Data Monitoring Committee to stop the study early for efficacy. We submitted an NDA for relutrigine for the treatment of SCN2A-DEE and SCN8A-DEE to the FDA, and it was accepted for priority review with a target PDUFA action date of September 27, 2026. In anticipation of potential approval, commercial preparations and pre-launch activities are ongoing. The EMERALD study, which is evaluating relutrigine in patients with broad DEEs, has completed recruitment, with topline results anticipated in the fourth quarter of 2026. Assuming approval of the NDA for relutrigine, we believe the EMERALD study, if positive, could serve as the basis for a supplemental NDA, or sNDA, submission in 2027.

For our vormatrigine program within the Cerebrum™ platform, our ENERGY program is ongoing, which consists of five studies to generate patient eligibility, efficacy, safety and pharmacokinetics, or PK, data in patients with focal onset seizures, or FOS, or generalized epilepsy. Studies under the ENERGY program include EMPOWER, RADIANT, POWER1, POWER2 and POWER3. In the second half of 2025, we announced positive results from our RADIANT Phase 2 open-label study evaluating the PK, safety and efficacy of vormatrigine in patients with FOS or generalized epilepsy. We initiated the EMPOWER study, an observational study of vormatrigine in patients with epilepsy, in the third quarter of 2024. For our POWER1 study, a double-blind, placebo-controlled, 12-week study in FOS, we expect to announce topline results in the second quarter of 2026. POWER2,

our third efficacy study, is enrolling patients, with completion expected in the second half of 2026 and topline results expected in 2027. We intend to initiate POWER3, a clinical trial evaluating vormatrigine as a single-agent treatment for FOS, in the first half of 2026.

For our most advanced product candidate under the Solidus™ platform, elsunersen, we announced positive topline results from the EMBRAVE Part A study evaluating the safety and efficacy of elsunersen versus sham procedure in April 2026 for treating SCN2A gain-of-function DEE. We are currently enrolling EMBRAVE3, a Phase 3 registrational study for SCN2A gain-of-function DEE, and expect to announce topline results in 2027.

We also have three earlier stage novel ASOs with preclinical proof of mechanism. PRAX-080 is focused on PCDH19 mosaic expression, PRAX-090 is targeting SYNGAP1 loss-of-function, or LoF, mutations which is a leading cause of severe intellectual disability and epilepsy in DEEs, and PRAX-100 is targeting SCN2A LoF mutations, the predominant genetic link to de novo autism spectrum disorders, or ASD. We anticipate nominating a development candidate for PRAX-080, PRAX-090 and PRAX-100 in the first half of 2026.

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, or IP, 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, contract development and manufacturing organizations and contract research organizations, or CROs, to conduct our preclinical and clinical activities. Since inception, we have financed our operations primarily with proceeds from the sale and issuance of equity securities.

We are a development stage company and we have not generated any revenue from product sales. All of our product candidates are in preclinical and clinical development or awaiting regulatory approval. 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 $92.6 million for the three months ended March 31, 2026. As of March 31, 2026, we had an accumulated deficit of $1.2 billion. We expect to incur significant expenses for the foreseeable future as we expand our research and development activities and prepare for commercialization of our product candidates. 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 be maintained or increased in connection with our ongoing activities, as we:

- prepare for the potential commercialization of ulixacaltamide, for which we have submitted an NDA to the FDA that has been accepted for review, after successful completion of our Phase 3 Essential3 program for ET;
- prepare for the potential commercialization of relutrigine, for which we have submitted an NDA to the FDA which has been accepted for review after a successful interim analysis in the EMBOLD clinical trial, and advance relutrigine in the EMERALD clinical trial;
- advance vormatrigine in the ENERGY clinical trials for FOS or generalized epilepsy;
- advance elsunersen in the EMBRAVE3 pivotal clinical trial;
- advance our preclinical candidates to clinical trials;
- further invest in our pipeline;
- further invest in our manufacturing capabilities;
- continue to seek regulatory approval for our product candidates;
- maintain, expand, protect and defend our IP portfolio;
- acquire or in-license technology;
- continue building our sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and

- increase our headcount to support our development and commercialization efforts.

As a result, we will need substantial capital 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 March 31, 2026, we had cash, cash equivalents and marketable securities of $1.4 billion. We expect that our cash, cash equivalents, and marketable securities as of March 31, 2026 will be sufficient to fund our operating expenditures and capital expenditure requirements necessary to advance our operating activities into 2028. The analysis included consideration of our current financial needs and ongoing research and development and commercialization plans. 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."

Financial Operations Overview

Revenue

We have not generated any revenue from the sale of products since inception and our ability to generate revenue from the sale of products in the future will be dependent on regulatory approval of our product candidates. 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 such collaboration or license agreements.

Operating Expenses

Research and Development Expenses

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 product candidates; 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 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 development and manufacturing organizations to acquire, develop and manufacture materials for preclinical and clinical studies; and
- depreciation, amortization and other direct and allocated expenses, including rent and other operating costs, such as information technology, 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 condensed consolidated balance sheets as prepaid expenses or accrued 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.

As a company operating in a virtual environment, a significant portion of our research and development costs have been external costs incurred by third-parties. We track direct external research and development expenses to specific platforms and product candidates upon commencement. Due to the number of ongoing studies and our ability to use resources across platforms, indirect or shared operating costs incurred for our research and development platforms, such as personnel, facility costs and certain consulting costs, are not recorded or maintained on a platform-specific basis.

The following table reflects our research and development expenses, including direct expenses summarized by platform and indirect or shared operating costs recognized as research and development expenses during the periods presented below (in thousands):

	Three Months Ended March 31,	
	2026	2025
Cerebrum™	$ 51,989	$ 42,832
Solidus™	4,334	1,348
Personnel-related (including stock-based compensation)	16,651	12,851
Other indirect research and development expenses	5,013	3,775
Total research and development expenses	$ 77,987	$ 60,806

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 be maintained or 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, if approved, 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 successfully develop, obtain regulatory approval for, and then successfully commercialize, our product candidates;
- our receipt of marketing approvals from applicable regulatory authorities;
- our ability to commercialize products, if approved, whether alone or in collaboration with others;
- our ability to add and retain key research and development personnel;
- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to successfully complete clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- our 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 IP protection and regulatory exclusivity for our product candidates, if approved; and
- the continued acceptable safety profiles of our product candidates.

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 require significant 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, commercial 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; commercial-related costs as we prepare for the potential launch of our product candidates; insurance costs; administrative travel expenses; and facility-related expenses, which include direct depreciation costs and allocated expenses for office rent and other operating costs, such as information technology. Costs to secure and defend our IP are expensed as incurred and are classified as general and administrative expenses. These costs relate to the operation of the business and are unrelated to the research and development function or any individual platform or product candidate.

We anticipate that our general and administrative expenses may increase in the future as we increase our headcount, when needed, to support the expected growth in our research and development activities and the potential commercialization of our product candidates. 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

Other Income, Net

Other income, net consists of interest income from our cash, cash equivalents and marketable securities and amortization of investment premiums and discounts.

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. There was no income tax provision recognized for the three months ended March 31, 2026 and 2025.

Results of Operations

Comparison of the Three Months Ended March 31, 2026 and 2025

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

	Three Months Ended March 31,		Change
	2026	2025	
Operating expenses:			
Research and development	$ 77,987	$ 60,806	$ 17,181
General and administrative	27,873	13,922	13,951
Total operating expenses	105,860	74,728	31,132
Loss from operations	(105,860)	(74,728)	(31,132)
Other income:			
Other income, net	13,299	5,432	7,867
Total other income	13,299	5,432	7,867
Net loss	$ (92,561)	$ (69,296)	$ (23,265)

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 March 31,		Change
	2026	2025	
Cerebrum™	$ 51,989	$ 42,832	$ 9,157
Solidus™	4,334	1,348	2,986
Personnel-related (including stock-based compensation)	16,651	12,851	3,800
Other indirect research and development expenses	5,013	3,775	1,238
Total research and development expenses	$ 77,987	$ 60,806	$ 17,181

The $17.2 million increase in research and development expenses was primarily attributable to the following:

- $9.2 million increase in expense related to our Cerebrum™ platform, driven primarily by:
  - a $6.9 million increase in spend for our relutrigine program, primarily related to our EMERALD trial activities and manufacturing spend to support commercialization;
  - a $6.9 million increase in spend for our vormatrigine program, primarily driven by spend for our ENERGY program;
  - a $1.7 million increase in activities for our earlier stage assets as we advance our portfolio; partially offset by,
  - a $6.3 million decrease in spend for our ulixacaltamide program, driven primarily by decreased activity in the Essential3 study, partially offset by increased manufacturing costs to support commercialization and the PDUFA fee paid in the first quarter of 2026.
- $3.8 million increase in personnel-related costs mainly due to increased headcount; and
- $3.0 million increase in expense related to our Solidus™ platform, driven by elsunersen program spend for EMBRAVE Part A clinical trial and our EMBRAVE3 pivotal clinical trial.

General and Administrative Expense

The $14.0 million increase in general and administrative expenses was primarily attributable to the following:

- $9.8 million increase in personnel-related costs mainly due to increased stock-based compensation expense and increased headcount; and

- $3.5 million increase in professional expenses, primarily attributable to increased commercial spend as we prepare for the potential launch of ulixacaltamide and relutrigine.

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 or awaiting regulatory review, and we may not generate meaningful revenue from product sales in the near term, if at all.

To date, we have financed our operations primarily with proceeds from the issuance of redeemable convertible preferred stock and from the sale of common stock through an initial public offering, common stock and pre-funded warrants through follow-on public offerings and common stock from at-the-market offerings under our shelf registration statement. From inception through March 31, 2026, we have raised $2.4 billion in aggregate cash proceeds from such transactions, net of issuance costs. As of March 31, 2026, we had cash, cash equivalents, and marketable securities of $1.4 billion.

In March 2024, we entered into an Open Market Sale Agreement, or the 2024 Sales Agreement, with Jefferies LLC, or Jefferies, to provide for the offering, issuance, and sale of up to an aggregate amount of $150.0 million of common stock in at-the-market offerings. In December 2024, we entered into an amendment to the 2024 Sales Agreement with Jefferies to provide for the offering, issuance and sale of up to an aggregate amount of $250.0 million of common stock from time to time in at-the-market offerings. During the three months ended March 31, 2025, we issued and sold an aggregate of 694,212 shares under the amended 2024 Sales Agreement for aggregate net proceeds of $54.9 million, after deducting commissions and offering expenses payable by us. The amended 2024 Sales Agreement was terminated in September 2025.

In September 2025, we entered into a Sales Agreement, or the 2025 Sales Agreement, with TD Securities (USA) LLC, or TD Cowen, to provide for the offering, issuance and sale of up to an aggregate of $250.0 million of common stock from time to time in at-the-market offerings. During the three months ended March 31, 2026, we did not issue or sell any shares of common stock under the 2025 Sales Agreement.

In October 2025, we completed a public offering of: (i) an aggregate of 3,527,072 shares of our common stock at a public offering price of $157.00 per share, including the underwriters' full exercise of their option to purchase 501,592 additional shares of common stock, and (ii) pre-funded warrants to purchase 318,470 shares of common stock at a public offering price of $156.9999 per share of common stock underlying the warrants. The purchase price per share of each pre-funded warrant represents the per share offering price for the common stock, less the $0.0001 per share exercise price of each underlying share. Total net proceeds generated from the offering were $567.1 million, after deducting underwriting discounts, commissions, and other offering expenses payable by us. As of March 31, 2026, none of the pre-funded warrants had been exercised and all remained outstanding.

In January 2026, we completed a public offering of 2,543,800 shares of our common stock at a public offering price of $260.00 per share, including the underwriters' full exercise of their option to purchase 331,800 additional shares of common stock. Total net proceeds generated from the offering were approximately $621.2 million, after deducting underwriting discounts, commissions and other offering expenses payable by us.

Cash Flows

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

	Three Months Ended March 31,	
	2026	2025
Net cash (used in) provided by:		
Operating activities	$ (86,152)	$ (53,010)
Investing activities	(344,275)	(51,064)
Financing activities	609,431	54,269
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 179,004	$ (49,805)

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 operating assets and liabilities.

During the three months ended March 31, 2026, net cash used in operating activities of $86.2 million was primarily due to our $92.6 million net loss and $10.3 million in changes in operating assets and liabilities primarily related to a decrease in accrued expenses, partially offset by $16.7 million of non-cash charges primarily related to stock-based compensation.

During the three months ended March 31, 2025, net cash used in operating activities of $53.0 million was primarily due to our $69.3 million net loss, partially offset by $8.4 million in changes in operating assets and liabilities primarily related to an increase in accounts payable and $7.9 million of non-cash charges primarily related to stock-based compensation.

Investing Activities

During the three months ended March 31, 2026, net cash used in investing activities of $344.3 million was primarily related to purchases of marketable securities offset by maturities of marketable securities.

During the three months ended March 31, 2025, net cash used in investing activities of $51.1 million was primarily related to purchases of marketable securities offset by maturities of marketable securities.

Financing Activities

During the three months ended March 31, 2026, net cash provided by financing activities of $609.4 million consisted primarily of net proceeds from our January 2026 follow-on offering.

During the three months ended March 31, 2025, net cash provided by financing activities of $54.3 million consisted primarily of net proceeds from our at-the-market offerings.

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, and our commercialization activities as we prepare for the potential approval of our product candidates. As a result, we expect to incur substantial operating expenses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue to build a sales, marketing, technology and distribution infrastructure and scale-up manufacturing capabilities to commercialize any products for which we may obtain regulatory approval;
- advance the clinical development of our clinical-stage product candidates within our Cerebrum™ and Solidus™ platforms;
- 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 IP portfolio and opportunistically acquire complementary IP;
- seek to obtain regulatory approvals for our product candidates;
- when needed, add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts and to support our operations as 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 cash, cash equivalents, and marketable securities as of March 31, 2026 will be sufficient to fund our operating expenditures and capital expenditure requirements into 2028. 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 potential collaborations with third parties for the development of our product candidates, 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 platforms and product candidates;
- the number and characteristics of product candidates 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 IP rights and defending any IP-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; 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. Accordingly, we may 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 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.

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. 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 Annual Report on Form 10-K filed with the SEC on February 19, 2026, other than as disclosed in Note 2 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

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

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because cash, cash equivalents and marketable securities we may hold at any time may be in the form of money market funds or marketable debt securities or may be invested in U.S. Treasury and U.S. government agency obligations. However, because of the low risk profile of the instruments in our portfolio at any given time, an immediate change in market interest rates of 100 basis points would not have a material impact on our financial position or results of operations.

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 (the "Exchange Act") as controls and other procedures of an issuer that are designed to ensure that information required to be disclosed by the issuer in the reports that it files or submits 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 by the issuer in the reports that it files or submits under the Exchange Act is accumulated and communicated to the issuer's management, including its 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 March 31, 2026. Based on the evaluation of our disclosure controls and procedures as of March 31, 2026, 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.

There have been no material changes from the risk factors previously disclosed in the section entitled "Risk Factors" of our Annual Report on Form 10-K for the year ended December 31, 2025 filed with the SEC on February 19, 2026.

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

Recent Sales of Unregistered Equity Securities

We did not make any sales of unregistered securities during the three months ended March 31, 2026.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

(a) Disclosure in lieu of reporting on a Current Report on Form 8-K.

None.

(b) Material changes to the procedures by which security holders may recommend nominees to the board of directors.

None.

(c) Insider Trading Arrangements and Policies.

During the three months ended March 31, 2026, no director or officer of the Company adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K, except as follows:

On March 25, 2026, Jill DeSimone, a member of our board of directors, adopted a “Rule 10b5-1 trading arrangement” providing for the sale from time to time of an aggregate of up to 11,600 shares of our common stock. The trading arrangement is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c). The duration of the trading arrangement is until December 31, 2026, or earlier if all transactions under the trading arrangement are completed.

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 Current Report on Form 8-K (File No. 001-39620) filed on October 20, 2020).
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed with the Securities and Exchange Commission on December 1, 2023)
3.3	Amended and Restated Bylaws of Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on January 7, 2022).
4.1	Form of Pre-funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on January 12, 2024)
4.2	Form of Pre-funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on March 29, 2024)
4.3	Form of Pre-funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on October 20, 2025)
10.1	Form of Performance-Based Restricted Stock Unit Award Agreement for employees under the 2020 Stock Option and Incentive Plan, as amended and restated (incorporated by reference to Exhibit 10.11 to the Registrant's Annual Report on Form 10-K (File No. 001-39620) filed on February 19, 2026).
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*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (embedded within the Inline XBRL 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.

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.

PRAXIS PRECISION MEDICINES, INC.

Date: May 7, 2026

By: /s/ Marcio Souza

Marcio Souza

Chief Executive Officer and Director (Principal Executive Officer)

Date: May 7, 2026

By: /s/ Timothy Kelly

Timothy Kelly

Chief Financial Officer (Principal Financial Officer)

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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (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: May 7, 2026

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, Timothy Kelly, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (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: May 7, 2026

By: /s/ TIMOTHY KELLY

Timothy Kelly
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 March 31, 2026 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 Section 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: May 7, 2026

By: /s/ MARCIO SOUZA
Marcio Souza
Chief Executive Officer
(Principal Executive Officer)

Date: May 7, 2026

By: /s/ TIMOTHY KELLY
Timothy Kelly
Chief Financial Officer
(Principal Financial Officer)