Larimar Therapeutics Reports FDA Clinical Hold on CTI-1601 and Termination of Recently Announced Private Placement Financing

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BALA CYNWYD, Pa., May 25, 2021 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (“Larimar”) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for Friedreich’s ataxia (FA) and other complex rare diseases, today announced that the United States Food and Drug Administration (FDA) has placed a clinical hold on the CTI-1601 clinical program and that the company will not be closing a previously announced private placement financing. CTI-1601 is a recombinant fusion protein intended to deliver human frataxin into the mitochondria of patients with FA who are unable to produce enough of this essential protein.

The clinical hold follows the previous notification by Larimar to the FDA of mortalities which occurred at the highest dose levels in an ongoing 180-day non-human primate (NHP) toxicology study, which is designed to support extended dosing of patients with CTI-1601. In the clinical hold letter, the FDA stated it needs a full study report from the ongoing NHP study and Larimar may not initiate additional clinical trials until the company has submitted the report and received notification from the agency that additional clinical trials may commence.

“While the notification of a formal clinical hold is disappointing, it does not change our previously stated clinical development strategy for CTI-1601,” said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar. “Patient safety is our top priority, and we will continue with our plan to complete the NHP toxicology study, assess the data, and discuss that data with FDA to obtain their consent prior to initiating our Jive and pediatric MAD trials. Based on all of the information we have regarding CTI-1601’s safety profile to date, we continue to believe there is a path forward toward the initiation of our Jive and pediatric MAD trials. However, due to the additional regulatory requirements that come with responding to a formal clinical hold, we believe there is a possibility that the initiation of these trials may be delayed into 2022. Regarding the termination of the previously announced private placement financing, as of March 31, 2021, we have $81.4 million in cash and investments, which provides cash runway through the first half of 2022.”

The safety of CTI-1601 was previously evaluated in Phase 1 single- and multiple-ascending dose clinical trials. Recently announced data from these trials indicate that repeated subcutaneous injections of CTI-1601 were generally well tolerated at doses up to 100 mg administered daily for 13 days. No serious adverse events, important medical events, or treatment-related severe adverse events were reported in the trial and the number and severity of adverse events did not increase with increasing exposure to CTI-1601. The most common adverse events were mild and moderate injection site reactions. Data from the MAD trial also showed that daily subcutaneous injections of CTI-1601 at doses of 50 mg or 100 mg resulted in frataxin levels in peripheral tissues (buccal cells) that were at or in excess of those that would be expected in phenotypically normal heterozygous carriers.

About CTI-1601
CTI-1601 is a recombinant fusion protein intended to deliver human frataxin into the mitochondria of patients with Friedreich’s ataxia who are unable to produce enough of this essential protein. Currently in Phase 1 clinical trials in the U.S., CTI-1601 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 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, CTI-1601, is currently being evaluated in a Phase 1 clinical program in the U.S. as a potential treatment for FA. 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 expectations regarding its ability to resolve the clinical hold imposed by the FDA related to CTI-1601, Larimar’s ability to develop and commercialize CTI-1601 and other planned product candidates, Larimar’s planned research and development efforts, and other matters regarding Larimar’s business strategies, 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, Larimar’s ability to successfully engage with the FDA and satisfactorily respond to requests from the FDA for further information and data regarding CTI-1601, the timing and outcome of Larimar’s planned interactions with the FDA concerning such the clinical hold on CTI-1601, the success, cost and timing of Larimar’s product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones; that 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 clinical trials, and assessments; the ongoing impact of the COVID-19 pandemic

on Larimar's future clinical trials, manufacturing, regulatory and nonclinical study timelines, ability to raise additional capital and general economic conditions; Larimar's ability to optimize and scale CTI-1601's manufacturing process; Larimar's ability to obtain regulatory approval 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 views 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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