Larimar Therapeutics Announces FDA has Removed Partial Clinical Hold for Nomlabofusp Program in Friedreich's Ataxia

May 20, 2024

- *Food and Drug Administration (FDA) removed partial clinical hold following review of Phase 2 dose exploration study data*
- *Ongoing open label extension (OLE) study initially evaluating 25 mg; Larimar plans to dose escalate to 50 mg following further characterization of frataxin pharmacodynamics (PD) at the 25 mg dose*
- *Interim data from OLE study remains on track for Q4 2024*
- *Biologics License Application (BLA) submission targeted for 2H 2025*

BALA CYNWYD, Pa., May 20, 2024 (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. FDA has removed the partial clinical hold previously placed on the company's nomlabofusp (CTI-1601) clinical program. Nomlabofusp is currently in development for the treatment of patients with Friedreich's Ataxia (FA). Nomlabofusp is a novel protein replacement therapy designed to address the root cause of FA by delivering frataxin to mitochondria. The FDA removed the partial clinical hold after a review of data from the Company's recently completed four-week, placebo-controlled Phase 2 dose exploration study. The review included data from both the 25 mg and 50 mg cohorts in patients who received daily dosing of nomlabofusp for 14 days followed by every other day dosing until day 28.

"We are very excited the FDA has removed the partial clinical hold on our nomlabofusp program following review of our Phase 2 data. Helping patients with FA is our top priority and we appreciate the attention and thorough review by the FDA of all submitted data," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Importantly, we are now cleared to dose escalate to the 50 mg dose in our ongoing OLE study which we plan to do following further characterization of frataxin PD at the 25 mg dose. The OLE study is evaluating the long-term safety as well as frataxin levels following daily administration of nomlabofusp and we look forward to interim data in the fourth quarter of the year."

In the Phase 2 dose exploration study, nomlabofusp was generally well-tolerated throughout the four-week treatment period. Nomlabofusp had a predictable pharmacokinetic profile and demonstrated dose-dependent increases in frataxin levels in skin and buccal cells. All patients with quantifiable levels at baseline and Day 14 in the 50 mg cohort achieved frataxin levels in skin cells over 33% of the average level observed in healthy volunteers at Day 14, and 3 patients achieved levels greater than 50% of the average healthy volunteer level.

The long-term safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues following nomlabofusp are currently being evaluated in the ongoing OLE study in patients with FA. The OLE study will initially evaluate daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. Larimar plans to dose escalate to 50 mg in the OLE study following additional characterization of frataxin PD at the 25 mg dose. Further dose escalation above 50 mg, if necessary, would require submission of additional data for FDA review to support the increased dose. Interim data from the OLE study is expected in the fourth quarter of 2024.

About Nomlabofusp (CTI-1601)
Nomlabofusp is a recombinant fusion protein intended to deliver human frataxin to the mitochondria of patients with Friedreich's ataxia who are unable to produce enough of this essential protein. Nomlabofusp has been granted Rare Pediatric Disease designation, Fast Track designation and Orphan Drug designation by the U.S. Food and Drug Administration (FDA), Orphan Drug Designation by the European Commission, and a PRIME designation by the European Medicines Agency.

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 (CTI-1601), 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 (also known as CTI-1601) and other planned product candidates, Larimar's planned research and development efforts, including the timing of its nomlabofusp clinical trials, interactions with the FDA 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 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 nomabofusp 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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Source: Larimar Therapeutics