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 June 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: 001-36510

LARIMAR THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**20-3857670**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
Three Bala Plaza East, Suite 506 Bala Cynwyd, PA	**19004**
(Address of principal executive offices)	**(Zip Code)**

(844) 511-9056

(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	LRMR	The Nasdaq Global Market

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

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

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

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

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

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

As of August 12 2025, there were 85,590,392 shares of the registrant's Common Stock, $0.001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Statements made in this Quarterly Report on Form 10-Q that are not statements of historical or current facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements discuss our business, operations and financial performance and conditions, as well as our plans, objectives and expectations for our business operations and financial performance and condition. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "predict," "positioned," "potential," "seek," "should," "target," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. In addition, statements that "we believe" or similar statements reflect our beliefs and opinions on the relevant subject only. These forward-looking statements, which are subject to risks, uncertainties and assumptions about us, may include projections of our future financial performance, our anticipated growth strategies and anticipated trends in our business.

You should understand that the following important factors could affect our future results and could cause those results or other outcomes to differ materially from those expressed or implied in our forward-looking statements:

- uncertainties in obtaining successful non-clinical or clinical results that reliably and meaningfully demonstrate safety, tolerability and efficacy profiles that are satisfactory to the U.S. Food and Drug Administration ("FDA"), European Medicines Agency ("EMA") and other comparable regulatory authorities for marketing approval for nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601) or any other product candidates that we may develop in the future and unexpected costs that may result therefrom;
- delays in patient recruitment for our clinical trials (including as a result of the impact of FDA approval of competitive products for the treatment of Friedreich's ataxia ("FA"), and/or the impact of other clinical trials of competitive products), delays as a result of clinical and non-clinical results and/or the FDA's request for additional information or studies (whether clinical or non-clinical), changes in clinical protocols, adverse events, regulatory restrictions, including additional clinical holds, and milestones for nomlabofusp;
- our ability to successfully execute our ongoing open label trial and our pediatric PK run-in trial, including the timing of site initiations and the rate of patient enrollment;
- our ability to benefit from participating in the FDA's Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") pilot program for the development of nomlabofusp;
- uncertainties associated with the clinical development and regulatory approval for nomlabofusp, including potential delays in the commencement, enrollment and completion of clinical trials, the timing of a potential Biologics License Application ("BLA") submission for accelerated approval, including our ability to supply to the FDA all required data for the FDA to review and accept an accelerated application, or any other product candidates that we may develop in the future;
- the difficulties and expenses associated with obtaining and maintaining regulatory approval for nomlabofusp or any other product candidates we may develop in the future, and the indication and labeling under any such approval;
- how long we can continue to fund our operations with our existing cash, cash equivalents and marketable securities and our estimates regarding future results of operations, financial position, research and development costs, capital requirements and our access and needs for additional financing;
- our expectations regarding the use of proceeds from the recently completed financing and any future financings;

•our ability, and the ability of third-party manufacturers we engage, to optimize, scale and validate nomlabofusp or any other product candidate's manufacturing process and to manufacture sufficient quantities of clinical supplies, and, if approved, commercial supplies of nomlabofusp or any other product candidates that we may develop in the future and our ability to maintain our relationships and contracts with our key vendors and to identify and contract with alternate or secondary key vendors;

•our ability to realize any value from nomlabofusp and/or any other product candidates we may develop in the future in light of inherent risks and difficulties involved in successfully bringing product candidates to market and the risk that the product candidates, if approved, will not achieve broad market acceptance;

•our ability to comply with regulatory requirements applicable to our business and other regulatory developments in the United States and other countries;

•the size and growth of the potential markets for nomlabofusp, if approved, or any other product candidates that we may develop in the future, the rate and degree of market acceptance of nomlabofusp or any other product candidate, if approved, that we may develop in the future and our ability to serve those markets;

•given both approved and competing therapies and products for the treatment of FA, our ability to obtain and maintain designations or eligibility for expedited regulatory programs, and to commercialize current and future product candidates, if approved, (including the impact of potential barriers to entry if a competitor is able to establish a strong market position before we are able to commercialize our products);

•our ability to obtain and maintain patent protection and defend our intellectual property rights against third parties;

•the performance and compliance with the rules and regulations of the FDA (and all other regulatory authorities) of third parties upon which we depend, including third-party contract research organizations ("CROs"), consultants, and third-party suppliers, manufacturers, distributors, and logistics providers;

•our ability to recruit and retain key scientific, technical, commercial, and management personnel and to retain our executive officers;

•our ability to maintain proper functionality and security of our internal computer and information systems and prevent or avoid cyber-attacks, malicious intrusion, breakdown, destruction, loss of data privacy or other significant disruption;

•the extent to which geopolitical tensions, including regional conflicts around the world, adverse macroeconomic events, including those due to inflationary pressures, rising interest rates, banking instability, monetary policy changes, changes in trade policies, including tariffs (including tariffs or trade protection measures that have been or may in the future be imposed by the U.S. or other countries), economic slowdowns or recessions, health epidemics, unforeseen emergencies and other outbreaks of communicable diseases that could disrupt our operations, the operations of third parties on which we rely or the operations of regulatory agencies we interact with in the development of nomlabofusp and any other product candidates that we may develop; and

•the potential impact of regulatory developments in the U.S., including regulatory developments due to changes in the U.S. presidential administration, healthcare reform in the United States, including the Inflation Reduction Act of 2022 ("IRA"), and measures being taken worldwide designed to reduce healthcare costs and limit the overall level of government expenditures.

These forward-looking statements are based on management's current expectations, estimates, forecasts and projections about our business and the industry in which we operate, and management's beliefs and assumptions are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe the expectations reflected in the forward-looking statements are reasonable, the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements may not be achieved

or occur at all. The factors that could cause or contribute to such differences include, but are not limited to, those discussed in our Annual Report on Form 10-K filed on March 24, 2025 and our Quarterly Report on Form 10-Q filed on April 30, 2025. All forward-looking statements are applicable only as of the date on which they were made and, except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise after the date of this Quarterly Report on Form 10-Q or to reflect the occurrence of any unanticipated events. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless expressed as such, and should only be viewed as historical data.

Larimar Therapeutics, Inc.

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PART I-FINANCIAL INFORMATION

Item 1. Financial Statements

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)
(Unaudited)

	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 20,587	$ 33,218
Short-term marketable securities	117,937	150,236
Prepaid expenses and other current assets	7,032	11,850
Total current assets	145,556	195,304
Property and equipment, net	797	881
Operating lease right-of-use assets	2,468	2,838
Restricted cash	606	606
Other assets	561	596
Total assets	$ 149,988	$ 200,225
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,169	$ 2,424
Accrued expenses	21,351	20,872
Operating lease liabilities, current	1,129	1,060
Total current liabilities	26,649	24,356
Operating lease liabilities	3,485	4,057
Total liabilities	30,134	28,413
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of June 30, 2025 and December 31, 2024; no shares issued and outstanding as of June 30, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2025 and December 31, 2024; 64,027,892 and 63,815,065 shares issued and outstanding as of June 30, 2025 and December 31, 2024, respectively	64	64
Additional paid-in capital	444,420	440,758
Accumulated deficit	(324,621)	(269,158)
Accumulated other comprehensive gain (loss)	(9)	148
Total stockholders' equity	119,854	171,812
Total liabilities and stockholders' equity	$ 149,988	$ 200,225

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 23,368	$ 19,682	$ 49,919	$ 32,621
General and administrative	4,424	4,917	9,060	8,712
Total operating expenses	27,792	24,599	58,979	41,333
Loss from operations	) (27,792	) (24,599	) (58,979	) (41,333
Other income, net	1,610	2,972	3,516	5,052
Net loss	$ (26,182)	$ (21,627)	$ (55,463)	$ (36,281)
Net loss per share, basic and diluted	$ (0.41)	$ (0.34)	$ (0.87)	$ (0.62)
Weighted average common shares outstanding, basic and diluted	64,027,892	63,801,792	63,996,126	58,677,749
Comprehensive loss:				
Net loss	$ (26,182)	$ (21,627)	$ (55,463)	$ (36,281)
Other comprehensive loss:				
Unrealized loss on marketable securities	(63)	(125)	(157)	(231)
Total other comprehensive loss	(63)	(125)	(157)	(231)
Total comprehensive loss	$ (26,245)	$ (21,752)	$ (55,620)	$ (36,512)

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional		Accumulated Other	Total
	Shares	Par Value	Paid-in Capital	Accumulated Deficit	Comprehensive Gain (Loss)	Stockholders' Equity
Balances as of December 31, 2024	63,815,065	$ 64	$ 440,758	$ (269,158)	$ 148	$ 171,812
Vesting of restricted stock units	212,827	—	—	—	—	—
Stock-based compensation expense	—	—	1,834	—	—	1,834
Unrealized loss on marketable securities	—	—	—	—	(94)	(94)
Net loss	—	—	—	(29,281)	—	(29,281)
Balances as of March 31, 2025	64,027,892	$ 64	$ 442,592	$ (298,439)	$ 54	$ 144,271
Stock-based compensation expense	—	—	1,828	—	—	1,828
Unrealized loss on marketable debt securities	—	—	—	—	(63)	(63)
Net loss	—	—	—	(26,182)	—	(26,182)
Balances as of June 30, 2025	64,027,892	$ 64	$ 444,420	$ (324,621)	$ (9)	$ 119,854

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(In thousands, except share data)
(Unaudited)
(Continued)

	Common Stock		Additional		Accumulated Other	Total
	Shares	Par Value	Paid-in Capital	Accumulated Deficit	Comprehensive Gain (Loss)	Stockholders' Equity
Balances as of December 31, 2023	43,909,069	$ 43	$ 270,150	$ (188,554)	$ 81	$ 81,720
Issuance of common stock, net	19,736,842	20	161,736	—	—	161,756
Vesting of restricted stock units	153,750	1	(1)	—	—	—
Exercise of stock options	356	—	—	—	—	—
Stock-based compensation expense	—	—	2,128	—	—	2,128
Unrealized loss on marketable securities	—	—	—	—	(106)	(106)
Net loss	—	—	—	(14,654)	—	(14,654)
Balances as of March 31, 2024	63,800,017	$ 64	$ 434,013	$ (203,208)	$ (25)	$ 230,844
Stock-based compensation expense	—	—	2,301	—	—	2,301
Exercise of stock options	2,500	—	11	—	—	11
Unrealized loss on marketable debt securities	—	—	—	—	(125)	(125)
Net loss	—	—	—	(21,627)	—	(21,627)
Balances as of June 30, 2024	63,802,517	$ 64	$ 436,325	$ (224,835)	$ (150)	$ 211,404

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)
(Unaudited)

	Six Months Ended June 30,	
	2025	2024
Cash flows from operating activities:		
Net loss	$ (55,463)	$ (36,281)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	3,662	4,429
Lease expense	(133)	(86)
Depreciation expense	175	167
Amortization of premium on marketable securities	(1,216)	(2,361)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	4,818	(1,681)
Accounts payable	1,722	1,626
Accrued expenses	479	9,765
Other assets	35	23
Net cash used in operating activities:	(45,921)	(24,399)
Cash flows from investing activities:		
Purchases of property and equipment	(68)	(274)
Purchases of marketable securities	(80,142)	(166,582)
Maturities of marketable securities	113,500	35,000
Net cash provided by (used in) investing activities	33,290	(131,856)
Cash flows from financing activities:		
Proceeds from issuance of equity securities, net of issuance costs	—	161,806
Proceeds from exercise of stock options and warrants	—	11
Net cash provided by financing activities	—	161,817
Net increase in cash, cash equivalents and restricted cash	(12,631)	5,562
Cash, cash equivalents and restricted cash at beginning of period	33,824	28,088
Cash, cash equivalents and restricted cash at end of period	$ 21,193	$ 33,650
Supplemental disclosure of non-cash investing and financing activities:		
Purchases of property and equipment included in accounts payable and accrued expenses	$ 23	$ 53
Offering costs included in accrued expense	$ 50	$ 50
Leased assets obtained in exchange for new operating lease liabilities	$ —	$ 465

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

LARIMAR THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1.Description of Business and Basis of Presentation

Larimar Therapeutics, Inc., together with its subsidiary (the "Company" or "Larimar"), is a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using its novel cell penetrating peptide technology platform. Larimar's lead product candidate, nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601), is a subcutaneously administered, recombinant fusion protein intended to deliver human frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality.

Larimar initiated human clinical studies of nomlabofusp in 2019. Since then, the Company has completed two Phase 1 clinical studies, a Phase 2 dose exploration study and a PK run-in study in adolescents (12-17 years old).

In 2024, the Company initiated its open label extension study in patients with FA. The open label extension study's eligibility criteria only allowed patients who previously participated in the Company's prior nomlabofusp Phase 1 or Phase 2 studies. In 2025, the Company changed the name of the open label extension study to the open label study, due to the inclusion of patients who had not participated in prior nomlabofusp clinical studies. The Company's ongoing open label study is currently screening and enrolling eligible adolescents that completed dosing in the recently completed PK run-in study into the open label study. In addition, the Company is also screening and enrolling patients with FA who have not participated in prior nomlabofusp clinical studies. The Company is also planning to enroll children (2-11 years of age) directly into the open label study.

The Company has engaged in multiple discussions and interactions with the U.S. Food and Drug Administration ("FDA") in connection with its clinical development of nomlabofusp and such communications remain ongoing. The Company currently participates in the FDA's Center for Drug Evaluation and Research ("CDER") Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") pilot program. The objective of this program is to accelerate the development of drugs for rare diseases that lead to significant disability or death by facilitating frequent advice and regular communication with the FDA staff to expedite the review process.

The Company has also had numerous interactions with the European Medicines Agency ("EMA") and the United Kingdom's Medicines and Healthcare Regulatory Agency ("MHRA") regarding the clinical development of nomlabofusp.

The Company has received Orphan Drug Designation and Fast Track Designation from the FDA. The Company has been granted orphan drug designation in the European Union (the "EU") and access to the EU's Priority Medicines Program ("PRIME") Scheme. The Company has also received access to the EMA's Innovative Licensing and Access Pathway ("ILAP"). All these programs are designed to facilitate drug development of therapeutics for rare and/or orphan indications.

Recent significant developments are as follows:

•In December 2024, the Company reported positive initial data from its ongoing open label study. This data included safety, FXN levels, clinical and pharmacokinetic data. Also in December 2024, the Company announced that they were increasing the dose in the open label study to 50 mg of nomlabofusp daily for then currently enrolled participants. All newly enrolled participants will start dosing at 50 mg nomlabofusp daily. In March 2025, the Company announced that its Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore they expect to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, the Company amended the open label study protocol to administer premedication with antihistamines for the first month of dosing.

•In January 2025, the Company initiated dosing of adolescents (12-17 years old) in its PK run-in study for patients with FA. Study participants were randomized 2:1 to receive either nomlabofusp at a

weight-base dose expected to match the PK of adults receiving the 50 mg dose, or placebo, daily for seven days. The Company completed dosing of 14 adolescents in March 2025.

•In February 2025, FDA accepted the data supporting the comparability of the lyophilized drug product to the frozen solution and agreed with the Company's plans to introduce the lyophilized product into its clinical development program. The frozen solution is the dosage form currently being used in the open label study. In May 2025, the Company began to introduce the lyophilized product formulation, which is intended for commercialization, into the open label study with new study participants.

•In March 2025, the Company announced that FDA stated as part of a START pilot program that it is open to considering the use of FXN concentration as a reasonably likely surrogate endpoint ("RLSE") and the acceptability of FXN concentration as an RLSE to support accelerated approval will be a matter of review in a future marketing application. FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that data the Company recently submitted appear to support a relationship between increased FXN concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA also acknowledged that the nonclinical studies the Company submitted were performed at relevant human doses. FDA also suggested that the Company consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic ("PD") markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as an RLSE. The Company continues to interact with the FDA under the START pilot program.

•In June 2025, the FDA provided safety database recommendations for a Biologics License Application, ("BLA"), seeking accelerated approval, which included evaluating safety in at least 30 participants with continuous study drug exposure for six months and a subset of at least 10 of those participants with continuous study drug exposure for one year, with the large majority of data coming from participants receiving the 50 mg dose. In a prior communication received in March, FDA stated that it is open to the use of skin FXN concentrations as a RLSE and acknowledged the submitted data appear to support a relationship between increased skin FXN and relevant tissues such as the heart, dorsal root ganglion, and skeletal muscle. Acceptability of increases in skin FXN for accelerated approval will be decided during future BLA review.

•In July 2025, the Company announced the publication of nonclinical data evaluating the mechanism of action, pharmacodynamics and pharmacology of nomlabofusp as a novel FXN protein replacement therapy designed to address the underlying cause of FA in two peer-reviewed articles. These data were included in the briefing package reviewed by the FDA in support of using skin FXN concentrations as a RLSE for the Company's registrational program seeking accelerated approval for nomlabofusp.

Nomlabofusp plans and milestones include the following:

•The ongoing open label study continues to enroll, and active study participants are currently receiving the 50 mg dose of nomlabofusp. The Company plans to provide an update in September 2025 on open label data on at least 30 to 40 study participants who received at least one dose of nomlabofusp. Safety and PK data from the recently completed adolescent PK run-in study are also expected as part of the September 2025 update.

•Adolescent participants from the PK run-in study are currently screening and enrolling in the open label study. Larimar is also screening and enrolling into the open label study patients with FA who have not participated in prior nomlabofusp clinical studies. In addition, Larimar plans to enroll children (2 to 11 years of age) directly into the open label study.

•In the second quarter of 2026, the Company plans to submit a BLA seeking accelerated approval.

•Following feedback from FDA and EMA on the study protocol, global Phase 3 study sites in the U.S., European Union, U.K., Canada, and Australia were identified and are currently being qualified and initiated. The Company expects to begin patient recruitment later this year.

The Company is subject to risks and uncertainties common to pre-commercial companies in the biotechnology industry, including, but not limited to, development and commercialization by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with governmental regulations, failure to secure regulatory approval for its drug candidates or any other product candidates and the ability to secure additional capital to fund its operations. Product candidates under development will require

extensive non-clinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, infrastructure and extensive compliance-reporting capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, it will realize significant revenue from product sales.

Basis of Presentation

The condensed consolidated financial statements include the accounts of Larimar and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated. The accompanying condensed consolidated financial statements have been prepared in conformity with U.S. Generally Accepted Accounting Principles ("GAAP").

The consolidated balance sheet as of December 31, 2024 was derived from the Company's audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited condensed consolidated financial statements as of June 30, 2025 and for the three and six months ended June 30, 2025 and 2024, have been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2024 included in the Company's Annual Report on Form 10-K filed with the SEC on March 24, 2025.

In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair statement of the Company's condensed consolidated financial position as of June 30, 2025, condensed consolidated results of operations for the three and six months ended June 30, 2025 and condensed consolidated statement of cash flows for the six months ended June 30, 2025 have been made. The results of operations for the three and six months ended June 30, 2025 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2025.

Liquidity and Capital Resources

The Company's condensed consolidated financial statements have been presented on the basis that it will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business.

Since its inception, the Company has incurred significant recurring operating losses and negative cash flows from operations. The Company has incurred net losses of $55.5 million and $36.3 million for the six months ended June 30, 2025 and 2024, respectively. In addition, as of June 30, 2025, the Company had an accumulated deficit of $324.6 million. The Company expects to continue to generate operating losses for the foreseeable future. As of June 30, 2025, the Company had approximately $138.5 million of cash, cash equivalents and marketable securities available for use to fund its operations and capital requirements.

The Company has funded its operations to date primarily with proceeds from sales of common stock and proceeds from the sale of prefunded warrants for the purchase of common stock, the acquisition in 2020 of cash, cash equivalents and marketable securities upon the merger with Zafgen, Inc. ("Zafgen") and, prior to the 2020 merger with Zafgen, capital contributions from Chondrial Holdings, LLC.

Subsequent to June 30, 2025, the Company completed an underwritten public offering of common stock raising net proceeds of approximately $65.1 million.

In accordance with Accounting Standards Update ("ASU") No. 2014-15, "Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern", the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that these condensed consolidated financial statements are issued. As of the issuance date of these condensed consolidated financial statements, the Company expects its cash, cash equivalents and marketable securities combined with the net proceeds of approximately $65.1 million from the Company's July 2025 public offering will be sufficient to fund its forecasted operating expenses and capital expenditure requirements for at least twelve months from the date these financial statements are issued, into the fourth quarter of 2026. If the timing of the Company's clinical assumptions are delayed or if there are other

forecasted assumption changes that negatively impact its operating plan, the Company would reduce expenditures in order to further extend cash resources.

The Company has not yet commercialized any products and does not expect to generate revenue from the commercial sale of any products for several years, if at all. The Company expects that its research and development and general and administrative expenses will continue to increase and, as a result, that it will need additional capital to fund its future operating and capital requirements. Unless and until the Company can generate substantial revenue, management continuously evaluates different strategies to obtain the required funding for future operations. These strategies include seeking additional funding through a combination of public or private equity offerings, debt or royalty financings, collaborations and licensing arrangements, strategic partnerships with pharmaceutical and/or larger biotechnology companies, or other sources. The incurrence of indebtedness would result in increased fixed payment obligations and the Company may be required to agree to certain restrictive covenants, such as limitations on its ability to incur additional debt, limitations on its ability to acquire, sell or license intellectual property rights, minimum required cash balances and other operating restrictions that could adversely impact the Company's ability to conduct its business. Any additional fundraising efforts may divert the Company's management from their day-to-day activities, which may adversely affect its ability to develop and commercialize its product candidates.

There can be no assurance that the Company will be able to raise sufficient additional capital on acceptable terms, if at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or if the Company does not have sufficient authorized shares, the Company may be required to delay, limit, or eliminate the development of business opportunities and its ability to achieve its business objectives, its competitiveness, and its business, financial condition, and results of operations will be materially adversely affected. The Company could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and it may be required to relinquish rights to some of its technologies or product candidates or otherwise agree to terms unfavorable to it, any of which may have a material adverse effect on the Company's business, operating results and prospects. In addition, geopolitical tensions, volatility of capital markets, and other adverse macroeconomic events, including those due to inflationary pressures, rising interest rates, bank instability and the ability of the U.S. government to manage federal debt limits as well as the potential impact of other health crises on the global financial markets may reduce the Company's ability to access capital, which could negatively affect its liquidity and ability to continue as a going concern.

If the Company is unable to obtain sufficient funding when needed and/or on acceptable terms, the Company may be required to significantly curtail, delay or discontinue one or more of its research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion, commercialization efforts and/or commercial operations, which could adversely affect its business prospects, or the Company may be unable to continue operations.

2.Summary of Significant Accounting Policies

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. This process involves reviewing open contracts and purchase orders, communicating with our personnel and outside vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual of research and development expense, the recording as prepaid expense of payments made in advance of the actual provision of goods or services, valuation of stock-based awards and valuation of leases. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions.

Research and Development Costs

Costs associated with internal research and development and external research and development services, including drug development, clinical studies and non-clinical studies, are expensed as incurred. Research and development expenses include costs for salaries, employee benefits, subcontractors, facility-related expenses, depreciation, stock-based compensation, third-party license fees, laboratory supplies, and external costs of outside vendors engaged to conduct discovery, non-clinical and clinical development activities and clinical trials as well as to manufacture clinical trial materials, and other costs. The Company recognizes external research and development

costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its key service providers.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such prepaid expenses are recognized as an expense when the goods have been delivered or the related services have been performed, or when it is no longer expected that the goods will be delivered, or the services rendered.

Upfront payments, milestone payments and annual maintenance fees under license agreements are currently expensed in the period in which they are incurred.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

The Company measures all stock-based awards granted to employees and directors based on the fair value on the date of grant using the Black-Scholes option-pricing model. Compensation expense of those awards is recognized over the requisite service period, which is the vesting period of the respective award. Typically, the Company issues awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in its consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Prior to May 28, 2020, the Company had been a private company and lacked company-specific historical and implied volatility information for its common stock. Prior to January 1, 2023, the Company estimated its expected common stock price volatility solely based on the historical volatility of publicly traded peer companies. Beginning on January 1, 2023, based on the availability of sufficient historical trading data of the Company's own common stock on the Nasdaq Global Market to calculate accurately its volatility, the Company began blending its volatility starting from June 2020 (following its merger with Zafgen in 2020) to the date of each stock-based award, and weighing the volatility of its peer group for the amount of time from May 31, 2020 backwards so that the blended volatility equals the expected term of the related stock-based award. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield considers the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

Net Loss Per Share

Basic net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period.

Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of common shares, including potentially dilutive common stock equivalents assuming the dilutive effect of outstanding stock options, outstanding restricted stock units, and unvested restricted common shares, as determined using the treasury stock method. For periods in which the Company has reported net losses (all periods since inception), diluted net loss per common share attributable to common stockholders is the same as basic net loss per common share attributable to common stockholders, since dilutive common stock equivalents are not assumed to have been issued if their effect is antidilutive.

The Company excluded 9,593,248 and 6,900,232 common stock equivalents outstanding as of June 30, 2025 and 2024, respectively, from the computation of diluted net loss per share for the three and six months ended

June 30, 2025 and 2024 because they had an anti-dilutive impact due to the net loss incurred for the periods presented.

Segment Information

Operating segments are defined as components of an enterprise for which separate discrete information is available for evaluation by the chief operating decision maker, or decision making group, in deciding how to allocate resources in assessing performance. The Company is managed on a consolidated basis and has one operating and reportable segment related to the development of clinical and preclinical product candidates for the development of the Company's proprietary new therapies, primarily nomlabofusp, the Company's life science segment. The Company's chief operating decision maker ("CODM") is the Company's chief executive officer ("CEO").

The accounting policies of the life science segment are the same as those described in the summary of significant accounting policies. The CODM assesses performance for the Company and its reportable segment based on net loss, which is reported on the consolidated Statements of Operations. The measure of segment assets is reported on the balance sheet as total consolidated assets. All long-lived assets are located in the U.S.

To date, the Company has not generated any product revenue. The Company expects to continue to incur significant expenses and operating losses for the foreseeable future as it advances product candidates through all stages of development and clinical trials and regulatory approval.

The CODM uses consolidated financial information, including consolidated net loss, to evaluate performance, forecast future period financial results, allocate resources for the company by, among other things, comparing budgeted to actual results.

The table below summarizes the significant expense categories regularly provided to the CODM for the three and six months ended June 30, 2025 and 2024:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				
Technical operations	$ 13,642	$ 12,525	$ 28,949	$ 19,470
Development [(a)(b)]	8,495	5,891	18,104	10,621
Nomlabofusp support	1,231	1,266	2,866	2,530
General and administrative [(c)(d)]	3,643	4,484	7,789	8,279
Commercial	781	433	1,271	433
Total operating expenses	$ 27,792	$ 24,599	$ 58,979	$ 41,333
Other income, net	(1,610)	(2,972)	(3,516)	(5,052)
Net loss	$ (26,182)	$ (21,627)	$ (55,463)	$ (36,281)

(a) Development expenses include research and development related stock compensation expense of approximately $0.9 million and $1.0 million for the three months ended June 30, 2025 and 2024, respectively.

(b) Development expenses include research and development related stock compensation expense of approximately $1.8 million and $1.9 million for the six months ended June 30, 2025 and 2024, respectively.

(c) General and administrative expenses include general and administrative related stock compensation expense of approximately $0.9 million and $1.3 million for the three months ended June 30, 2025 and 2024, respectively.

(d) General and administrative expenses include general and administrative related stock compensation expense of approximately $1.8 million and $2.5 million for the six months ended June 30, 2025 and 2024, respectively.

Recently Issued and Adopted Accounting Pronouncements

From time to time, new accounting guidance is issued by the FASB or other standard setting bodies that is adopted by us as of the effective date or, in some cases where early adoption is permitted, in advance of the effective

date. We have assessed the recently issued guidance that is not yet effective and believe the new guidance will not have a material impact on the condensed consolidated results of operations, cash flows or financial position.

In November 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2023-07, Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures ("ASU 2023-07"). ASU 2023-07 requires disclosure of incremental segment information on an annual and interim basis. The amendments also require companies with a single reportable segment to provide all disclosures required by this amendment and all existing segment disclosures in ASC 280, Segment Reporting. The amendments are effective for fiscal years beginning after December 15, 2023, and interim periods beginning after December 15, 2024. The Company adopted ASU 2023-07, effective December 31, 2024, in these consolidated financial statements. ASU 2023-07 only impacted the disclosures and did not impact the consolidated financial statements. See Note 2, Segment Information, for disclosures related to the adoption of ASU 2023-07.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, "Improvements to Income Tax Disclosures (Topic 740)," which requires entities to disclose in their rate reconciliation table additional categories of information about federal, state and foreign income taxes as well as additional information about reconciling items if certain quantitative thresholds are met. This ASU will require all entities to disclose income taxes paid, net of refunds, disaggregated by federal (national), state and foreign taxes for annual periods and to disaggregate the information by jurisdiction based on a quantitative threshold. All entities are required to apply the guidance prospectively, with the option to apply it retrospectively. The ASU is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company will adopt ASU-203-09 beginning with our annual reporting period ending December 31, 2025, the adoption of which is not expected to have a material impact on the Company's consolidated results of operations or cash flows.

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", which requires disaggregated disclosures in the notes of the financial statements of certain categories of expenses that are included in expense line items on the face of the income statement. This ASU is effective for annual periods beginning after December 15, 2026, and interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company will evaluate the impact adopting ASU 2024-03 will have on the Company's consolidated financial statements and disclosures.

Income Taxes

We recognize deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial reporting and tax basis of assets and liabilities, as well as for operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using the tax rates that are expected to apply to taxable income for the years in which those tax assets and liabilities are expected to be realized or settled. We record valuation allowances to reduce deferred tax assets to the amount we believe is more likely than not to be realized. During the three and six months ended June 30, 2025 and June 30, 2024, the Company recorded no income tax benefits for the net operating losses incurred in each period due to the uncertainty of realizing a benefit from those items

On July 4, 2025, Public Law 119-21 ("The Law") became effective. The Law includes significant provisions, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. The Company is currently assessing The Law's impact on its consolidated financial statements; however, it does not expect The Law to have a material impact on its estimated annual effective tax rate or cash flows in 2025.

3.Fair Value Measurements and Marketable Securities

Fair Value Measurements

The Company's assets and liabilities that are measured at fair value on a recurring basis as of June 30, 2025 and December 31, 2024 are measured in accordance with the standards of ASC 820, "*Fair Value Measurements and Disclosures*", which establishes a three-level valuation hierarchy for measuring fair value and expands financial statement disclosures about fair value measurements. The valuation hierarchy is based on the transparency of inputs to the valuation of an asset or liability as of the measurement date. The three levels are defined as follows:

Level – 1	Inputs to the valuation methodology are quoted prices (unadjusted) for identical assets or liabilities in active markets.
Level – 2	Inputs to the valuation methodology include quoted prices for similar assets and liabilities in active markets, and inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the financial instrument.
Level – 3	Inputs to the valuation methodology are unobservable and significant to the fair value measurement.

The Company's financial instruments consist primarily of cash, cash equivalents, marketable securities, accounts payable and accrued liabilities. For accounts payable and accrued liabilities, the carrying amounts of these financial instruments as of June 30, 2025 and December 31, 2024 were considered representative of their fair values due to their short term to maturity.

The following tables summarize the Company's cash equivalents and marketable securities as of June 30, 2025 and December 31, 2024:

	Total	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
		(in thousands)		
June 30, 2025				
Cash equivalents:				
Money market funds invested in government securities	$ 19,715	$ 19,715	$ —	$ —
Total cash equivalents	19,715	19,715	—	—
Marketable securities:				
U.S. Treasury Bills	11,404	11,404	—	—
U.S. Government securities	106,533	—	106,533	—
Total marketable securities	117,937	11,404	106,533	—
Total cash equivalents and marketable securities	$ 137,652	$ 31,119	$ 106,533	$ —
December 31, 2024				
Cash equivalents:				
Money market funds invested in government securities	$ 26,702	$ 26,702	$ —	$ —
Total cash equivalents	26,702	26,702	—	—
Marketable securities:				
U.S. Treasury Bills	2,947	2,947	—	—
U.S. Government securities	147,289	—	147,289	—
Total marketable securities	150,236	2,947	147,289	—
Total cash equivalents and marketable securities	$ 176,938	$ 29,649	$ 147,289	$ —

The accrued interest receivable related to the Company's investments was $1.0 million and $1.1 million as of June 30, 2025 and December 31, 2024, respectively, and is included in prepaid expenses and other current assets on the condensed consolidated balance sheet.

The Company classifies its money market funds and U.S. treasury bills, which are valued based on quoted market prices in active markets with no valuation adjustment, as Level 1 assets within the fair value hierarchy.

The Company classifies its investments in U.S. government and agency securities, corporate commercial paper, and corporate bonds, if any, as Level 2 assets within the fair value hierarchy. The fair values of these investments are estimated by taking into consideration valuations obtained from third-party pricing services. The pricing services utilize industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

As of June 30, 2025 and December 31, 2024, the unrealized losses for available-for-sale investments were non-credit related, and the Company does not intend to sell the investments that were in an unrealized loss position, nor will it be required to sell those investments before recovery of their amortized cost basis, which may be maturity. As of June 30, 2025 and December 31, 2024, no allowances for credit losses for the Company's investments were recorded. During the three and six months ended June 30, 2025 and 2024, the Company did not recognize any impairment losses related to investments.

As of June 30, 2025 and December 31, 2024, the Company's cash equivalents and marketable securities consisted of a U.S. government money market fund, U.S. Treasury Bills and U.S. government and agency securities, all held in our name in a separate custody account with U.S. Bank. The U.S. government money market fund has same-day liquidity access and the U.S. government and agency securities all have maturities of 360 days or less.

Marketable Securities

The following table summarizes the Company's marketable securities as of June 30, 2025 and December 31, 2024:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
		(in thousands)		
June 30, 2025				
Assets:				
U.S. Treasury Bills	$ 11,406	$ —	$ (2)	$ 11,404
U.S. Government securities	106,539	15	(21)	106,533
Total marketable securities	$ 117,945	$ 15	$ (23)	$ 117,937
December 31, 2024				
Assets:				
U.S. Treasury Bills	$ 2,945	$ 2	$ —	$ 2,947
U.S. Government securities	147,143	166	(20)	147,289
Total marketable securities	$ 150,088	$ 168	$ (20)	$ 150,236

As of June 30, 2025 and December 31, 2024, the Company held no investments that have been in a continuous loss position for 12 months or longer.

4.Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	June 30, 2025	December 31, 2024
	(in thousands)	
Prepaid research and development expenses	$ 5,475	$ 9,780
Interest receivable	983	1,054
Prepaid insurance	252	499
Other prepaid expenses and other assets	322	517
	$ 7,032	$ 11,850

5.Fixed Assets

Fixed assets, net consisted of the following:

	Useful Life	June 30, 2025	December 31, 2024
		(in thousands)	
Computer equipment	5 years	$ 130	$ 117
Lab equipment	5 years	1,737	1,707
Furniture and fixtures	7 years	555	555
Leasehold improvements	lease term	93	45
		2,515	2,424
Less: Accumulated depreciation		(1,718)	(1,543)
		$ 797	$ 881

Depreciation expense was $0.1 million and $0.2 million for the three and six months ended June 30, 2025, respectively. Depreciation expense was $0.1 million for the three and six months ended June 30, 2024. In addition, for the three and six months ended June 30, 2025 and 2024, there was less than $0.1 million of depreciation related to sublet assets recorded as other expense.

6.Accrued Expenses

Accrued expenses consisted of the following:

	June 30, 2025	December 31, 2024
	(in thousands)	
Accrued research and development expenses	$ 18,538	$ 17,057
Accrued payroll and related expenses	2,151	3,254
Accrued other	662	561
	$ 21,351	$ 20,872

7.Stockholders' Equity and Stock Options

Common Stock and Prefunded Warrants

On May 28, 2020, the Company entered into a securities purchase agreement with certain accredited investors (the "Purchasers") for the sale by the Company in a private placement of 6,105,359 shares of the Company's common stock and prefunded warrants to purchase an aggregate of 628,403 shares of the Company's common stock, for a price of $11.88 per share of the common stock and $11.87 per prefunded warrant. The prefunded warrants were exercisable at an exercise price of $0.01 and were exercisable indefinitely. In August 2023, the 628,403 shares of prefunded warrants were exercised and the Company received cash proceeds of six thousand two hundred and eighty-four dollars. The private placement closed on June 1, 2020. The aggregate gross proceeds for the issuance and sale of the common stock and prefunded warrants were $80.0 million, transaction costs totaled $4.6 million and resulted in net proceeds of $75.4 million. The Company's Registration Statement on Form S-3, filed with the SEC on June 26, 2020, registered the resale of 6,105,359 shares of common stock sold and the 628,403

shares of common stock underlying the prefunded warrants. MTS Health Partners served as placement agent to the Company in connection with the private placement. As partial compensation for these services, the Company issued MTS Health Partners 35,260 shares of common stock.

As of June 30, 2025, the Company's Ninth Amended and Restated Certificate of Incorporation, as amended, authorized the Company to issue up to 115,000,000 shares of common stock, par value $0.001 per share, of which 64,027,892 shares were issued and outstanding, and up to 5,000,000 shares of undesignated preferred stock, par value $0.001 per share, of which no shares were issued or outstanding. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the preferred stock. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors of the Company (the "Board"), if any. No cash dividends have been declared or paid to date.

In February 2024, the Company completed an underwritten public offering in which the Company issued and sold 19,736,842 shares of its common stock at a public offering price of $8.74 per share. The Company received net proceeds of approximately $161.8 million after deducting underwriting discounts, commissions and other offering expenses.

In July 2025, the Company completed an underwritten public offering in which the Company issued and sold 21,562,500 shares of its common stock including the exercise in full of the underwriters' option to purchase additional shares of common stock, at a public offering price of $3.20 per share. The Company received net proceeds of approximately $65.1 million.

ATM Agreement

In November 2022, the Company entered into a sales agreement (the "2022 ATM Agreement") with Guggenheim Securities, LLC in connection with the establishment of an "at-the-market" offering program under which the Company could sell up to an aggregate of $50.0 million of shares of common stock (the "ATM Shares") from time to time.

In February 2024, in connection with the underwritten public offering described above, the Company terminated the 2022 ATM Agreement. No ATM Shares were ever sold pursuant to the 2022 ATM Agreement.

In May 2024, the Company entered into a sales agreement (the "2024 ATM Agreement") with Guggenheim Securities, LLC in connection with the establishment of an "at-the-market" offering program under which the Company could sell up to an aggregate of $100 million of shares of common stock (the "ATM Shares") from time to time. To date, no sales of common stock have been made under the 2024 ATM Agreement.

2020 Equity Incentive Plan

The Board adopted the 2020 Equity Incentive Plan (the "2020 Plan") on July 16, 2020 and the stockholders of the Company approved the 2020 Plan on September 29, 2020. The 2020 Plan replaced the predecessor plans (the "Prior Plans") that the Company assumed following its merger with Zafgen in May 2020. Options outstanding under the Prior Plans will remain outstanding, unchanged, and subject to the terms of the Prior Plans and the respective award agreements, and no further awards will be made under the Prior Plans. However, if any award previously granted under the Prior Plans, expires, terminates, is canceled, or is forfeited for any reason after the approval of the 2020 Plan, the shares subject to that award will be added to the 2020 Plan share pool so that they can be utilized for new grants under the 2020 Plan.

The 2020 Plan provides for the grant of incentive stock options ("ISOs"), nonstatutory stock options ("NSOs"), stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based restricted stock units, and cash or other stock-based awards. ISOs may be granted only to the Company's employees, including the Company's officers, and the employees of the Company's affiliates. All other awards may be granted to the Company's employees, including the Company's officers, the Company's non-employee directors and consultants, and the employees and consultants of the Company's affiliates.

The maximum number of shares that may be issued in respect of any awards under the 2020 Plan is the sum of: (i) 1,700,000 shares plus (ii) an annual increase on January 1, 2021 and each anniversary of such date thereafter through January 1, 2030, equal to the lesser of (A) 4% of the shares issued and outstanding on the last day of the immediately preceding fiscal year, or (B) such smaller number of shares as determined by the Board (collectively, the "Plan Limit"). The maximum aggregate number of shares that may be issued under the 2020 Plan is 8,000,000 over the ten-year term of the 2020 Plan.

As permitted by the 2020 Plan, the Company added 2,552,603 and 1,756,363 shares available for grant to the 2020 Plan on January 1, 2025 and January 1, 2024, respectively. As of June 30, 2025, 508,844 shares of common stock were available for grant under the 2020 Plan.

During the six months ended June 30, 2025 and twelve months ended December 31, 2024, options to purchase 333 and 1,242 shares, respectively, issued under the Prior Plans were cancelled and became available for grant under the 2020 Plan.

Stock Option Valuation

The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees during the six months ended June 30, 2025:

	June 30, 2025
Risk-free interest rate	4.45%
Expected term (in years)	6.22
Expected volatility	94%
Dividend yield	0.00%

Stock Options

The following table summarizes the Company's stock option activity for the six months ended June 30, 2025 (amounts in millions, except for share, contractual term, and per share data):

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (a) (in millions)
Outstanding as of December 31, 2024	6,436,387	$ 7.78	7.6	
Options granted	2,344,137	3.32		
Options forfeited/expired	(197,067)	6.10		
Outstanding as of June 30, 2025	8,583,457	$ 6.60	7.7	$ 0.2
Exercisable as of June 30, 2025	4,096,695	$ 9.16	6.3	$ —
Vested and expected to vest as of June 30, 2025	8,583,457	$ 6.60	7.7	$ 0.2

(a)The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were "in the money" at June 30, 2025.

Option Grants

During the six months ended June 30, 2025, the Company granted options to purchase 2,344,137 shares of common stock to employees and directors under the 2020 Plan. The options have an exercise price equal to the closing stock price as of the grant date. Of the 2,344,137 options granted, 2,249,137 were granted to employees and vest over four years, with 25% vesting on the first anniversary of the grant and the remainder vesting in equal monthly installments thereafter. The remaining 95,000 options were annual grants to the Company's directors and vest on the earlier of (i) one year from the grant date or (ii) the date of the Company's next annual meeting of stockholders. The options have an exercise price equal to the closing stock price as of the grant date. The weighted-average grant date fair value of options granted under the 2020 Plan during the six months ended June 30, 2025 was $2.63.

As of June 30, 2025, total unrecognized compensation expense related to unvested stock options granted under the 2020 Plan was $13.3 million, which is expected to be recognized over a weighted average period of 2.72 years.

Inducement Stock Option Grant

There were no inducement awards granted in the six months ended June 30, 2025.

As of June 30, 2025, total unrecognized compensation expense related to unvested inducement options granted was $0.6 million, which is expected to be recognized over a weighted average period of 1.79 years.

Restricted Stock Units Grants

In January 2025, RSUs were granted under the 2020 Plan to certain of the Company's employees in order to maintain retention of key employees. The value of an RSU award is based on the Company's stock price on the date of grant. The shares underlying the RSUs are not issued until the RSUs vest.

Activity with respect to the Company's RSUs and performance-based RSUs during the six months ended June 30, 2025 was as follows (in millions, except share, contractual term, and per share data):

	Number of Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (a) (in millions)
Outstanding as of December 31, 2024	699,003	$ 4.69	1.2	
Restricted stock units granted	544,988	3.46		
Restricted stock units vested	(212,827)	4.73		
Restricted stock units forfeited	(21,373)	4.27		
Outstanding as of June 30, 2025	1,009,791	$ 4.02	1.7	$ 2.9
Unvested and expected to vest as of June 30, 2025	1,009,791	$ 4.02	1.7	$ 2.9

Restricted Stock Unit Grants

During the six months ended June 30, 2025, the Company granted 544,988 shares of RSUs to employees under the 2020 Plan. The RSUs vest annually over four years and have a weighted-average grant date fair value of $3.46 per unit.

As of June 30, 2025, total unrecognized compensation expense for RSUs was $2.8 million, which is expected to be recognized over a weighted average period of 2.53 years.

Performance-Based Restricted Stock Unit Grants

In January 2025, the Company granted performance-based RSUs (the "January 2025 PSU Awards") to each of the executive officers of the Company under the 2020 Plan. Each award was expressed as a target number of RSUs. With respect to the January 2025 PSU Awards, the Board established specified regulatory-related performance criteria and a corresponding performance period over which such performance criteria must be achieved, the satisfaction of which are conditions to earning the January 2025 PSU Awards and vesting of the underlying RSUs. As of June 30, 2025, the January 2025 PSU Awards were outstanding covering 200,000 common stock underlying RSUs.

The grant date fair value of the performance-based RSUs was $3.46 per unit based on the grant date closing price per share. As of June 30, 2025, the underlying performance criteria of the January 2025 PSU Awards were determined to be not probable of achievement, and no stock-based compensation expense was recognized for the three and six months ended June 30, 2025.

Stock-Based Compensation

Stock-based compensation expense was classified in the condensed consolidated statements of operations as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Research and development	$ 887	$ 987	$ 1,812	$ 1,898
General and administrative	941	1,314	1,850	2,531
	$ 1,828	$ 2,301	$ 3,662	$ 4,429

8.Commitments and Contingencies

Intellectual Property Licenses

The Company is party to an exclusive License Agreement (the "WFUHS License"), dated November 30, 2016, as amended, with Wake Forest University Health Sciences ("WFUHS") and an exclusive License Agreement (the "IU License"), dated November 30, 2016, as amended, with Indiana University ("IU"). Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology used by the Company with respect to the development of nomlabofusp. Both agreements continue from their effective date through the last to date of expiration of the licensed patents, unless earlier terminated by either party in accordance with their terms.

In partial consideration for the right and license granted under these agreements, the Company will pay each of WFUHS and IU a royalty of a low single digit percentage of net sales of licensed products depending on whether there is a valid patent covering such products. As additional consideration for these agreements, the Company is obligated to pay each of WFUHS and IU certain milestone payments of up to $2.6 million in the aggregate upon the achievement of certain developmental milestones, which commenced with the enrollment of the first patient in a Phase 1 clinical trial. The Company enrolled the first patient in its SAD trial on December 11, 2019 and paid WFUHS and IU less than $0.1 million. The Company will also pay each of WFUHS and IU sublicensing fees ranging from a high-single digit to a low double-digit percentage of sublicense consideration depending on the Company's achievement of certain regulatory milestones as of the time of receipt of the sublicense consideration. The Company is also obligated to reimburse WFUHS and IU for patent-related expenses. In the event that the Company disputes the validity of any of the licensed patents, the royalty rate would be tripled during such dispute. The Company is also obligated to pay to IU a minimum annual royalty of less than $0.1 million per annum.

In the event that the Company is required to pay IU consideration, then the Company may deduct 20% of such IU consideration on a dollar-for-dollar basis from the consideration due to WFUHS. In the event that the Company is required to pay WFUHS consideration, then the Company may deduct 60% of such WFUHS consideration on a dollar-for-dollar basis from the consideration due to IU.

In October 2022, the Company initiated dosing of a Phase 2 study. Pursuant to the terms of both the WFUHS License and the IU License, the company recognized milestone expense of $0.3 million within research and development expenses.

Both agreements continue from their effective date through the last date of expiration of the licensed patents, unless earlier terminated by either party in accordance with their terms.

Leases

Bala Cynwyd Office Space

On August 8, 2019, the Company entered into an operating lease for office space in Bala Cynwyd, Pennsylvania, effective as of December 15, 2019, for a period of three years and six months with an option to extend the lease for three additional years. Due to required tenant improvements to be completed by the landlord, the Company did not take immediate possession of the leased property and the lease term commenced on February 15, 2020.

On March 9, 2023, the Company executed a lease extension agreement on its original 4,642 square footage of office space in Bala Cynwyd, Pennsylvania (which was set to expire in August 2023) and agreed to lease an additional 3,462 square feet of office space from the same landlord.

The lease extension on the original 4,642 square footage commenced on September 1, 2023 and the Company recorded a right of use asset and lease liability of $0.5 million as of that date.

The new lease on 3,462 additional square footage commenced on October 1, 2023 and the Company recorded a right of use asset and lease liability of $0.3 million as of that date.

The right of use assets and lease liabilities with both these leases are reflected in the financial statements for six months ended June 30, 2025 as are the right of use asset and lease liability of the Company's Boston office space discussed below.

Boston Office Lease

In connection with the Company's 2020 merger with Zafgen, on May 28, 2020, the Company acquired a non-cancellable operating lease for approximately 17,705 square feet of office space (the "Premises"). The lease expires on October 30, 2029. As part of the agreement, the Company was initially required to maintain a letter of credit, of $1.3 million. In October of 2024, upon the agreement with the landlord that we had achieved certain clinical development milestones required in the lease, this letter of credit was reduced to $0.6 million. During both periods presented, this cash deposit is classified as restricted cash within the condensed consolidated financial statements. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The right-of-use asset is being amortized to other income/(expense) over the remaining lease term as a result of the sublease described below.

On October 27, 2020, the Company entered into a sublease agreement (the "Sublease") with Massachusetts Municipal Association, Inc. (the "Subtenant"), whereby the Company sublet the entire Premises to the Subtenant. The initial term of the Sublease commenced on December 4, 2020 and continues until October 30, 2029. In connection with the Sublease, the Company evaluated the need for impairment under ASC 360 "Impairment Testing: Long-Lived Assets Classified as Held and Used," and determined there was no impairment.

The Sublease provided for an initial annual base rent of $0.8 million, which increases annually up to a maximum annual base rent of $1.0 million. The Subtenant also is responsible for paying to the Company future increases in operating costs (commencing on January 1, 2022), future increases in annual tax costs (commencing July 1, 2021) and all utility costs (commencing March 1, 2021) attributable to the Premises during the term of the Sublease. As part of the Sublease, the subtenant deposited a letter of credit in the amount of $0.8 million to assure their performance under the sublease. If there are no uncured events of default under the sublease, the amount of this security deposit decreases over time to $0.4 million on the sixth anniversary of the Sublease. The Company records sublease income on this sublease on a straight-line basis as a component of other income/(expense).

Lab Space

On November 5, 2018, the Company entered into an operating lease for office and lab space in Philadelphia, Pennsylvania, effective as of January 1, 2019, and expiring on December 31, 2020 with an option to extend the lease for two additional years. On August 4, 2020, the Company executed the first option to extend the lease for an additional year, expiring on December 31, 2021. On August 9, 2021, the Company executed the remaining option to extend the lease for an additional year, expiring on December 31, 2022. In September 2023, the Company extended this lease for an additional year with the option to terminate with four months' notice. On March 28, 2024, the Company gave the requisite notice and vacated the property in May 2024.

On October 16, 2023, the Company entered into an operating lease for lab space in King of Prussia, Pennsylvania for a period of four years. Due to required tenant improvements to be completed by the landlord, the Company did not take immediate possession of the leased property. The actual lease term commenced on May 10, 2024. Upon commencement of the lease term, the Company recorded a right of use asset and lease liability of $0.5 million which are reflected in these condensed consolidated financial statements.

Lease Expense

Expense arising from operating leases was $0.1 million and $0.3 million during the three and six months ended June 30, 2025, respectively. Expense arising from operating leases was $0.1 million and $0.3 million during the three and six months ended June 30, 2024, respectively. For operating leases, the weighted-average remaining lease term for leases at June 30, 2025 and December 31, 2024 was 4.0 and 4.5 years, respectively. For operating leases, the weighted average discount rate for leases at June 30, 2025 and December 31, 2024 was 11.0%. The Company has not entered into any financing leases.

Maturities of lease liabilities due under these lease agreements as of June 30, 2025 are as follows:

(in thousands)		Operating Leases
Six months ending December 31, 2025	$	777
Year ended December 31, 2026		1,478
Year ended December 31, 2027		1,273
Year ended December 31, 2028		1,188
Year ended December 31, 2029		959
Thereafter		—
Total lease payments		5,675
Less: imputed interest		(1,061)
Present value of lease liabilities	$	4,614

Legal Proceedings

The Company is not currently a party to any litigation, nor is management aware of any pending or threatened litigation against the Company, that it believes would materially affect the Company's business, operating results, financial condition or cash flows.

9.Related Party

In May 2024, the Company entered into an agreement with the Friedreich's Ataxia Research Alliance (FARA) to join the TRACK-FA Neuroimaging Consortium that includes pharmaceutical, biotechnology, academic and clinical partners. The consortium will conduct a natural history study designed to establish disease-specific neuroimaging biomarkers to track disease progression in the brain and spinal cord and provide a basis for utilizing these biomarkers in clinical trials. As an industry partner, the Company will help fund the study and contribute to the study design, research activities, and analysis. The Company will have access to all study data for use in its regulatory filings, as appropriate. During the twelve months ended December 31, 2024, the Company incurred $0.9 million of costs related to the Track-FA program and will fund future costs going forward. One of the Company's Directors is also a director of FARA. During the six months ended June 30, 2025, the Company incurred less than $0.1 million of costs related to the Track-FA program.

Additionally, during the twelve months ended December 31, 2024, the Company sponsored patient and caregiver awareness events held by FARA for a cumulative $0.1 million.

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 should be read in conjunction with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q ("Quarterly Report"), and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2024 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 24, 2025 (the "2024 Annual Report"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks, uncertainties, and assumptions. These statements are based on our beliefs and expectations about future outcomes and are subject to risks and uncertainties that could cause our actual results to differ materially from anticipated results. We undertake no obligation to publicly update these forward-looking statements, whether as a result of new information, future events or otherwise. You should read the "Risk Factors" section included in our 2024 Annual Report, in addition to the "Risk Factors" and "Cautionary Note Regarding Forward-Looking Statements" sections of this Quarterly Report for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using our novel cell penetrating peptide ("CPP") technology platform. Our lead product candidate, nomlabofusp (nomlabofusp is the International Nonproprietary Name and the United States Adopted Name for CTI-1601), is a subcutaneously administered, recombinant fusion protein intended to deliver tissue frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive, and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality. Currently, there are no treatment options that address the core deficit of FA, low levels of FXN. Nomlabofusp represents the first potential therapy designed to systemically increase FXN levels in patients with FA.

We believe that our CPP platform, which enables a therapeutic molecule to cross a cell membrane in order to reach intracellular targets, has the potential to enable the treatment of other rare and orphan diseases. We intend to use our proprietary platform to target additional orphan indications characterized by deficiencies in or alterations of intracellular content or activity.

Since our inception, we have devoted substantially all of our resources to developing nomlabofusp, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, developing sales and marketing capacities, and providing general and administrative support for such operations.

Nomlabofusp Program Update

We initiated human clinical studies of nomlabofusp in 2019. Since then, we have completed two Phase 1 clinical studies, a Phase 2 dose exploration study and a PK run-in study in adolescents (12-17 years old).

In 2024, we initiated our open label extension study in patients with FA. The open label extension study's eligibility criteria only allowed patients who previously participated in our prior nomlabofusp Phase 1 or Phase 2 studies. In 2025, we changed the name of the open label extension study to the open label study, due to the inclusion of patients who had not participated in prior nomlabofusp clinical studies. Our ongoing open label study is currently screening and enrolling eligible adolescents that completed dosing in the recently completed PK run-in study into the open label study. In addition, we are also screening and enrolling patients with FA who have not participated in prior nomlabofusp clinical studies. We are also planning to enroll children (2-11 years of age) directly into the open label study.

We have engaged in multiple discussions and interactions with the U.S. Food and Drug Administration ("FDA") in connection with our clinical development of nomlabofusp. Communications remain ongoing. We currently participate in the FDA's Center for Drug Evaluation and Research ("CDER") Support for Clinical Trials Advancing Rare Disease Therapeutics ("START") pilot program. The objective of this program is to accelerate the development of drugs for rare diseases that lead to significant disability or death by facilitating frequent advice and regular communication with the FDA staff to expedite the review process.

We have also had numerous interactions with the European Medicines Agency ("EMA") and the United Kingdom's Medicines and Healthcare Regulatory Agency ("MHRA") regarding the clinical development of nomlabofusp.

We have received Orphan Drug Designation and Fast Track Designation from the FDA. We have been granted orphan drug designation in the European Union (the "EU") and access to the EU's Priority Medicines Program ("PRIME") Scheme. We have also received access to the EMA's Innovative Licensing and Access Pathway ("ILAP"). All these programs are designed to facilitate drug development of therapeutics for rare and/or orphan indications.

Recent significant developments are as follows:

•In December 2024, we reported positive initial data from our ongoing open label study. This data included safety, FXN levels, clinical and pharmacokinetic data. Also in December 2024, we announced that we were increasing the dose in the open label study to 50 mg of nomlabofusp daily for then currently enrolled participants. All newly enrolled participants will start dosing at 50 mg of nomlabofusp daily. In March 2025, we announced that our Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, we expect to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, we amended the open label study protocol to administer premedication with antihistamines for the first month of dosing.

•In January 2025, we initiated dosing of adolescents (12-17 years old) in our PK run-in study for patients with FA. Study participants were randomized 2:1 to receive either nomlabofusp at a weight-base dose expected to match the PK of adults receiving the 50 mg dose, or placebo, daily for seven days. We completed dosing of 14 adolescents in March 2025.

•In February 2025, the FDA accepted the data supporting the comparability of the lyophilized drug product to the frozen solution and agreed with our plans to introduce the lyophilized product into our clinical development program. The frozen solution is the dosage form currently being used in the open label study. In May 2025, we began to introduce the lyophilized product formulation, which is intended for commercialization, into the open label study with new study participants.

•In March 2025, we announced that FDA stated as part of a START pilot program that it is open to considering the use of FXN concentration as a reasonably likely surrogate endpoint ("RLSE") and the acceptability of FXN concentration as an RLSE to support accelerated approval will be a matter of review in a future marketing application. The FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that data we recently submitted appear to support a relationship between increased FXN concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA also acknowledged that the nonclinical studies we submitted were performed at relevant human doses. FDA also suggested that we consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic ("PD") markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as an RLSE. We continue to interact with the FDA under the START pilot program.

•In June 2025, the FDA provided safety database recommendations for a Biologics License Application, ("BLA") seeking accelerated approval, which included evaluating safety in at least 30 participants with continuous study drug exposure for six months and a subset of at least 10 of those participants with continuous study drug exposure for one year, with the large majority of data coming from participants receiving the 50 mg dose. In a prior communication received in March, FDA stated that it is open to the use of skin FXN concentrations as a RLSE and acknowledged the submitted data appear to support a relationship between increased skin FXN and relevant tissues such as the heart, dorsal root ganglion, and skeletal muscle. Acceptability of increases in skin FXN for accelerated approval will be decided during future BLA review.

•In July 2025, we announced the publication of nonclinical data evaluating the mechanism of action, pharmacodynamics and pharmacology of nomlabofusp as a novel FXN protein replacement therapy designed to address the underlying cause of FA in two peer-reviewed articles. These data were included in the briefing package reviewed by the U.S. FDA in support of using skin FXN concentrations as a RLSE for our registrational program seeking accelerated approval for nomlabofusp.

Nomlabofusp plans and milestones include the following:

•The ongoing open label study continues to enroll, and active study participants are currently receiving the 50 mg dose of nomlabofusp. We plan to provide an update in September 2025 on open label data on at least 30 to 40 study participants who received at least one dose of nomlabofusp. Safety and PK data from the recently completed adolescent PK run-in study are also expected as part of the September 2025 update.

•Adolescent participants from the PK run-in study are currently screening and enrolling in the open label study. We are also screening and enrolling into the open label study patients with FA who have not participated in prior nomlabofusp clinical studies. In addition, we plan to enroll children (2 to 11 years of age) directly into the open label study.

•In the second quarter of 2026, we plan to submit a BLA seeking accelerated approval.

•Following feedback from FDA and EMA on the study protocol, global Phase 3 study sites in the U.S., European Union, U.K., Canada, and Australia were identified and are currently being qualified and initiated. We expect to begin patient recruitment later this year.

Financing Activities, including Recent Material Financings

We have funded our operations to date primarily with proceeds from sales of common stock, proceeds from the sale of prefunded warrants for the purchase of common stock, the acquisition in 2020 of cash, cash equivalents, marketable securities and restricted cash upon the merger with Zafgen, Inc. ("Zafgen") and, prior to the 2020 merger with Zafgen, capital contributions from Chondrial Holdings, LLC.

In February 2024, we completed an underwritten public offering in which we issued and sold 19,736,842 shares of our common stock at a public offering price of $8.74 per share. We received net proceeds of approximately $161.8 million after deducting underwriting discounts, commissions and other offering expenses.

In May 2024, we entered into a Sales Agreement (the "2024 ATM Agreement") with Guggenheim Securities, LLC in connection with the establishment of an "at-the-market" offering program providing for the sale of up to an aggregate of $100 million of shares of our common stock from time to time. To date, we have made no sales under the 2024 ATM agreement.

Subsequent to June 30, 2025, we completed an underwritten public offering in which we issued and sold 21,562,500 shares of our common stock, including the exercise in full of the underwriters' option to purchase additional shares, at a public offering price of $3.20 per share. We received net proceeds of approximately $65.1 million.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed, consolidated financial statements are prepared in accordance with Generally Accepted Accounting Principles ("GAAP"). The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, costs and expenses, and related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our consolidated financial statements and, therefore, consider these to be our critical accounting policies. We evaluate these estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

Research and Development Expense

Costs for certain research and development activities, such as manufacturing, non-clinical studies and clinical trials are generally recognized based on the evaluation of the progress of completion of specific tasks using information and data provided by our vendors and collaborators, and accordingly, are considered an area of significant judgment and management's review of manufacturing, non-clinical and clinical expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel and outside vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. We work with vendors and suppliers to ensure that our estimates of our research and development expenses are reasonable. We expect to increase our investment in research and development in order to advance nomlabofusp through additional clinical trials. As a result, we expect that our research and development expenses will continue to increase in the foreseeable future as we pursue clinical development of nomlabofusp and/or any other product candidates we develop.

Stock Compensation Expense

We measure all stock-based awards granted to employees and directors based on the fair value on the date of grant using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of highly subjective assumptions which determine the fair value of stock-based awards. The assumptions used in our option-pricing model represent management's best estimates. These estimates are complex, involve a number of variables, uncertainties and assumptions and the application of management's judgment, and thus are inherently subjective. If factors change and different assumptions are used, our stock-based compensation expense could be materially different in the future.

Prior to May 28, 2020, we were a private company and lacked company-specific historical and implied volatility information for our common stock. Prior to January 1, 2023, we estimated our expected common stock price volatility solely based on the historical volatility of publicly traded peer companies with comparable characteristics including enterprise value, risk profiles and position within the industry. Beginning on January 1, 2023, we began blending our historical data starting in June 2020 (following our merger with Zafgen in 2020) with its historical peer group. We regularly evaluate our peer group to assess changes in circumstances where identified companies may no longer be similar to us, in which case, more suitable companies whose share prices are publicly available would be utilized in the calculation. We expect to continue to do so until we have full historical data regarding the volatility of our own traded stock price.

The expected term of our stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield considers the fact that we have never paid cash dividends on common stock and do not expect to pay any cash dividends in the foreseeable future.

Compensation expense of those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. Typically, we issue awards with only service-based vesting conditions and record the expense for these awards using the straight-line method. We account for forfeitures as they occur.

In January, 2025, our Board of Directors ("Board") approved the issuance of an aggregate of 200,000 performance-based restricted stock units ("RSUs") to certain of our executive officers (the "January 2025 PSU Awards"). The Board established specified performance criteria and a corresponding performance period over which such performance criteria must be achieved, the satisfaction of which are conditions to earning the January 2025 PSU Awards and vesting of the underlying RSUs. As of June 30, 2025, the underlying performance criteria of the January 2025 PSU Awards were determined to be not probable of achievement, and no stock-based compensation expense was recognized for the three months ended June 30, 2025.

We classify stock-based compensation expense in our consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales, and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts result in clinical success and regulatory approval or collaboration agreements with third parties for our product candidates, we may generate revenue from those product candidates or collaborations.

Operating Expenses

The majority of our operating expenses since inception have consisted primarily of research and development activities, and general and administrative costs.

Research and Development Expenses

Research and development expenses, which consist primarily of costs associated with our product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

- third-party contract costs relating to research, formulation, manufacturing, non-clinical studies and clinical trial activities;
- employee related costs, including salaries, benefits and stock-based compensation expenses for employees engaged in scientific research and development functions;
- external costs of outside consultants and vendors;
- payments made under our third-party licensing agreements;
- sponsored research agreements;
- laboratory consumables; and
- allocated facility-related costs.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the clinical and commercial development of nomlabofusp, or any other product candidates we develop. We are also unable to predict when, if ever, material net cash inflows will commence from sales of our product candidates. The duration, costs, and timing of clinical trials and development of nomlabofusp or any other product candidates we develop will depend on a variety of factors, including:

- the scope, rate of progress and expense of clinical trials and other research and development activities;
- clinical trial results;
- uncertainties in clinical trial enrollment rate or design;
- significant and changing government regulation;
- the timing and receipt of any regulatory approvals;
- the influence of the FDA or other regulatory authorities on our clinical trial design and timing;
- establishing manufacturing capabilities or making arrangements with third-party manufacturers and risk involved with development of manufacturing processes, FDA pre-approval inspection practices and successful completion of manufacturing batches for clinical development and other regulatory purposes;
- our ability to obtain and maintain patent and trade secret protection and regulatory exclusivity for our product candidates; and
- our ability to recruit and retain key research and development personnel.

A change in the outcome of one or more of these variables with respect to the development of a product candidate could significantly change the costs, timing and viability associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct additional non-clinical or clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, consisting of salaries, related benefits and stock-based compensation, costs related to our executive, finance, information technology, and costs related to other administrative functions. General and administrative expenses also include insurance expenses and professional fees for auditing, tax, and legal services, including legal expenses to pursue patent protection for our intellectual property. We expect that our general and administrative expenses will increase in the foreseeable future as we hire additional employees to implement, improve and scale our operational, financial, commercial and management systems.

Results of Operations

Comparison of three months ended June 30, 2025 and 2024

The following table summarizes our results of operations for the three months ended June 30, 2025 and 2024:

	Three Months Ended June 30,		
	2025	2024	Increase (Decrease)
		(in thousands)	
Statement of Operations Data:			
Operating expenses:			
Research and development	$ 23,368	$ 19,682	$ 3,686
General and administrative	4,424	4,917	(493)
Total operating expenses	27,792	24,599	3,193
Loss from operations	(27,792)	(24,599)	(3,193)
Other income (expense), net	1,610	2,972	(1,362)
Net loss	$ (26,182)	$ (21,627)	$ (4,555)

Research and Development Expenses

Research and development expenses for the three months ended June 30, 2025 increased $3.7 million compared to the three months ended June 30, 2024. The increase in research and development expenses was primarily attributable to an increase of $2.4 million in professional consulting fees associated with ongoing clinical studies and worldwide regulatory activities, an increase of $1.3 million in personnel costs associated with increased headcount, an increase of $0.6 million in clinical costs primarily associated with the start of an anticipated confirmatory study that would be required as part of an accelerated approval of our planned BLA submission expected in the second quarter of 2026, partially offset by a decrease of $0.8 million in costs incurred under related to the Friedreich's Ataxia Research Alliance (FARA) TRACK-FA program (a neuroimaging consortium that includes pharmaceutical, biotechnology, academic and clinical partners. The consortium will conduct a natural history study designed to establish disease-specific neuroimaging biomarkers to track disease progression in the brain and spinal cord and provide a basis for utilizing these biomarkers in clinical trials).

General and Administrative expenses

General and administrative expenses for the three months ended June 30, 2025 decreased $0.5 million compared to the three months ended June 30, 2024. The decrease in general and administrative expenses was primarily due to a decrease of $0.4 million in stock compensation costs related to the full vesting of stock options granted at higher value stock options and a decrease of $0.3 million in professional service expenses primarily related to legal services performed, partially offset by an increase of $0.1 million of personnel costs associated with increased headcount.

Other Income (expense), net

Other income (expense), net was $1.6 million income in the three months ended June 30, 2025 compared to $3.0 million income in the three months ended June 30, 2024. The decrease relates to a lower levels of investable cash, cash equivalents and marketable securities.

Comparison of six months ended June 30, 2025 and 2024

The following table summarizes our results of operations for the six months ended June 30, 2025 and 2024:

	Six Months Ended June 30,		
	2025	2024	Increase (Decrease)
		(in thousands)	
Statement of Operations Data:			
Operating expenses:			
Research and development	$ 49,919	$ 32,621	$ 17,298
General and administrative	9,060	8,712	348
Total operating expenses	58,979	41,333	17,646
Loss from operations	(58,979)	(41,333)	(17,646)
Other income (expense), net	3,516	5,052	(1,536)
Net loss	$ (55,463)	$ (36,281)	$ (19,182)

Research and Development Expenses

Research and development expenses for the six months ended June 30, 2025 increased $17.3 million compared to the six months ended June 30, 2024. The increase in research and development expenses was primarily attributable to an increase of $7.1 million in nomlabofusp manufacturing costs, an increase of $3.5 million in professional consulting fees associated with increasing clinical, regulatory and quality operations, an increase of $3.4 million in clinical costs primarily associated with the start of an anticipated confirmatory study that would be required as part of an accelerated approval of our planned BLA submission expected in the second quarter of 2026, an increase of $2.9 million in personnel costs, an increase of $0.8 million in nonclinical costs related to assay development, and partially offset by a decrease of $0.8 million in costs incurred under the TRACK-FA program discussed above.

General and Administrative expenses

General and administrative expenses for the six months ended June 30, 2025 increased $0.3 million compared to the six months ended June 30, 2024. The increase in general and administrative expenses was primarily due to an increase of $0.8 million in personnel costs and an increase of $0.3 million in professional services primarily related to consulting fees associated with ongoing pre-commercial pre-launch activities, partially offset by a decrease of $0.7 million in stock compensation costs related to the full vesting of stock options granted at higher value stock options higher value stock options than those that have recently been granted.

Other Income (expense), net

Other income (expense), net was $3.5 million income in the six months ended June 30, 2025 compared to $5.1 million income in the six months ended June 30, 2024. The decrease primarily relates to a lower levels of investable cash, cash equivalents and marketable securities.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from our operations. We have devoted substantially all of our resources to developing nomlabofusp, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, capital raising, and providing general and administrative support for such operations.

Cash Flows

The following table summarizes our sources and uses of cash for each of the periods presented below:

	Six Months Ended June 30,	
	2025	2024
	(in thousands)	
Net cash used in operating activities	$ (45,921)	$ (24,399)
Net cash provided by (used in) investing activities	33,290	(131,856)
Net cash provided by financing activities	—	161,817
Net increase in cash, cash equivalents and restricted cash	$ (12,631)	$ 5,562

Net cash used in operating activities

During the six months ended June 30, 2025, operating activities used $45.9 million of cash, resulting from our net loss of $55.5 million, adjusted for noncash expenses of $2.5 million and changes in our operating assets and liabilities resulting in a source of cash of $7.1 million. Our net loss was primarily attributed to research and development activities related to our nomlabofusp program and our general and administrative expenses as described above. Noncash expenses primarily relate to stock-based compensation expenses. The change in operating assets and liabilities was primarily due to a increases in prepaid expenses, accounts payable and accrued expenses.

During the six months ended June 30, 2024, operating activities used $24.4 million of cash, resulting from our net loss of $36.3 million, adjusted for noncash expenses of $2.1 million and changes in our operating assets and liabilities resulting in a source of cash of $9.7 million. Our net loss was primarily attributed to research and development activities related to our nomlabofusp program and our general and administrative expenses as described above. Noncash expenses are primarily stock-based compensation expenses. The change in operating assets and liabilities was primarily due to an increase in accrued expenses and accounts payable partially offset by a decrease in prepaid expense.

Net cash provided by (used in) investing activities

During the six months ended June 30, 2025, investing activities provided $33.3 million. This source of cash resulted from the maturities of $113.5 million of marketable securities partially offset by purchases of $80.1 million of marketable securities.

During the six months ended June 30, 2024, investing activities used $131.9 million of cash. This net use of cash was due to the purchase of $166.6 million of marketable securities, partially offset by $35.0 million of cash provided by maturities of marketable securities.

Net cash provided by financing activities

During the six months ended June 30, 2025, there were no financing activities.

During the six months ended June 30, 2024, financing activities provided $161.8 million of cash flows primarily from an offering of common stock.

Operating Capital Requirements

We have not yet commercialized any products and do not expect to generate revenue from the commercial sale of any products for several years, if at all.

We have to date incurred net losses. We incurred net losses of approximately $55.5 million and $36.3 million for the six months ended June 30, 2025 and 2024, respectively. As of June 30, 2025, we had an accumulated deficit of $324.6 million and cash cash equivalents and marketable securities of $138.5 million, excluding restricted cash of $0.6 million.

Losses have resulted principally from costs incurred in connection with research and development activities, and general and administrative costs associated with the development of nomlabofusp and our operations. We expect to incur significant expenses and operating losses for the foreseeable future as we expect to continue to incur expenses in connection with our ongoing activities, if and as we:

- continue to advance the development of nomlabofusp through additional clinical trials, including related manufacturing costs;
- seek to identify and advance development of additional product candidates into clinical development and identify additional indications for our product candidates;
- seek to obtain regulatory approvals for nomlabofusp and other potential product candidates;
- identify, acquire or in-license other product candidates and technologies;
- maintain, leverage and expand our intellectual property portfolio; and
- expand our operational, financial, commercial and management systems and personnel, including personnel to support our clinical development and future commercialization efforts and our operations as a public company.

We anticipate that current cash, cash equivalents and marketable securities as of June 30, 2025,combined with the net proceeds of $65.1 million from our July, 2025 underwritten public offering, will fund operations into the fourth quarter of 2026. If we encounter unexpected delays in our clinical trials or if there are other unanticipated changes to our operating plan from our current assumptions that negatively impact our operations, we may reduce expenditures in order to further extend our existing cash resources. Until we can generate substantial revenue, if ever, we expect to seek additional funding through a combination of public or private equity offerings, debt/royalty financings, collaborations, strategic alliances and licensing arrangements or other sources. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, minimum cash balances, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms, if at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or we do not have sufficient authorized shares, we may be required to delay, limit, or eliminate the development of business opportunities and our ability to achieve our business objectives, our competitiveness, and our business, financial condition, and results of operations will be materially adversely affected. We could also be required to seek funds through arrangements with collaborative partners, strategic alliances or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects. In addition, geopolitical tensions including regional conflicts around the world, adverse macroeconomic events, including those due to inflationary pressures, rising interest rates, banking instability, monetary policy changes, changes in trade policies, including tariffs (including tariffs and trade protection measures that have been or may in the future be imposed by the U.S. or other countries), new laws and regulations, including, but not limited to the recently enacted Public Law 119-21,economic slowdowns or recessions, health epidemics, unforeseen emergencies and other outbreaks of communicable diseases could disrupt our operations, the operations of third parties on which we rely or the operations of regulatory agencies we interact with in the development of nomlabofusp and any other product candidates that we may develop which may reduce our ability to access capital, which could negatively affect our liquidity and ability to continue as a going concern.

If we are unable to obtain sufficient funding when needed and/or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion and/or pre commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. Certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our

business. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

Off-Balance Sheet Arrangements

During the periods presented we did not have, and we currently do not have, any off-balance sheet arrangements, as defined under applicable SEC rules, such as relationships with unconsolidated entities or financial partnerships, which are often referred to as structured finance or special purpose entities, established for the purpose of facilitating financing transactions that are not required to be reflected on our balance sheets.

Recently Issued Accounting Pronouncements

Please read Note 2 to our condensed consolidated financial statements included in Part I of Item 1 of this Quarterly Report on Form 10-Q for a description of recent accounting pronouncements applicable to our business, if any.

Other Company Information

None.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

We are a "smaller reporting company" as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and are not required to provide the information under this item.

Item 4. Controls and Procedures

We maintain “disclosure controls and procedures,” as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, to allow timely decisions regarding required disclosure.

The design of any disclosure controls and procedures also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

With respect to the quarter ended June 30, 2025, under the supervision and with the participation of our management, we conducted an evaluation of the effectiveness of the design and operations of our disclosure controls and procedures. Based upon this evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures are effective.

Management does not expect that our internal control over financial reporting will prevent or detect all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control systems are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in a cost-effective control system, no evaluation of internal control over financial reporting can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been or will be detected.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the fiscal quarter ended June 30, 2025 which 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 are subject to claims in legal proceedings arising in the normal course of business. To our knowledge, during the six months ended June 30, 2025, there were no, and as of the date of this Quarterly Report, there are no, threatened or pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

Item 1A. Risk Factors

You should carefully consider the risk factors described in our 2024 Annual Report under the caption "Item 1A. Risk Factors." The risks described in our 2024 Annual Report are not the only risks facing our company. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial also may materially adversely affect our business, financial condition or future results.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Rule 10b5-1 Trading Arrangements

During the quarter ended June 30, 2025, none of our directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" (as each term is defined in Item 408(a) of Regulation S-K).

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report are set forth on the Exhibit Index, which is incorporated herein by reference.

EXHIBIT INDEX

Exhibit No.	Description
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	Inline XBRL Instance Document- the instance document does not appear in the Interactive Data File because its XBRL tag re embedded within the Inline XBRL document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Filed herewith.

** Furnished herewith.

SIGNATURES

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

LARIMAR THERAPEUTICS, INC.

Date: August 14, 2025

By: /s/ Carole S. Ben-Maimon, M.D.
Carole S. Ben-Maimon, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 14, 2025

By: /s/ Michael Celano
Michael Celano
Chief Financial Officer
(Principal Financial and Accounting Officer)