Larimar Therapeutics Reports Positive Open Label Data and Submission of First Module of Rolling BLA for Accelerated Approval of Nomlabofusp for Friedreich's Ataxia

June 29, 2026

- *FDA alignment on submission of BLA data package in multi-disciplinary Type B pre-BLA meeting minutes; first module of rolling BLA submitted with remaining modules expected 2H 2026*
- *Daily nomlabofusp increased and sustained skin FXN levels at 1 year and 18 months; 100% (9/9) of participants achieved and maintained levels over 50% of mean levels in healthy volunteers (comparable to asymptomatic heterozygous carriers) at 1-year*
- *Continued directional improvement across mFARS, FARS-ADL, 9-HPT, MFIS observed at 1 year of nomlabofusp treatment (n = 13) relative to a worsening in a FACOMS natural history study reference population*
- *One of six non-ambulatory participants at baseline became ambulatory after 1 year of dosing; none of the seven ambulatory participants progressed to non-ambulatory at 1 year*
- *Well-characterized safety profile; long-term dosing continues to be generally well-tolerated; anaphylaxis occurred in 10/43 patients with 9 of the 10 having exposure to nomlabofusp in a prior study*
- *11 participants had no exposure to nomlabofusp in a prior study; 1 experienced anaphylaxis*
- *Dosing of first patient in global confirmatory Phase 3 study expected Q3 2026*
- *Company management to host webcast and conference call today at 7:45 a.m. EDT*

BALA CYNWYD, Pa., June 29, 2026 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced it has submitted the first module of its rolling Biologics License Application (BLA) submission to the Food and Drug Administration (FDA) for accelerated approval of nomlabofusp; the remaining modules are expected to be submitted in the second half of 2026. The submission was made after obtaining FDA meeting minutes of a Type B multidisciplinary pre-BLA meeting. The Company also announced positive data from the ongoing long-term open label (OL) study evaluating daily subcutaneous injections of nomlabofusp in adolescent and adult patients with Friedreich's ataxia (FA). FA is a rare, progressive, and fatal neurological disease with no approved disease modifying therapies that address the root cause of the disease.

"Today's data represent a pivotal milestone for Larimar and, most importantly, for the FA community," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar. "In the Type B multi-disciplinary pre-BLA meeting minutes, FDA confirmed that our existing data package appears to be sufficient to support a BLA submission seeking accelerated approval based on skin frataxin as a potential novel surrogate endpoint and approval will be a matter of review. We are very excited to share some unique and important initial observations in our publicly available program update deck and on our conference call later this morning. These data together reinforce the strong clinical and biomarker evidence we have built to date for nomlabofusp. With compelling and consistent OL study data in hand, rolling BLA submission initiated, and dosing of the first patient in our global confirmatory Phase 3 study approaching, we are executing on all fronts to bring what could be the first disease-modifying therapy to pediatric and adult patients living with FA."

Dr. Rusty Clayton, Chief Medical Officer of Larimar added, "With longer-term treatment of more patients at the 50 mg dose, we continue to see improvements in multiple clinical outcome measures reinforcing the positive benefit-risk profile of nomlabofusp. Moreover, study participants who received nomlabofusp treatment for at least one year were able to improve FA disease progression. Further improvement was observed in participants who completed 18 months of dosing. Collectively, sustained elevations in skin FXN concentrations with concomitant directional improvements in key clinical outcomes relative to a FA natural reference population, along with a well-characterized safety profile, support the potential of nomlabofusp to meaningfully alter the course of this devastating disease."

The OL study is evaluating the safety and tolerability, pharmacokinetics (PK), and frataxin (FXN) levels in skin and buccal cells, along with exploratory pharmacodynamic (PD) markers (lipid profiles and gene expression) and clinical outcomes following long-term subcutaneous administration of nomlabofusp. The OL study protocol has now been amended to include children 2-11 years of age, adolescents and adults who have not participated in a prior nomlabofusp study.

As of June 2026, 43 adolescent and adult participants in the OL study had received at least one dose of nomlabofusp and 22 participants remain in the study with a maximum treatment duration of more than 800 days. Among the study participants, approximately 49% were non-ambulatory at baseline.

Consistent Long-term Safety Profile

- Long-term daily dosing was generally well tolerated with 13 adults on treatment for 1 year, 7 for 18 months, and 3 for 2 years
- >10,000 doses of nomlabofusp have been administered in the OL study

- Most common adverse events are local injection site reactions that were mild to moderate, decreased in frequency over time, and did not lead to any withdrawals from the study
- 21 participants discontinued
    - 10 of 41 participants in OL study experienced anaphylaxis
        - 9 had prior exposure to nomlabofusp, one had no prior exposure
        - All participants returned to their usual state of health after receiving standard treatment with no further sequelae
    - 3 experienced generalized urticaria (no new occurrences since initiating antihistamine therapy)
    - 8 withdrew (3 due to other adverse events)
- 11 participants with no prior exposure; one had anaphylaxis

Skin FXN Increased and Reached Steady State with Long-term Daily Nomlabofusp

*Absolute Mean Skin FXN Levels pg/µg (Range)					
Baseline	**1 month**	**3 months**	**6 months**	**1 year**	**18 months**
3.7 (1.5, 8.8), *n = 27*	**8.9** (2.9, 22.9), *n = 26*	**12.5** (5.6, 37.1), *n = 20*	**12.3** (5.6, 26.7), *n = 11*	**12.1** (8.1, 16.1), *n = 9*	**10.7** (9.9, 11.8), *n = 3*

Note: 8.2 pg/µg represents 50% of the average FXN concentration of healthy volunteers
* 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
Data are presented as of the March 2026 cutoff date

Majority of Participants Achieved Skin FXN Levels Similar to Levels Reported in Asymptomatic Heterozygous Carriers Following Nomlabofusp for 6 Months

% of Participants with Skin FXN Levels in the Range of Asymptomatic Heterozygous Carriers (> 8.2 pg/µg; ~50% of Mean Healthy Volunteer FXN Concentration)					
Baseline	**1 month**	**3 months**	**6 months**	**12 months**	**18 months**
4% 1/27	**38%** 10/26	**50%** 10/20	**82%** 9/11	**100%** 9/9	**100%** 3/3

*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
Data are presented as of the March 2026 cutoff date

Nomlabofusp OL Data Shows Potential for Clinical Benefit vs. FACOMS Reference Population

- Directional improvement across mFARS, FARS-ADL, and 9-HPT at 1 year of nomlabofusp treatment (n = 13) relative to a worsening in a Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS), natural history study reference population
- At 1 year: a mean 1.0-point improvement in mFARS with nomlabofusp treatment compared to a mean 1.6-point worsening in FACOMS reference group, resulting in a 2.6-point difference
- At 18 months: a mean 2.3-point improvement in mFARS with nomlabofusp treatment compared to a calculated mean 2.3-point worsening in FACOMS reference group, resulting in a calculated 4.6-point difference
- In participants entering the OL study, key clinical outcome measures had worsened when compared to baseline in prior studies over an average of 2.5 years. Improvement in these same participants occurred during the first year of participation in the OL study
- In the 13 participants completing 1 year of dosing with nomlabofusp, 6 participants were non-ambulatory and 7 were ambulatory at baseline. At the 1 year timepoint, none of the ambulatory patients had progressed to non-ambulatory status, and 1 patient who was non-ambulatory became ambulatory.
- Improvement in clinical outcomes was associated with increased skin FXN levels
- These clinical findings support that increase in tissue FXN levels after treatment with nomlabofusp may lead to clinical benefit across a broad spectrum of patients with FA, including those with advanced disease

FACOMS Reference Population is Based on Characteristics Similar to OL Study

- FACOMS, a longitudinal natural history study, includes patients with confirmed FA diagnosis
- Reference population is based on the range of baseline characteristics of participants in the OL study and FDA recommendations on the methodology to generate the reference population

Clinical Outcome Measure Change from Baseline, Mean (Range)							
	mFARS[3] [0- 93]		**FARS-ADL**[3] [0- 36]		**9-HPT: Dominant Hand**[3] [Seconds]		**MFIS**[3] [0- 84][2]
	Nomlabofsp	FACOMS[1]	Nomlabofusp	FACOMS[1]	Nomlabofusp	FACOMS[1]	Nomlabofusp
1 year	**-1.0** (-6.5, 3.0) *n=13*	**1.6** (-15.7, 18.0) *n=189*	**-1.1** (-9.0, 2.5) *n=13*	**1.5** (-5.5, 9.0) *n=211*	**-15.6** (-46.7, 15.4) *n=12*	**6.1** (-40.1, 203.7) *N=194*	**-5.2** (-25.0, 10.0) *n=13*
18 mos	**-2.3** (-10.0, 4.5) *n=7*	*2.3*[2]	**-0.3** (-1.5, 1.0) *n=7*	*NA*	**-11.8** (-13.6, 6.5) *n=7*	*NA*	**0.6** (-16.0, 15.0) *n=7*

Range = min, max

[1] 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

[2] Data collected annually in FACOMS; 18-month value was interpolated using a linear equation constructed with annual data up to 3 years

[3]Modified Friedreich Ataxia Rating Scale (mFARS), FARS-Activities of Daily Living (FARS-ADL), 9-hole peg test (9-HPT)
Data are presented as of the March 2026 cutoff date

Dr. Marshall Summar, Chief Executive Officer (CEO) of Uncommon Cures, past Founding Director of the Rare Disease Institute and Margaret O'Malley Chair of Genetic Medicine at Children's National Hospital added, "As the CEO of a key clinical site in the OL study and a career Medical Geneticist, I have seen firsthand the significant burden that FA places on patients and their families. The data generated to date suggest that nomlabofusp has the potential to meaningfully impact the underlying biology of the disease and translate into clinically relevant benefits. The clinical improvements observed so far are promising and mark a meaningful step toward what could become the first disease-modifying therapy for a patient population with significant unmet medical needs."

FDA Alignment for Submission of the BLA Package for Accelerated Approval in Multi-disciplinary Type B Pre-BLA Meeting Minutes

- **Sufficient Data Package:** FDA reviewed OL data submitted in a briefing package and confirmed that the existing data package appears capable of supporting submission and review of a BLA seeking accelerated approval; approval will be a matter of review.
- **FXN as Surrogate Endpoint:** FDA had reaffirmed willingness to consider FXN as a novel surrogate endpoint and confirmed that Larimar's exposure-response analysis linking nomlabofusp exposures to clinical outcomes is the type that could support the BLA submission.
- **Gene Expression and Lipid Data:** FDA noted that prospectively collected exploratory gene expression and lipid biomarkers may support the biomarker data and provide additional support to the novel surrogate endpoint and mechanism of action of nomlabofusp beyond tissue FXN concentrations.
- **Rolling BLA Submission:** FDA agreed to a rolling BLA submission, and the first module has been submitted.

Key Upcoming Milestones

- Dosing of first patient in global confirmatory Phase 3 study planned Q3 2026
- Submission of final modules for rolling BLA submission expected 2H 2026
- Targeting mid-2027 launch, if approved

Webcast Details
To access the webcast on Monday, June 29, 2026, at 7:45 am EDT, please visit this link to the event. 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 press 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, including the dosing of a first patient in a global confirmatory Phase 3 study, interactions and filings with the FDA, the safety and therapeutic potential of nomlabofusp , expectations regarding the timing of completion 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; 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 non-clinical or 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; Larimar's ability to submit BLA modules on the intended timeline; Larimar's ability to realize the benefits of Breakthrough Therapy Designation; 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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