Larimar Therapeutics Completes Rolling BLA Submission Seeking Accelerated Approval of Nomlabofusp for Friedreich's Ataxia

November 9, 2026

- *Completed BLA submission marks a major milestone in advancing nomlabofusp as the first potential disease-modifying therapy to address the underlying cause of FA*
- *Submission follows FDA alignment on key elements of the BLA package for submission, including willingness to consider skin frataxin as a potential novel surrogate endpoint*
- *Previously reported open label data showed sustained increases in skin frataxin with directional clinical improvements observed in multiple outcomes relative to a natural history reference population*
- *Patients currently enrolling in FORWARD-FA, the global confirmatory Phase 3 study for nomlabofusp*

BALA CYNWYD, Pa., Nov. 09, 2026 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced completion of its rolling Biologics License Application (BLA) submission to the U.S. Food and Drug Administration (FDA) seeking accelerated approval of nomlabofusp for the treatment of patients with Friedreich's ataxia (FA). Nomlabofusp is an investigational protein replacement therapy designed to address the underlying cause of FA, frataxin deficiency, by delivering human frataxin to mitochondria.

Completion of the submission follows previously disclosed FDA feedback on key elements of the BLA and represents a significant milestone for Larimar. Larimar is currently enrolling patients in FORWARD-FA, its global confirmatory Phase 3 study, a double-blind, placebo-controlled study that will evaluate daily subcutaneous use of nomlabofusp over 18 months in ambulatory participants ages 2 to 40 who are new to the drug. Approximately two thirds of the participants will be under the age of 21.

"Completing our BLA submission is a major milestone for Larimar and the FA community, bringing years of development work together in an application seeking accelerated approval of nomlabofusp," said Carole Ben-Maimon, MD, Chief Executive Officer of Larimar. "Our confidence in the program is grounded in the sustained increases in skin frataxin and consistent directional improvements observed in multiple clinical outcomes in patients. It is important to note that patients with FA are expected to experience continued neurologic decline, not improvement. After treatment with nomlabofusp, we have not observed worsening in most participants but rather signs of improvement. Our previously disclosed discussions with FDA have helped inform the content of the submission. As we support the potential FDA review and advance FORWARD-FA, our confirmatory Phase 3 study, our team is preparing for a potential U.S. launch with the goal of making nomlabofusp available to patients as quickly as possible following approval, if granted. In parallel, we continue to evaluate options to optimize our global commercialization strategy."

The BLA seeks accelerated approval based on increases in skin frataxin (FXN) concentrations as a potential novel surrogate endpoint and is supported by data from the ongoing long-term open label (OL) study. Previously reported findings showed sustained increases in skin FXN, with all evaluable participants at one year achieving skin FXN levels within the range expected in asymptomatic heterozygous carriers. Directional improvements were observed at one year across multiple clinical outcomes relative to worsening in a natural history reference population. Directional improvement of exploratory pharmacodynamic markers (plasma lipids and peripheral tissue gene transcription) compared to placebo treated patients were also observed. Nomlabofusp has the potential to be the first disease-modifying therapy for patients with FA.

"Larimar's completion of its BLA submission for nomlabofusp marks an important and exciting milestone for the Friedreich's ataxia community. As the first potential frataxin replacement therapy, nomlabofusp is designed to address the underlying cause of FA. The fact that the submission includes data from adolescents ages 12 to 17 is especially meaningful, as there are currently no approved therapies for children under 16 years of age. FA is a complex and variable disease, and patients and families need multiple treatment options that can address their individual needs across different ages and stages of disease," said Jen Farmer, M.S., Chief Executive Officer, Friedreich's Ataxia Research Alliance (FARA).

Submission Highlights:

- Data Package Supporting Submission and Review: FDA reviewed OL study data submitted in a briefing package and confirmed in minutes from the multidisciplinary Type B pre-BLA meeting that the existing data package appears capable of supporting submission and review of a BLA seeking accelerated approval of nomlabofusp as a treatment for patients with FA. Approval will be a matter of review.
- FXN as a Potential Surrogate Endpoint: In those previously disclosed interactions, FDA reaffirmed its willingness to consider FXN as a novel surrogate endpoint and indicated that Larimar's exposure-response analysis linking nomlabofusp exposure to clinical outcomes is the type of analysis that could support the BLA submission.

- Additional Biological Evidence: FDA previously noted that prospectively collected exploratory gene expression and lipid pharmacodynamic markers data may help further characterize the biological activity of nomlabofusp beyond tissue FXN concentrations.

Potential Upcoming Milestones

- **BLA Submission Acceptance:** Early 2027
- **Open Label Study Safety Update:** Q1 2027
- **U.S. Approval:** Mid-2027
- **Obtaining & Monetizing the Rare Pediatric Disease Priority Review Voucher:** Mid-2027, if approved
- **U.S. Launch:** Targeting Q3 2027 launch, if approved.

Nomlabofusp has received FDA Breakthrough Therapy Designation for the treatment of FA in adults and children as well as Fast Track, Rare Pediatric Disease, and Orphan Drug designations. Nomlabofusp was also selected for FDA's Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program, which has enabled more frequent interactions with FDA to support our development and regulatory strategy.

About FORWARD-FA
FORWARD-FA, Larimar's global confirmatory Phase 3 study, is designed to assess safety and clinical outcomes and to verify the clinical benefit of nomlabofusp as part of the Company's accelerated approval strategy. The double-blind, placebo-controlled study will evaluate daily subcutaneous use of nomlabofusp over 18 months in ambulatory participants ages 2 to 40 who are new to the drug. Approximately two thirds of the participants will be under the age of 21. The focus on younger participants reflects the tendency for more rapid disease progression in this population and underscores the need for evaluating potential treatments for early intervention.

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; the potential of nomlabofusp to address the underlying cause of FA and provide clinical benefit; the interpretation of clinical, biomarker and safety data and their ability to support the BLA; expectations regarding the FDA's acceptance for filing, review and potential approval of the BLA, including consideration of skin FXN as a potential novel surrogate endpoint; Larimar's planned research and development efforts, including the design, timing and conduct of FORWARD-FA; interactions with FDA; expectations regarding the potential for, accelerated approval or accelerated access and the impact of such accelerated access on the FDA's review timeline Larimar's preparations for and timing of a potential launch, if approved; the potential award and future sale of a Rare Pediatric Priority Review Voucher; and Larimar's overall development plans, 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 or determine that skin FXN is not an acceptable surrogate endpoint for accelerated approval; that completion of the BLA submission does not assure acceptance for filing or accelerated approval and that FDA may require additional information or studies; 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 successfully 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 jallaire@lifesciadvisors.com (212) 915-2569	Chief Financial Officer mcelano@larimartx.com (484) 414-2715

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