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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2025

or

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

For the transition period from _____ to_____

Commission File Number: 001-42365

CAMP4 Therapeutics Corporation
(Exact name of registrant as specified in its charter)

Delaware	**81-1152476**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

One Kendall Square
Building 1400 West, 3rd Floor
Cambridge, Massachusetts 02139
(Address of Principal Executive Offices)

(617) 651-8867
(Registrant's telephone number)

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

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

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

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

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

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

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

Title of Each Class	**Trading symbol**	**Name of Exchange on which registered**
Common stock, par value $0.0001 per share	**CAMP**	**Nasdaq Global Market**

As of November 3, 2025, there were 46,881,134 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Quarterly Report on Form 10-Q ("Quarterly Report") contains forward-looking statements that involve substantial risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. All statements other than statements of historical fact contained in this Quarterly Report, including statements regarding our strategy, future operations, future financial position, prospects, plans, objectives of management and expected growth, are forward-looking statements. These statements are based on our current beliefs, expectations and assumptions regarding our intentions, beliefs or current expectations concerning, among other things, the future of our business, future plans and strategies, our operational results and other future conditions. Forward-looking statements involve known and unknown risks, uncertainties and other important 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 the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "estimate," "believe," "predict," "potential" or "continue" or the negative of these terms or other similar expressions intended to identify statements about the future, although not all forward-looking statements contain these identifying words. These forward-looking statements include, without limitation, statements about the following:

- the initiation, timing, progress, results and costs of our research and development ("R&D") programs and of our current and future preclinical studies and clinical trials of our product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, as well as the period during which the results of the trials are expected become available;
- the timing of our planned good laboratory practices toxicology studies and regulatory submissions, initiation of planned clinical trials and timing of expected clinical results for our current and future product candidates;
- the timing of any submissions of filings for regulatory approval of, and our ability to obtain and maintain regulatory approvals for, any product candidates we may develop;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- our expectations regarding the size of the patient populations, market acceptance and opportunity for and clinical utility of our product candidates, if approved for commercial use;
- our reliance on third party manufacturing partners to comply with significant regulations with respect to manufacturing our products;
- our expectations regarding the scope of any approved indication for any product candidates we may develop;
- our ability to successfully commercialize our product candidates, if approved;
- our ability to leverage our RAP Platform to identify and develop future product candidates;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements, and our need for or ability to obtain additional funding before we can expect to generate any revenue from product sales;
- our ability to establish or maintain strategic collaborations or arrangements, including potential business development opportunities and potential licensing partnerships, and our ability to attract collaborators with development, regulatory and commercialization expertise;
- our ability to identify, recruit and retain key personnel;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;

- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- the sufficiency of our existing cash and cash equivalents to fund our future operating expenses and capital expenditure requirements;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our estimates regarding future expenses and needs for additional financing;
- the impact of laws and regulations;
- the effect of changes in international trade policies and general economic, industry, geopolitical and market conditions, such as uncertainties related to military conflict or war, tariffs (including tariffs that have been or may in the future be imposed by the United States or other countries), sanctions, trade protection measures or other trade barriers, inflation and financial institution instability, or pandemic or epidemic disease outbreaks, many of which are beyond our control, as well as the value of our common stock and our ability to access capital markets; and
- our expectations regarding the time during which we will be an emerging growth company and smaller reporting company under the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act").

Although we base these forward-looking statements on assumptions that we believe are reasonable when made, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from those made in or suggested by the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, those results or developments may not be indicative of results or developments in subsequent periods.

Given these risks and uncertainties, you are cautioned not to place undue reliance on these forward-looking statements. Any forward-looking statement that we make in this Quarterly Report speaks only as of the date of such statement. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, whether as a result of new information, future events or otherwise. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless specifically expressed as such, and should only be viewed as historical data. 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.

Unless otherwise indicated, market and industry data contained in this Quarterly Report, including potential market opportunities, is based on our management's estimates and research, as well as industry and general publications and research and studies conducted by third parties. Although we believe that the information from these third-party publications, research and studies included in this Quarterly Report is reliable, and we are responsible for the accuracy of such information, we have not independently verified the accuracy or completeness of this information. Management's estimates are derived from publicly available information, their knowledge of our industry and their assumptions based on such information and knowledge, which we believe to be reasonable. This data involves a number of assumptions and limitations and the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in Part II, Item 1A. "Risk Factors" in this Quarterly Report. These and other factors could cause our future performance to differ materially from our assumptions and estimates.

NOTE REGARDING TRADEMARKS

"CAMP4," "RAP Platform," "RNA Actuator" and our other registered or common law trademarks, trade names or service marks appearing in this Quarterly Report are the property of CAMP4 Therapeutics Corporation and are registered as trademarks in the United States and other countries. This Quarterly Report also contains references to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Quarterly Report,

including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate in any way that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other entities' trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, any other entity.

PART I —FINANCIAL INFORMATION

Item 1. Financial Statements

CAMP4 Therapeutics Corporation

Unaudited Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)

	September 30, 2025	December 31, 2024
Assets		
Cash and cash equivalents	$ 75,255	$ 64,039
Prepaid expenses and other current assets	2,453	2,344
Total current assets	77,708	66,383
Restricted cash	1,624	1,624
Property and equipment, net	2,895	3,858
Operating lease right-of-use assets	4,078	6,015
Finance lease right-of-use assets	90	427
Total assets	$ 86,395	$ 78,307
Current liabilities:		
Accounts payable	$ 585	$ 1,210
Accrued expenses	4,065	4,833
Deferred revenue, short-term	—	385
Operating lease liabilities, current portion	3,226	2,994
Finance lease liabilities, current portion	27	91
Financing liability, current portion	—	85
Total current liabilities	7,903	9,598
Long-term liabilities:		
Operating lease liabilities, net of current portion	3,046	5,493
Finance lease liabilities, net of current portion	47	70
Derivative tranche and other liabilities	16,732	2
Total liabilities	27,728	15,163
Commitments and contingencies (Note 4)		
Stockholders' equity:		
Common stock, $0.0001 par value per share, 175,000,000 shares authorized as of September 30, 2025 and December 31, 2024, 46,880,185 and 20,161,072 shares issued and 46,880,185 and 20,145,129 shares outstanding as of September 30, 2025 and December 31, 2024, respectively	5	2
Additional paid-in capital	310,534	274,895
Accumulated deficit	(251,872)	(211,753)
Total stockholders' equity	58,667	63,144
Total liabilities and stockholders' equity	$ 86,395	$ 78,307

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

CAMP4 Therapeutics Corporation

Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except for share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue				
Research and collaboration revenue	$ 795	$ —	$ 3,150	$ —
Operating Expenses:				
Research and development	9,356	9,702	29,845	28,821
General and administrative	4,596	3,814	12,590	10,233
Impairment of right-of-use asset	494	—	494	—
Total operating expenses	14,446	13,516	42,929	39,054
Loss from operations	(13,651)	(13,516)	(39,779)	(39,054)
Other (expense) income, net:				
Interest income	423	94	1,464	720
Change in fair value of derivative tranche liability	(1,800)	—	(1,800)	—
Other expense	(71)	(62)	(4)	(178)
Total other (expense) income, net	(1,448)	32	(340)	542
Net loss attributable to common stockholders and comprehensive loss	$ (15,099)	$ (13,484)	$ (40,119)	$ (38,512)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.55)	$ (24.19)	$ (1.78)	$ (76.50)
Weighted-average shares of common stock outstanding, basic and diluted	27,274,721	557,437	22,554,427	503,455

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

CAMP4 Therapeutics Corporation

Unaudited Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(In thousands, except share amounts)

	Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' equity
	Shares	Amount			
Balance as of December 31, 2024	20,145,129	$ 2	$ 274,895	$ (211,753)	$ 63,144
Stock option exercises	1	—	—	—	—
Vesting of restricted common stock	11,666	—	—	—	—
Stock-based compensation expense	—	—	861	—	861
Net loss	—	—	—	(12,433)	(12,433)
Balance as of March 31, 2025	20,156,796	2	275,756	(224,186)	51,572
Vesting of restricted common stock	4,277	—	—	—	—
Stock-based compensation expense	—	—	1,002	—	1,002
Net loss	—	—	—	(12,587)	(12,587)
Balance as of June 30, 2025	20,161,073	$ 2	$ 276,758	$ (236,773)	$ 39,987
Issuance of common stock, net of issuance costs	26,717,414	3	29,188	—	29,191
Issuance of pre-funded warrants, net of issuance costs	—	—	3,601	—	3,601
Stock option exercises	1,698	—	3	—	3
Stock-based compensation expense	—	—	984	—	984
Net loss	—	—	—	(15,099)	(15,099)
Balance as of September 30, 2025	46,880,185	$ 5	$ 310,534	$ (251,872)	$ 58,667

	Convertible preferred stock		Common stock		Additional paid-in capital	Accumulated deficit	Total stockholders' deficit
	Shares	Amount	Shares	Amount			
Balance as of December 31, 2023	130,648,426	$ 162,147	460,704	$ 1	$ 36,231	$ (159,962)	$ (123,730)
Stock option exercises	—	—	971	—	2	—	2
Vesting of restricted common stock	—	—	14,589	—	—	—	—
Stock-based compensation expense	—	—	—	—	856	—	856
Net loss	—	—	—	—	—	(12,456)	(12,456)
Balance as of March 31, 2024	130,648,426	162,147	476,264	1	37,089	(172,418)	(135,328)
Issuance of common stock	—	—	88	—	—	—	—
Vesting of restricted common stock	—	—	14,525	—	—	—	—
Stock-based compensation expense	—	—	—	—	786	—	786
Net loss	—	—	—	—	—	(12,572)	(12,572)
Balance as of June 30, 2024	130,648,426	$ 162,147	490,877	$ 1	$ 37,875	$ (184,990)	$ (147,114)
Stock option exercises	—	—	500,531	1	30	—	31
Vesting of restricted common stock	—	—	14,522	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,592	—	1,592
Net loss	—	—	—	—	—	(13,484)	(13,484)
Balance as of September 30, 2024	130,648,426	$ 162,147	1,005,930	$ 2	$ 39,497	$ (198,474)	$ (158,975)

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

CAMP4 Therapeutics Corporation

Unaudited Condensed Consolidated Statements of Cash Flows
(In thousands)

	Nine Months Ended September 30,	
	2025	2024
Operating activities		
Net loss	$ (40,119)	$ (38,512)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,267	1,278
Stock-based compensation expense	2,847	3,235
Change in fair value of derivative tranche liability	1,800	—
Impairment of right-of-use asset	494	—
Loss on the sale of property and equipment	67	—
Non-cash lease expense	1,443	1,285
Non-cash interest and other expenses	17	68
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(109)	(343)
Accounts payable	(561)	1,024
Accrued expenses and other liabilities	(393)	(310)
Deferred revenue	(385)	—
Operating lease assets and liabilities	(2,214)	(1,999)
Net cash used in operating activities	(35,846)	(34,274)
Investing activities		
Purchases of property and equipment	(279)	(178)
Proceeds from the sale of property and equipment	36	—
Net cash used in investing activities	(243)	(178)
Financing activities		
Proceeds from issuance of common stock and pre-funded warrants, net	32,562	—
Proceeds allocated to the derivative tranche liability	14,930	—
Proceeds from exercise of stock options	3	33
Principal payments on finance leases	(93)	(268)
Principal payments on financing obligation	(97)	(345)
Payments of deferred offering costs	—	(820)
Net cash provided by (used in) financing activities	47,305	(1,400)
Net change in cash, cash equivalents, and restricted cash	11,216	(35,852)
Cash, cash equivalents and restricted cash – beginning of year	65,663	40,004
Cash, cash equivalents and restricted cash – end of period	$ 76,879	$ 4,152
Supplemental disclosures of cash flow information:		
Finance lease right-of-use asset converted to fixed asset	$ 290	$ —
Issuance costs in accounts payable and accrued expenses	$ 230	$ —
Deferred offering costs in accounts payable and accrued expenses	$ —	$ 2,504

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

CAMP4 Therapeutics Corporation
Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Basis of Presentation and Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying condensed consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") and applicable rules and regulations of the Securities and Exchange Commission for interim financial reporting, consistent in all material respects with those applied in the Company's Annual Report on Form 10-K for the year ended December 31, 2024 ("2024 Form 10-K") and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's consolidated financial position, consolidated results of operations, and consolidated cash flows for the interim periods presented. Any reference in these notes to applicable guidance is meant to refer to the authoritative standards of U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB"). This report should be read in conjunction with the audited consolidated financial statements in our 2024 Form 10-K.

These condensed consolidated financial statements include CAMP4 Therapeutics Corporation and its wholly-owned subsidiary, CAMP4 Therapeutics Pty Ltd (together, the "Company"). All intercompany balances and transactions have been eliminated in consolidation. The significant accounting policies used in the preparation of these condensed consolidated financial statements for the three and nine months ended September 30, 2025 are consistent with those described in the Company's 2024 Form 10-K. The results of operations for the three and nine months ended September 30, 2025 are not necessarily indicative of the operating results to be expected for the full fiscal year or future operating periods.

Reverse Stock Split

On October 3, 2024, the Company effected a one-for-11.2158 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in the accompanying condensed consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios. The par value and the number of authorized shares of the convertible preferred stock and common stock were not adjusted in connection with the reverse stock split.

Initial Public Offering

On October 15, 2024, the Company completed its initial public offering ("IPO"), pursuant to which it issued and sold an aggregate of 6,820,000 shares of its common stock to the public at a price of $11.00 per share, resulting in net proceeds of $65.8 million, after deducting underwriting discounts and commissions of $5.3 million and other offering expenses of $4.0 million. In addition, on November 1, 2024, the Company received proceeds of $6.6 million, after deducting underwriting discounts and commissions of $0.5 million, pursuant to the partial exercise by the underwriters of their option to purchase 643,762 additional shares in the IPO. Collectively, the Company received aggregate net proceeds of $72.4 million. Upon the closing of the IPO, the Company's outstanding convertible preferred stock automatically converted into 11,648,582 shares of common stock.

In connection with the completion of its IPO, on October 15, 2024, the Company's certificate of incorporation was amended and restated to authorize the issuance of up to 175,000,000 shares of common stock, par value $0.0001 per share and 25,000,000 shares of preferred stock, par value of $0.0001 per share.

Private Placement

On September 9, 2025, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with investors pursuant to which the Company agreed to sell certain securities, in up to two closings in a private placement transaction (the "Private Placement"). The initial closing of the Private Placement occurred on September 11, 2025 (the "Initial Closing"). At the Initial Closing, the Company issued 26,717,414 shares of the Company's common stock and

6,003,758 pre-funded warrants for net cash proceeds of $46.7 million, after deducting $3.3 million in issuance costs (see Notes 2 and 6).

Liquidity and Going Concern

As of September 30, 2025, the Company had approximately $75.3 million of cash and cash equivalents and working capital of approximately $69.8 million. The Company has a relatively limited operating history, and the revenue and income potential of the Company's business and market are unproven. The Company has experienced net losses and negative cash flows from operations since its inception and, as of September 30, 2025, the Company had an accumulated deficit of $251.9 million. During the nine months ended September 30, 2025, the Company incurred a net loss of $40.1 million and had negative cash flows from operating activities of $35.8 million. The Company will continue to incur significant costs and expenses related to its ongoing operations until it successfully develops, obtains regulatory approval for and gains market acceptance of a product candidate and achieves revenues adequate to support the Company's operations.

From inception to September 30, 2025, the Company has funded its operations primarily with proceeds from the sale of its securities, including in its IPO and the Private Placement, as well as through revenues from its license and collaboration agreements. Based on its current operating plan, the Company estimates that its cash and cash equivalents as of September 30, 2025 will be sufficient to fund operations through at least the next twelve months from the date of issuance of these condensed consolidated financial statements. As the Company continues to pursue its business plan, it expects to finance its operations through potential public or private equity offerings, debt financings or other capital sources, including current or potential future collaborations, licenses and other similar arrangements. However, there can be no assurance that any additional financing or strategic arrangements will be available to the Company on acceptable terms, if at all. If events or circumstances occur such that the Company does not obtain additional funding, it may be necessary to significantly reduce its scope of operations to reduce its rate of spending through actions such as reductions in staff and delaying, limiting, reducing or terminating product development or future commercialization efforts or grant rights to develop and market product candidates that it would otherwise prefer to develop and market itself, which could have a material adverse effect on the Company's business, results of operations or financial condition.

Use of Estimates

The preparation of its condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses in the condensed consolidated financial statements and the related disclosures in the accompanying notes. The Company bases its estimates on historical experience and various relevant assumptions believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting periods that are not readily apparent from other sources. Changes in estimates are recorded in the financial results of the period in which the new information becomes available. The actual results experienced may differ materially from the Company's estimates.

Restricted Cash

The Company includes its restricted cash balance in the cash, cash equivalents and restricted cash reconciliation of operating, investing and financing activities in the condensed consolidated statements of cash flows. The restricted cash is collateral for the letters of credit. The following table provides a reconciliation of cash, cash equivalents and restricted cash reported in the condensed consolidated balance sheets to the corresponding amounts shown in the condensed consolidated statements of cash flows (in thousands):

	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 75,255	$ 64,039
Restricted cash	1,624	1,624
Total cash, cash equivalents, and restricted cash	$ 76,879	$ 65,663

Comprehensive Loss

There were no differences between net loss and comprehensive loss presented in the condensed consolidated statements of operations for the three and nine months ended September 30, 2025 and 2024.

Derivative Tranche Liability

The Private Placement includes a right provided to the investors to purchase the Company's securities in two tranches. The second tranche was determined to be a freestanding instrument and accounted for as derivative tranche liability within the condensed consolidated balance sheets. The derivative tranche liability was recorded at its fair value on issuance and is subsequently remeasured at each reporting period with changes in fair value recorded in the condensed consolidated statements of operations until settlement.

Recently Issued Accounting Standards

Accounting standards not listed below were assessed and determined not to be applicable or are expected to have a minimal impact on the Company's condensed consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, Income Taxes ("Topic 740"): *Improvements to Income Tax Disclosures*. ASU 2023-09 requires disaggregated information about a reporting entity's effective tax rate reconciliation as well as additional information on income taxes paid. The standard requires entities to disclose federal, state, and foreign income taxes in their rate reconciliation tables and elaborate on reconciling items that exceed a quantitative threshold. Additionally, it requires an annual disclosure of income taxes paid, net of refunds, categorized by jurisdiction based on a quantitative threshold. For public business entities, the guidance is effective for annual periods beginning after December 15, 2024. ASU 2023-09 will result in the required additional disclosures being included in the Company's annual consolidated financial statements.

In November 2024, the FASB issued ASU 2024-03, Income Statement-Reporting Comprehensive Income-Expense Disaggregation Disclosures (Subtopic 220-40): *Disaggregation of Income Statement Expenses*. ASU 2024-03 requires disclosure of additional information about specific expense categories in the notes to financial statements for interim and annual reporting periods. For public business entities, the guidance is effective for interim and annual periods beginning after December 15, 2026. Early adoption is permitted for annual consolidated financial statements that have not yet been issued or made available for issuance. The guidance may be applied on a prospective basis or retrospectively for all prior periods presented in the financial statements. ASU 2024-03 will result in the required additional disclosures being included in the Company's consolidated financial statements once adopted.

2. Fair Value Measurements

Financial Assets

The following tables present the financial asset instruments carried at fair value on a recurring basis as of September 30, 2025 and December 31, 2024 (in thousands) in accordance with the ASC 820 hierarchy.

	Fair Value Measurements as of September 30, 2025			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 70,239	$ —	$ —	$ 70,239

	Fair Value Measurements as of December 31, 2024			
	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents	$ 60,819	$ —	$ —	$ 60,819

Derivative Tranche Liability

The following tables present the derivative tranche liability (see Note 6) carried at fair value on a recurring basis as of September 30, 2025 (in thousands) in accordance with the ASC 820 hierarchy and was based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. There was no derivative tranche liability as of December 31, 2024.

	Fair Value Measurements as of September 30, 2025			
	Level 1	Level 2	Level 3	Total
Liabilities				
Derivative tranche liability	$ —	$ —	$ 16,730	$ 16,730

The derivative tranche liability is valued using a Monte Carlo simulation model which uses certain assumptions, including annual volatility. The range of volatilities of comparable public companies utilized was 51.6% - 149.7% as of September 30, 2025. The volatility utilized in the Monte Carlo option-pricing model was determined by using the median. The following table presents the assumptions used to determine the fair value of the derivative tranche liability for the issuance date, September 11, 2025, and as of September 30, 2025:

	September 11, 2025	September 30, 2025
Expected volatility	73.0 %	73.0 %
Risk-free interest rate	3.50 %	3.60 %
Expected term (in years)	1.33	1.28
Probability of achieving specified conditions	25.0 %	25.0 %
Discount rate	3.53 %	3.59 %
Share price	$ 2.78	$ 3.00

The following table provides a roll-forward of the aggregate fair value of the derivative tranche liability categorized with Level 3 inputs (in thousands):

	Derivative Tranche Liability
Balance as of December 31, 2024	$ —
Issuance	14,930
Change in fair value	1,800
Balance as of September 30, 2025	$ 16,730

The change in fair value of tranche liability was primarily due to the increase in the price per share of the Company's common stock from the issuance date to September 30, 2025.

The carrying amounts reflected in the condensed consolidated balance sheet for prepaid expenses and other current assets, accounts payable and accrued expenses and other liabilities are shown at their historical values, which approximate their fair values.

3. Other Balance Sheet Components

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
R&D expenses	$ 1,776	$ 979
Insurance expense	8	591
Software and subscriptions expense	347	337
Variable lease expense	118	118
Federal R&D tax credit receivable	—	108
Other	204	211
Total prepaid expenses and other current assets	$ 2,453	$ 2,344

Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Leasehold improvements	$ 4,518	$ 4,518
Laboratory equipment	4,480	4,078
Computer and software	938	969
Furniture and fixtures	524	524
Total property and equipment	10,460	10,089
Less: accumulated depreciation and amortization	(7,565)	(6,231)
Total property and equipment, net	$ 2,895	$ 3,858

The Company incurred depreciation and amortization expense of $0.4 million for each of the three months ended September 30, 2025 and 2024. The Company incurred depreciation and amortization expense of $1.2 million for each of the nine months ended September 30, 2025 and 2024. For each of the three and nine months ended September 30, 2025 and 2024, depreciation and amortization expense included nominal finance lease right-of-use ("ROU") asset amortization (see Note 4). As of September 30, 2025, the Company has vacated the Boulder, Colorado lease location (see Note 4). As a result, the Company sold laboratory equipment with a net book value of $0.3 million and recognized a nominal loss on the sale.

Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Employee compensation and benefits	$ 2,050	$ 3,111
Professional fees	654	661
External R&D expenses	1,167	524
Other	194	537
Total accrued expenses	$ 4,065	$ 4,833

4. Commitments and Contingencies

Operating Leases

The Company continues to lease office and laboratory space in Cambridge, Massachusetts, which lease expires on June 30, 2027. As of September 30, 2025, the Company has vacated its Boulder, Colorado lease location, which lease expires on September 30, 2028. The Company is currently pursuing sublease opportunities for the space. As of September 30, 2025, it was determined that the market rate for similar space is less than the rate the Company is paying under the lease. As a result, the Company has recognized in the condensed consolidated statement of operations an impairment charge of $0.5 million related to the right of use ("ROU") asset.

The table below summarizes the Company's operating lease costs for the three and nine months ended September 30, 2025 and 2024 (in thousands except for lease terms and borrowing rates):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating lease costs:				
Lease expense	$ 598	$ 609	$ 1,816	$ 1,827
Short-term lease expense	14	14	42	40
Variable lease expense	358	344	1,016	1,017
Total operating lease costs	$ 970	$ 967	$ 2,874	$ 2,884
Other information:				
Operating cash flows used for operating leases	$ 876	$ 770	$ 2,587	$ 2,523
Weighted-average remaining lease term in years	1.9	2.9	1.9	2.9
Weighted-average incremental borrowing rate	6.73 %	6.72 %	6.73 %	6.72 %

Maturities of operating lease liabilities as of September 30, 2025 were as follows (in thousands):

2025 remaining	$ 877
2026	3,558
2027	1,980
2028	264
Total lease payments	6,679
Less: amount representing imputed interest	(407)
Total future minimum lease obligations	$ 6,272

Finance Leases

The Company leases certain specialized lab equipment under several finance lease agreements with maturities ranging from October 2028 to November 2028. The table below summarizes the Company's finance lease costs for the three and nine months ended September 30, 2025 and 2024 (in thousands except for lease terms and borrowing rates):

		Three Months Ended September 30,		Nine Months Ended September 30,	
	Classification	2025	2024	2025	2024
Finance lease costs:					
Amortization of ROU assets	Depreciation and amortization	$ 7	$ 49	$ 46	$ 146
Interest on lease liabilities	Other expense	2	6	6	22
Total finance lease costs		$ 9	$ 55	$ 52	$ 168
Other information:					
Operating cash flows used for finance leases		$ 14	$ 89	$ 93	$ 268
Weighted-average remaining lease term in years		3.1	1.8	3.1	1.8
Weighted-average incremental borrowing rate		7.74 %	8.24 %	7.74 %	8.24 %

Maturities of finance lease liabilities as of September 30, 2025 were as follows (in thousands):

2025 remaining	$	18
2026		31
2027		31
Total lease payments		80
Less: amount representing imputed interest		(6)
Total future minimum lease obligations	$	74

Legal Proceedings

There are no matters currently outstanding for which any liabilities have been accrued or require disclosure.

5. Collaboration and License Agreements

In-License Agreements

Children's Medical Center Corporation

In April 2018, the Company entered into a development and license agreement (the "CMCC Agreement") with Children's Medical Center Corporation ("CMCC"). The CMCC Agreement allows the Company to use CMCC's proprietary intellectual property to conduct research, development and commercialization of products utilizing CMCC's proprietary intellectual property in return for specified payments. The proprietary intellectual property licensed pursuant to this agreement is related to certain legacy programs the Company is not pursuing which were subsequently sublicensed to Fulcrum Therapeutics, Inc. ("Fulcrum"), as described below. As part of the CMCC Agreement, the Company issued a total of 15,123 shares of common stock to CMCC and its affiliates based on the fair value of the common stock on the date of issuance.

The Company is obligated to pay potential development milestone payments under the terms of the CMCC Agreement of up to $7.7 million for the first licensed target, $3.9 million for the second licensed target and $1.9 million for the third licensed target upon the achievement of certain specified contingent events. If commercial sales of a licensed product commence, the Company will pay CMCC royalties at percentage rates ranging in the low- to mid-single digits on net sales of licensed products in countries where such product is protected by patent rights. The Company incurred de minimis royalties owed to CMCC for each of the three and nine months ended September 30, 2025 and 2024 under the CMCC Agreement and recorded the amounts in R&D expense in the condensed consolidated statements of operations and comprehensive loss. In May 2025, the Company received a $0.6 million milestone payment pursuant to the Fulcrum Agreement, which is in part, a sublicense of rights granted to the Company under the CMCC Agreement. For the nine months ended September 30, 2025, the Company incurred a ten percent sublicense fee on the $0.6 million Fulcrum milestone under the CMCC Agreement. No such sublicense fee was incurred during three months ended September 30, 2025 or the three and nine months ended September 30, 2024.

The Company re-evaluates the likelihood of achieving future milestones under the CMCC Agreement at the end of each reporting period. As of September 30, 2025, the Company determined that the likelihood of achieving future milestones under the CMCC Agreement was not probable.

Whitehead Institute for Biomedical Research

In October 2019, the Company entered into a patent license agreement (as subsequently amended, the "Whitehead Agreement") with the Whitehead Institute for Biomedical Research ("Whitehead"). Under the Whitehead Agreement, the Company was granted a worldwide, royalty-bearing, sublicensable license under certain patent rights owned or controlled by Whitehead. Upon entering into the Whitehead Agreement, the Company paid an initial $0.1 million license issuance fee and has paid de minimis additional fees in connection with subsequent amendments to the Whitehead Agreement. In addition, the Company is obligated to pay certain filing, prosecution and maintenance fees with respect to certain patent rights licensed.

The Company is also obligated to pay potential development milestone payments to Whitehead of up to $1.9 million upon the achievement of certain specified contingent events. In addition, if the Company successfully commercializes a product under the Whitehead Agreement, it is obligated to pay tiered royalties at percentage rates ranging from less than one percent to the mid-single digits of net sales or of running royalties of net sales, subject to specified reductions, until either the last-to-expire valid claim of a Whitehead patent covering the product or seven years after the first commercial sale, in each case on a product-by-product and country-by-country basis.

The Company incurred de minimis fees under the Whitehead Agreement for each of the three and nine months ended September 30, 2025 and 2024. These fees are recorded in R&D expense in the Company's condensed consolidated statements of operations and comprehensive loss.

Sublicense Agreement

Fulcrum Therapeutics, Inc.

In July 2023, the Company entered into a license agreement (the "Fulcrum Agreement") with Fulcrum. Under the Fulcrum Agreement, the Company granted an exclusive license related to the Company's intellectual property ("IP") and granted a non-exclusive sublicense for IP obtained through the CMCC Agreement. In exchange for the license rights, Fulcrum paid the Company a $0.4 million upfront payment. In the event that Fulcrum achieves certain development and commercial milestones, Fulcrum is obligated to pay the Company one-time milestone payments ranging from $0.6 million to $20.0 million, depending on the product and milestone achieved. In addition, under the Fulcrum Agreement there are potential de minimis minimum annual royalty payments as well as sales-based royalties of up to the low-double digits upon commercialization.

The Company assessed this arrangement in accordance with ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), and concluded that the contract counterparty, Fulcrum, is a customer. In accordance with ASC 606, the Company determined that there is one performance obligation in the Fulcrum Agreement, consisting of the exclusive and non-exclusive license rights granted to Fulcrum. The transaction price was comprised of the fixed consideration of $0.4 million and was recognized upon transfer of control of the licenses at a point in time upon contract execution. The arrangement includes significant variable consideration primarily in the form of milestone payments, which was fully constrained at the inception of the contract. All variable consideration is remeasured and reassessed each reporting period. As of and for the three and nine months ended September 30, 2025 and 2024, the Company determined the remaining variable consideration was fully constrained.

The sales-based royalty fee qualifies for the royalty constraint exception and does not require an estimate of the future transaction price.

In May 2025, the Company received a $0.6 million milestone payment pursuant to the Fulcrum Agreement. As of the effective date of the Fulcrum Agreement, this milestone was considered fully constrained variable consideration under ASC 606. The Company included $0.6 million in the transaction price and recognized $0.6 million as research and collaboration revenue for the nine months ended September 30, 2025. No such research and collaboration revenue was recorded under the Fulcrum agreement for the three months ended September 30, 2025 or the three and nine months ended September 30, 2024.

All variable consideration is remeasured and reassessed each reporting period. As of and for the three and nine months ended September 30, 2025 and 2024, the Company determined all other remaining variable consideration was fully constrained.

Collaborative Arrangement

Eli Lilly and Company

In July 2023, the Company executed a Material Transfer Agreement (as subsequently amended, the "MTA") with Eli Lilly and Company ("Eli Lilly"). As part of the MTA, the Company and Eli Lilly agreed to perform R&D activities to generate up to three antisense oligonucleotides ("ASOs") in accordance with a prescribed workplan. The Company evaluated the MTA under ASC 808 and concluded that it is a collaboration arrangement. The Company and Eli Lilly are jointly overseeing the R&D activities under the MTA. In addition, both parties are exposed to significant risks and potential rewards under the MTA. For both the three months ended September 30, 2025 and 2024, the Company recorded de

minimis amounts as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss as a result of the earned R&D reimbursement from Eli Lilly. For the nine months ended September 30, 2025 and 2024, the Company recorded $0.1 million and $0.3 million, respectively, as a reduction in R&D expense in the condensed consolidated statement of operations and comprehensive loss as a result of the earned R&D reimbursement from Eli Lilly. Additionally, the Company had an unbilled receivable of less than $0.1 million recorded within prepaid expenses and other current assets on the condensed consolidated balance sheets as of December 31, 2024. There were no unbilled receivables as of September 30, 2025.

Research and Collaboration Arrangement

BioMarin Pharmaceutical Inc.

In September 2024, the Company entered into a Collaboration and License Agreement (the "BioMarin Agreement") with BioMarin Pharmaceutical Inc. ("BioMarin"). Under the terms of the BioMarin Agreement, BioMarin paid the Company an upfront, nonrefundable payment of $1.0 million, and will reimburse the Company for certain research activities. On a per-program basis, the Company will be eligible to receive up to $5.0 million in future contingent preclinical milestones, up to $75.0 million in future contingent development and regulatory milestones and up to $105.0 million in commercial sales milestones. The Company will be further eligible to receive tiered royalties at percentage rates ranging from the low to high single digits of net sales, subject to specified reductions, until either the last-to-expire valid claim of a patent covering the product, ten years after the first commercial sale, or the expiration of any applicable regulatory exclusivity obtained for the product, in each case on a product-by-product and country-by-country basis. The agreement may be terminated in its entirety or on a program-by-program basis for convenience by BioMarin. The agreement may also be terminated by either the Company or BioMarin under certain other circumstances, including material breach, as set forth in the agreement. The notice periods for termination provisions range from 30 days to 270 days depending on the reason for termination.

The Company assessed this arrangement in accordance with ASC 606 and concluded that BioMarin is a customer and there is one performance obligation, consisting of a bundle of R&D activities and license right grants. The performance obligation also includes participation in a joint steering committee, the grant of an exclusive license to BioMarin, the grant of a non-exclusive license to the data resulting from the R&D activities, performing the R&D activities in accordance with the contractual work plan, and providing quarterly progress reports. The transaction price was comprised of fixed consideration of $3.8 million and will be recognized over time based upon a cost-to-cost method. Any deferred revenue recorded will be classified as short-term. The Company believes this work will be completed within 12 months of contract execution and the related revenue will be recognized over that period.

The arrangement includes significant variable consideration primarily in the form of milestone payments, which is fully constrained at the inception of the contract. All variable consideration is remeasured and reassessed each reporting period. As of and for the three and nine months ended September 30, 2025, the Company determined the variable consideration was fully constrained.

For the three and nine months ended September 30, 2025, the Company recognized $0.8 million and $2.6 million in collaboration revenue under the BioMarin Agreement, respectively. Additionally, the Company had an unbilled receivable of $0.4 million as of September 30, 2025 and deferred revenue of $0.3 million as of December 31, 2024 recorded on the consolidated balance sheet, respectively.

6. Derivative Tranche Liability

Pursuant to the Purchase Agreement, subject to the occurrence of the second closing triggers, the Company has agreed to sell and the investors have agreed to purchase at a closing (the "Second Closing") up to 32,681,866 shares of the Company's common stock or pre-funded warrants in lieu thereof at a purchase price of $1.53 per share and $1.5299 per pre-funded warrant, and directors, consultants and members of management have agreed to purchase an additional 39,306 shares of the Company's common stock at a purchase price of $1.65 per share, for gross proceeds to the Company of up to approximately $50.1 million. The Second Closing triggers include a certain regulatory milestone and either of the following:

1. the achievement of a volume weighted average price per share of equal to or greater than $7.50, as defined in the purchase agreement, measured during any ten consecutive trading days during the thirty trading days following the date of the Company's first announcement of the regulatory milestone (the "Price Threshold"), or

2. the Company's receipt of a signed written notice from investors holding a majority of the Securities outstanding that waives the Price Threshold for purposes of the Second Closing in which case, only the waiving investors will be obligated to participate.

The obligation to issue and purchase securities was concluded to be a forward contract derivative liability and was measured at fair value using a probability weighted model at the issuance date. The initial fair value (Level 3) of the forward contract was $14.9 million and was recorded as a derivative tranche liability and will be remeasured at each subsequent reporting period (see Note 2).

7. Stockholders' Equity

Common and Preferred Stock

The Company's amended and restated certificate of incorporation authorizes the issuance of up to 175,000,000 shares of $0.0001 par value common stock and up to 25,000,000 shares of $0.0001 par value undesignated preferred stock. The Board of Directors of the Company (the "Board") may designate the rights, preferences, privileges, and restrictions of the preferred stock, including dividend rights, conversion rights, voting rights, terms of redemption, liquidation preference, and number of shares constituting any series or the designation of any series. The issuance of preferred stock could have the effect of restricting dividends on the Company's common stock, diluting the voting power of the Company's common stock, impairing the liquidation rights of the Company's common stock, or delaying or preventing a change in control. As of September 30, 2025, no shares of preferred stock were outstanding.

Each share of common stock entitles the holder to one vote, together with the holders of any preferred stock, on all matters submitted to the stockholders for a vote. Common stockholders are also entitled to receive dividends. As of September 30, 2025, no cash dividends have been declared or paid.

Private Placement

The Initial Closing of the Private Placement occurred on September 11, 2025. At the Initial Closing, the Company issued and sold 26,681,053 shares of the Company's common stock priced at $1.53 to certain investors, some of which were affiliated with directors of the Company, and 36,361 shares of common stock priced at $1.65 to directors, management and consultants of the Company (collectively, the "Shares"). In lieu of common stock, certain investors were sold pre-funded warrants to purchase 6,003,758 shares of common stock (the "Warrant Shares," and together with the Shares, the "Securities") at an offering price of $1.5299 per pre-funded warrant. Gross proceeds to the Company totaled $50.1 million before deducting placement agent fees and other expenses totaling $3.3 million.

The pre-funded warrants in the Initial Closing, and the potential Second Closing, have an exercise price of $0.0001 per Warrant Share, subject to customary adjustments, will be exercisable at any time after original issuance will not expire until exercised in full, and will be exercisable on a net exercise "cashless" basis. The pre-funded warrants may not be exercised if the aggregate number of shares of common stock beneficially owned by the holder thereof immediately following such exercise would exceed a specified beneficial ownership limitation, not to exceed 19.99%.

The Purchase Agreement contains customary representations, warranties and covenants. The Securities have not been registered under the Securities Act of 1933, as amended, and were offered pursuant to the exemption from registration provided in Section 4(a)(2) under the Securities Act of 1933, as amended. In connection with the Private Placement, the Company has filed an initial registration statement on Form S-3 (the "Initial Registration Statement") with the SEC to register for resale the Shares and, as applicable, the Warrant Shares, in connection with the Initial Closing. If applicable, within 30 days of the Second Closing and subject to certain allowable delays, the Company will prepare and file a second registration statement (the "Second Registration Statement") with the SEC to register for resale the shares and, as applicable, the warrant shares, in each case that were issued in connection with the Second Closing. The Company will use reasonable best efforts to cause the Initial Registration Statement or any Second Registration Statement and any amendments to promptly become effective, but in no event later than the earlier of (i) the 75th calendar day following the Initial Registration Statement or any Second Registration Statement filing date and (ii) the fifth business day after the date the Company is notified by the SEC that the Initial Registration Statement or any Second Registration Statement, as applicable, will not be "reviewed" or will not be subject to further review.

The Company concluded that the pre-funded warrants issued in the Initial Closing met the conditions to be classified as equity instruments under ASC 815-40.

Reserved Shares

As of September 30, 2025, the Company has reserved the following shares of common stock for future issuance:

Shares reserved for future issuance under the 2024 Equity Incentive and Employee Stock Purchase Plan	2,234,136
Stock options outstanding	2,885,206
Pre-funded warrants outstanding	6,003,758
Shares issuable upon settlement of tranche liability in Private Placement	32,681,866
Warrants outstanding	142
Total	43,805,108

8. Stock-Based Compensation

2024 Equity Incentive Plan

In October 2024, the Company adopted the 2024 Equity Incentive Plan (the "2024 Plan"), which became effective in connection with the Company's IPO. The 2024 Plan provides for the grant of stock options, stock appreciation rights, restricted and unrestricted stock awards, restricted stock unit awards, and other stock-based awards to employees, directors, and non-employee service providers of the Company.

Awards granted under the 2024 Plan expire no later than ten years from the date of grant. The price of stock options shall not be less than 100% of the estimated fair value on the date of grant and typically vest over a four-year period, although awards may be granted with different vesting terms. The 2024 Plan initially reserved 2,143,039 shares of common stock for the issuance of future awards and provides for an automatic annual increase in the number of shares of common stock reserved for future issuance by the lesser of (i) 5% of the number of shares of the Company's common stock outstanding as of the close of business on the immediately preceding December 31 and (ii) the number of shares determined by the Board on or prior to such date for such year. Pursuant to the terms of the 2024 Plan, an additional 604,832 shares of common stock were added to the number of available shares, effective January 1, 2025. As of September 30, 2025, there were 1,818,223 shares of common stock reserved and available for issuance pursuant to the 2024 Plan.

2024 Employee Stock Purchase Plan

In October 2024, the Company adopted the 2024 Employee Stock Purchase Plan (the "2024 ESPP"), which became effective in connection with the Company's IPO. The 2024 ESPP initially reserved 214,303 shares of common stock for the issuance of future awards and provides for an automatic annual increase in the number of shares of common stock reserved for future issuance by the lesser of (1) 1% of the number of shares of the Company's common stock outstanding as of the close of business on the immediately preceding December 31 and (2) the number of shares determined by the Board on or prior to such date for such year. Pursuant to the terms of the 2024 ESPP, an additional 201,610 shares of common stock were added to the number of available shares, effective January 1, 2025. As of September 30, 2025, there were 415,913 shares of common stock reserved and available for issuance pursuant to the 2024 ESPP.

The 2024 ESPP permits eligible employees to enroll in six-month offering periods. Participants may purchase shares of the Company's common stock, through payroll deductions, at a price equal to 85% of the fair market value of the common stock on the first or last day of the applicable offering period, whichever is lower. Purchase dates under the 2024 ESPP occur on or around January 1 and July 1 each year. As of September 30, 2025, there have been no completed offering periods.

Stock Option Activity

The following table summarizes stock option activity for the nine months ended September 30, 2025 (in thousands, except share and per share amounts):

	Number of outstanding options	Weighted average exercise price	Weighted average remaining contractual term in years	Aggregate intrinsic value
Balance as of December 31, 2024	2,119,425	$ 7.87	7.98	$ 751
Granted	948,446	$ 3.30		
Forfeited/Cancelled	(180,966)	$ 5.44		
Exercises	(1,699)	$ 2.12		$ 2
Balance as of September 30, 2025	2,885,206	$ 6.52	7.01	$ —
Vested and expected to vest as of September 30, 2025	2,885,206	$ 6.52	7.05	$ —
Exercisable as of September 30, 2025	1,512,812	$ 7.01	6.51	$ —

Restricted Stock Award Activity

A summary of restricted stock award activity for the nine months ended September 30, 2025 is as follows:

	Number of shares	Weighted average fair value
Unvested as of December 31, 2024	15,943	$ 1.75
Vested	(15,943)	$ 1.75
Unvested as of September 30, 2025	—	$ —

All restricted common stock awards were initially issued at a price determined to be fair value on the date of grant. The Company recognizes forfeitures of restricted common stock as they occur. As of September 30, 2025, all outstanding restricted common stock awards were fully vested.

Stock-Based Compensation Expense

The following table presents the components and classification of stock-based compensation expense for the three and nine months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Options	$ 984	$ 1,561	$ 2,813	$ 3,143
Restricted stock awards	—	31	34	92
Total	$ 984	$ 1,592	$ 2,847	$ 3,235
Research and development	$ 495	$ 519	$ 1,459	$ 1,424
General and administrative	489	1,073	1,388	1,811
Total	$ 984	$ 1,592	$ 2,847	$ 3,235

The Company has an aggregate $5.8 million of gross unrecognized stock-based compensation expense as of September 30, 2025 remaining to be recognized over a weighted average period of 2.3 years.

9. Income Taxes

No provision for federal, state, or foreign income taxes has been recorded for the three and nine months ended September 30, 2025 and 2024 and the effective tax rate was zero. The Company has incurred net operating losses for all the periods presented and has not reflected any benefit for such net operating loss carryforwards in the accompanying condensed consolidated financial statements due to uncertainty around utilizing these tax attributes within their respective carryforward periods. The Company has recorded a full valuation allowance against its deferred tax assets as it is more likely than not that such assets will not be realized in the near future.

The Company had no reserves related to uncertain tax positions as of September 30, 2025 and December 31, 2024. For the three and nine months ended September 30, 2025 and 2024, the Company has not recognized any potential interest or penalties. The Company is not currently subject to any tax assessment from an income tax examination in the U.S. or any other taxing jurisdiction.

On July 4, 2025, the One Big Beautiful Bill Act (the "OBBBA") was enacted in the United States. The OBBBA includes several changes to the federal tax law that generally allow for more favorable deductibility of certain business expenses beginning in 2025, including the restoration of immediate expensing of domestic R&D expenditures, reinstatement of 100% bonus depreciation, and more favorable rules for determining the limitation on business interest expense. The OBBBA also includes certain changes to the US taxation of foreign activity.

The Company evaluated the provisions of the OBBBA and determined that the enactment of the legislation did not have a material impact on its income tax provision, net deferred tax assets or liabilities, or estimated annual effective tax rate for the three and nine months ended September 30, 2025.

10. Segment Reporting

The Company manages its business activities on a consolidated basis and operates as a single operating segment, R&D, which engages in the business of developing a new class of RNA-targeting therapeutics to treat a broad range of genetic diseases. The Company's operations are primarily in the United States. The chief operating decision maker does not review assets in evaluating the results and therefore, such information is not presented.

The operating financial results of the Company's R&D segment for the three and nine months ended September 30, 2025 and 2024 are as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue	$ 795	$ —	$ 3,150	$ —
Less: Significant segment expenses				
Personnel-related expenses	4,922	5,242	15,701	14,795
Clinical and preclinical expenses	4,313	4,589	13,502	13,283
Facilities-related and overhead	2,090	1,937	6,088	5,655
Professional and consulting fees	2,204	1,312	5,390	4,128
Corporate expenses	297	317	1,334	814
Impairment of right-of-use asset	494	—	494	—
Travel and entertainment	126	119	420	379
Plus: Other segment (loss) income	(1,448)	32	(340)	542
Segment net loss	$ (15,099)	$ (13,484)	$ (40,119)	$ (38,512)

11. Net Loss Per Share Attributable to Common Stockholders

Weighted-average common shares outstanding includes 6,003,758 of common shares issuable upon the exercise of pre-funded warrants issued in the Initial Closing. Basic and diluted net loss per share was calculated as follows (in thousands, except share and per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Numerator:				
Net loss attributable to common stockholders	$ (15,099)	$ (13,484)	$ (40,119)	$ (38,512)
Denominator:				
Weighted-average common shares outstanding, basic and diluted	27,274,721	557,437	22,554,427	503,455
Net loss per share attributable to common stockholders, basic and diluted	$ (0.55)	$ (24.19)	$ (1.78)	$ (76.50)

The Company excluded the following from the computation of diluted net loss per share attributable to common stockholders as of September 30, 2025 and 2024 because their inclusion would have had an anti-dilutive effect:

		As of September 30,	
		2025	2024
Conversion of preferred stock		—	11,648,582
Shares reserved for future issuance under the 2024 Equity Incentive Plan and ESPP		2,234,136	—
Stock options outstanding		2,885,206	2,078,470
Restricted stock vesting		—	30,607
Shares issuable upon settlement of tranche liability in Private Placement		32,681,866	—
Warrants outstanding	Warrants outstanding	142	142
Total		37,801,350	13,757,801

12. Related Parties

In September 2015, the Company entered into consulting agreements with its two founders, related parties who hold shares of the Company's common stock, to provide R&D and strategic services. For both the three months ended September 30, 2025 and 2024, the Company recognized R&D expense of less than $0.1 million related to work performed under the founder agreements. For both the nine months ended September 30, 2025 and 2024, the Company recognized R&D expense of less than $0.2 million related to work performed under the founder agreements. For both the three months ended September 30, 2025 and 2024, the Company recognized stock-based compensation expense of less than $0.1 million related to the consulting agreements. For both the nine months ended September 30, 2025 and 2024, the Company recognized stock-based compensation expense of less than $0.1 million related to the consulting agreements. The Company had no amounts due to the founders as of September 30, 2025.

In March 2019, the Company entered into a consulting agreement with an executive consultant, a related party who holds shares of the Company's common stock. For both the three and nine months ended September 30, 2025, the Company recognized G&A expense totaling less than $0.1 million related to work performed under the consulting agreement. For both the three and nine months ended September 30, 2024, the Company recognized G&A expense of less than $0.1 million related to work performed under the consulting agreement. For the three and nine months ended September 30, 2025, the Company recognized stock-based compensation expense of less than $0.1 million related to the consulting agreement. For the three and nine months ended September 30, 2024, the Company recognized stock-based compensation expense of less than $0.1 million related to the consulting agreement. The Company had no amounts due to the consultant as of September 30, 2025.

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

The following discussion and analysis of our financial condition and results of operations and the unaudited interim condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q (the "Quarterly Report") should be read in conjunction with the audited financial statements and related notes thereto in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, which was filed with the Securities and Exchange Commission (the "SEC") on March 27, 2025 (the "2024 Form 10-K"). This discussion and analysis and other parts of this Quarterly Report contain forward-looking statements. Our actual results and the timing of selected events could differ materially from those described in or implied by these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the sections of this Quarterly Report titled "Special Note Regarding Forward-Looking Statements," and those risk factors described in "Part I, Item 1A, Risk Factors" of our 2024 Form 10-K and in "Part II, Item 1A, Risk Factors" in this Quarterly Report.

Overview

We are a clinical-stage biopharmaceutical company pioneering the discovery and development of a new class of RNA-targeting therapeutics with the goal of upregulating gene expression and restoring healthy protein levels to treat a broad range of genetic diseases. Regulatory RNAs ("regRNAs") play a central role in the regulation of every protein-coding gene by contributing to gene activation and suppression. Our approach is designed to amplify messenger RNA ("mRNA") expression by harnessing the power of regRNAs that form localized complexes with transcription factors and regulate gene expression. Our proprietary RNA Actuating Platform, or RAP Platform, allows us to rapidly and systematically identify and characterize the active regulatory elements controlling every expressed gene and tens of thousands of druggable enhancer and promoter regRNA sequences that control protein-coding genes. Once a disease-associated target gene is identified, we apply our RAP Platform to identify the controlling regRNA and rapidly generate novel antisense oligonucleotide ("ASO") candidates, which we refer to as RNA Actuators. These ASOs are designed to bind to the identified regRNA and amplify the expression of the target gene in a specific and controllable way. We are primarily focused on diseases of the central nervous system with validated disease biology, and we believe our RAP Platform allows us to address a broad range of rare and prevalent genetic diseases in which a modest increase in protein expression has the potential to be clinically meaningful.

We are leveraging our RAP Platform to advance a preclinical program for the treatment of synaptic Ras GTPase activating protein 1 ("SYNGAP1")-related disorders, a group of neurodevelopmental conditions caused by pathogenic variants in the SYNGAP1 gene that result in a haploinsufficient state, reducing SYNGAP protein levels by up to 50%. SYNGAP plays a critical role in cognitive development and synaptic function. Epilepsy is a common characteristic of these disorders, and nearly all patients experience some degree of developmental delay and cognitive impairment. Incidence estimates vary significantly, ranging from one to 40 per 100,000 individuals. While we believe that SYNGAP1-related disorders remain underdiagnosed, we estimate that there are more than 10,000 individuals living with these disorders in the United States. SYNGAP1-related disorders are reported to represent 0.5% to 1.0% of all intellectual disability cases, making them among the most common causes of intellectual disability in patients with epilepsy, and indicating that the patient population may be significantly larger than incidence estimates suggest. There are no FDA-approved, disease-modifying therapies for SYNGAP1-related disorders. Treatment is often limited to supportive physical, occupational and speech therapy. A combination of non-specific anti-seizure medications may be prescribed, though SYNGAP1-related disorders have proven difficult to control with available therapeutics. Up to 50% of patients do not adequately respond to medication, in which case implantable devices, such as those for vagus nerve stimulation, may offer incremental therapeutic benefit. We are advancing our SYNGAP program to address the significant unmet need by targeting the direct cause of SYNGAP1-related disorders, haploinsufficiency, which we believe is amenable to targeting through regRNAs. Our novel approach targets the SYNGAP1 gene at the transcriptional level to restore SYNGAP function and improve symptoms by utilizing an intrathecally delivered ASO. Upregulation of SYNGAP1 gene expression may increase SYNGAP protein levels in amounts sufficient to yield therapeutic benefit. Our preclinical studies demonstrated a dose-dependent increase in SYNGAP mRNA levels accompanied by an increase in SYNGAP protein expression. On May 16, 2025, we presented preclinical data from our SYNGAP program in an oral presentation at the 28th American Society of Gene and Cell Therapy Annual Meeting. We observed that intracerebroventricular injection of our lead development candidate, CMP-002 (formerly known as CMP-SYNGAP-01), restored SYNGAP protein levels to near normal range in haploinsufficient mice carrying a single copy of the human SYNGAP1 gene after a single dose and rescued motor defects and spatial learning defects following two doses. Additionally, we observed that biweekly intrathecal injections of CMP-002 were well tolerated in cynomolgus monkeys and resulted in a significant increase in SYNGAP protein levels across multiple brain regions clinically relevant to the disease, with dose-linear increases of CMP-002 in disease-relevant brain regions. We have initiated GLP toxicology studies for CMP-002 to enable the filing of a clinical trial application. Pending successful completion of GLP toxicology studies and regulatory clearance, we intend to initiate a global Phase 1/2 clinical trial in individuals with SYNGAP1-related disorders as early as the second half of 2026.

Our pipeline also includes our clinical candidate, CMP-001 (formerly known as CMP-CPS-001), for the treatment of the most prevalent urea cycle disorders ("UCDs"), for which we have completed a Phase 1 clinical trial in healthy volunteers. UCDs are a group of severe, inherited metabolic diseases caused by mutations in the genes that encode one or more of the eight enzymes and transporters necessary to convert ammonia into urea. The inability of the body to properly metabolize ammonia leads to the accumulation of toxic levels in circulation, ultimately resulting in severe health outcomes, such as neurologic disability, seizure and death. CMP-001 is designed to improve urea cycle activity by amplifying expression of carbamoyl phosphate synthetase 1 ("CPS1"), an enzyme that catalyzes the first step of the urea cycle, by binding to a CPS1-specific regRNA. Our preclinical studies have demonstrated that modulating the activity of the target regRNA increases expression of the CPS1 gene, resulting in increased CPS1 enzyme levels, which allows for more ammonia to be converted into urea, thereby lowering ammonia levels to normal, healthy ranges. These preclinical studies also demonstrated that CMP-001 can increase the level of, or upregulate, the production of multiple enzymes responsible for converting ammonia into urea, potentially allowing us to address more than 85% of patients with UCDs. We have received approval of a clinical trial application in the Netherlands to enable the initiation of a Phase 1b expansion to enroll female participants who are heterozygous for a mutation of the OTC gene. We estimate that there are approximately 2,000 diagnosed female OTC heterozygotes in the United States who have inherited one copy of a gene with OTC-related changes and experience potentially addressable UCD symptoms.

We have completed the analysis of safety, pharmacokinetic, and pharmacodynamic biomarker data from all four cohorts of the single ascending dose ("SAD") portion of the clinical trial and from the three completed cohorts of the multiple ascending dose ("MAD") portion of the clinical trial. In total, 86 healthy volunteer participants were observed, including 51 receiving CMP-001. CMP-001 demonstrated a favorable safety profile in both the SAD and MAD portions of the clinical trial, with no serious adverse events or discontinuations due to adverse events. Pharmacokinetic data similarly was observed to be consistent with expectations, demonstrating a dose-dependent increase in exposure (Cmax and AUC) with clear separation between groups and low-to-moderate variability in key pharmacokinetic parameters at all dose levels. There were no conclusive determinations of pharmacodynamic activity or non-activity in this healthy volunteer population, which we believe may have resulted from intrinsic variability in the ureagenesis rates of healthy individuals measured with the investigational 13C-sodium acetate test used in the study. We have made the strategic decision to pause new investment in our UCD program and to prioritize the development of our SYNGAP program. We continue to believe that CMP-001 has the potential to be the first disease-modifying therapy for the most prevalent UCDs and intend to pursue partnership opportunities to support the further development of CMP-001.

In addition, we are party to a strategic research collaboration with BioMarin Pharmaceutical Inc. ("BioMarin") to advance novel therapeutics that increase protein levels by targeting regRNA sequences for two genetic targets.

Since our inception in 2015, we have focused substantially all our resources primarily on developing our RAP Platform, identifying, developing and progressing our product candidates through preclinical and clinical development, organizing and staffing our company, research and development ("R&D") activities, establishing and protecting our intellectual property portfolio, and raising capital. To date, we have primarily funded our operations with proceeds from the sale of convertible preferred stock and common stock, including our private placement, the initial closing of which occurred on September 11, 2025, and our initial public offering ("IPO"), which closed on October 15, 2024, as well as through revenues from our license and collaboration agreements. Through September 30, 2025, we have received net proceeds of $46.7 million from the initial closing of our private placement, $72.4 million from our IPO and $188.3 million from the sale of our convertible preferred stock. In addition, through September 30, 2025, we have recognized $21.2 million in research collaboration and license revenue through our development and license agreements. Our ability to generate any product revenue and, in particular, our ability to generate product revenue sufficient to achieve profitability, will depend on the successful development and eventual commercialization of product candidates.

We have incurred significant operating losses and negative cash flows from operations since our inception. Our net losses were $40.1 million and $38.5 million for the nine months ended September 30, 2025 and 2024, respectively. As of September 30, 2025, we had an accumulated deficit of $251.9 million. Substantially all of our net losses have resulted from costs incurred in connection with our R&D programs and, to a lesser extent, from general and administrative ("G&A") costs associated with our operations. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and preclinical studies, our other R&D activities and capital expenditures, and the timing and amount of any milestone or royalty payments due under our existing or future license or collaboration agreements. In addition, we incur additional costs associated with operating as a public company, including significant legal, audit, accounting, regulatory and tax-related services associated with maintaining compliance with exchange listing and requirements of the SEC, director and officer liability insurance costs, investor and public relations costs, and other expenses that we did not incur as a private company. If we obtain regulatory approval for our product

candidates, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution.

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

- finalize preclinical development for our program in SYNGAP1-related disorders;
- advance current and future product candidates through preclinical and clinical studies;
- expand the capabilities of our RAP Platform and seek to identify and develop additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- contract with third-party manufacturers for preclinical and clinical supply to support any future product candidates we may develop and for commercial supply with respect to any such product candidates that receive regulatory approval;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

Because of the numerous risks and uncertainties associated with the development of therapeutics, we are unable to accurately predict the timing or amount of increased expenses and 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 as planned and may be forced to reduce or terminate our operations.

We do not have any products approved for sale and have not generated any revenue from product sales. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our current or any future product candidates, which we expect will take a number of years or may never occur. 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 equity offerings, debt financings, or other capital sources, including current and potential future collaborations, license agreements, and other similar arrangements. However, 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 or arrangements as, and when needed, we may delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise develop and market ourselves, or even cease operations.

As of September 30, 2025, we had cash and cash equivalents of $75.3 million. Based on our current operating plan, we estimate that our existing cash and cash equivalents will be sufficient to fund our projected operating expenses and capital expenditure requirements into 2027. See the sections titled “Liquidity and Capital Resources” included elsewhere in this Quarterly Report and “Risk Factors - Risks Related to our Financial Position and Need for Additional Capital” included elsewhere in this Quarterly Report and in our 2024 Form 10-K.

We do not own or operate and currently have no plans to establish any manufacturing facilities. We rely, and expect to continue to rely, on third parties for clinical supply as well as commercial supply if we obtain marketing approval. In addition, we rely on third parties to package, label, store, and distribute our clinical supply and we intend to rely on third parties to conduct the same activities for our commercial products if we obtain regulatory approval. We believe that this strategy allows us to maintain a more efficient infrastructure by eliminating the need for us to invest in our

own manufacturing facilities, equipment, and personnel while also enabling us to focus our expertise and resources on the development of product candidates and continued enhancement of our RAP Platform.

Results of Operations

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

The following table summarizes our results of operations for the three months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Revenue			
Research and collaboration revenue	$ 795	$ —	$ 795
Operating expenses:			
Research and development	9,356	9,702	(346)
General and administrative	4,596	3,814	782
Impairment of right-of-use asset	494	—	494
Total operating expenses	14,446	13,516	930
Loss from operations	(13,651)	(13,516)	(135)
Other (expense) income, net:			
Interest income	423	94	329
Change in fair value of derivative tranche liability	(1,800)	—	(1,800)
Other expense	(71)	(62)	(9)
Total other (expense) income, net	(1,448)	32	(1,480)
Net loss	$ (15,099)	$ (13,484)	$ (1,615)

Research and Collaboration Revenue

We recognized $0.8 million in research and collaboration revenue during the three months ended September 30, 2025. We did not recognize any research and collaboration revenue during the three months ended September 30, 2024. The increase of $0.8 million was due to revenue recognized under our Collaboration and License Agreement with BioMarin.

Research and Development Expenses

The following table summarizes our R&D expenses for the three months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Clinical and preclinical expenses	$ 4,313	$ 4,589	$ (276)
Personnel-related expenses	2,976	2,888	88
Facilities-related and overhead expense	1,527	1,575	(48)
Professional and consulting fees	389	469	(80)
Other expenses	151	181	(30)
Total R&D expenses	$ 9,356	$ 9,702	$ (346)

R&D expenses were $9.4 million for the three months ended September 30, 2025 compared to $9.7 million for the three months ended September 30, 2024. The decrease of $0.3 million was primarily due to a decrease of $0.3 million in clinical and preclinical expenses related to the Company's UCD program. Other R&D expenses remained relatively comparable with the same period in the previous year.

General and Administrative Expenses

The following table summarizes our G&A expenses for the three months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Personnel-related expenses	$ 1,946	$ 2,354	$ (408)
Facilities-related and overhead expense	563	362	201
Professional and consulting fees	1,815	843	972
Other expenses	272	255	17
Total G&A expenses	$ 4,596	$ 3,814	$ 782

G&A expenses were $4.6 million for the three months ended September 30, 2025 compared to $3.8 million for the three months ended September 30, 2024. The increase of $0.8 million was partially due to an increase of $1.0 million in professional and consulting fees primarily due to private placement fees associated with our tranche liability and $0.2 million in facility-related and overhead expense due to increases in insurance premiums. These increases were partially offset by a $0.4 million decrease in personnel-related expenses primarily due to higher stock-based compensation for the three months ended September 30, 2024 associated with the forgiveness of promissory notes originally issued to certain executive officers.

Impairment of Right-of-Use ("ROU") Asset

During the three months ended September 30, 2025, upon vacating our Boulder, Colorado location and pursuing a sublessee, it was determined that the market rate for similar space is less than the base rent the Company is paying under its current lease. As a result, the Company impaired the related ROU asset and recognized an impairment charge of $0.5 million.

Other (Expense) Income, Net

Other (expense) income, net for the three months ended September 30, 2025 was a $1.4 million expense compared to nominal income for the three months ended September 30, 2024. The change was primarily due to a $1.8 million expense for the change in fair value of our derivative tranche liability associated with the second tranche of our private placement. This expense was partially offset by a $0.3 million increase in interest income due to higher average invested cash balances during the three months ended September 30, 2025.

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

The following table summarizes our results of operations for the nine months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Revenue			
Research and collaboration revenue	$ 3,150	$ —	$ 3,150
Operating expenses:			
Research and development	29,845	28,821	1,024
General and administrative	12,590	10,233	2,357
Impairment of right-of-use asset	494	—	494
Total operating expenses	42,929	39,054	3,875
Loss from operations	(39,779)	(39,054)	(725)
Other (expense) income, net:			
Interest income	1,464	720	744
Change in fair value of derivative tranche liability	(1,800)	—	(1,800)
Other expense	(4)	(178)	174
Total other (expense) income, net	(340)	542	(882)
Net loss	$ (40,119)	$ (38,512)	$ (1,607)

Research and Collaboration Revenue

We recognized $3.2 million in research and collaboration revenue during the nine months ended September 30, 2025. We did not recognize any research and collaboration revenue during the nine months ended September 30, 2024. The increase of $3.2 million was due to revenue recognized under our Collaboration and License Agreement with BioMarin of $2.6 million and a $0.6 million milestone payment earned under the Fulcrum Agreement.

Research and Development Expenses

The following table summarizes our R&D expenses for the nine months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Clinical and preclinical expenses	$ 13,502	$ 13,283	$ 219
Personnel-related expenses	9,468	9,043	425
Facilities-related and overhead expense	4,475	4,557	(82)
Professional and consulting fees	1,782	1,382	400
Other expenses	618	556	62
Total R&D expenses	$ 29,845	$ 28,821	$ 1,024

R&D expenses were $29.8 million for the nine months ended September 30, 2025 compared to $28.8 million for the nine months ended September 30, 2024. The increase of $1.0 million was primarily due to an increase of $0.4 million in personnel-related expenses due to lower offsetting personnel reimbursements under one of our collaboration agreements as well as severance and related costs resulting from separation agreements with certain former employees. We also incurred an increase of $0.4 million in professional and consulting fees due to increased utilization of external support for clinical operations. In addition, we incurred an increase of $0.2 million in clinical and preclinical expenses related to the Company's UCD clinical trial and SYNGAP preclinical work.

General and Administrative Expenses

The following table summarizes our G&A expenses for the nine months ended September 30, 2025 and 2024 (in thousands):

	2025	2024	Change ($)
Personnel-related expenses	6,233	5,752	$ 481
Professional and consulting fees	3,608	2,746	862
Facilities-related and overhead expense	1,613	1,098	515
Other expenses	1,136	637	499
Total G&A expenses	$ 12,590	$ 10,233	$ 2,357

G&A expenses were $12.6 million for the nine months ended September 30, 2025 compared to $10.2 million for the nine months ended September 30, 2024. The increase of $2.4 million was partially due to an increase of $0.5 million in facility-related and overhead expense due to increases in insurance premiums. In addition, there was an increase of $0.5 million in other expenses due to an increase in state franchise taxes, as well as costs associated with operating as a public company. There was also an increase of $0.5 million in personnel-related expenses due to annual merit-related salary increases, partially offset by decreases in stock compensation expense primarily due to higher stock-based compensation for the nine months ended September 30, 2024 associated with the forgiveness of promissory notes originally issued to certain executive officers. Finally, there was an increase of $0.9 million in professional and consulting-related expenses due to costs associated with operating as a public company.

Impairment of Right-of-Use Asset

During the nine months ended September 30, 2025, upon vacating our Boulder, Colorado location and pursuing a sublessee, it was determined that the market rate for similar space is less than the base rent the Company is paying under its current lease. As a result, the Company impaired the related ROU asset and recognized an impairment charge of $0.5 million.

Other (Expense) Income, Net

Other (expense) income, net was an expense of $0.3 million for the nine months ended September 30, 2025 compared to income of $0.5 million for the nine months ended September 30, 2024. The change of $0.9 million was primarily due to a $1.8 million expense for the change in fair value of our derivative tranche liability associated with the second tranche of our private placement. This was partially offset by a $0.7 million increase in interest income due to higher average invested cash equivalent balances during the nine months ended September 30, 2025 and a decrease in other expenses of $0.2 million primarily due to lower foreign exchange losses.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from operations. We expect to incur significant expenses and operating losses in the foreseeable future as we advance the development of product candidates. Through September 30, 2025, we have primarily funded our operations with proceeds from the sale of our equity securities and revenues from our license and collaboration agreements.

As of September 30, 2025, we had cash and cash equivalents of $75.3 million. Based on our current operating plan, we estimate that our cash and cash equivalents as of September 30, 2025 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 deplete our capital resources sooner than we currently expect. Our future viability is dependent on our ability to generate cash from our operating activities or to raise additional capital to finance our operations. There is no assurance that we will succeed in obtaining sufficient funding on terms acceptable to us to fund continuing operations, if at all.

Future Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we continue our development of, seek regulatory approval for, and potentially commercialize our product candidates and seek to discover and develop additional product candidates, conduct our ongoing and planned clinical trials and preclinical studies, continue our R&D activities, hire additional personnel, expand and protect our intellectual property, and incur additional costs associated with being a public company.

The timing and amount of our funding requirements will depend on many factors, including:

- the initiation, type, number, scope, progress, expansions, results, costs and timing of preclinical studies and clinical trials of our product candidates and any future product candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;
- the costs and timing of manufacturing for our product candidates, including commercial manufacturing at sufficient scale, if any product candidate is approved;
- the costs, timing and outcome of regulatory meetings and reviews of product candidates or any future product candidates, including requirements of regulatory authorities in any additional jurisdictions in which we may seek approval and any future product candidates;
- the costs of obtaining, maintaining, enforcing and protecting our patents and other intellectual property and proprietary rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company, including enhanced internal control over financial reporting;
- the costs associated with hiring additional personnel and consultants as our clinical and preclinical activities increase and as we operate as a public company;
- the timing and payment of milestone, royalty or other payments we must make or may receive pursuant to our existing and potential future license or collaboration agreements with third parties;
- the costs and timing of establishing or securing sales and marketing capabilities if our product candidates or any future product candidate is approved;
- our ability to achieve sufficient market acceptance, coverage, and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- patients' ability and willingness to pay out-of-pocket for any approved products in the absence of coverage and/or adequate reimbursement from third-party payors;
- the terms and timing of establishing and maintaining collaborations, licenses, and other similar arrangements; and
- costs associated with any products or technologies that we may in-license or acquire.

Our operating plans and other demands for our cash resources may change because of many factors currently unknown to us, and we may need to seek additional funds sooner than planned.

We have no other committed sources of capital. Until such time, if ever, we can generate substantial product revenues, we expect to finance our operations through the sale of equity securities, debt financings, working capital lines of credit, strategic alliances and/or license arrangements, grant funding, interest income earned on invested cash balances or a combination of two or more of these sources. However, we may be unable to raise additional funds or enter into such other arrangements when needed, on favorable terms or at all. To the extent we raise additional capital through the sale of equity or convertible debt securities, investors' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of a common stockholder. 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, engaging in acquisition, merger or collaboration transactions, selling or licensing our assets, making capital expenditures, redeeming our stock, making certain investments or declaring dividends. If we raise additional funds through collaborations or license agreements 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, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, or even cease operations.

Contractual and Other Obligations

As of September 30, 2025, other than those disclosed within Note 4, 5, and 6 to our condensed consolidated financial statements, there have been no material changes to our contractual obligations and commitments from those described under "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our 2024 Form 10-K.

Cash Flows

For the Nine Months Ended September 30, 2025 and 2024

The following table provides information regarding our cash flows for the nine months ended September 30, 2025 and 2024 (in thousands):

	2025	2024
Net cash used in operating activities	$ (35,846)	$ (34,274)
Net cash used in investing activities	(243)	(178)
Net cash provided by (used in) financing activities	47,305	(1,400)
Net change in cash, cash equivalents, and restricted cash	$ 11,216	$ (35,852)

Operating Activities

Cash flows used in operations are primarily attributed to clinical and preclinical expenses, employee compensation, rent and variable rent expenses for our Cambridge and Boulder leases as well as professional fees. Our cash flows from operating activities are significantly affected by our use of cash for operating expenses and working capital to support the business.

Net cash used in operating activities for the nine months ended September 30, 2025 was $35.8 million and consisted of net loss of $40.1 million, non-cash adjustments of $7.9 million and a net change in assets and liabilities of $3.7 million. Non-cash items primarily included stock-based compensation of $2.8 million, loss from the change in fair value of our tranche liability of $1.8 million, non-cash operating lease expense of $1.4 million, depreciation and amortization of $1.3 million, and impairment of the Boulder lease ROU asset of $0.5 million. The net change in assets and liabilities was primarily due to $2.6 million in lease payments, a decrease in accounts payable of $0.6 million due to the timing of payments, a decrease in accrued expenses of $0.4 million due to the payout of annual employee bonuses and cash paid to certain executive officers to offset the tax impact associated with the forgiveness of promissory notes from 2024, partially offset by the current year accrual for annual employee bonuses and higher accrued R&D expenses.

Net cash used in operating activities for the nine months ended September 30, 2025, was $1.6 million higher than the same period in 2024. The increase in cash used for operations was primarily due to the $2.0 million higher net change in assets and liabilities, partially offset by higher non-cash adjustments of $2.1 million and a lower net loss of $1.6 million. The higher net change in assets and liabilities was primarily due to the $1.6 million change in accounts payable due to the timing of payments and the increased costs of becoming a public company. The higher non-cash adjustment was primarily due to loss on the change in fair value of our tranche liability of $1.8 million and the impairment of the Boulder lease ROU asset of $0.5 million.

Investing Activities

Our primary investing activities consist of capital expenditures for laboratory and computer equipment as well as software. Net cash used in investing activities for the nine months ended September 30, 2025 was $0.2 million, due to purchases of laboratory equipment, partially offset by the sale of equipment used in our Boulder location. Net cash used in investing activities increased by $0.1 million during the nine months ended September 30, 2025, compared to the same period in 2024, primarily attributable to the timing of cash payments for laboratory equipment, partially offset by lower spend for the nine months ended September 30, 2025.

Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2025 was $47.3 million. Our primary financing activity for the nine months ended September 30, 2025 was our private placement, which resulted in cash net of issuance costs totaling $32.6 million for securities sold in the initial close and $14.9 million allocated to the derivative tranche liability. Excluding the cash provided by the private placement, net cash used in financing activities decreased $16.1 million during the nine months ended September 30, 2025, compared to the same period in 2024. The decrease was primarily attributable to cash used in 2024 of $0.8 million for deferred offering costs and a $0.4 million decrease in repayments of our finance leases and financing liability.

Critical Accounting Policies and Significant Judgments and Estimates

There have been no significant changes to our critical accounting estimates in the preparation of our condensed consolidated financial statements during the nine months ended September 30, 2025 compared to those disclosed in our 2024 Form 10-K except for the following.

Valuation of Derivative Tranche Liability

In connection with our Private Placement in September 2025, we had a commitment and investors had an obligation to purchase securities in a second closing at a fixed price, if specified conditions are met. The obligation to issue additional securities at a future date was determined to be a freestanding derivative instrument and is accounted for as a liability. The derivative tranche liability was accounted for at fair value at the issuance date and remeasured at the end of each reporting period until the shares are issued or the obligation expires. Changes in the fair value of the derivative tranche liability are recognized in the condensed consolidated statement of operations.

The fair value of the derivative tranche liability was determined using a probability weighted model, which considered as inputs the probability of achieving tranche closing conditions, the estimated fair value of our common stock and a discount rate. We recognized a $1.8 million loss for the nine months ended September 30, 2025, related to the change in fair value of the derivative tranche liability.

Recently Issued Accounting Standards

See Note 2 to our condensed consolidated financial statements included elsewhere in this Quarterly Report for more information.

Emerging Growth Company and Smaller Reporting Company Status

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"), and we may remain an emerging growth company until December 31, 2029 or until such earlier time that we are no longer an emerging growth company. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved and an exemption from compliance with the requirements regarding the communication of critical audit matters in the auditor's report on financial statements.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have elected to avail ourselves of this

extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies. As a result of this election, our financial statements may not be comparable to those of companies that are not emerging growth companies.

We will remain an emerging growth company until the earliest to occur of: (i) the last day of the fiscal year in which we have at least $1.235 billion in annual revenue; (ii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer," as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded $700.0 million as of the last business day of the second fiscal quarter of such year; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt securities during the prior three-year period; and (iv) December 31, 2029.

We are also a "smaller reporting company," meaning that the market value of our shares held by non-affiliates is less than $700.0 million and our annual revenue was less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either: (i) the market value of our shares held by non-affiliates is less than $250.0 million; or (ii) our annual revenue was less than $100.0 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Not applicable to a smaller reporting company.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 as of September 30, 2025. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by us in the reports that we file 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 a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2025, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2025 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

From time to time, we may be subject to legal proceedings. We are not currently a party to or aware of any proceedings that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

Item 1A. Risk Factors

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider the factors discussed in Part I, "Item 1A. Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2024 (the "2024 Form 10-K"), which could materially affect our business, financial condition or future results. The risk factors disclosure in our 2024 Form 10-K is qualified by the information that is described in this Quarterly Report on Form 10-Q. The risks described in our 2024 Form 10-K are not our only risks. Additional risks and uncertainties not presently known to us or that we currently believe to be immaterial also may materially adversely affect our business, financial condition or future results. There have been no material changes to our risk factors as previously disclosed in the 2024 Form 10-K except as follows:

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.

We are a clinical-stage biopharmaceutical company in the early stages of development with a limited operating history. Since our inception, we have focused primarily on developing our proprietary RNA Actuating Platform, or RAP Platform, identifying, developing and progressing our product candidates through preclinical and clinical development, organizing and staffing our company, research and development activities, establishing and protecting our intellectual property portfolio, and raising capital. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We are still in the early stages of development of our product candidates and our most advanced product candidate is only in a Phase 1 clinical trial. We have no products licensed for commercial sale and have not generated any revenue to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. For the years ended December 31, 2024 and 2023, we reported net losses of $51.8 million and $49.3 million, respectively, and for the nine months ended September 30, 2025 and 2024, we reported net losses of $40.1 million and $38.5 million, respectively. As of September 30, 2025, we had an accumulated deficit of $251.9 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue the research and development of and seek regulatory approvals for our current product candidates and any other future product candidates we may develop.

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

- finalize preclinical development for our program in SYNGAP1-related disorders;
- advance current and future product candidates through preclinical and clinical studies;
- expand the capabilities of our RAP Platform and seek to identify and develop additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend, and enforce our intellectual property portfolio;
- hire additional clinical, regulatory, and scientific personnel;
- contract with third-party manufacturers for preclinical and clinical supply to support any future product candidates we may develop and for commercial supply with respect to any such product candidates that receive regulatory approval;

- ultimately establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and

- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, and future commercialization efforts, as well as to support our operations as a public company.

Even if we obtain regulatory approval for, and are successful in commercializing, one or more of any of our current and any future product candidates, we will continue to incur substantial research and development and other costs to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require substantial additional capital to finance our operations, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce, or terminate our development programs, commercialization efforts or other operations.

Our operations have consumed substantial amounts of cash since inception. We expect our expenses to substantially increase in connection with our ongoing activities, particularly as we conduct our ongoing and planned clinical trials and preclinical studies and potentially seek regulatory approval for our product candidates and any future product candidates we may develop. If we obtain regulatory approval for any of our product candidates, we also expect to incur significant commercialization expenses related to product manufacturing, marketing, sales, and distribution. Because the outcome of any clinical trial or preclinical study is highly uncertain, we cannot reasonably estimate the actual amount of capital necessary to successfully complete the development and commercialization of our product candidates.

As of September 30, 2025, we had cash and cash equivalents of $75.3 million. Based on our current operating plan, we estimate that our cash and cash equivalents as of September 30, 2025 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 deplete our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we do not expect that our existing cash and cash equivalents will be sufficient to complete development of any of our product candidates, or any future product candidates we may identify, and we will require substantial capital in order to advance our product candidates through clinical trials, regulatory approval and commercialization. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. Our ability to raise additional funds may be adversely impacted by global economic conditions, disruptions to, and volatility in, the credit and financial markets in the United States and worldwide, and diminished liquidity and credit availability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce, or eliminate our research and development programs or any future commercialization efforts, or even cease operations. We expect to finance our cash needs through public or private equity or debt financings or other capital sources, including potential collaborations, licenses, and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Attempting to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop our product candidates.

Our future capital requirements will depend on many factors, including, but not limited to:

- the initiation, type, number, scope, progress, expansions, results, costs and timing of preclinical studies and clinical trials of our product candidates and any future product candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;

- the costs and timing of manufacturing for our product candidates, including commercial manufacturing at sufficient scale, if any product candidate is approved;

- the costs, timing and outcome of regulatory meetings and reviews of our product candidates or any future product candidates, including requirements of regulatory authorities in any additional jurisdictions in which we may seek approval and any future product candidates;
- the costs of obtaining, maintaining, enforcing, and protecting our patents and other intellectual property and proprietary rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company, including enhanced internal control over financial reporting;
- the costs associated with hiring additional personnel and consultants as our clinical and preclinical activities increase;
- the timing and payment of milestone, royalty, or other payments we must make or may receive pursuant to our existing and potential future license or collaboration agreements with third parties;
- the costs and timing of establishing or securing sales and marketing capabilities if our product candidates or any product candidate is approved;
- our ability to achieve sufficient market acceptance, coverage, and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- patients' ability and willingness to pay out-of-pocket for any approved products in the absence of coverage and/or adequate reimbursement from third-party payors;
- the terms and timing of establishing and maintaining collaborations, licenses, and other similar arrangements; and
- costs associated with any products or technologies that we may in-license or acquire.

Conducting clinical trials and preclinical studies and discovering potential product candidates using our RAP Platform is an expensive and uncertain process, and we may never generate the necessary data or results required to obtain regulatory approval and commercialize our product candidates. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will initially be derived from sales of products that we do not expect to be commercially available for many years, if at all.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of common stockholders. Any debt financing or 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, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

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 or other arrangements when needed or on terms acceptable to us, we may be required to delay, limit, reduce or eliminate some or all of our research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. We recently announced that we plan to pursue partnership opportunities to support the further development of CMP-001 (formerly known as CMP-CPS-001). However, we cannot provide assurance that a partnership will be consummated.

Risks Related to the Research and Development of Our Product Candidates

Our business is highly dependent on our lead development candidate, CMP-002 (formerly known as CMP-SYNGAP-01), and we must complete clinical testing before we can seek regulatory approval and begin commercialization of any of our product candidates. If we are unable to obtain regulatory approval for, and successfully commercialize, our current product candidates, our business may be materially harmed, and such failure may affect the viability of any future product candidates.

There is no guarantee that any of our product candidates will proceed in preclinical or clinical development or achieve regulatory approval. The process for obtaining marketing approval for any product candidate is very long and risky and there will be significant challenges for us to address in order to obtain marketing approval as planned, if at all.

There is no guarantee that we will be able to advance CMP-002, our lead development candidate, into clinical trials, nor that the results obtained in our planned future clinical trials will be sufficient to obtain regulatory approval for any current or future product candidates. In addition, because our product candidates will be based on our RAP Platform and antisense oligonucleotide ("ASO") technology, if any of our current product candidates encounters safety or efficacy problems, developmental delays, regulatory issues, or other problems, our development plans and business related to our other current or future product candidates could be significantly harmed. A failure of one of our current product candidates may affect the ability to obtain regulatory approval to continue or conduct clinical programs for our other current or future product candidates.

We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications that we believe can be addressed by our technology among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. We recently announced our intention, pending successful completion of GLP toxicology studies and regulatory clearance, to initiate a global Phase 1/2 clinical trial in individuals with SYNGAP1-related disorders as early as the second half of 2026 and to pursue partnership opportunities to support the further development of CMP-001 for individuals with urea cycle disorders. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Our Business Operations and Industry

International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and growth prospects.

We rely on third-party suppliers located in, and conduct clinical trials in, countries outside the United States. There is inherent risk, based on the complex relationships among the United States and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The U.S. government recently announced tariffs affecting a wide range of products and jurisdictions and has indicated an intention to continue developing new trade policies, including with respect to the pharmaceutical industry. In response, certain foreign governments have announced or implemented retaliatory tariffs and other protectionist measures. These developments have created a dynamic and unpredictable trade landscape, which is adversely impacting, and may continue to adversely impact, our business.

We do not own or operate and currently have no plans to establish any manufacturing facilities. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates for clinical testing, as well as for the manufacture of any products that we may commercialize, if approved. Current or future tariffs are likely to result in

increased research and development expenses, including with respect to increased costs associated with active pharmaceutical ingredients, raw materials, laboratory equipment and research materials and components. In addition, such tariffs could increase our supply chain complexity, disrupt our existing supply chain and cause delays in our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence, negatively impacting our ability to secure additional financing on favorable terms or at all. In addition, if we succeed in commercializing any drug products, tariffs and trade restrictions could hinder our ability to establish cost-effective production capabilities, negatively impacting our growth prospects.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, results of operations and financial condition. In addition, trade developments have and may continue to heighten the risks related to other risk factors described in our 2024 Form 10-K.

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

(a) Sales of Unregistered Securities

None.

(b) Use of Proceeds from Public Offering of Common Stock

On October 10, 2024, our Registration Statement on Form S-1, as amended (File No. 333-282241), was declared effective in connection with our initial public offering ("IPO"). The aggregate net proceeds from our IPO, after deducting underwriting discounts and commissions and expenses payable by us, were $72.4 million. There has been no material change in the planned use of proceeds from our IPO as described in the final prospectus for our IPO filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the SEC on October 11, 2024. We are holding the balance of the net proceeds in cash and cash equivalents.

(c) Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

During the quarter ended September 30, 2025, no "Rule 10b5-1 plans" or "non-Rule 10b5-1 trading arrangements," as each term is defined in Item 408(a) of Regulation S-K, were adopted, modified, or terminated by officers or directors of the Company.

Item 6. Exhibits

See Exhibit Index.

EXHIBIT INDEX

Exhibit No.	Description
3.1	Fifth Amended and Restated Certificate of Incorporation of CAMP4 Therapeutics Corporation (incorporated by reference to Exhibit 3.1 to the Form 8-K filed on October 15, 2024, File No. 001-42365)
3.2	Amended and Restated Bylaws of CAMP4 Therapeutics Corporation (incorporated by reference to Exhibit 3.2 to the Form 8-K filed on October 15, 2024, File No. 001-42365)
4.1	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Form 8-K filed on September 10, 2025, File No. 001-42365)
10.1	Securities Purchase Agreement, dated September 9, 2025, by and among the Company and the investors party thereto (incorporated by reference to Exhibit 10.1 to the Form 8-K filed on September 10, 2025, File No. 001-42365)
10.2	Registration Rights Agreement, dated September 9, 2025, by and among the Company and the investors party thereto (incorporated by reference to Exhibit 10.2 to the Form 8-K filed on September 10, 2025, File No. 001-42365)
31.1†	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2†	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1†*	Certification of Chief Executive Officer Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2†*	Certification of Chief Financial Officer Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS†	XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
101.SCH†	XBRL Taxonomy Extension Schema Document
101.CAL†	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF†	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB†	XBRL Taxonomy Extension Label Linkbase Document
101.PRE†	XBRL Taxonomy Extension Presentation Linkbase Document
104†	Cover Page Interactive Data File (formatted in Inline XBRL and contained in Exhibit 101)

† Filed herewith.

* This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent specifically incorporated by reference into such filing.

SIGNATURES

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

Date: November 6, 2025

CAMP4 Therapeutics Corporation

By:	/s/ Josh Mandel-Brehm
Name:	Josh Mandel-Brehm
Title:	President and Chief Executive Officer
	(Principal Executive Officer)

By:	/s/ Kelly Gold
Name:	Kelly Gold
Title:	Chief Financial Officer
	(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

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

I, Josh Mandel-Brehm, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CAMP4 Therapeutics Corporation;

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

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

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

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

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

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

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

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

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

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

Dated: November 6, 2025

By: /s/ Josh Mandel-Brehm

Name: Josh Mandel-Brehm

Title: President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

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

I, Kelly Gold, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CAMP4 Therapeutics Corporation;

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

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

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

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

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

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

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

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

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

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

Dated: November 6, 2025

By: /s/ Kelly Gold

Name: Kelly Gold

Title: Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of CAMP4 Therapeutics Corporation (the "Company") hereby certifies, to the best of my knowledge, that:

(i) the accompanying Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2025 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934; and

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

Dated: November 6, 2025

By: /s/ Josh Mandel-Brehm

Name: Josh Mandel-Brehm

Title: President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of CAMP4 Therapeutics Corporation (the "Company") hereby certifies, to the best of my knowledge, that:

(i) the accompanying Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2025 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934; and

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

Dated: November 6, 2025

By: /s/ Kelly Gold

Name: Kelly Gold

Title: Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)