

**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, 2024

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

(State or other jurisdiction of incorporation or organization)

47-5195942

(I.R.S. Employer Identification No.)

99 High Street, 30th Floor

Boston, MA

(Address of principal executive offices)

02110

(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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

As of November 1, 2024, the registrant had 18,637,777 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 section titled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2023. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking

statement is a guarantee of future performance. You should read this Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed with the Securities and Exchange Commission as exhibits hereto completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this Quarterly Report on Form 10-Q represent our views as of the date of this Quarterly Report on Form 10-Q. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should therefore not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Quarterly Report on Form 10-Q.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for our product candidates. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from our own internal estimates and research as well as from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. While we are not aware of any misstatements regarding any third-party information presented in this Quarterly Report on Form 10-Q, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties and are subject to change based on various factors, including those discussed under the section titled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2023.

Table of Contents

	<u>Page</u>
PART I.	<u>FINANCIAL INFORMATION</u>
Item 1.	<u>Financial Statements (Unaudited)</u>
	<u>Condensed Consolidated Balance Sheets as of September 30, 2024 and December 31, 2023</u> 1
	<u>Condensed Consolidated Statements of Operations for the Three and Nine Months Ended September 30, 2024 and 2023</u> 2
	<u>Condensed Consolidated Statements of Comprehensive Loss for the Three and Nine Months Ended September 30, 2024 and 2023</u> 3
	<u>Condensed Consolidated Statements of Stockholders' Equity for the Three and Nine Months Ended September 30, 2024 and 2023</u> 4
	<u>Condensed Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2024 and 2023</u> 6
	<u>Notes to Condensed Consolidated Financial Statements</u> 7
Item 2.	<u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u> 18
Item 3.	<u>Quantitative and Qualitative Disclosures About Market Risk</u> 29
Item 4.	<u>Controls and Procedures</u> 29
PART II.	<u>OTHER INFORMATION</u>
Item 1.	<u>Legal Proceedings</u> 31
Item 1A.	<u>Risk Factors</u> 31
Item 2.	<u>Unregistered Sales of Equity Securities and Use of Proceeds</u> 31
Item 3.	<u>Defaults Upon Senior Securities</u> 31
Item 4.	<u>Mine Safety Disclosures</u> 31
Item 5.	<u>Other Information</u> 31
Item 6.	<u>Exhibits</u> 32
	<u>Signatures</u> 33

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, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	\$ 168,645	\$ 81,300
Marketable securities	188,387	—
Prepaid expenses and other current assets	3,016	3,580
Total current assets	360,048	84,880
Long-term marketable securities	54,141	—
Property and equipment, net	277	588
Operating lease right-of-use assets	1,374	2,064
Other non-current assets	416	416
Total assets	<u>\$ 416,256</u>	<u>\$ 87,948</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 15,010	\$ 5,815
Accrued expenses	15,457	7,416
Operating lease liabilities	1,224	1,126
Current portion of deferred revenue	1,170	1,392
Total current liabilities	32,861	15,749
Long-term liabilities:		
Non-current portion of operating lease liabilities	436	1,369
Non-current portion of deferred revenue	293	1,161
Total liabilities	33,590	18,279
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, 2024 and December 31, 2023	—	—
Common stock, \$0.0001 par value; 150,000,000 shares authorized; 17,785,697 shares issued and outstanding as of September 30, 2024, and 8,791,877 shares issued and outstanding as of December 31, 2023	14	13
Additional paid-in capital	1,159,382	723,577
Accumulated other comprehensive gain	1,331	—
Accumulated deficit	(778,061)	(653,921)
Total stockholders' equity	382,666	69,669
Total liabilities and stockholders' equity	<u>\$ 416,256</u>	<u>\$ 87,948</u>

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,	
	2024	2023	2024	2023
Collaboration revenue	\$ 302	\$ 468	\$ 1,090	\$ 1,932
Operating expenses:				
Research and development	41,881	17,260	96,125	68,378
General and administrative	15,256	8,724	41,174	32,121
Total operating expenses	<u>57,137</u>	<u>25,984</u>	<u>137,299</u>	<u>100,499</u>
Loss from operations	(56,835)	(25,516)	(136,209)	(98,567)
Other income:				
Other income, net	4,925	884	12,069	2,168
Total other income	<u>4,925</u>	<u>884</u>	<u>12,069</u>	<u>2,168</u>
Net loss	<u>\$ (51,910)</u>	<u>\$ (24,632)</u>	<u>\$ (124,140)</u>	<u>\$ (96,399)</u>
Net loss per share attributable to common stockholders, basic and diluted ¹	<u>\$ (2.75)</u>	<u>\$ (2.72)</u>	<u>\$ (7.21)</u>	<u>\$ (16.73)</u>
Weighted average common shares outstanding, basic and diluted ¹	<u>18,884,562</u>	<u>9,039,427</u>	<u>17,210,604</u>	<u>5,763,121</u>

¹ Results have been retroactively adjusted to reflect the 1-for-15 reverse stock split effected on November 28, 2023. See Note 2 for details.

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,	
	2024	2023	2024	2023
Net loss	\$ (51,910)	\$ (24,632)	\$ (124,140)	\$ (96,399)
Change in unrealized gain on marketable securities, net of tax	1,402	—	1,331	173
Comprehensive loss	\$ (50,508)	\$ (24,632)	\$ (122,809)	\$ (96,226)

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 Income (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	<u>13,258,047</u>	<u>\$ 13</u>	<u>\$ 923,141</u>	<u>\$ (693,474)</u>	<u>\$ 3</u>	<u>\$ 229,683</u>
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	<u>17,755,506</u>	<u>\$ 14</u>	<u>\$ 1,145,308</u>	<u>\$ (726,151)</u>	<u>\$ (71)</u>	<u>\$ 419,100</u>
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	<u>17,785,697</u>	<u>\$ 14</u>	<u>\$ 1,159,382</u>	<u>\$ (778,061)</u>	<u>\$ 1,331</u>	<u>\$ 382,666</u>

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

PRAXIS PRECISION MEDICINES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (continued)
(Unaudited)
(Amounts in thousands, except share data)

	Common Stock ¹		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2022	3,292,163	\$ 5	\$ 606,918	\$ (530,644)	\$ (173)	\$ 76,106
Stock-based compensation expense	—	—	7,593	—	—	7,593
Issuance of common stock from at-the-market public offerings, net of commission costs of \$560	560,253	1	18,095	—	—	18,096
Vesting of restricted stock units	11,516	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(2,875)	—	(127)	—	—	(127)
Issuance of common stock upon exercise of stock options	2,970	—	101	—	—	101
Change in unrealized loss on marketable securities, net of tax	—	—	—	—	154	154
Net loss	—	—	—	(37,455)	—	(37,455)
Balance at March 31, 2023	<u>3,864,027</u>	<u>\$ 6</u>	<u>\$ 632,580</u>	<u>\$ (568,099)</u>	<u>\$ (19)</u>	<u>\$ 64,468</u>
Stock-based compensation expense	—	—	5,775	—	—	5,775
Issuance of common stock from follow-on public offering and accompanying pre-funded warrants, net of underwriting discounts, commissions and offering costs of \$4,484	4,296,646	6	63,433	—	—	63,439
Issuance of common stock from at-the-market public offerings, net of issuance costs	392,541	1	6,031	—	—	6,032
Issuance of common stock under employee stock purchase plan	15,663	—	208	—	—	208
Vesting of restricted stock units	720	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(249)	—	(4)	—	—	(4)
Change in unrealized loss on marketable securities, net of tax	—	—	—	—	19	19
Net loss	—	—	—	(34,312)	—	(34,312)
Balance at June 30, 2023	<u>8,569,348</u>	<u>\$ 13</u>	<u>\$ 708,023</u>	<u>\$ (602,411)</u>	<u>\$ —</u>	<u>\$ 105,625</u>
Stock-based compensation expense	—	—	5,763	—	—	5,763
Vesting of restricted stock units	233	—	—	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(70)	—	(1)	—	—	(1)
Issuance of common stock upon exercise of stock options	311	—	1	—	—	1
Net loss	—	—	—	(24,632)	—	(24,632)
Balance at September 30, 2023	<u>8,569,822</u>	<u>\$ 13</u>	<u>\$ 713,786</u>	<u>\$ (627,043)</u>	<u>\$ —</u>	<u>\$ 86,756</u>

¹ Results have been retroactively adjusted to reflect the 1-for-15 reverse stock split effected on November 28, 2023. See Note 2 for details.

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,	
	2024	2023
Cash flows from operating activities:		
Net loss	\$ (124,140)	\$ (96,399)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	311	321
Stock-based compensation expense	32,785	19,131
Non-cash operating lease expense	690	619
Amortization of premiums and discounts on marketable securities, net	(3,620)	47
Non-cash collaboration and license agreement expense	2,500	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	564	8,109
Accounts payable	9,195	(7,556)
Accrued expenses	8,005	(8,871)
Operating lease liabilities	(835)	(744)
Deferred revenue	(1,090)	(1,932)
Net cash used in operating activities	<u>(75,635)</u>	<u>(87,275)</u>
Cash flows from investing activities:		
Purchases of marketable securities	(302,101)	—
Maturities of marketable securities	64,525	39,000
Net cash (used in) provided by investing activities	<u>(237,576)</u>	<u>39,000</u>
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	63,439
Issuance of common stock from collaboration and license agreement	14,765	—
Proceeds from at-the-market offerings, net of issuance and commission costs	7,647	24,128
Payments of tax withholdings related to vesting of restricted stock units	(151)	(132)
Proceeds from exercise of stock options and employee stock purchase plan purchases	659	310
Net cash provided by financing activities	<u>400,556</u>	<u>87,745</u>
Increase in cash, cash equivalents and restricted cash	87,345	39,470
Cash, cash equivalents and restricted cash, beginning of period	81,716	62,031
Cash, cash equivalents and restricted cash, end of period	<u>\$ 169,061</u>	<u>\$ 101,501</u>
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	168,645	101,085
Restricted cash	416	416
Total cash, cash equivalents and restricted cash	<u>\$ 169,061</u>	<u>\$ 101,501</u>
Supplemental disclosures of non-cash activities:		
Issuance costs from at-the-market offering included in accrued expenses	\$ 36	\$ —
Offering costs from follow-on offering included in accounts payable	\$ —	\$ 184
Purchases of property and equipment included in accounts payable	\$ —	\$ 50

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

PRAXIS PRECISION MEDICINES, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1. Nature of the Business

Praxis Precision Medicines, Inc. ("Praxis" or the "Company") is a clinical-stage biopharmaceutical company translating 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. For the Company's most advanced product candidate under the Cerebrum™ platform, ulixacaltamide, our Phase 3 Essential3 clinical trials in essential tremor are ongoing. The Company is planning to conduct an interim analysis in the first quarter of 2025. Within the Company's vortrigine program (formerly known as PRAX-628), it announced positive results from its Photo-Paroxysmal Response ("PPR") study in the first quarter of 2024, and the Company has initiated or plans to initiate four studies to generate patient eligibility, efficacy, safety and pharmacokinetics (PK) data for the program. The Company initiated an observational study of vortrigine in patients with epilepsy in the third quarter of 2024, and has initiated or plans to initiate three efficacy studies. The first efficacy study will be an open label eight-week study in patients with focal onset seizures or generalized epilepsy expected to be initiated in the fourth quarter of 2024, with topline results expected in the first half of 2025. The Company also initiated a double-blind, placebo-controlled, 12-week study in focal onset seizures in the fourth quarter of 2024, with topline results expected in the second half of 2025, and plans to initiate a third efficacy study in the first half of 2025. Within the Company's relutrigine (formerly known as PRAX-562) program, it announced positive topline results from the first cohort of the EMBOLD study in the third quarter of 2024, and has initiated enrollment of the second cohort, with topline results expected in the first half of 2026. For the Company's most advanced product candidate under the Solidus™ platform, elsunersen (formerly known as PRAX-222), it shared results from Part 1 of the EMBRAVE study in the fourth quarter of 2023, and has initiated the first arm of the global confirmatory study in Brazil. It is currently completing multiple global regulatory interactions to further expand the pivotal phase of the program later in 2024.

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 and at-the-market offerings under its shelf registration statement, and from follow-on public offerings of common stock and pre-funded warrants to purchase common stock. From inception through September 30, 2024, the Company raised \$1.0 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 \$124.1 million for the nine months ended September 30, 2024. In addition, as of September 30, 2024, the Company had an accumulated deficit of \$778.1 million. 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, 2024 of \$411.2 million will be sufficient to fund its operating expenditures and capital expenditure requirements necessary to advance its research efforts and clinical trials for at least one year from the date of issuance of these condensed consolidated financial statements. The future viability of the Company is dependent on its ability to 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, 2024 are consistent with those discussed in Note 2 to the consolidated financial statements included in the Company's 2023 Annual Report on Form 10-K, other than as noted below.

Reverse Stock Split

On November 28, 2023, the Company filed a Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a 1-for-15 reverse stock split of its common stock (the "Reverse Stock Split"). The Reverse Stock Split became effective at 5:00 p.m., Eastern Time, on November 28, 2023 (the "Effective Time").

As a result of the Reverse Stock Split, every 15 shares of the Company's issued and outstanding common stock were automatically reclassified into one validly issued, fully-paid and non-assessable share of common stock. Any fractional post-split shares as a result of the Reverse Stock Split were rounded down to the nearest whole post-split share. The Reverse Stock Split did not affect the number of authorized shares of common stock or the par value of the common stock. All share amounts and per share amounts for the periods prior to the Effective Time disclosed in this Quarterly Report on Form 10-Q have been restated to reflect the Reverse Stock Split on a retroactive basis.

Marketable Securities Classification

The Company may invest its excess cash in money market funds and debt instruments of the U.S. Treasury, financial institutions, corporations and U.S. government agencies with strong credit ratings and an investment grade rating at or above A-1 or P-1 by two of the three nationally recognized statistical rating organizations. The Company does not believe that it is exposed to more than a nominal amount of credit risk related to any marketable securities

it may invest in. The Company has established guidelines relative to diversification and maturities that maintain safety and liquidity, and periodically reviews and modifies these guidelines to maximize trends in yields and interest rates without compromising safety and liquidity.

The Company classifies its investments in debt instruments as available-for-sale. Available-for-sale investments are reported at fair value at each balance sheet date, and include any unrealized holding gains and losses in accumulated other comprehensive loss, a component of stockholders' equity. Realized gains and losses are included in the Company's condensed consolidated statements of operations. All of the Company's available-for-sale securities are available for use in its current operations. The Company classifies marketable securities as either cash equivalents, short-term, or long-term based on their stated maturity dates as it is more-likely-than-not that the Company will hold these assets through to their maturity dates.

Unaudited Interim Condensed Consolidated Financial Information

The accompanying condensed consolidated balance sheet as of September 30, 2024, the condensed consolidated statements of operations for the three and nine months ended September 30, 2024 and 2023, the condensed consolidated statements of comprehensive loss for the three and nine months ended September 30, 2024 and 2023, the condensed consolidated statements of cash flows for the nine months ended September 30, 2024 and 2023 and the condensed consolidated statements of stockholders' equity for the three and nine months ended September 30, 2024 and 2023 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, 2024, the results of its operations for the three and nine months ended September 30, 2024 and 2023 and its cash flows for the nine months ended September 30, 2024 and 2023. Financial statement disclosures for the three and nine months ended September 30, 2024 and 2023 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, 2024 are not necessarily indicative of results to be expected for the year ended December 31, 2024, any other interim periods, or any future year or period.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, accrued and prepaid research and development expense, collaboration revenue, stock-based compensation expense and the recoverability of the Company's net deferred tax assets and related valuation allowance. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from those estimates.

Common Stock Warrants

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

Net Loss per Share

Basic net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding for the period. The calculation of weighted average number of common shares outstanding excludes shares of restricted common stock that are not vested but includes shares of common stock underlying pre-funded

warrants. Diluted net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding for the period, after giving consideration to the dilutive effect of potentially dilutive common shares. For purposes of this calculation, outstanding options to purchase shares of common stock, unvested shares of restricted common stock and potential shares issuable under the 2020 ESPP are considered potentially dilutive common shares. The Company has generated a net loss in all periods presented so the basic and diluted net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

Recent Accounting Pronouncements

Recently Issued Accounting Pronouncements

On November 27, 2023, the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280) - Improvements to Reportable Segment Disclosures*. ASU No. 2023-07 improves reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. The amendments included in this ASU apply to all public entities that are required to report segment information in accordance with Topic 280, including those with a single reportable segment. The amendments are effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted and should be applied retrospectively to all prior periods presented in the financial statements. Upon transition, the segment expenses categories and amounts disclosed in the prior periods should be based on the significant segment expense categories identified and disclosed in the period of adoption. The Company only has one reportable segment and this ASU impacts disclosures only. The Company has determined that the effects of adopting the amendments in ASU 2023-07 will only impact its disclosures and not have a material impact on its consolidated financial position and the results of its operations when such amendment is adopted.

On December 14, 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 incomes taxes paid information. For public companies, the amendments are effective for annual periods beginning after December 15, 2024 and should be applied prospectively. 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.

3. Cash Equivalents and Marketable Securities

The following is a summary of the Company's investment portfolio as of September 30, 2024 (in thousands). The Company did not hold any cash equivalents or marketable securities as of December 31, 2023.

	As of September 30, 2024			
	Cost	Gross Unrealized		Estimated
		Gains	Losses	Fair Value
Cash equivalents:				
Money market funds	\$ 81,359	\$ —	\$ —	\$ 81,359
Available-for-sale marketable securities:				
Debt securities issued by U.S. government agencies	143,283	900	—	144,183
Corporate debt securities	74,715	370	—	75,085
Commercial paper	18,543	45	—	18,588
Other debt securities	4,656	16	—	4,672
Total cash equivalents and marketable securities	\$ 322,556	\$ 1,331	\$ —	\$ 323,887

As of September 30, 2024, the Company had 3 securities with a total fair market value of \$8.0 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, 2024	December 31, 2023
Within one year	\$ 188,387	\$ —
After one year through five years	54,141	—
Total	<u>\$ 242,528</u>	<u>\$ —</u>

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, 2024 and 2023.

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, 2024 (in thousands). The Company did not hold any financial assets measured at fair value on a recurring basis as of December 31, 2023.

	As of September 30, 2024			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	\$ 81,359	\$ —	\$ —	\$ 81,359
Available-for-sale marketable securities:				
Debt securities issued by U.S. government agencies	144,183	—	—	144,183
Corporate debt securities	—	75,085	—	75,085
Commercial paper	—	18,588	—	18,588
Other debt securities	—	4,672	—	4,672
	<u>\$ 225,542</u>	<u>\$ 98,345</u>	<u>\$ —</u>	<u>\$ 323,887</u>

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Accrued external research and development expenses	\$ 11,756	\$ 2,957
Accrued personnel-related expenses	3,162	3,716
Accrued other expenses	539	743
Total accrued expenses	<u>\$ 15,457</u>	<u>\$ 7,416</u>

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 other non-current assets on the accompanying condensed consolidated balance sheets as of September 30, 2024 and December 31, 2023. 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 will be 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 recognizes revenue for its research services performance obligation over time using an input method over the duration of the research services. During the three and nine months ended September 30, 2024, the Company recognized \$0.3 million and \$1.1 million, respectively, in collaboration revenue related to the Collaboration Agreement in the condensed consolidated statement of operations. As of September 30, 2024, \$1.5 million was included in deferred revenue in the condensed consolidated balance sheet, of which \$1.2 million was classified as current.

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, 2024, the Company had not achieved any development or sales milestones or earned 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 in research and development expense in the accompanying condensed consolidated statement of operations for the three months ended March 31, 2024. 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, 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, 2024 because no qualifying costs were incurred. During the three and nine months ended September 30, 2024, the Company did not recognize any revenue associated with the combined arrangement.

8. Common Stock and Preferred Stock

Reverse Stock Split

On November 28, 2023, the Company filed a Certificate of Amendment to the Company's Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a 1-for-15 reverse stock split of its common stock. The Reverse Stock Split became effective at 5:00 p.m., Eastern Time, on November 28, 2023.

As a result of the Reverse Stock Split, every 15 shares of the Company's issued and outstanding common stock were automatically reclassified into one validly issued, fully-paid and non-assessable share of common stock. Any fractional post-split shares as a result of the Reverse Stock Split were rounded down to the nearest whole post-split share. The Reverse Stock Split did not affect the number of authorized shares of common stock or the par value of the common stock. All share amounts and per share amounts for the period prior to the Effective Time disclosed in this Quarterly Report on Form 10-Q have been restated to reflect the Reverse Stock Split on a retroactive basis.

Common Stock

As of September 30, 2024 and December 31, 2023, the authorized capital stock of the Company included 150,000,000 shares of common stock, \$0.0001 par value.

As of September 30, 2024 and December 31, 2023, the Company did not hold any treasury shares.

Follow-On Public Offerings

June 2023 Public Offering

On June 21, 2023, the Company completed a public offering of: (i) an aggregate of 4,296,646 shares of its common stock at a public offering price of \$14.25 per share, including the underwriters' full exercise of their option to purchase 619,979 additional shares of common stock, and (ii) pre-funded warrants to purchase 470,000 shares of common stock at a public offering price of \$14.2485 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 \$0.0015 per share exercise price for each underlying share. Total net proceeds generated from the offering were approximately \$63.4 million, after deducting underwriting discounts, commissions and other offering expenses payable by the Company.

During the nine months ended September 30, 2024, all pre-funded warrants associated with this offering were exercised via a cashless exercise, resulting in 469,981 shares of common stock issued. No cash proceeds associated with the exercise were received by the Company. As of September 30, 2024, there were no pre-funded warrants associated with this offering outstanding.

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 nine months ended September 30, 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. As of September 30, 2024, a total of 904,580 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, 2024, none of the pre-funded warrants had been exercised and all remained outstanding.

The Company determined that the pre-funded warrants related to the June 2023, 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, 2024	December 31, 2023
Shares reserved for exercise of outstanding stock options	1,829,324	666,163
Shares reserved for exercise of pre-funded warrants related to the June 2023 Financing	—	470,000
Shares reserved for exercise of pre-funded warrants related to the January 2024 Financing	904,580	—
Shares reserved for exercise of pre-funded warrants related to the April 2024 Financing	221,238	—
Shares reserved for future awards under the 2020 Stock Option and Incentive Plan	115,058	148,264
Shares reserved for future awards under the 2020 Employee Stock Purchase Plan	115,883	53,111
Shares reserved for future awards under the 2024 Inducement Plan	112,774	—
Shares reserved for vesting of restricted stock units	222,674	47,145
Total shares of authorized common stock reserved for future issuance	3,521,531	1,384,683

Preferred Stock

As of September 30, 2024 and December 31, 2023, 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. The total shares of common stock initially authorized for issuance under the Inducement Plan was 1,000,000. In June 2024, the common stock authorized for issuance under the Inducement Plan was reduced by 870,000 shares following stockholder approval of an increase in the shares available for grant under the 2020 Stock Option and Incentive Plan (the "2020 Plan"). The total number of shares of common stock authorized for issuance under the Inducement Plan as of September 30, 2024 was 130,000 shares.

2020 Stock Option and Incentive Plan

The total number of shares of common stock authorized for issuance under the 2020 Plan as of September 30, 2024 and December 31, 2023 was 1,970,833 shares and 661,240 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, 2024 and December 31, 2023 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, 2024 and December 31, 2023 was 175,145 shares and 87,227 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, 2023	47,145	\$ 202.27
Issued	197,097	51.98
Vested	(11,856)	309.13
Forfeited	(9,712)	54.86
Unvested as of September 30, 2024	222,674	\$ 69.98

As of September 30, 2024, total unrecognized compensation cost related to unvested restricted stock units was \$11.9 million, which is expected to be recognized over a weighted-average period of 2.84 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 (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding as of December 31, 2023	666,163	\$ 160.19		
Granted	1,195,546	51.43		
Exercised	(8,753)	39.75		\$ 134
Cancelled or Forfeited	(23,632)	127.76		
Outstanding as of September 30, 2024	1,829,324	\$ 90.08	8.71	\$ 12,800
Exercisable as of September 30, 2024	878,111	\$ 121.03	7.93	\$ 6,963
Vested and expected to vest as of September 30, 2024	1,829,324	\$ 90.08	8.71	\$ 12,800

Valuation of Stock Options

The weighted-average assumptions that the Company used in the Black-Scholes option pricing model to determine the grant-date fair value of stock options granted to employees and non-employees on the date of grant were as follows for the three and nine months ended September 30, 2024:

	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2024
Risk-free interest rate	4.06 %	4.01 %
Expected term (in years)	6.00	6.00
Expected volatility	101.00 %	96.48 %
Expected dividend yield	— %	— %
Weighted average grant-date fair value per share	\$ 45.95	\$ 40.73

As of September 30, 2024, total unrecognized compensation cost related to unvested stock options was \$43.6 million, which is expected to be recognized over a weighted-average period of 2.55 years.

Stock-Based Compensation

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	\$ 4,640	\$ 2,035	\$ 12,298	\$ 6,176
General and administrative	7,792	3,728	20,487	12,955
Total stock-based compensation expense	\$ 12,432	\$ 5,763	\$ 32,785	\$ 19,131

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,	
	2024	2023	2024	2023
Outstanding stock options	1,829,324	658,417	1,829,324	658,417
Unvested restricted stock units	222,674	42,241	222,674	42,241
Potential shares issuable under the 2020 ESPP	7,391	10,780	7,391	10,780
	2,059,389	711,438	2,059,389	711,438

Common shares issuable upon exercise of the pre-funded warrants that were sold in connection with the June 2023, January 2024, and April 2024 underwritten public offering are included in the calculation of basic weighted average number of common shares outstanding for the three and nine months ended September 30, 2024. 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. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the condensed consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. The Company has concluded that no subsequent events have occurred that require disclosure.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K, which was filed with the Securities and Exchange Commission (the "SEC") on March 5, 2024. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2023, 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 most advanced product candidate under the Cerebrum™ platform, ulixacaltamide, our Phase 3 Essential3 clinical trials in essential tremor, or ET, are ongoing. We are planning to conduct an interim analysis in the first quarter of 2025. Within our vformatrigine program (formerly known as PRAX-628), we announced positive results from our Photo-Paroxysmal Response ("PPR") study in the first quarter of 2024, and have initiated or plan to initiate four studies to generate patient eligibility, efficacy, safety and pharmacokinetics (PK) data for the program. We initiated an observational study of vformatrigine in patients with epilepsy in the third quarter of 2024, and initiated or plan to initiate three efficacy studies. The first efficacy study will be an open label eight-week study in patients with focal onset seizures or generalized epilepsy to be initiated in the fourth quarter of 2024, with topline results expected in the first half of 2025. We have also initiated a double-blind, placebo-controlled, 12-week study in focal onset seizures in the fourth quarter of 2024, with topline results expected in the second half of 2025, and plan to initiate a third efficacy study in the first half of 2025. Within 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 have initiated enrollment of the second cohort, with topline results expected in the first 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, and have initiated the first arm of our global confirmatory study in Brazil. We are currently completing multiple global regulatory interactions to further expand the pivotal phase of the program later in 2024.

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 \$124.1 million for the nine months ended September 30, 2024. As of September 30, 2024, we had an accumulated deficit of \$778.1 million. We expect to incur significant expenses and operating losses for the foreseeable future as we expand our research and development activities. In addition, our losses from operations may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities. We anticipate that our expenses will be maintained or increased in connection with our ongoing activities, as we:

- advance our lead product candidate, ulixacaltamide, through the Phase 3 Essential3 clinical trial program for ET;
- advance relutrigine (formally PRAX-562) in the EMBOLD and EMERALD clinical trials;
- advance elsunersen (formerly PRAX-222) into the pivotal stage of the program;
- advance vormatrigine (formerly PRAX-628) into efficacy clinical trials for focal onset seizures or generalized epilepsy;
- 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, 2024, we had cash, cash equivalents and marketable securities of \$411.2 million. We expect that our cash, cash equivalents and marketable securities as of September 30, 2024 will be sufficient to fund our operating expenditures and capital expenditure requirements necessary to advance our research efforts and clinical trials into 2027. The analysis included consideration of our current financial needs and ongoing research and

development plans. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See “—Liquidity and Capital Resources.”

Reverse Stock Split

On November 28, 2023, we filed a Certificate of Amendment to our Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a 1-for-15 reverse stock split of our common stock, or the Reverse Stock Split. The Reverse Stock Split became effective at 5:00 p.m., Eastern Time, on November 28, 2023, or the Effective Time.

As a result of the Reverse Stock Split, every 15 shares of our issued and outstanding common stock were automatically reclassified into one validly issued, fully-paid and non-assessable share of common stock, subject to the treatment of fractional shares as described below, without any action on the part of the holders thereof. The Reverse Stock Split did not affect the number of authorized shares of common stock or the par value of the common stock.

No fractional shares were issued in connection with the Reverse Stock Split. Stockholders who would otherwise be entitled to receive fractional shares as a result of the Reverse Stock Split were entitled to a cash payment in lieu thereof at a price equal to the fraction to which the stockholder would otherwise be entitled, multiplied by the closing price per share of the common stock (as adjusted for the Reverse Stock Split) on the Nasdaq Global Select Market on November 28, 2023, the last trading day immediately preceding the Effective Time.

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. We recognized \$0.3 million and \$1.1 million, respectively, of collaboration revenue from the Collaboration Agreement during the three and nine months ended September 30, 2024, compared to \$0.5 million and \$1.9 million, respectively, during the three and nine months ended September 30, 2023.

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:

- 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,	
	2024	2023	2024	2023
Cerebrum™	\$ 27,474	\$ 5,832	\$ 55,226	\$ 23,857
Solidus™	1,327	2,806	4,197	16,330
Personnel-related (including stock-based compensation)	10,863	6,840	30,265	21,744
Other indirect research and development expenses	2,217	1,782	6,437	6,447
Total research and development expenses	\$ 41,881	\$ 17,260	\$ 96,125	\$ 68,378

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

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales 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, 2024 and 2023.

Results of Operations

Comparison of the Three Months Ended September 30, 2024 and 2023

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

	Three Months Ended September 30,		Change
	2024	2023	
Collaboration revenue	\$ 302	\$ 468	\$ (166)
Operating expenses:			
Research and development	41,881	17,260	24,621
General and administrative	15,256	8,724	6,532
Total operating expenses	57,137	25,984	31,153
Loss from operations	(56,835)	(25,516)	(31,319)
Other income:			
Other income, net	4,925	884	4,041
Total other income	4,925	884	4,041
Net loss	\$ (51,910)	\$ (24,632)	\$ (27,278)

Collaboration Revenue

The \$0.2 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. Changes in revenue recognized each period reflect timing of research services provided and costs incurred.

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
	2024	2023	
Cerebrum™	\$ 27,474	\$ 5,832	\$ 21,642
Solidus™	1,327	2,806	(1,479)
Personnel-related (including stock-based compensation)	10,863	6,840	4,023
Other indirect research and development expenses	2,217	1,782	435
Total research and development expenses	\$ 41,881	\$ 17,260	\$ 24,621

The \$24.6 million increase in research and development expenses was primarily attributable to the following:

- \$21.6 million increase in expense related to our Cerebrum™ platform, driven primarily by:
 - a \$15.7 million increase in spend for our ulixacaltamide program, primarily due to Essential3 study spend, as well as Phase 1 trial spend, partially offset by completion of our Essential1 study in the prior year;
 - a \$5.4 million increase in spend for our vortmatrigine program, primarily related to current quarter POWER1, RADIANT and EMPOWER study startup activities, Phase 1 trial spend and manufacturing spend; and
 - a \$0.5 million increase in spend for our relutrigine program, primarily related to the EMBOLD Phase 2 clinical trial.
- \$4.0 million increase in personnel-related costs, including stock-based compensation expense, due to increased headcount;
- \$0.4 million increase in indirect expenses driven primarily by consulting spend; and
- \$1.5 million decrease in expense related to our Solidus™ platform, driven by prior year spend associated with our elsunersen program.

General and Administrative Expense

The \$6.5 million increase in general and administrative expenses was primarily attributable to the following:

- \$4.6 million increase in personnel-related costs, mainly due to increased stock-based compensation expense;
- \$1.4 million increase in professional expenses mainly due to increased legal, audit and other consulting expenses; and
- \$0.5 million increase in other expenses, none of which were individually significant.

Other Income

Other income for the three months ended September 30, 2024 was comprised of interest income on our cash, cash equivalents, and marketable securities and investment premium and discount amortization. Other income for the three months ended September 30, 2023 was comprised of interest income on our cash and cash equivalents.

Results of Operations

Comparison of the Nine Months Ended September 30, 2024 and 2023

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

	Nine Months Ended September 30,		Change
	2024	2023	
Collaboration revenue	\$ 1,090	\$ 1,932	\$ (842)
Operating expenses:			
Research and development	96,125	68,378	27,747
General and administrative	41,174	32,121	9,053
Total operating expenses	137,299	100,499	36,800
Loss from operations	(136,209)	(98,567)	(37,642)
Other income:			
Other income, net	12,069	2,168	9,901
Total other income	12,069	2,168	9,901
Net loss	\$ (124,140)	\$ (96,399)	\$ (27,741)

Collaboration Revenue

The \$0.8 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. Changes in revenue recognized each period reflect timing of research services provided and costs incurred.

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
	2024	2023	
Cerebrum™	\$ 55,226	\$ 23,857	\$ 31,369
Solidus™	4,197	16,330	(12,133)
Personnel-related (including stock-based compensation)	30,265	21,744	8,521
Other indirect research and development expenses	6,437	6,447	(10)
Total research and development expenses	\$ 96,125	\$ 68,378	\$ 27,747

The \$27.7 million increase in research and development expenses was primarily attributable to the following:

- \$31.4 million increase in expense related to our Cerebrum™ platform, driven primarily by:
 - a \$29.5 million increase in spend for our ulixacaltamide program, primarily due to clinical-related activities for our Phase 3 program in ulixacaltamide and Phase 1 trial spend, partially offset by prior year Essential1 study activity;
 - a \$3.2 million increase in clinical-related spend for our vortmatrigine program, primarily driven by current quarter POWER1, RADIANT, and EMPOWER study startup activities and manufacturing spend;
 - a \$0.3 million increase in spend for our relutrigine program related to our EMBOLD study; and
 - a \$1.6 million decrease in activities for our earlier stage assets.
- \$12.1 million decrease in expense related to our Solidus™ platform, driven by a prior year \$6.9 million milestone payment due to Ionis Pharmaceuticals Inc., or Ionis, upon initiation of our elsunersen EMBRAVE study, as well as prior year study activity; and
- \$8.5 million increase in personnel-related costs, primarily stock-based compensation expense.

General and Administrative Expense

The \$9.1 million increase in general and administrative expenses was primarily attributable to the following:

- \$8.3 million increase in personnel-related costs mainly due to increased stock-based compensation expense;
- \$0.3 million increase in professional fees, primarily audit fees; and
- \$0.5 million increase in other expenses, none of which were individually significant.

Other Income

Other income for the nine months ended September 30, 2024 and 2023 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 do not expect to generate revenue from sales of any products for several years, if at all.

To date, we have financed our operations primarily with proceeds from 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, 2024, we have raised \$1.0 billion in aggregate cash proceeds from such transactions, net of issuance costs. As of September 30, 2024, we had cash, cash equivalents and marketable securities of \$411.2 million.

In November 2021, we entered into an Open Market Sale Agreement, or the 2021 Sales Agreement, with Jefferies LLC, or Jefferies, to provide for the offering, issuance and sale of up to an aggregate amount of \$125.0 million of common stock from time to time in at-the-market offerings for which Jefferies acted as sales agent. We terminated the 2021 Sales Agreement in June 2023.

On June 21, 2023, we completed a public offering of: (i) an aggregate of 4,296,646 shares of common stock at a public offering price of \$14.25 per share, including the underwriters' full exercise of their option to purchase 619,979 additional shares of common stock, and (ii) pre-funded warrants to purchase 470,000 shares of common stock at a public offering price of \$14.2485 per share. The purchase prices per share for each pre-funded warrant represents the per share offering price for the common stock, less the \$0.0015 per share exercise price for each underlying share. Total net proceeds generated from the offering were approximately \$63.4 million, after deducting

underwriting discounts, commissions and other offering expenses payable by us. As of June 30, 2024, all warrants associated with this offering were exercised on a cashless basis with no proceeds received by the Company.

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 in at-the-market offerings. The 2023 Sales Agreement was terminated in January 2024. During the three months ended March 31, 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.

On January 16, 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, 2024, 152,145 warrants associated with this offering were exercised on a cashless basis with no cash proceeds received by the Company.

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.

On April 2, 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.

Cash Flows

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

	Nine Months Ended September 30,	
	2024	2023
Net cash (used in) provided by:		
Operating activities	\$ (75,635)	\$ (87,275)
Investing activities	(237,576)	39,000
Financing activities	400,556	87,745
Net increase in cash, cash equivalents and restricted cash	<u>\$ 87,345</u>	<u>\$ 39,470</u>

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, 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.

During the nine months ended September 30, 2023, net cash used in operating activities of \$87.3 million was primarily due to our \$96.4 million net loss and \$11.0 million in changes in operating assets and liabilities primarily related to decreases in accrued expenses and accounts payable, partially offset by \$20.1 million of non-cash charges primarily related to stock-based compensation.

Investing Activities

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.

During the nine months ended September 30, 2023, net cash provided by investing activities of \$39.0 million was related to maturities of marketable securities.

Financing Activities

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.

During the nine months ended September 30, 2023, net cash provided by financing activities of \$87.7 million consisted primarily of net proceeds from at-the-market offerings and our June 2023 follow-on public offering.

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;
- potentially 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, 2024 will be sufficient to fund our operating expenses and capital expenditure requirements into 2027. 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 March 5, 2024, 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, 2024. Based on the evaluation of our disclosure controls and procedures as of September 30, 2024, 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, 2023 filed with the SEC on March 5, 2024.

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, 2024.

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, 2024, 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	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
10.1	Praxis Precision Medicines, Inc. 2020 Stock Option and Incentive Plan, as amended and restated (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on June 6, 2024).
10.2	Employment Agreement Amendment Letter, dated October 11, 2024, by and between Marcio Souza and Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on October 15, 2024)
10.3	Employment Agreement Amendment Letter, dated October 11, 2024, by and between Timothy Kelly and Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on October 15, 2024)
10.4	Employment Agreement Amendment Letter, dated October 11, 2024, by and between Alex Nemiroff and Praxis Precision Medicines, Inc. (incorporated by reference to Exhibit 10.3 to the Registrant's Current Report on Form 8-K (File No. 001-39620) filed on October 15, 2024)
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.

