Larimar Therapeutics Provides Nomlabofusp Development Update and Reports Fourth Quarter and Full Year 2024 Financial Results

March 24, 2025

- *FDA stated in written correspondence for 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*
- *FDA recommended measuring skin FXN concentrations to support evidence of effectiveness for accelerated approval pathway and acknowledged submitted data appear sufficient to support relationship between increased skin FXN concentrations and relevant tissues such as heart, dorsal root ganglia and skeletal muscle*
- *Larimar is continuing discussions with FDA on the adequacy of the safety data set to support BLA submission*
- *BLA seeking accelerated approval targeted for submission by year-end 2025*
- *FDA and EMA feedback obtained on global Phase 3 study protocol; study planned to initiate in mid-2025*
- *Topline 50 mg dose data from the OLE study and available data from the adolescent cohort of the PK run-in study planned for program update in September 2025*
- *Strong balance sheet of $183.5 million cash, cash equivalents and marketable securities as of December 31, 2024, with projected cash runway into second quarter of 2026*

BALA CYNWYD, Pa., March 24, 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 fourth quarter and full year 2024 operating and financial results.

“The strong clinical and regulatory progress across our nomlabofusp program reinforces the timing of our planned Biologics License Application (BLA) submission expected by the end of 2025 to seek accelerated approval. Importantly, we are enthusiastic about the recent FDA interactions and their openness to consider skin frataxin (FXN) concentrations as a potential novel surrogate endpoint reasonably likely to predict clinical benefit in patients with Friedreich’s ataxia (FA). Acceptability of FXN as a novel surrogate endpoint will be based on review of the data by the Food and Drug Administration (FDA) in the future BLA. As we previously disclosed, we have been collecting skin FXN concentration data throughout the development program,” said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. “Overall, FDA’s recommendations are in line with our current approach, and we appreciate the frequent dialogue via our participation in the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program as we further refine our registrational plan.”

Dr. Ben-Maimon continued, “Our open label extension (OLE) study continues to advance, with some participants on daily treatment for up to one year. We continue to actively enroll study participants, and all participants are currently receiving the 50 mg dose. The 25 mg initial data we presented in December showed increased and sustained FXN levels over time and early trends in improved clinical outcomes in patients with FA. Our pediatric pharmacokinetic (PK) run-in study continues to progress, and we expect to complete the dosing of the adolescent cohort by the end of this month and report available data in September 2025. We also plan to enroll children 2-11 years of age in the clinical program. We have received feedback from both FDA and European Medicines Agency (EMA) on the protocol for our global Phase 3 trial and are on track to initiate this trial mid-2025. We believe our near-term data package strongly positions us to bring the first potential disease modifying therapy to patients with FA.”

Recent Highlights

- **Potential for Accelerated Approval Pathway Based on FXN Concentrations as a Reasonably Likely Surrogate Endpoint (RSLE):** FDA stated in written correspondence associated with a meeting through the START pilot program that they are open to considering the use of FXN concentration as a RLSE and the acceptability of FXN’s use as an RLSE would ultimately be a matter of review of the data in a future marketing application.
- **Increases in Skin FXN as Evidence of Effectiveness:** 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 recently 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 were performed at relevant human doses.

- **Additional Analyses Under Consideration:** FDA also suggested that Larimar consider 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 is continuing discussions with FDA regarding 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 all 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, some of whom have been receiving nomlabofusp for over one year, in September 2025.

- **OLE Protocol Amended to Include Premedication:** Larimar's Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, Larimar expects to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, Larimar has amended the OLE protocol to administer premedication for the first month of dosing.

- **Continuing Dosing of Adolescents in Pharmacokinetic (PK) Run-In Study:** In January 2025, Larimar initiated dosing of adolescents (12-17 years of age) in a PK run-in study for pediatric patients with FA. In this study, adolescents are receiving a weight-based dose expected to match the 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. Larimar anticipates the dosing of this cohort will be completed by the end of this month. The data from this cohort is expected to be presented during the nomlabofusp program update in September 2025.

- **FDA Acceptance to Transition to Lyophilized Form of Nomlabofusp:** In February 2025, FDA accepted the data supporting the comparability of the lyophilized drug product to the frozen solution and agreed with Larimar's plans to introduce the lyophilized product into our clinical development program in mid-2025. The frozen solution is the dosage form currently being used in the OLE. The lyophilized drug product is the formulation that Larimar intends to commercialize.

- **BLA Submission and Initiation of Global Phase 3 Study on Track:** Larimar has obtained feedback from both FDA and EMA on the global Phase 3 study protocol and is on track to initiate the study by mid-2025 with potential sites in the U.S., Europe, U.K., Canada, and Australia. Larimar is targeting the BLA submission to seek accelerated approval by the end of 2025.

Fourth Quarter and Full Year 2024 Financial Results

As of December 31, 2024, the Company had cash, cash equivalents and marketable securities totaling $183.5 million.

The Company reported a net loss for the fourth quarter of 2024 of $28.8 million, or $0.45 per share, compared to a net loss of $13.0 million, or $0.30 per share, for the fourth quarter of 2023.

Research and development expenses for the fourth quarter of 2024 were $26.7 million compared to $10.6 million for the fourth quarter of 2023. The increase in research and development expenses was primarily driven by an increase of $15.0 million in nomlabofusp manufacturing costs including lyophilization development, production scaling costs and manufacturing costs related to producing doses to be used in ongoing and planned clinical trials, an increase of $1.3 million in personnel expense due to increased headcount, an increase of $1.0 million of professional fees related to consulting costs, and an increase of $0.5 million in stock compensation costs associated with 2024 grants, partially offset by a decrease of $1.7 million in clinical trial costs primarily associated with decreased activity in the dose exploration study in 2024.

General and administrative expenses for the fourth quarter of 2024 were $4.6 million compared to $3.5 million for the fourth quarter of 2023. The increase in general and administrative expenses was primarily driven by an increase of $0.6 million in professional fees primarily related to consulting costs related to commercial activity and other public company related expenses, and an increase of $0.4 million in personnel expense due to increased headcount.

Other income (expense), net was $2.5 million of income in the three months ended December 31, 2024 compared to $1.2 million of income in the three months ended December 31, 2023. The increase primarily relates to interest income on a higher investment base and higher investment yields on that base during the current period.

For the full year 2024, the Company reported a net loss of $80.6 million, or $1.32 per share, compared to a net loss of $36.9 million, or $0.84 per share, for the same period in 2023.

Research and development expenses for the full year 2024 were $73.3 million compared to $27.7 million for the same period in 2023. The increase in research and development expenses was primarily driven by an increase of $36.1 million in nomlabofusp manufacturing costs including lyophilization development, production scaling costs and manufacturing costs related to producing doses to be used in ongoing and planned clinical trials, an increase of $4.5 million in personnel expense due to increased headcount, an increase of $1.3 million of professional fees related to consulting costs, an increase of $1.2 million in clinical costs primarily associated with the ongoing OLE study which began dosing participants in the first quarter of 2024, an increase of $1.0 million in stock compensation costs associated with 2024 equity compensation grants, an increase of $0.9 million related to the

Friedreich's Ataxia Research Alliance (FARA) Track-FA program, an increase of $0.3 million in internal lab costs and an increase of $0.2 million in facility costs associated with the new lab space.

General and administrative expenses for the full year 2024 were $17.6 million compared to $14.1 million for 2023. The increase in general and administrative expenses was primarily driven by an increase of $1.5 million in professional fees primarily related to consulting costs related to commercial activity and other public company related expenses, an increase of $1.4 million in personnel expense due to increased headcount, an increase of $0.4 million of other expense related to computer software, information technology services and recruiting and an increase of $0.2 million in stock compensation costs associated with 2024 equity compensation grants.

Other income (expense), net was $10.3 million of income in the twelve months ended December 31, 2024 compared to $4.8 million of income in the twelve months ended December 31, 2023. The increase primarily relates to increased interest income on a higher investment base and higher investment yields on that base during 2024.

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; delays in patient recruitment, including as a result of changes in clinical protocols and adverse events; 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.

Investor Contact:
Joyce Allaire
LifeSci Advisors
jallaire@lifesciadvisors.com
(212) 915-2569

Company Contact:
Michael Celano
Chief Financial Officer
mcelano@larimartx.com
(484) 414-2715

Larimar Therapeutics, Inc.
Consolidated Balance Sheets

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 33,218	$ 26,749
Marketable securities	150,236	60,041
Prepaid expenses and other current assets	11,850	3,385
Total current assets	195,304	90,175
Property and equipment, net	881	684
Operating lease right-of-use assets	2,838	3,078
Restricted cash	606	1,339

Other assets	596	659
Total assets	$ 200,225	$ 95,935
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,424	$ 1,283
Accrued expenses	20,872	7,386
Operating lease liabilities, current	1,060	837
Total current liabilities	24,356	9,506
Operating lease liabilities	4,057	4,709
Total liabilities	28,413	14,215
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of December 31, 2024 and December 31, 2023; no shares issued and outstanding as of December 31, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of December 31, 2024 and December 31, 2023; 63,815,065 and 43,909,069 shares issued and outstanding as of December 31, 2024 and December 31, 2023, respectively	64	43
Additional paid-in capital	440,758	270,150
Accumulated deficit	(269,158)	(188,554)
Accumulated other comprehensive gain (loss)	148	81
Total stockholders' equity	171,812	81,720
Total liabilities and stockholders' equity	$ 200,225	$ 95,935

Larimar Therapeutics, Inc.

Consolidated Statements of Operations

(In thousands, except share and per share data)

	Three Months Ended December 31,		Three Months Ended December 31,	
	2024	**2023**	**2024**	**2023**
Operating expenses:				
Research and development	$ 26,738	$ 10,648	$ 73,278	$ 27,670
General and administrative	4,555	3,514	17,612	14,088
Total operating expenses	31,293	14,162	90,890	41,758
Loss from operations	(31,293)	(14,162)	(90,890)	(41,758)
Other income, net	2,469	1,169	10,286	4,809
Net loss	$ (28,824)	$ (12,993)	$ (80,604)	$ (36,949)
Net loss per share, basic and diluted	$ (0.45)	$ (0.30)	$ (1.32)	$ (0.84)
Weighted average common shares outstanding, basic and diluted	63,810,823	43,906,281	61,256,084	43,901,241

Source: Larimar Therapeutics