Larimar Therapeutics Reports First Quarter 2025 Financial Results

April 30, 2025

- *FDA stated as part of a START pilot program meeting that it is open to considering skin FXN concentration as a reasonably likely surrogate endpoint in support of an accelerated approval*
- *BLA seeking accelerated approval planned to be submitted by year-end 2025; global Phase 3 study planned to initiate in mid-2025*
- *Completed dosing in adolescent PK run-in study; topline 50 mg dose data from the OLE study and data from adolescent cohort planned for program update in September 2025*
- *Strong balance sheet of $157.5 million cash, cash equivalents and marketable securities as of March 31, 2025, with projected cash runway into second quarter of 2026*

BALA CYNWYD, Pa., April 30, 2025 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its first quarter 2025 operating and financial results.

“Our strong execution across our nomlabofusp clinical development program continues and we are focused on delivering the first potential disease modifying therapy to patients with FA. Importantly, with the robust preclinical and clinical data package we have in hand and the additional clinical data readouts coming later this year, coupled with the positive FDA feedback and recommendations in line with our current approach, we are on track to submit our planned Biologics License Application (BLA) by the end of 2025 to seek accelerated approval,” said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. “We continue to have frequent communication with the FDA via our START pilot program participation including interactions regarding the adequacy of the safety data set required to support BLA submission. In mid-2025 we expect to initiate our global Phase 3 study following global regulatory feedback on the study protocol, and we also expect to transition to the lyophilized formulation of nomlabofusp. We completed dosing of adolescents in our pharmacokinetic (PK) run-in study and expect to report adolescent data from that study, along with data from participants receiving the 50 mg dose of nomlabofusp daily in our long-term open label extension (OLE) study. This program update will be in September 2025. We look forward to these important near-term catalysts as we work to bring nomlabofusp towards potential registration.”

Recent Highlights

- **Completed Dosing of Adolescents in Pharmacokinetic (PK) Run-In Study:** Today Larimar announced completion of dosing of adolescents (12-17 years of age) in a PK run-in study for pediatric patients with FA. Adolescents received a weight-based dose expected to match PK of adults receiving the 50 mg dose. Following assessment of safety and PK data, participants will be eligible to screen for the OLE study. The data from this cohort are expected to be presented during the nomlabofusp program update in September 2025.
- **Potential for Accelerated Approval Pathway Based on Skin FXN Concentrations:** In March 2025, Larimar announced that the FDA is open to the use of FXN concentrations as a reasonably likely surrogate endpoint (RLSE) and the acceptability of FXN concentrations as an RLSE to support approval will be a matter of review in a future marketing application. FDA recommended focusing on assessments of skin FXN concentrations and acknowledged that submitted data 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 suggested exploring the relationship between increases in FXN in skin and changes in pharmacodynamic markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as a RLSE.
- **Planned Upcoming Regulatory Discussions:** Larimar continues to interact with FDA under the START pilot program, including seeking feedback on the adequacy of the safety data set required to support BLA submission.
- **50 mg OLE Data Expected in September 2025:** The ongoing OLE study continues to enroll, and active study participants are currently receiving the 50 mg dose of nomlabofusp. Larimar plans to provide an update on OLE data on at least 30 to 40 study participants, including 50 mg dose data, in September 2025.

- **Planned Transition to Lyophilized Form of Nomlabofusp:** Larimar plans to introduce the lyophilized product formulation intended for commercialization into the clinical development program in mid-2025.
- **BLA Submission and Initiation of Global Phase 3 Study on Track:** Global Phase 3 study is on track to initiate in mid-2025 with potential sites in the U.S., Europe, U.K., Canada, and Australia. Larimar plans to submit the BLA submission seeking accelerated approval by the end of 2025 to seek accelerated approval.

First Quarter 2025 Financial Results

As of March 31, 2025, the Company had cash, cash equivalents and marketable securities totaling $157.5 million.

The Company reported a net loss for the first quarter of 2025 of $29.3 million, or $0.46 per share, compared to a net loss of $14.7 million, or $0.27 per share, for the first quarter of 2024.

Research and development expenses for the first quarter of 2025 were $26.6 million, compared to $12.9 million for the first quarter of 2024. The increase in research and development expenses was primarily driven by an increase of $7.1 million in nomlabofusp manufacturing costs, an increase of $2.8 million in clinical costs primarily associated with the PK run-in study and start-up costs related to the Company's planned global Phase 3 study, an increase of $1.6 million in personnel expense due to increased headcount, and an increase of $1.2 million in consulting fees.

General and administrative expenses were $4.6 million in the first quarter of 2025, compared to $3.8 million in the first quarter of 2024. The increase in general and administrative expenses was primarily driven by an increase of $0.7 million in personnel expense, an increase of $0.5 million in consulting fees primarily associated with commercial activities, partially offset by a decrease of $0.3 million in stock compensation expense.

About Larimar Therapeutics
Larimar Therapeutics, Inc. (Nasdaq: LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. Larimar's lead compound, nomlabofusp, is being developed as a potential treatment for Friedreich's ataxia. Larimar also plans to use its intracellular delivery platform to design other fusion proteins to target additional rare diseases characterized by deficiencies in intracellular bioactive compounds. For more information, please visit: https://larimartx.com.

Forward-Looking Statements
This press release contains forward-looking statements that are based on Larimar's management's beliefs and assumptions and on information currently available to management. All statements contained in this release other than statements of historical fact are forward-looking statements, including but not limited to statements regarding Larimar's ability to develop and commercialize nomlabofusp and any other planned product candidates, Larimar's planned research and development efforts, including the timing of its nomlabofusp clinical trials, interactions and filings with the FDA, expectations regarding potential for accelerated approval or accelerated access and time to market and overall development plans and other matters regarding Larimar's business strategies, ability to raise capital, use of capital, results of operations and financial position, and plans and objectives for future operations.

In some cases, you can identify forward-looking statements by the words "may," "will," "could," "would," "should," "expect," "intend," "plan," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue," "ongoing" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, the success, cost and timing of Larimar's product development activities, nonclinical studies and clinical trials, including nomlabofusp clinical milestones and continued interactions with the FDA; that preliminary clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of nomlabofusp may not be predictive of the results or success of later clinical trials, and assessments; that the FDA may not ultimately agree with Larimar's nomlabofusp development strategy; the potential impact of public health crises on Larimar's future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and general economic conditions; Larimar's ability and the ability of third-party manufacturers Larimar engages, to optimize and scale nomlabofusp's manufacturing process; Larimar's ability to obtain regulatory approvals for nomlabofusp and future product candidates; Larimar's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators, and to successfully commercialize any approved product candidates; Larimar's ability to raise the necessary capital to conduct its product development activities; and other risks described in the filings made by Larimar with the Securities and Exchange Commission (SEC), including but not limited to Larimar's periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. These forward-looking statements are based on a combination of facts and factors currently known by Larimar and its projections of the future, about which it cannot be certain. As a result, the forward-looking statements may not prove to be accurate. The forward-looking statements in this press release represent Larimar's management's views only as of the date hereof. Larimar undertakes no obligation to update any forward-looking statements for any reason, except as required by law.

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Larimar Therapeutics, Inc.
Condensed Consolidated Balance Sheets

(unaudited)

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 21,126	$ 33,218
Marketable securities	136,400	150,236
Prepaid expenses and other current assets	7,959	11,850
Total current assets	165,485	195,304
Long-term marketable securities		—
Property and equipment, net	855	881
Operating lease right-of-use assets	2,647	2,838
Restricted cash	606	606
Other assets	582	596
Total assets	$ 170,175	$ 200,225
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,274	$ 2,424
Accrued expenses	18,764	20,872
Operating lease liabilities, current	1,096	1,060
Total current liabilities	22,134	24,356
Operating lease liabilities	3,770	4,057
Total liabilities	25,904	28,413
Commitments and contingencies		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of March 31, 2025 and December 31, 2024; no shares issued and outstanding as of March 31, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of March 31, 2025 and December 31, 2024; 64,027,892 and 63,815,065 shares issued and outstanding as of March 31, 2025 and December 31, 2024, respectively	64	64
Additional paid-in capital	442,592	440,758
Accumulated deficit	(298,439)	(269,158)
Accumulated other comprehensive gain	54	148
Total stockholders' equity	144,271	171,812
Total liabilities and stockholders' equity	$ 170,175	$ 200,225

Larimar Therapeutics, Inc.
Consolidated Statements of Operations
(In thousands, except share and per share data)
(unaudited)

	Three Months Ended March 31,	
	2025	2024
Operating expenses:		
Research and development	$ 26,552	$ 12,939
General and administrative	4,636	3,795
Total operating expenses	31,188	16,734
Loss from operations	(31,188)	(16,734)
Other income, net	1,907	2,080
Net loss	$ (29,281)	$ (14,654)
Net loss per share, basic and diluted	$ (0.46)	$ (0.27)
Weighted average common shares outstanding, basic and diluted	63,964,008	53,553,707
Comprehensive loss:		
Net loss	$ (29,281)	$ (14,654)
Other comprehensive (loss):		
Unrealized (loss) on marketable securities	(94)	(106)
Total other comprehensive (loss)	(94)	(106)
Total comprehensive loss	$ (29,375)	$ (14,760)



Source: Larimar Therapeutics