Larimar Therapeutics Announces FDA Recommendations on Safety Database, and Other Details of Nomlabofusp BLA Submission for Friedreich's Ataxia Program

June 23, 2025

- *Interactions with FDA over the past year have provided clear expectations for the path to submission of the nomlabofusp BLA*
- *Written FDA recommendations for safety database include a total of at least 30 participants with continuous exposure for 6 months including a subset of at least 10 with 1-year; large majority of the exposure should be on the 50 mg dose*
- *BLA submission seeking accelerated approval planned in the second quarter of 2026 to allow for inclusion of the recommended safety data for adults and children*
- *OLE data expected in September 2025 from 30-40 participants who received at least one dose of nomlabofusp; data will include participants on the 50 mg dose*
- *Adolescent PK run-in data expected in September 2025 from 14 participants (some on placebo); participants currently screening and enrolling into OLE*
- *Global Phase 3 study activities are ongoing with identification and qualification of sites in U.S., Europe, U.K., Canada, and Australia*
- *Company management to host webcast and conference call today at 8:00 a.m. ET*

BALA CYNWYD, Pa., June 23, 2025 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced FDA safety database recommendations and refined timeline for Biologics License Application (BLA) submission to allow for the inclusion of the recommended safety data from adults and children with Friedreich's Ataxia (FA). This comes following written responses from the U.S. Food and Drug Administration (FDA) based on discussions under the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program.

"We are thrilled to have clarity from FDA on the safety database recommendations following submission of safety information included in a briefing package from our nomlabofusp program. Importantly, we now have written recommendations from FDA on critical elements of the BLA submission including the safety database as well as the use of skin frataxin (FXN) concentrations as a reasonably likely surrogate endpoint (RLSE). Enrollment in our open label extension (OLE) study continues to progress and we have recently expanded enrollment to include adolescents and patients who have not participated in prior clinical studies and therefore have not been exposed to nomlabofusp previously. Based on the FDA's safety database recommendations and our plan to request approval to treat a broad population of patients including adults and children, we now plan to submit our BLA seeking accelerated approval in the second quarter of 2026," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Our participation in the START program has been incredibly valuable and continues to help us expedite clinical and regulatory development for the nomlabofusp program. We are on track to report data in September 2025 including data on the 50 mg dose from our OLE study, as well as adolescent pharmacokinetic (PK) run-in data. Nomlabofusp has the potential to be the first disease modifying therapy for FA and we look forward to expanding the clinical program to patients around the world with the initiation of our global Phase 3 study."

Dr. Rusty Clayton, Chief Medical Officer of Larimar added, "Our long-term OLE study is further advancing, with some participants now on treatment for up to 15 months. This includes exposure at both the 25 mg and 50 mg doses. The high adherence rates we are seeing for daily subcutaneous injections in participants over the long term is very encouraging. We have begun transitioning adolescents from the PK run-in study and have amended the protocol to include patients who have never participated in any of our prior clinical trials. Overall, we are pleased with our progress and the recommendations we now have from FDA on the safety database to achieve our near-term registrational goals."

Clear FDA Expectations for Path to Submission of Nomlabofusp BLA Seeking Accelerated Approval

- **Safety Database:** FDA recommended to evaluate safety in at least 30 participants with continuous study drug exposure for 6-months and a subset of at least 10 of those participants with continuous study drug exposure for 1-year; the large majority of safety data should be from participants receiving the 50 mg dose.

- **Use of Skin FXN Concentrations as a Surrogate Endpoint:** FDA is open to the use of skin FXN concentrations as a RLSE and acknowledged the submitted data appear to support a relationship between increased skin FXN and relevant tissues such as the heart, dorsal root ganglion, and skeletal muscle. Acceptability of increases in skin FXN for accelerated approval will be decided during future BLA review.

- **Clinical Data Package:** Includes clinical data from the successfully completed and ongoing clinical trials.
    - **Phase 1 and 2 Studies:** Completed single ascending-dose (SAD) and multiple ascending dose (MAD) Phase 1

studies, and the Phase 2 dose exploration study
    - **FA Adolescent PK Run-In Study:** PK data from 14 adolescents 12-17 years old dosed once daily for 7 days with a weight-based dose equivalent to the 50 mg adult dose of nomlabofusp or placebo. Dosing has been completed, and data are expected in September 2025. Participants are now screening and enrolling for the OLE study.
    - **Ongoing OLE Study:** Evaluating safety and tolerability, PK, and FXN levels in buccal and skin cells, along with exploratory pharmacodynamic markers (lipid profiles and gene expression data) and clinical outcomes following long-term once daily subcutaneous administration of nomlabofusp. Enrollment is ongoing and all active participants are currently receiving the 50 mg dose. In addition, screening and enrollment of adolescents is ongoing. Expansion of the study is planned to include patients who have never participated in any of our prior clinical trials and have never been exposed to nomlabofusp.
- **Global Phase 3 Study:** Activities are ongoing with the identification and qualification of sites in U.S., Europe, U.K., Canada, and Australia. The Phase 3 study is expected to be underway at the time of BLA submission and is currently intended as the confirmatory study to verify clinical benefit as required by FDA's accelerated approval pathway.
- **Pharmacology and Toxicology:** Nonclinical data supporting the use of FXN as a novel surrogate endpoint, complete toxicology package including juvenile toxicology study and data supporting improvements in patient lipid profiles and gene expression
- **Chemistry Manufacturing and Controls (CMC):** Required data supporting the lyophilized drug product, which is stable at room temperature, data on batches manufactured at a commercial scale and analytical methods and proposed specifications

Near-term Milestones

- OLE data expected in September 2025 from 30-40 participants who received at least one dose of nomlabofusp, including participants on the 50 mg dose. Adolescent PK run-in data are also expected in September 2025 from 14 participants (some on placebo)
- Data from the nonclinical package to be published in a peer reviewed journal this summer
- BLA seeking accelerated approval planned to be submitted in the second quarter 2026

Conference Call and Webcast

Larimar will host a conference call and webcast today, June 23, 2025, at 8:00 a.m. ET. 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 13754491 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; 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:
Joyce Allaire
LifeSci Advisors
jallaire@lifesciadvisors.com
(212) 915-2569

Company Contact:
Michael Celano
Chief Financial Officer
mcelano@larimartx.com
(484) 414-2715

Source: Larimar Therapeutics