Larimar Therapeutics Announces FDA Breakthrough Therapy Designation for Nomlabofusp in FA and Reiterates Planned BLA Submission in June 2026

February 24, 2026

- *Nomlabofusp program granted Breakthrough Therapy Designation for the treatment of adults and children with FA based on FDA's review of available clinical data from open label study*
- *FDA written communications after recent START meeting continue to align with use of skin FXN to support BLA submission seeking accelerated approval*
- *Topline open label study data to support BLA submission expected in Q2 2026*
- *Planned BLA submission seeking accelerated approval on track for June 2026; U.S. launch targeted for first-half 2027, if approved*

BALA CYNWYD, Pa., Feb. 24, 2026 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) to nomlabofusp, a frataxin (FXN) protein replacement therapy with disease modifying potential, for the treatment of adults and children with Friedreich's ataxia (FA). Additionally, after a recent Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program meeting with FDA, the Company announced continued alignment with the FDA to consider the use of skin FXN as a novel surrogate endpoint reasonably likely to predict clinical benefit to support a planned Biologics License Application (BLA) submission seeking accelerated approval. There was also agreement on the relevant clinical outcomes with consistent directional improvement in all four clinical outcomes assessed and the type of analyses required to support the exposure response relationships for the nomlabofusp program. The FDA stated that the adequacy of the safety database will be a matter of review at the time of BLA submission. The planned BLA submission is targeted for June 2026.

Breakthrough Therapy Designation and FDA feedback on BLA submission were based on the FDA's review of available clinical data from the Company's ongoing open label (OL) study evaluating nomlabofusp in adult and pediatric patients with FA.

Breakthrough Therapy Designation Granted by FDA for Nomlabofusp for the Treatment of FA

Breakthrough Therapy Designation is intended to expedite the development and regulatory review of a drug intended to treat a serious condition. A drug is eligible for BTD if preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available treatments in one or more clinically significant endpoints.

The Breakthrough Therapy Designation request for nomlabofusp included preliminary clinical data from the OL study demonstrating increases in skin FXN to levels expected in asymptomatic carriers and consistent directional improvement across four key clinical outcomes including modified Friedreich Ataxia Rating Scale (mFARS) score, FARS-Activities of Daily Living (ADL), 9 Hole Peg Test (9-HPT), and Modified Fatigue Impact Scale (MFIS) after 1-year on treatment. These findings reinforce the potential of nomlabofusp to improve FA's disease course relative to a worsening observed in a reference group from the Friedrich's Ataxia Clinical Outcomes Measure Study (FACOMS) natural history study.

"There continues to be a substantial burden of disease affecting the estimated 5,000 children and adults in the U.S. living with FA. Receiving Breakthrough Therapy Designation underscores the FDA's recognition of the high unmet medical needs and the potential for nomlabofusp to demonstrate a substantial improvement over available therapy on clinically significant endpoints," said Dr. Rusty Clayton, Chief Medical Officer of Larimar. "As part of the BTD request, the FDA reviewed preliminary nomlabofusp data demonstrating improvements in mFARS score, ADL, 9-HPT performance, as well as decreased fatigue, in the context of increased tissue FXN to levels similar to those observed in asymptomatic carriers with no signs of disease. We are encouraged by the increasing body of clinical data supporting the potential of nomlabofusp to modify disease progression by targeting the root cause of FA, FXN deficiency. We look forward to continued collaboration with the FDA as we proceed toward potential registration."

FDA Meeting Comments Support a Planned BLA Submission for Nomlabofusp in June 2026

Dr. Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar added, "We are pleased to have continued engagement with the FDA on our planned BLA submission for nomlabofusp and we appreciate FDA's thorough review of the preliminary clinical data. This regulatory progress supports our BLA readiness seeking accelerated approval and allows us to focus on continued execution. We are committed to ensuring a robust and comprehensive data package that captures the favorable benefit-risk profile of nomlabofusp and its potential to meaningfully improve outcomes for patients with FA. We continue to plan for a June 2026 BLA submission seeking accelerated approval and are excited to initiate our confirmatory Phase 3 study in the U.S., E.U., U.K., Canada and Australia. We are proud to have clinical trial applications related to our Phase 3 study currently under review in France and Canada, with submission to U.K. regulatory authorities soon to follow."

In connection with a recent START meeting, the FDA reviewed preliminary clinical data for the nomlabofusp program and continued to provide encouraging feedback on BLA content:

- **FXN as Surrogate Endpoint:** FDA reaffirmed willingness to consider use of FXN as novel surrogate endpoint and

confirmed Larimar's exposure-response analysis exploring the relationship between nomlabofusp exposures and clinical outcome measures is the type that could support the future BLA submission.

- **Reference Population:** FDA confirmed the process proposed for selecting a reference population based on matched subjects from the FACOMS database for the natural history comparisons of clinical endpoints to be used for BLA submission and offered to provide advance review and comment on the statistical plan.

- **Safety Dataset:** FDA stated that the adequacy of the safety dataset will be a matter of review at the time of BLA submission.

- **Global Phase 3 Study:** FDA is aligned with plans to have the global confirmatory Phase 3 study underway at the time of BLA submission and confirmed that change from baseline in the Upright Stability Score (USS) (a subscale of mFARS) is a reasonable and clinically relevant primary endpoint for the planned Phase 3 study.

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."

Expected Near-term Milestones

- Topline OL study data to support BLA submission expected in Q2 2026
- Plan to initiate screening in global confirmatory Phase 3 study in Q2 2026; dosing of first patient expected mid-2026
- BLA seeking accelerated approval planned to be submitted in June 2026
- U.S. launch targeted for first-half 2027, if approved

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", "target" 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; 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; 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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