Larimar Therapeutics Receives FDA Clearance to Proceed to 50 mg Cohort in CTI-1601's Phase 2 Friedreich's Ataxia Trial and to Initiate Open Label Extension Trial

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- *Top-line safety, pharmacokinetic, and pharmacodynamic (frataxin level) data from Phase 2 trial's 50 mg cohort expected in 1H 2024*
- *Initiation of open label extension trial with 25 mg daily dosing expected in Q1 2024; interim data expected in Q4 2024*
- *Company management hosting a webcast and conference call today at 8:00 a.m. ET*

BALA CYNWYD, Pa., July 25, 2023 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Company's four-week, placebo-controlled, Phase 2 dose exploration trial of CTI-1601 in patients with Friedreich's ataxia (FA) to proceed to a 50 mg cohort in which participants will be dosed daily for the first 14 days, and then every other day until day 28. In addition, Larimar's open label extension (OLE) trial was also cleared for initiation by the FDA. Participants in the OLE will receive 25 mg of CTI-1601 daily. CTI-1601 is a novel protein replacement therapy designed to deliver frataxin to the mitochondria of patients with FA who have low levels of frataxin.

Larimar received clearance to advance its Phase 2 trial to a 50 mg cohort and initiate its OLE trial following a review by the FDA of Larimar's complete response to its partial clinical hold that included unblinded safety, pharmacokinetic (PK), and pharmacodynamic (PD) data from the Phase 2 trial's completed 25 mg cohort. Data from the completed 25 mg cohort (n = 13) indicated that CTI-1601 was generally well tolerated and showed increases in frataxin (FXN) levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14 (the final day of daily dosing in the trial). Further dose escalation in the Phase 2 and OLE trials and the initiation of additional U.S. clinical trials evaluating CTI-1601 are contingent on FDA review of results from the Phase 2 trial's 50 mg cohort in accordance with a partial clinical hold.

"Gaining clearance to advance to a 50 mg cohort in our Phase 2 trial and initiate the OLE trial are crucial steps in CTI-1601's development as potentially the first therapy to increase frataxin levels in patients with FA," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Given the inability of current treatments to address the frataxin deficiency underlying Friedreich's ataxia, we believe CTI-1601 has the potential to improve the treatment paradigm for this devastating disease. We now look forward to data from our Phase 2 trial's 50 mg cohort in the first half of 2024, which will help us further characterize the safety and PK profiles of CTI-1601 and its ability to increase frataxin levels in a dose-dependent fashion as seen in our earlier Phase 1 studies."

Participants who complete treatment in the Phase 2 dose exploration trial, or who previously completed a prior clinical trial of CTI-1601, are eligible to screen for Larimar's OLE trial. Participants in the OLE trial will receive subcutaneous injections of 25 mg of CTI-1601 administered daily. The trial is expected to begin in Q1 2024 with interim data expected in Q4 2024.

Dr. Ben-Maimon continued, "We are pleased to have clearance to begin our OLE trial and look forward to what we expect will be important interim data from the study in Q4 2024. Alongside our efforts to advance both our OLE and Phase 2 trials in the United States, we have also begun to engage with regulators and investigators outside the U.S. as we prepare to expand our clinical program to additional geographies. With approximately 75% of individuals with FA living outside the U.S., establishing global clinical trial capabilities is important for addressing the pressing unmet needs of the FA community."

CTI-1601 has been granted Orphan Drug (U.S. and Europe), Rare Pediatric Disease (U.S.), Fast Track (U.S.), and PRIME (Europe) designations for FA.

Webcast and Conference Call
Larimar will host a conference call and webcast today, July 25, 2023 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 13740205 or click on this link and request a return call. Following the live event, the archived webcast will be available on the "Events & Presentations" page of the Larimar website. Following the live event, the archived webcast will be available for 90 days.

About the Phase 2 Trial
The Phase 2 trial is a placebo-controlled, four-week, dose exploration trial designed to further characterize CTI-1601's safety, pharmacokinetic, and pharmacodynamic profiles. Eligible participants include ambulatory and non-ambulatory individuals with FA who are at least 18 years old. Participants enrolled in each cohort of the trial are randomized 2:1 to receive CTI-1601 or placebo daily via subcutaneous injections for the first 14 days, and then every other day until day 28. The trial currently includes two cohorts. Cohort 1 included 13 participants (9 on active treatment and 4 on placebo) and evaluated a 25 mg dose of CTI-1601. Cohort 2, which is initiating, will include 12-15 participants randomized 2:1 to CTI-1601 or placebo and will evaluate a 50 mg dose of CTI-1601. Key endpoints include safety assessments, pharmacokinetic assessments, as well as measures of frataxin levels and other pharmacodynamic markers (e.g., lipid profiles and gene expression data) in peripheral tissues. For more information on the trial, please visit clinicaltrials.gov under the identifier: NCT05579691.

About the Open Label Extension Trial
The open label extension trial is a multi-center study designed to enroll patients with FA who have previously completed a clinical trial of CTI-1601.

Participants in the trial will receive daily subcutaneous injections of 25 mg of CTI-1601 with injections being self-administered or administered by a caregiver. The safety and pharmacodynamic objectives of the open-label extension trial are to evaluate the safety, tolerability, and pharmacokinetics of long-term subcutaneous administration of CTI-1601 as well as measures of frataxin levels and other pharmacodynamic markers (e.g., lipid profiles and gene expression data) in peripheral tissues. Other objectives include evaluation of the effects of long-term subcutaneous administration of CTI-1601 on measures of clinical function. Data collected during the trial will be compared to a matched set of untreated patients derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS) database.

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, 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 Larimar's ability to develop and commercialize CTI-1601 and other planned product candidates, Larimar's planned research and development efforts, including the timing of its CTI-1601 clinical trials 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 CTI-1601 clinical milestones and continued interactions with the FDA regarding the partial clinical hold; that preliminary clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of CTI-1601 may not be predictive of the results or success of later clinical trials, and assessments; 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 CTI-1601's manufacturing process; Larimar's ability to obtain regulatory approvals for CTI-1601 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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