UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2025

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)**

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

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 October 31, 2025, the registrant had 25,007,171 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:

- 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 obtain funding for our operations, including funding 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, 2024 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 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 sections titled “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2024, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2025 and elsewhere in this Quarterly Report on Form 10-Q.

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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)

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 149,527	$ 215,372
Marketable securities	117,630	177,195
Prepaid expenses and other current assets	6,687	11,805
Total current assets	273,844	404,372
Long-term marketable securities	122,008	76,961
Property and equipment, net	174	230
Operating lease right-of-use assets	362	1,131
Other non-current assets	—	416
Total assets	$ 396,388	$ 483,110
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 30,819	$ 12,528
Accrued expenses	21,628	23,763
Operating lease liabilities	436	1,259
Total current liabilities	52,883	37,550
Long-term liabilities:		
Non-current portion of operating lease liabilities	—	110
Total liabilities	52,883	37,660
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 September 30, 2025 and December 31, 2024	—	—
Common stock, $0.0001 par value; 150,000,000 shares authorized; 21,165,933 shares issued and outstanding as of September 30, 2025, and 19,422,358 shares issued and outstanding as of December 31, 2024	14	14
Additional paid-in capital	1,394,088	1,281,522
Accumulated other comprehensive gain	500	654
Accumulated deficit	(1,051,097)	(836,740)
Total stockholders' equity	343,505	445,450
Total liabilities and stockholders' equity	$ 396,388	$ 483,110

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Collaboration revenue	$ —	$ 302	$ —	$ 1,090
Operating expenses:				
Research and development	65,797	41,881	189,609	96,125
General and administrative	12,562	15,256	39,545	41,174
Total operating expenses	78,359	57,137	229,154	137,299
Loss from operations	(78,359)	(56,835)	(229,154)	(136,209)
Other income:				
Other income, net	4,425	4,925	14,797	12,069
Total other income	4,425	4,925	14,797	12,069
Net loss	$ (73,934)	$ (51,910)	$ (214,357)	$ (124,140)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.36)	$ (2.75)	$ (9.97)	$ (7.21)
Weighted average common shares outstanding, basic and diluted	21,977,268	18,884,562	21,506,021	17,210,604

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net loss	$ (73,934)	$ (51,910)	$ (214,357)	$ (124,140)
Change in unrealized gain (loss) on marketable securities, net of tax	105	1,402	(154)	1,331
Comprehensive loss	$ (73,829)	$ (50,508)	$ (214,511)	$ (122,809)

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	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
Stock-based compensation expense	—	—	7,770	—	—	7,770
Issuance of common stock from at-the-market offerings, net of commission costs of $579	657,477	—	28,360	—	—	28,360
Issuance of common stock from exercise of pre-funded warrants	26,509	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	9,319	—	294	—	—	294
Vesting of restricted stock units	5,860	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(608)	—	(23)	—	—	(23)
Change in unrealized gain on marketable securities, net of tax	—	—	—	—	(264)	(264)
Net loss	—	—	—	(71,127)	—	(71,127)
Balance at June 30, 2025	21,040,474	$ 14	$ 1,380,978	$ (977,163)	$ 395	$ 404,224
Stock-based compensation expense	—	—	7,447	—	—	7,447
Issuance of common stock from at-the-market offerings, net of issuance and commission costs of $359	102,364	—	4,907	—	—	4,907
Vesting of restricted stock units	3,746	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(1,106)	—	(52)	—	—	(52)
Issuance of common stock upon exercise of stock options	20,455	—	808	—	—	808
Change in unrealized gain on marketable securities, net of tax	—	—	—	—	105	105
Net loss	—	—	—	(73,934)	—	(73,934)
Balance at September 30, 2025	21,165,933	$ 14	$ 1,394,088	$ (1,051,097)	$ 500	$ 343,505

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	8,791,877	$ 13	$ 723,577	$ (653,921)	$ —	$ 69,669
Stock-based compensation expense	—	—	14,475	—	—	14,475
Issuance of common stock from follow-on public offering and accompanying pre-funded warrants, net of underwriting discounts, commissions and offering costs of $10,836	3,802,025	—	161,649	—	—	161,649
Issuance of common stock from at-the-market public offerings, net of issuance and commission costs of $376	209,852	—	6,169	—	—	6,169
Issuance of common stock from a collaboration and license agreement	443,253	—	17,265	—	—	17,265
Vesting of restricted stock units	11,095	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(3,689)	—	(137)	—	—	(137)
Issuance of common stock upon exercise of stock options	3,634	—	143	—	—	143
Change in unrealized gain on marketable securities, net of tax	—	—	—	—	3	3
Net loss	—	—	—	(39,553)	—	(39,553)
Balance at March 31, 2024	13,258,047	$ 13	$ 923,141	$ (693,474)	$ 3	$ 229,683
Stock-based compensation expense	—	—	5,878	—	—	5,878
Issuance of common stock from follow-on public offering and accompanying pre-funded warrants, net of underwriting discounts, commissions and offering costs of $14,013	3,849,558	1	215,987	—	—	215,988
Issuance of common stock from exercise of pre-funded warrants	622,123	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	25,146	—	311	—	—	311
Vesting of restricted stock units	726	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(242)	—	(13)	—	—	(13)
Issuance of common stock upon exercise of stock options	148	—	4	—	—	4
Change in unrealized loss on marketable securities, net of tax	—	—	—	—	(74)	(74)
Net loss	—	—	—	(32,677)	—	(32,677)
Balance at June 30, 2024	17,755,506	$ 14	$ 1,145,308	$ (726,151)	$ (71)	$ 419,100
Stock-based compensation expense	—	—	12,432	—	—	12,432
Issuance of common stock from at-the-market public offerings, net of issuance and commission costs of $70	25,189		1,442			1,442
Issuance of common stock from exercise of stock options	4,971		201			201
Vesting of restricted stock units	51	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(20)	—	(1)	—	—	(1)
Change in unrealized loss on marketable securities, net of tax	—	—	—	—	1,402	1,402
Net loss	—	—	—	(51,910)	—	(51,910)
Balance at September 30, 2024	17,785,697	$ 14	$ 1,159,382	$ (778,061)	$ 1,331	$ 382,666

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

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)

(Amounts in thousands)

	Nine Months Ended September 30,	
	2025	**2024**
Cash flows from operating activities:		
Net loss	$ (214,357)	$ (124,140)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	112	311
Stock-based compensation expense	24,003	32,785
Non-cash operating lease expense	769	690
Amortization of premiums and discounts on marketable securities, net	(2,921)	(3,620)
Non-cash collaboration and license agreement expense	—	2,500
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	5,534	564
Accounts payable	18,291	9,195
Accrued expenses	(3,228)	8,005
Operating lease liabilities	(933)	(835)
Deferred revenue	—	(1,090)
Other	(13)	—
Net cash used in operating activities	(172,743)	(75,635)
Cash flows from investing activities:		
Purchases of property and equipment	(56)	—
Purchases of marketable securities	(233,537)	(302,101)
Maturities of marketable securities	250,836	64,525
Net cash provided by (used in) investing activities	17,243	(237,576)
Cash flows from financing activities:		
Issuance of common stock from follow-on public offering and accompanying pre-funded warrants, net of underwriting discounts, commissions and offering costs	—	377,636
Issuance of common stock from collaboration and license agreement	—	14,765
Proceeds from at-the-market offerings, net of issuance and commission costs	89,263	7,647
Payments of tax withholdings related to vesting of restricted stock units	(1,270)	(151)
Proceeds from exercise of stock options and employee stock purchase plan purchases	1,662	659
Net cash provided by financing activities	89,655	400,556
(Decrease) increase in cash, cash equivalents and restricted cash	(65,845)	87,345
Cash, cash equivalents and restricted cash, beginning of period	215,788	81,716
Cash, cash equivalents and restricted cash, end of period	$ 149,943	$ 169,061
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	149,527	168,645
Restricted cash	416	416
Total cash, cash equivalents and restricted cash	$ 149,943	$ 169,061
Supplemental disclosures of non-cash activities:		
Issuance costs from at-the-market offering included in accrued expenses	$ 221	$ 36

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 clinical-stage biopharmaceutical company translating insights from genetic epilepsies into the development of therapies for central nervous system ("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, or 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 September 30, 2025, the Company raised $1.2 billion in aggregate cash proceeds from these transactions, net of issuance costs.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Programs currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

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 $214.4 million for the nine months ended September 30, 2025. In addition, as of September 30, 2025, the Company had an accumulated deficit of $1.1 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 September 30, 2025 of $389.2 million, together with net proceeds of $567.0 million from the October 2025 financing, will be sufficient to fund its operating expenditures and capital expenditure requirements necessary to advance its program activities for at least one year from the date of issuance of these condensed consolidated financial statements. The future viability of the Company is dependent on its ability to raise additional capital to finance its operations. The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurance that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America (“GAAP”). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (“ASC”) and ASUs of the FASB.

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three and nine months ended September 30, 2025 are consistent with those discussed in Note 2 to the consolidated financial statements included in the Company's 2024 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 September 30, 2025, the condensed consolidated statements of operations for the three and nine months ended September 30, 2025 and 2024, the condensed consolidated statements of comprehensive loss for the three and nine months ended September 30, 2025 and 2024, the condensed consolidated statements of cash flows for the nine months ended September 30, 2025 and 2024 and the condensed consolidated statements of stockholders’ equity for the three and nine months ended September 30, 2025 and 2024 are unaudited. The unaudited interim financial statements have been prepared on the same basis as the audited annual consolidated financial statements, and in the opinion of management reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company’s financial position as of September 30, 2025, the results of its operations for the three and nine months ended September 30, 2025 and 2024 and its cash flows for the nine months ended September 30, 2025 and 2024. Financial statement disclosures for the three and nine months ended September 30, 2025 and 2024 are condensed and do not include all disclosures required for an annual set of financial statements in accordance with GAAP.

The results for the three and nine months ended September 30, 2025 are not necessarily indicative of results to be expected for the year ended December 31, 2025, any other interim periods, or any future year or period.

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.

Recently Enacted Tax Legislation

The One Big Beautiful Bill Act (“OBBBA”) was enacted in the U.S. on July 4, 2025. The OBBBA legislation provides for the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, revisions to the international tax framework and the reinstatement of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented in future

periods. While tax changes in the OBBBA do not have a material impact to the Company's effective tax rate, we anticipate that certain tax provisions in the OBBBA, namely the provision allowing for immediate expensing of certain research and development expenses, will result in accelerated deductions of previously capitalized expenses of approximately $203.9 million. We continue to evaluate the tax and other provisions of the OBBBA and the potential effects on our financial position, results of operations, and cash flows.

Recent Accounting Pronouncements

Recently Issued Accounting Pronouncements

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740) - Improvements to Income Tax Disclosures*. ASU No. 2023-09 provides more transparency about income tax information through improvements to income tax disclosures primarily related to the rate reconciliation and income taxes paid information. For public companies, the amendments are effective for annual periods beginning after December 15, 2024 and can be applied prospectively or retrospectively. The Company has determined that the effects of adopting the amendments in ASU 2023-09 will only impact its disclosures and will not have a material impact on its consolidated financial position and the results of its operations when such amendment is adopted.

In November 2023, the FASB issued ASU No. 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*, 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.

3. Cash Equivalents and Marketable Securities

The following is a summary of the Company's investment portfolio as of September 30, 2025 and December 31, 2024 (in thousands):

	As of September 30, 2025			
	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 76,033	$ —	$ —	$ 76,033
Available-for-sale securities:				
Debt securities issued by U.S. government agencies	98,033	204	—	98,237
Corporate debt securities	99,583	240	—	99,823
Commercial paper	1,928	1	—	1,929
Other debt securities	39,594	55	—	39,649
Total cash equivalents and marketable securities	$ 315,171	$ 500	$ —	$ 315,671

	As of December 31, 2024			
	Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 128,652	$ —	$ —	$ 128,652
Debt securities issued by U.S. government agencies	15,953	2	—	15,955
Available-for-sale securities:				
Debt securities issued by U.S. government agencies	128,215	475	—	128,690
Corporate debt securities	107,695	157	—	107,852
Commercial paper	8,775	19	—	8,794
Other debt securities	8,819	1	—	8,820
Total cash equivalents and marketable securities	$ 398,109	$ 654	$ —	$ 398,763

As of September 30, 2025, the Company had 4 securities with a total fair market value of $16.1 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):

	September 30, 2025	December 31, 2024
Within one year	$ 117,630	$ 177,195
After one year through five years	122,008	76,961
Total	$ 239,638	$ 254,156

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 and nine months ended September 30, 2025 and 2024.

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 September 30, 2025 and December 31, 2024 (in thousands):

	As of September 30, 2025			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$ 76,033	$ —	$ —	$ 76,033
Debt securities issued by US. government agencies	98,237	—	—	98,237
Corporate debt securities	—	99,823	—	99,823
Commercial paper	—	1,929	—	1,929
Other debt securities	—	39,649	—	39,649
	$ 174,270	$ 141,401	$ —	$ 315,671

	As of December 31, 2024			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market funds	$ 128,652	$ —	$ —	$ 128,652
Debt securities issued by US. government agencies	144,644	—	—	144,644
Corporate debt securities	—	107,852	—	107,852
Commercial paper	—	8,794	—	8,794
Other debt securities	—	8,821	—	8,821
	$ 273,296	$ 125,467	$ —	$ 398,763

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Accrued external research and development expenses	$ 15,624	$ 13,363
Accrued personnel-related expenses	3,683	9,365
Accrued other expenses	2,321	1,035
Total accrued expenses	$ 21,628	$ 23,763

6. Commitments and Contingencies

In May 2021, the Company entered into a sublease agreement for office space located in Boston, Massachusetts that expires on January 31, 2026, with no option to renew or terminate early. The base rent increases by approximately 2% annually. 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 and other non-current assets on the accompanying condensed consolidated balance sheets as of September 30, 2025 and December 31, 2024, respectively. This lease 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.

The Company did not recognize any collaboration revenue associated with this Collaboration Agreement during the three and nine months ended September 30, 2025. As of September 30, 2025, there was no deferred revenue recorded in the condensed consolidated balance sheet. During the three and nine months ended September 30, 2024, the Company recognized $0.3 million and $1.1 million, respectively, related to the Collaboration Agreement in the condensed consolidated statement of operations. As of December 31, 2024, there was no deferred revenue recorded in the consolidated balance sheet.

Tenacia Collaboration and License Agreement

On January 4, 2024, the Company entered into an exclusive collaboration and license Agreement ("the 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 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 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 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 License Agreement, both of which were received in January 2024. During the three and nine months ended September 30, 2025 and 2024, the Company did not achieve any development or sales milestones or earn any royalties under the 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 collaboration and 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 September 30, 2025 and December 31, 2024, 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 September 30, 2025 because no qualifying costs were incurred. During the three and nine months ended September 30, 2025 and 2024, the Company did not recognize any revenue associated with the combined arrangement.

8. Common Stock and Preferred Stock

Common Stock

As of September 30, 2025 and December 31, 2024, the authorized capital stock of the Company included 150,000,000 shares of common stock, $0.0001 par value.

As of September 30, 2025 and December 31, 2024, 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, 2024, 152,145 pre-funded warrants were exercised via a cashless exercise, resulting in 152,142 shares of common stock issued. No cash proceeds associated with the exercise were received by the Company.

During the nine months ended September 30, 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 September 30, 2025, 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 September 30, 2025, none of the pre-funded warrants have been exercised and all remained outstanding.

The Company determined that the pre-funded warrants related to the January 2024 and April 2024 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.

Shares Reserved for Future Issuance

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

	September 30, 2025	December 31, 2024
Shares reserved for exercise of outstanding stock options	2,070,371	1,809,343
Shares reserved for exercise of pre-funded warrants related to the January 2024 Financing	704,225	904,580
Shares reserved for exercise of pre-funded warrants related to the April 2024 Financing	221,238	221,238
Shares reserved for future awards under the 2020 Stock Option and Incentive Plan	462,188	91,343
Shares reserved for future awards under the 2020 Employee Stock Purchase Plan	294,552	109,648
Shares reserved for future awards under the 2024 Inducement Plan	950,600	112,774
Shares reserved for vesting of restricted stock units	516,119	224,473
Total shares of authorized common stock reserved for future issuance	5,219,293	3,473,399

Preferred Stock

As of September 30, 2025 and December 31, 2024, 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 September 30, 2025 and December 31, 2024 was 1,000,000 shares and 130,000 shares, respectively.

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 September 30, 2025 and December 31, 2024 was 2,941,950 shares and 1,970,833 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 September 30, 2025 and December 31, 2024 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 September 30, 2025 and December 31, 2024 was 369,368 shares and 175,145 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, 2024	224,473	$ 71.71
Issued	387,431	61.51
Vested	(65,339)	99.63
Forfeited	(30,446)	59.46
Unvested as of September 30, 2025	516,119	$ 61.24

As of September 30, 2025, total unrecognized compensation cost related to unvested restricted stock units was $25.0 million, which is expected to be recognized over a weighted-average period of 3.09 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, 2024	1,809,343	$ 90.84		
Granted	324,670	60.03		
Exercised	(34,877)	39.21		$ 738
Cancelled or Forfeited	(28,765)	67.76		
Outstanding as of September 30, 2025	2,070,371	$ 87.20	7.94	$ 8,662
Exercisable as of September 30, 2025	1,197,365	$ 108.73	7.28	$ 6,571
Vested and expected to vest as of September 30, 2025	2,070,371	$ 87.20	7.94	$ 8,662

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 nine months ended September 30, 2025. No stock options were granted during the three months ended September 30, 2025.

	Nine Months Ended September 30,
	2025
Risk-free interest rate	4.45 %
Expected term (in years)	6.00
Expected volatility	93.08 %
Expected dividend yield	— %
Weighted average grant-date fair value per share	$ 46.74

As of September 30, 2025, total unrecognized compensation cost related to unvested stock options was $36.4 million, which is expected to be recognized over a weighted-average period of 2.24 years.

Stock-Based Compensation

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 2,959	$ 4,640	$ 9,969	$ 12,298
General and administrative	4,488	7,792	14,034	20,487
Total stock-based compensation expense	$ 7,447	$ 12,432	$ 24,003	$ 32,785

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Outstanding stock options	2,070,371	1,829,324	2,070,371	1,829,324
Unvested restricted stock units	516,119	222,674	516,119	222,674
Potential shares issuable under the 2020 ESPP	13,795	7,391	13,795	7,391
	2,600,285	2,059,389	2,600,285	2,059,389

Common shares issuable upon exercise of the pre-funded warrants that were sold in connection with the January 2024 and April 2024 underwritten public offerings are included in the calculation of basic weighted average number of common shares outstanding for the three and nine months ended September 30, 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 operating segment's revenue is derived from a single domestic collaboration agreement from which the segment licenses certain development or product candidates and has performed research and development services. 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 and nine months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Collaboration revenue	$ —	$ 302	$ —	$ 1,090
Program-specific expenses:				
Ulixacaltamide	11,644	19,976	53,542	43,160
Vormatrigine	20,899	6,613	52,589	9,205
Relutrigine	14,965	1,263	30,851	3,514
Elsunersen	2,219	380	4,297	1,586
Other early stage assets	3,142	1,179	5,664	3,242
Personnel-related expenses	12,116	9,472	37,059	27,582
Stock-based compensation expense	7,447	12,432	24,003	32,786
Depreciation expense	19	92	112	311
Other segment expenses[(a)]	5,908	5,730	21,037	15,913
Interest income	4,425	4,925	14,797	12,069
Net loss	$ (73,934)	$ (51,910)	$ (214,357)	$ (124,140)

(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 consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. The Company has concluded that no subsequent events have occurred that require disclosure other than those noted below.

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 $567.0 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

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, which was filed with the Securities and Exchange Commission, or the SEC, on February 28, 2025. 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" sections of our Annual Report on Form 10-K for the year ended December 31, 2024 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 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 clinical-stage biopharmaceutical company translating insights from genetic epilepsies into the development of therapies for central nervous system, or 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, we announced positive topline results for the two studies in the Phase 3 Essential3 program in essential tremor in October 2025. Following the FDA's receipt of the Essential3 topline results, the FDA granted a Type B pre-NDA meeting that is scheduled to take place in the fourth quarter of 2025.

For our vormatrigine program (formerly known as PRAX-628) within the Cerebrum™ platform, we announced positive results from our Photo-Paroxysmal Response, or PPR, study in the first quarter of 2024, and initiated the ENERGY program to generate patient eligibility, efficacy, safety and pharmacokinetics data. The ENERGY program includes five studies: EMPOWER, RADIANT, POWER1, POWER2 and POWER3. We initiated the EMPOWER study, an observational study of vormatrigine in patients with epilepsy, in the third quarter of 2024. In August 2025, we announced positive topline results from the first cohort of our first efficacy study, RADIANT, an open label study in patients with focal onset seizures or generalized epilepsy. We plan to share additional RADIANT study results at the American Epilepsy Society Annual Meeting in December 2025. We have completed recruitment of the POWER1 study, a double-blind, placebo-controlled, 12-week study in focal onset seizures, and expect to announce topline results in the first half of 2026. POWER2, our third efficacy study, has started, with enrollment expected to be completed in the second half of 2026. We intend to initiate POWER3, a clinical trial evaluating vormatrigine as a single-agent treatment for focal onset seizures, in the first half of 2026.

For our relutrigine program (formerly known as PRAX-562), we announced positive topline results from the first cohort of our EMBOLD study in the third quarter of 2024, and disclosed updated data from the study's open-label extension through 11 months in May 2025. In July 2025, the FDA granted breakthrough therapy designation for relutrigine for the treatment of seizures associated with SCN2A and SCN8A developmental and epileptic encephalopathies, or DEEs. Following a comprehensive Type B meeting with the FDA, we will be performing an interim analysis of the second cohort of the ongoing EMBOLD study in the fourth quarter of 2025. If positive, these

study results may support an NDA filing in early 2026. We have also started enrollment of the EMERALD study that is investigating relutrigine in a broader DEE patient population, and expect to be completed in the second half of 2026.

For our most advanced product candidate under the Solidus™ platform, elsunersen (formerly known as PRAX-222), we shared results from Part 1 of the EMBRAVE study in the fourth quarter of 2023. We are currently enrolling the second cohort of the EMBRAVE study in Brazil, with topline results expected in the first half of 2026. We have initiated EMBRAVE3, a Phase 3 registrational study for SCN2A gain-of-function DEE, and expect to complete in 2026.

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 generic 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, and do not expect to do so for several years, if at all. All of our product candidates are still in preclinical and clinical development. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our product candidates, if approved. We have incurred recurring operating losses since inception, including a net loss of $214.4 million for the nine months ended September 30, 2025. As of September 30, 2025, we had an accumulated deficit of $1.1 billion. We expect to incur significant expenses and operating losses for the foreseeable future as we expand our research and development activities. In addition, our losses from operations may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities. We anticipate that our expenses will be maintained or increased in connection with our ongoing activities, as we:

- prepare for potential commercialization of ulixacaltamide, following our positive Phase 3 results in the Essential3 clinical trial program for ET;
- advance vormatrigine in the ENERGY program;
- advance relutrigine in the EMBOLD and EMERALD clinical trials;
- advance elsunersen in the EMBRAVE and EMBRAVE3 clinical trials;
- advance our preclinical candidates to clinical trials;
- further invest in our pipeline;
- further invest in our manufacturing capabilities;
- seek regulatory approval for our product candidates;
- maintain, expand, protect and defend our IP portfolio;
- acquire or in-license technology;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- when needed, increase our headcount to support our development efforts and any future commercialization efforts.

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

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

As of September 30, 2025, we had cash, cash equivalents and marketable securities of $389.2 million. We expect that our cash, cash equivalents, and marketable securities as of September 30, 2025, together with proceeds from our follow-on pubic offering in October 2025, will be sufficient to fund our operating expenditures and capital expenditure requirements necessary to advance our program activities into 2028. The analysis included consideration of our current financial needs and ongoing research, development and commercialization plans. We have based this estimate on assumptions that may provide 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 do not expect to generate any revenue from the sale of products for several years, if at all. As discussed in Note 7 to our condensed consolidated financial statements, we entered into an Option and License Agreement, or the Collaboration Agreement, with UCB Biopharma SRL, or UCB, in December 2022. During the three and nine months ended September 30, 2024, we recognized $0.3 million and $1.1 million of collaboration revenue, respectively. In December 2024, UCB exercised its option to in-license global development and commercialization rights under the terms of the Collaboration Agreement. Accordingly, we did not recognize any collaboration revenue from the Collaboration Agreement during the three and nine months ended September 30, 2025. We have no further research service obligations under the terms of the Collaboration Agreement.

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 each period presented (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Cerebrum™	$ 49,182	$ 27,474	$ 138,991	$ 55,226
Solidus™	3,244	1,327	6,571	4,197
Personnel-related (including stock-based compensation)	11,069	10,863	35,379	30,265
Other indirect research and development expenses	2,302	2,217	8,668	6,437
Total research and development expenses	$ 65,797	$ 41,881	$ 189,609	$ 96,125

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 and expand into additional therapeutic areas.

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

- our ability to add and retain key research and development personnel;
- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to successfully complete clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize, our product candidates;

- our successful enrollment in and completion of clinical trials;
- the costs associated with the development of any additional product candidates we identify in-house or acquire through collaborations;
- our ability to discover, develop and utilize biomarkers to demonstrate target engagement, pathway engagement and the impact on disease progression of our product candidates;
- our ability to establish and maintain agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if our product candidates are approved;
- the terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder;
- our ability to obtain and maintain patent, trade secret and other IP protection and regulatory exclusivity for our product candidates, if approved;
- our receipt of marketing approvals from applicable regulatory authorities;
- our ability to commercialize products, if approved, whether alone or in collaboration with others; 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 to support market assessments and scenario planning; 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 and nine months ended September 30, 2025 and 2024.

Results of Operations

Comparison of the Three Months Ended September 30, 2025 and 2024

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

	Three Months Ended September 30,		Change
	2025	2024	
Collaboration revenue	$ —	$ 302	$ (302)
Operating expenses:			
Research and development	65,797	41,881	23,916
General and administrative	12,562	15,256	(2,694)
Total operating expenses	78,359	57,137	21,222
Loss from operations	(78,359)	(56,835)	(21,524)
Other income:			
Other income, net	4,425	4,925	(500)
Total other income	4,425	4,925	(500)
Net loss	$ (73,934)	$ (51,910)	$ (22,024)

Collaboration Revenue

The $0.3 million decrease in collaboration revenue is associated with a decrease in the revenue recorded under the Collaboration Agreement with UCB that was executed in December 2022. In December 2024, UCB exercised its option to in-license global development and commercialization rights for a development candidate as part of the Collaboration Agreement. As such, we have no further research service obligations under the terms of the Collaboration Agreement and did not record any associated revenue during the three months ended September 30, 2025.

Research and Development Expense

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

	Three Months Ended September 30,		Change
	2025	2024	
Cerebrum™	$ 49,182	$ 27,474	$ 21,708
Solidus™	3,244	1,327	1,917
Personnel-related (including stock-based compensation)	11,069	10,863	206
Other indirect research and development expenses	2,302	2,217	85
Total research and development expenses	$ 65,797	$ 41,881	$ 23,916

The $23.9 million increase in research and development expenses was primarily attributable to the following:

- $21.7 million increase in expense related to our Cerebrum™ platform, driven primarily by:
  - a $14.6 million increase for our vormatrigine program, primarily driven by POWER1 and RADIANT study activities;
  - a $13.4 million increase in spend for our relutrigine program, primarily related to EMBOLD and EMERALD study activities and manufacturing spend;
  - a $1.9 million increase in development activities for our earlier stage assets as we advance our portfolio, partially offset by;
  - a $8.2 million decrease in spend for our ulixacaltamide program, primarily due to decreased activity for Essential3 program and phase 1 spending.

- $1.9 million increase in expense related to our Solidus™ platform, driven primarily by elsunersen program spend for EMBRAVE3; and
- $0.3 million increase in personnel-related costs and indirect expenses.

General and Administrative Expense

The $2.7 million decrease in general and administrative expenses was primarily attributable to the following:

- $2.6 million decrease in personnel-related costs due to decreased stock compensation expense;
- $0.6 million decrease in indirect expenses; and
- $0.5 million increase in professional expenses.

Other Income

Other income for the three months ended September 30, 2025 and 2024 was comprised of interest income on our cash, cash equivalents and marketable securities and investment premium and discount amortization.

Results of Operations

Comparison of the Nine Months Ended September 30, 2025 and 2024

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

	Nine Months Ended September 30,		Change
	2025	2024	
Collaboration revenue	$ —	$ 1,090	$ (1,090)
Operating expenses:			
Research and development	189,609	96,125	93,484
General and administrative	39,545	41,174	(1,629)
Total operating expenses	229,154	137,299	91,855
Loss from operations	(229,154)	(136,209)	(92,945)
Other income:			
Other income, net	14,797	12,069	2,728
Total other income	14,797	12,069	2,728
Net loss	$ (214,357)	$ (124,140)	$ (90,217)

Collaboration Revenue

The $1.1 million decrease in collaboration revenue is associated with a decrease in the revenue recorded under the Collaboration Agreement with UCB that was executed in December 2022. In December 2024, UCB exercised its option to in-license global development and commercialization rights for a development candidate as part of the Collaboration Agreement. As such, we have no further research service obligations under the terms of the Collaboration Agreement and did not record any associated revenue during the nine months ended September 30, 2025.

Research and Development Expense

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

	Nine Months Ended September 30,		Change
	2025	2024	
Cerebrum™	$ 138,991	$ 55,226	$ 83,765
Solidus™	6,571	4,197	2,374
Personnel-related (including stock-based compensation)	35,379	30,265	5,114
Other indirect research and development expenses	8,668	6,437	2,231
Total research and development expenses	$ 189,609	$ 96,125	$ 93,484

The $93.5 million increase in research and development expenses was primarily attributable to the following:

- $83.8 million increase in expense related to our Cerebrum™ platform, driven primarily by:
  - a $43.5 million increase in spend for our vormatrigine program, primarily driven by POWER1 and RADIANT study spend as well as phase 1 study activities;
  - a $27.0 million increase in spend for our relutrigine program, primarily related to our EMBOLD and EMERALD trial activities, manufacturing spend and Phase 1 trial spending;
  - a $10.5 million increase in spend for our ulixacaltamide program, primarily due to Essential3 study activity; and
  - a $2.8 million increase in activities for our earlier stage assets as we advance our portfolio.
- $5.1 million increase in personnel-related costs due to increased headcount;
- $2.4 million increase in expense related to our Solidus™ platform, driven by elsunersen program spend; and
- $2.2 million increase in indirect expenses driven primarily by software and consulting spend to support operations.

General and Administrative Expense

The $1.6 million decrease in general and administrative expenses was primarily attributable to the following:

- $3.8 million decrease in personnel-related costs mainly due to decreased stock-based compensation expense;
- $1.3 million increase in professional expenses primarily related to legal and audit expenses; and
- $0.9 million increase in indirect expenses, including increased sponsorships and advocacy activities.

Other Income

Other income for the nine months ended September 30, 2025 and 2024 was comprised of interest income on our cash, cash equivalents, and marketable securities and investment premium and discount amortization.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have incurred significant losses in each period. We have not yet commercialized any of our product candidates, which are in various phases of preclinical and clinical development, and we may not generate revenue from sales of any products for several years, 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 September 30, 2025, we have raised $1.2 billion in aggregate cash

proceeds from such transactions, net of issuance costs. As of September 30, 2025, we had cash, cash equivalents and marketable securities of $389.2 million.

In December 2023, we entered into an Open Market Sale Agreement, or the 2023 Sales Agreement, with Jefferies, to provide for the offering, issuance and sale of up to an aggregate amount of $75.0 million of common stock from time to time in at-the-market offerings. The 2023 Sales Agreement was terminated in January 2024. During the nine months ended September 30, 2024, we issued and sold an aggregate of 192,190 shares under the 2023 Sales Agreement for aggregate net proceeds of $5.3 million, after deducting commissions and offering expenses payable by us.

In January 2024, we completed a public offering of: (i) an aggregate of 3,802,025 shares of our 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 us. As of September 30, 2025, 352,500 warrants associated with this offering were exercised on a cashless basis with no cash proceeds received by us.

In March 2024, we entered into an Open Market Sale Agreement, or the 2024 Sales Agreement, with 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. During the nine months ended September 30, 2024, we issued and sold an aggregate of 42,851 shares under the 2024 Sales Agreement for aggregate net proceeds of $2.3 million, after deducting commissions and offering expenses payable by us.

In April 2024, we completed a public offering of: (i) an aggregate of 3,849,558 shares of our 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 us. As of September 30, 2025, none of the pre-funded warrants have been exercised and all remained outstanding.

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 of $250.0 million of common stock from time to time in at-the-market offerings. During the nine months ended September 30, 2025, we issued and sold an aggregate of 1,351,689 shares under the amended 2024 Sales Agreement for aggregate net proceeds of $83.3 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 nine months ended September 30, 2025, we issued and sold an aggregate of 119,364 shares under the 2025 Sales Agreement for aggregate net proceeds of $5.8 million, after deducting commissions and offering expenses payable by us.

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 approximately $567.0 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):

	Nine Months Ended September 30,	
	2025	2024
Net cash (used in) provided by:		
Operating activities	$ (172,743)	$ (75,635)
Investing activities	17,243	(237,576)
Financing activities	89,655	400,556
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (65,845)	$ 87,345

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 our 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, which are primarily the result of increased expenses and timing of vendor payments.

During the nine months ended September 30, 2025, net cash used in operating activities of $172.7 million was primarily due to our $214.4 million net loss, partially offset by $19.7 million in changes in operating assets and liabilities primarily related to an increase in accounts payable and $22.0 million of non-cash charges primarily related to stock-based compensation.

During the nine months ended September 30, 2024, net cash used in operating activities of $75.6 million was primarily due to our $124.1 million net loss, partially offset by $15.8 million in changes in operating assets and liabilities primarily related to increases in accounts payable and accrued expenses, and $32.7 million of non-cash charges primarily related to stock-based compensation.

Investing Activities

During the nine months ended September 30, 2025, net cash provided by investing activities of $17.2 million was primarily related to maturities of marketable securities offset by purchases of marketable securities.

During the nine months ended September 30, 2024, net cash used in investing activities of $237.6 million was primarily related to purchases of marketable securities partially offset by maturities of marketable securities.

Financing Activities

During the nine months ended September 30, 2025, net cash provided by financing activities of $89.7 million consisted primarily of net proceeds from our at-the-market offerings.

During the nine months ended September 30, 2024, net cash provided by financing activities of $400.6 million consisted primarily of net proceeds from our January 2024 and April 2024 follow-on public offerings, our at-the-market offerings and our collaboration and license agreement with Tenacia.

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. As a result, we expect to incur substantial operating losses and negative operating cash flows for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- advance the clinical development of our 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;
- establish a sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize any products for which we may obtain regulatory approval;
- when needed, add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and potential future commercialization efforts and to support our 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 September 30, 2025, together with proceeds from our follow-on public offering in October 2025, will be sufficient to fund our operating expenses 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. Our commercial revenues, if any, will be derived

from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

Adequate additional funds may not be available to us on acceptable terms, or at all. We do not currently have any committed external source of funds. Market volatility 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 28, 2025, 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 September 30, 2025. Based on the evaluation of our disclosure controls and procedures as of September 30, 2025, 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, 2024 filed with the SEC on February 28, 2025 and in the section entitled "Risk Factors" of our Quarterly Report on Form 10-Q for the quarter ended June 30, 2025 filed with the SEC on August 4, 2025.

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 September 30, 2025.

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 September 30, 2025, 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.

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
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: November 5, 2025

By: /s/ Marcio Souza

Marcio Souza

Chief Executive Officer and Director (Principal Executive Officer)

Date: November 5, 2025

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: November 5, 2025

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: November 5, 2025

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 September 30, 2025 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: November 5, 2025

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

Date: November 5, 2025

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