Larimar Therapeutics Reports Third Quarter 2025 Financial Results

November 5, 2025

- *In the open label (OL) study, after 6-months of daily nomlabofusp administration, 100% of participants (n = 10) achieved skin FXN levels similar to asymptomatic carriers*
- *Consistent directional improvement across mFARS, FARS-ADL, 9-HPT and MFIS after 1-year in OL study reinforces the potential of nomlabofusp to alter FA's disease course relative to a worsening in a FACOMS natural history study reference population*
- *Of 39 participants in OL study (and of 65 total participants who received at least 1 dose in all nomlabofusp studies), 7 experienced anaphylaxis in the first 6 weeks of dosing and returned to usual state of health after standard treatment; excluding these events, long term dosing of nomlabofusp was generally well tolerated including 8 participants on treatment for over 1 year*
- Anaphylaxis is more common upon re-exposure to a drug after a gap in dosing; in the OL study, of the 10 participants who had not had prior exposure to nomlabofusp only 1 experienced anaphylaxis (this reaction was one of the 7 events discussed above).
- *Implementing a modified starting dose regimen designed to mitigate the risk of anaphylaxis events as agreed to by the FDA*
- *Modified starting dose regimen is also being incorporated into the Phase 3 protocol; Larimar continues to qualify global Phase 3 sites and prepare for study initiation and patient enrollment*
- *BLA submission seeking accelerated approval targeted in the second quarter of 2026*
- *$175.4 million in cash, cash equivalents and marketable securities as of September 30, 2025, with projected cash runway into the fourth quarter of 2026*

BALA CYNWYD, Pa., Nov. 05, 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 third quarter 2025 operating and financial results.

"We were pleased to recently share exciting long-term data from our open label study showing consistent directional improvement across four key clinical outcome measures relative to a Friedrich's Ataxia Clinical Outcomes Measure Study (FACOMS) reference population, and increased skin frataxin (FXN) levels similar to asymptomatic carriers. These findings underscore the potential of daily nomlabofusp treatment to help change the disease course of patients living with Friedreich's ataxia (FA), including those with advanced disease," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "We are implementing a modification to the starting dose regimen to help mitigate the risk of occurrence of anaphylactic reactions which seem to be more common in participants with prior exposure to nomlabofusp. Long-term treatment with daily nomlabofusp, including 8 participants for over 1 year, was generally well-tolerated."

Dr. Ben-Maimon continued, "With this positive data in hand, and a targeted path to registration, we continue to target our Biologics License Application (BLA) submission in the second quarter of 2026 seeking accelerated approval. We plan to provide a nomlabofusp development program update that will include status on our regulatory discussions and OL study in the first quarter of 2026. We are focused on execution of our near-term milestones that will advance nomlabofusp as the first potential disease modifying therapy designed to address the root cause of FA."

Highlights

Positive 50 mg Data from the Ongoing OL Study: In September, Larimar presented long-term data from the OL study showing 100% of participants (n=10) with data at 6 months achieved skin FXN levels over 50% of median levels in healthy volunteers (which is similar to levels in asymptomatic carriers). A 2.25 median improvement in modified Friedreich Ataxia Rating Scale (mFARS) score, a primary outcome measure in other clinical studies, was observed in participants after 1 year relative to a worsening of a median 1.00 observed in a FACOMS natural history reference population. Additionally, consistent directional improvements observed after 1 year across 4 key clinical outcomes (mFARS, FARS-Activities of Daily Living (ADL), 9 Hole Peg Test (9-HPT), and Modified Fatigue Impact Scale (MFIS)) suggest potential for clinical benefit relative to a worsening in the FACOMS reference population. Of the 39 participants in the OL study (and of 65 total participants who received at least 1 dose in all nomlabofusp studies), 7 experienced anaphylaxis in the first 6 weeks of dosing and returned to their usual state of health after standard treatment. Anaphylaxis is more common upon re-exposure to a drug after a gap in dosing; in the OL study, of the 10 participants who had not had prior exposure to nomlabofusp only 1 experienced anaphylaxis (this reaction was one of the 7 events discussed above). Excluding these events, long term dosing of nomlabofusp was generally well tolerated including 14 on treatment for at least 6 months and 8 on treatment for over 1 year.

- **Similar PK and Exposure in Adolescents and Adults:** Exposure and PK in 14 adolescents spanning 12 to 17 years of age (n = 14, 9 on nomlabofusp, 5 on placebo) who received a weight-based equivalent of 50 mg for 7 days were similar to adults on 50 mg of nomlabofusp.

- **Modifying Starting Dose Regimen:** Larimar is implementing a modified starting dose regimen that is designed to mitigate the risk of anaphylactic reactions. The new dosing regimen includes a 5 mg test dose followed by a 25 mg dose one hour later under observation. Nomlabofusp 25 mg will then be administered once daily through Day 30 and then the dose will be increased to 50 mg once daily.
- **Expanding Enrollment of OL Study:** Participants who completed treatment in a Phase 1 or Phase 2 study evaluating nomlabofusp were the first group of eligible patients to screen for the OL study. The OL study protocol has now been amended to include adolescent and adult patients who have not participated in a prior nomlabofusp study. Larimar plans to enroll children (2 to 11 years of age) directly into the open label study.
- **Amending Phase 3 Protocol and Qualifying Global Sites:** The modified starting dose regimen is being incorporated into the Phase 3 protocol. Larimar continues to qualify sites globally and prepare for study initiation and patient enrollment.
- **Advancing Chemistry Manufacturing and Control (CMC):** Received agreement from FDA on analytical testing requirements including potency testing of nomlabofusp. Process performance qualification (PPQ) on the commercial scale drug substance is planned in the fourth quarter of 2025, in preparation of data for BLA submission. Drug substance manufactured during PPQ activities are expected to be used as the initial commercial launch supply.
- **Nomlabofusp Program Update in the First Quarter of 2026**: Larimar plans to provide an update on regulatory discussions and open label study status in the first quarter of 2026.
- **BLA Submission on Track:** BLA seeking accelerated approval targeted in the second quarter of 2026; U.S. launch targeted for early 2027.

Third Quarter 2025 Financial Results

As of September 30, 2025, the Company had cash, cash equivalents and marketable securities totaling $175.4 million with a projected cash runway into the fourth quarter of 2026. Cash includes the $65.0 million net proceeds from the Company's July 2025 common stock public offering.

Third quarter of 2025 compared to the third quarter of 2024

The Company reported a net loss for the third quarter of 2025 of $47.7 million, or $0.61 per share, compared to a net loss of $15.5 million, or $0.24 per share, for the third quarter of 2024.

Research and development expenses for the third quarter of 2025 were $44.9 million compared to $13.9 million for the third quarter of 2024. The increase in research and development expenses was primarily attributable to an increase of $25.8 million in nomlabofusp manufacturing costs, an increase of $1.8 million in professional consulting fees associated with ongoing clinical studies and worldwide regulatory activities, an increase of $1.5 million in clinical costs primarily associated with the start of an anticipated global 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 $1.0 million in personnel costs associated with increased headcount and an increase of $0.9 million in nonclinical costs related to assay development.

General and administrative expenses were $4.6 million in the third quarter of 2025 compared to $4.3 million in the third quarter of 2024. The increase in general and administrative expenses was primarily due to an increase of $0.3 million of personnel costs associated with increased headcount and an increase of $0.3 million in professional service expenses primarily related to legal services performed, partially offset by a decrease of $0.2 million in stock compensation costs related to the full vesting of stock options granted at higher value stock options.

Nine months ended September 30, 2025, compared to the nine months ended September 30, 2024

The Company reported a net loss for the first nine months of 2025 of $103.2 million, or $1.50 per share, compared to a net loss of $51.8 million, or $0.86 per share, for the first nine months of 2024.

Research and development expenses for the nine months ended September 30, 2025, were $94.9 million compared to $46.5 million for the nine months ended September 30, 2024. The increase in research and development expenses was primarily attributable to an increase of $32.9 million in nomlabofusp manufacturing costs, an increase of $5.4 million in professional consulting fees associated with ongoing clinical studies and worldwide regulatory activities, an increase of $4.8 million in clinical costs primarily associated with the start of an anticipated global 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 $3.9 million in personnel costs associated with increased headcount and an increase of $1.7 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.

General and administrative expenses were $13.6 million for the first nine months of 2025 compared to $13.1 million for the nine months ended September 30, 2024. The increase in general and administrative expenses was primarily due to an increase of $1.1 million of personnel costs associated with increased headcount and an increase of $0.5 million in professional service expenses, partially offset by a decrease of $0.8 million in stock compensation costs related to the full vesting of stock options granted at higher value stock options.

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 the timing of the BLA submission, the expectations of the timing of, and potential for, accelerated approval or accelerated

access, time to launch and 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 and Larimar's ability to timely implement the revised dosing regimen in its clinical program for nomlabofusp; 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.

Investor Contact:	**Company Contact:**
Joyce Allaire	Michael Celano
LifeSci Advisors	Chief Financial Officer
jallaire@lifesciadvisors.com	mcelano@larimartx.com
(212) 915-2569	(484) 414-2715

Larimar Therapeutics, Inc.
Consolidated Balance Sheets
(In thousands except share data)
(unaudited)

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 90,140	$ 33,218
Short-term marketable securities	85,295	150,236
Prepaid expenses and other current assets	7,788	11,850
Total current assets	183,223	195,304
Property and equipment, net	709	881
Operating lease right-of-use assets	2,270	2,838
Restricted cash	606	606
Other assets	542	596
Total assets	$ 187,350	$ 200,225
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 11,100	$ 2,424
Accrued expenses	32,865	20,872
Operating lease liabilities, current	1,151	1,060
Total current liabilities	45,116	24,356
Operating lease liabilities	3,196	4,057
Total liabilities	48,312	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 September 30, 2025 and December 31, 2024; no shares issued and outstanding as of September 30, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of September 30, 2025 and December 31, 2024; 85,590,392 and 63,815,065 shares issued and outstanding as of September 30, 2025 and		

December 31, 2024, respectively	86	64
Additional paid-in capital	511,219	440,758
Accumulated deficit	(372,333)	(269,158)
Accumulated other comprehensive gain	66	148
Total stockholders' equity	139,038	171,812
Total liabilities and stockholders' equity	$ 187,350	$ 200,225

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Operating expenses:				
Research and development	$ 44,931	$ 13,919	$ 94,851	$ 46,540
General and administrative	4,568	4,345	13,628	13,057
Total operating expenses	49,499	18,264	108,479	59,597
Loss from operations	(49,499)	(18,264)	(108,479)	(59,597)
Other income, net	1,787	2,765	5,304	7,817
Net loss	$ (47,712)	$ (15,499)	$ (103,175)	$ (51,780)
Net loss per share, basic and diluted	$ (0.61)	$ (0.24)	$ (1.50)	$ (0.86)
Weighted average common shares outstanding, basic and diluted	78,324,767	63,806,158	68,824,826	60,399,697
Comprehensive loss:				
Net loss	$ (47,712)	$ (15,499)	$ (103,175)	$ (51,780)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	75	508	(82)	277
Total other comprehensive gain (loss)	75	508	(82)	277
Total comprehensive loss	$ (47,637)	$ (14,991)	$ (103,257)	$ (51,503)

Source: Larimar Therapeutics