Larimar Therapeutics Announces Positive Data from Ongoing Long-term Open Label Study and Updates to Nomlabofusp Program for Friedreich's Ataxia

September 29, 2025

- *In 4 completed studies and the ongoing OL study, 65 participants received at least 1 dose of nomlabofusp, including 39 in the OL study, with 14 on treatment for at least 6 months and 8 for over 1 year in the OL study*
- *Increases in skin FXN levels with short- and long-term daily nomlabofusp; 10/10 participants 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)*
- *Consistent directional improvement across 4 key clinical outcomes (mFARS, FARS-ADL, 9-HPT, MFIS) observed after 1 year of nomlabofusp treatment could suggest potential for clinical benefit relative to a worsening in a FACOMS natural history study reference population*
- *Anaphylaxis has been reported in 7 participants in the OL study, with most events occurring on the initial day of administration and all occurring within the first 6 weeks of dosing; excluding these events, long term dosing of nomlabofusp was generally well tolerated*
- *Following the 2 most recent cases of anaphylaxis, Larimar consulted its experts and decided to modify its starting dose regimen; Larimar provided the FDA a full update on the clinical development program including the safety, FXN, and clinical data in this press release and FDA agreed with our approach*
- *BLA submission seeking accelerated approval targeted in Q2 2026*
- *Company management to host webcast and conference call today at 8:00 a.m. ET*

BALA CYNWYD, Pa., Sept. 29, 2025 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced positive 25 mg and 50 mg data from the ongoing long-term open label (OL) study evaluating daily subcutaneous injections of nomlabofusp self-administered or administered by a caregiver in participants with Friedreich's ataxia (FA), a rare, progressive, and systemic disease with neurologic deterioration. The Company also provided a nomlabofusp development program update.

"We are excited to announce the consistent directional improvements across 4 key clinical outcomes observed in the OL study relative to a Friedrich's Ataxia Clinical Outcomes Measure Study (FACOMS) reference population and the observed increase in skin frataxin (FXN) levels. These new data, as well as the improvement in abnormal lipid profiles observed in prior completed studies, provide support that nomlabofusp increases FXN in patients with FA and that the strategy of FXN replacement has the potential to result in a clinical benefit. Importantly, achieving tissue FXN levels equivalent to more than 50% of those found in healthy volunteers means participants are at levels found in asymptomatic carriers who do not develop the disease," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Long-term treatment with daily nomlabofusp, including 8 participants for over 1 year, was generally well-tolerated. Through the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program, Larimar has regularly updated the FDA regarding all aspects of the clinical development program, including the data presented in this press release. We continue to hear strong interest in the nomlabofusp clinical program directly from patients with FA and their parents. The long-term clinical data presented today reinforce our conviction in the potential of nomlabofusp to address the root cause of FA and be the first potential disease modifying therapy."

Dr. Rusty Clayton, Chief Medical Officer of Larimar added, "The changes observed in skin FXN levels, lipid profiles, and clinical outcomes after nomlabofusp administration across diverse participants with FA – including individuals with advanced disease – are all directionally consistent and suggest a potential treatment effect. Allergic reactions, including anaphylaxis, are a known risk associated with nomlabofusp, similar to many other approved therapies, particularly proteins. To date, all anaphylaxis events have occurred within the first 6 weeks of nomlabofusp administration. In this rare neurodegenerative disease with limited therapeutic options, patients with FA continue to express interest in having access to new potentially disease modifying agents."

Jennifer Farmer, Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA) added, "FA is a relentlessly progressive disease that is life-altering and can be life-shortening. Treatment approaches, like nomlabofusp, that target the root cause of FA by FXN supplementation are of great interest to the FA community. We are encouraged by the increases in FXN protein and improved clinical outcomes relative to the FACOMS reference

population observed in the individuals who have maintained nomlabofusp therapy. FA patients and their families are informed and engaged. They understand that therapies come with side effects and risks that must be evaluated in the context of potential benefit. We appreciate Larimar's commitment to patient safety and their regular communications and updates on study outcomes."

The OL study is evaluating the safety and tolerability, pharmacokinetics (PK), and FXN levels in skin and buccal cells, along with exploratory pharmacodynamic (PD) markers (lipid profiles) and clinical outcomes following long-term subcutaneous administration of nomlabofusp. Participants were initially administered 25 mg of nomlabofusp daily. The dose was increased to 50 mg in the fourth quarter of 2024, with all newly enrolled patients receiving the 50 mg dose since November of 2024. 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.

As of August 27, 2025, 39 participants in the OL study had received at least one dose of nomlabofusp and 25 participants (19 adults, 6 adolescents) were receiving daily dosing of nomlabofusp for up to 527 days (mean 154 days). This includes the time from the initial dose of 25 or 50 mg to the last dose of nomlabofusp prior to the data cut off. Among the study participants, approximately 50% were non-ambulatory at baseline.

Long-term Safety with Daily Nomlabofusp in OL Study

- In participants receiving long term continuous treatment, including 14 participants on nomlabofusp for at least 6 months, 8 of whom continue to be on nomlabofusp for over 1 year, daily administration of nomlabofusp was generally well-tolerated.
- Most common adverse events continue to be local injection site reactions that were mild to moderate and did not lead to any participant withdrawal from the study.
- 65 patients have received at least one dose of nomlabofusp in 4 completed studies and the ongoing OL study. Seven OL study participants experienced anaphylaxis and were withdrawn from the study.[1] Most of the events occurred on the initial day of administration and all occurred within the first 6 weeks of dosing; all participants returned to their usual state of health after receiving standard treatment.
- Larimar consulted its experts and decided to modify its starting dose regimen. Larimar provided the FDA with a full update on the clinical development program including the safety, FXN, and clinical data in this press release and FDA agreed with our proposal.

[1]Other discontinuations include 3 cases of generalized urticaria, 1 seizure (the same event as reported in December 2024), 1 vasovagal event, and 2 non-treatment related discontinuations

Observed Increases in FXN with Long-term Daily Nomlabofusp in All Participants

Absolute Median Skin FXN Levels pg/µg (IQR), n*			
Baseline	**1 month**	**3 months**	**6 months**
2.70 (2.14, 4.13), *n = 18*	**6.87** (5.34, 10.37), *n = 18*	**7.50** (6.99, 13.73), *n = 14*	**13.44** (10.10, 26.71), *n = 10*

FXN = frataxin; IQR = interquartile range
Note: Median skin FXN levels in Larimar's noninterventional healthy volunteer study= 16.34 pg/µg
* Data include all participants with quantifiable FXN levels at each measurement point who had received 25 mg, 50 mg or had the dose increased from 25 mg to 50 mg

All Participants who Received Nomlabofusp for 6 months Achieved Skin FXN Levels Similar to Levels Found in Asymptomatic Carriers without Disease

Percentage of Participants* with Skin FXN Levels > 8.2 pg/µg (50% of the median FXN concentration found in Larimar's healthy volunteer study)**			
Baseline	**1 month**	**3 months**	**6 months**
0% (0/18)	**33%** (6/18)	**43%** (6/14)	**100%** (10/10)

*Data include all participants with quantifiable FXN levels at each measurement point who had received 25 mg, 50 mg or had the dose increased from 25 mg to 50 mg
**8.2 pg/µg represents 50% of the median FXN concentration

Consistent Directional Improvement Across 4 Key Clinical Outcomes

- Trends towards improvement were observed in modified Friedreich Ataxia Rating Scale (mFARS), FARS-Activities of Daily Living (ADL), 9 Hole Peg Test (9-HPT), and Modified Fatigue Impact Scale (MFIS) at 1 year relative to baseline
- These clinical findings support that FXN increases after treatment with nomlabofusp may lead to a potential clinical benefit across a broad spectrum of patients with FA, including those with advanced disease

OL Study Clinical Data Relative to FA Natural History Study Data Supports Potential for Clinical Benefit with Nomlabofusp

- Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS), a longitudinal natural history study (N = 955), includes patients with confirmed FA diagnosis
- Based on the range of baseline characteristics of participants in the OL study, Larimar identified patients from the

FACOMS dataset with similar characteristics using data recorded over the last 4 years for each patient

- mFARS has been used as a primary outcome measure in other clinical trials. OL study participants treated for 1 year with nomlabofusp daily demonstrated a median improvement in mFARS score of 2.25 relative to a worsening of 1.00 observed in patients in the FACOMS reference population. Directional improvements in the other three clinical outcomes (FARS-ADL, 9-HPT, MFIS) were also observed in OL study participants, while worsening in these outcomes was observed in the FACOMS reference population.

Median (IQR) Clinical Outcome Measure Change from Baseline at 1 year							
mFARS [0- 93]		FARS-ADL [0- 36]		9-HPT: Dominant Hand		MFIS [0- 84]	
Nomlabofusp *n = 8*	FACOMS *n = 185*	Nomlabofusp *n = 8*	FACOMS *n = 237*	Nomlabofusp *n = 7*	FACOMS *n = 219*	Nomlabofusp *n = 8*	FACOMS[a] *n = 136*
-2.25 (-3.8, -0.3)	**1.00** (-1.5, 4.0)	**-0.50** (-2.0, 1.0)	**0.50** (-1.0, 2.5)	**-7.40** (-38.8, -2.5)	**3.4** (-4.5, 18.0)	**-6.5** (-17.5, 4.0)	**1.5** (-9.5, 11.0)

IQR = interquartile range

[a]MFIS presented here is at 2 years because it was not assessed at 1 year

Long-term Pharmacokinetic Profile Consistent with Prior Studies

- Rapid absorption after subcutaneous administration
- Exposure appeared to reach steady state in plasma by Day 30 at both the 25 mg and 50 mg doses with no further accumulation
- Pharmacokinetic profile consistent with Phase 1 and Phase 2 studies

Nomlabofusp Program Updates

- **Informative Adolescent PK Run-In Data:** Adolescents 12 to 17 years of age received a weight-based equivalent of 50 mg for 7 days. Exposure and PK in adolescents spanning 12 to 17 years of age (n = 14, 5 on placebo) were similar to adults on 50 mg of nomlabofusp. Six adolescents are currently enrolled in the OL study
- **Planned Enrollment in OL Study:** The OL study protocol was amended to include adolescent and adult patients who have not participated in a prior nomlabofusp study. Recently, Larimar modified the starting dose regimen and is implementing this change. The new dosing regimen will include 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. Larimar plans to enroll patients 2-11 years of age directly into the OL study in the future
- **Global Phase 3 Study:** Sites are identified and being qualified in the U.S., Europe, U.K., Canada, and Australia
- **Developments in 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 Q4 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

Key Upcoming Milestones

- Implement the new dosing regimen in the OL study in Q4 2025
- PPQ on commercial scale drug substance planned in Q4 2025
- BLA submission seeking accelerated approval targeted in Q2 2026

Conference Call and Webcast

Larimar will host a conference call and webcast today, September 29, 2025, at 8:00 a.m. EDT. To access the webcast, please visit this link to the event. To participate by phone, please dial 1-877-407-9716 (domestic) or 1-201-493-6779 (international) and refer to conference ID 13756144 or click on this link and request a return call. Following the live event, an archived webcast will be available on the "Events & Presentations" page of the Larimar website.

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 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 plan 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 and regulatory 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.

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