Larimar Therapeutics Announces Dosing of Patients in Third Cohort of Phase 1 SAD Trial of CTI-1601 for Treatment of Friedreich's Ataxia

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BALA CYNWYD, Pa., July 20, 2020 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Nasdaq:LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced that patients have been dosed in the third cohort of a Phase 1 clinical trial to evaluate the safety and tolerability of single ascending doses (SAD) of CTI-1601 for the treatment of Friedreich's ataxia (FA). The trial was previously delayed due to the impact of the COVID-19 pandemic. 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 double-blind, placebo-controlled trial is evaluating the safety, tolerability and pharmacokinetics of single ascending doses of subcutaneously administered CTI-1601 in patients over age 18 with FA. To date, two cohorts of patients have completed the Phase 1 clinical trial. Topline results are planned for the first half of 2021.

"We're pleased that our Phase 1 clinical trial has resumed and we can continue to move forward with our lead product candidate, CTI-1601, which has the potential to become the first frataxin replacement therapy for patients with FA," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar Therapeutics. "Our highest priority remains the health of our employees and patients especially given the COVID-19 pandemic. We have thoughtfully re-engaged with our clinical site to mitigate the safety risks."

Additional information on the trial can be found on www.clinicaltrials.gov using the identifier NCT04176991.

About CTI-1601
CTI-1601 is a recombinant fusion protein intended to deliver human frataxin into the mitochondria of patients with Friedreich's ataxia (FA) who are unable to produce enough of this essential protein. Currently in a Phase 1 clinical trial, 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). To date, two cohorts of patients have completed the single ascending dose (SAD) Phase 1 clinical trial. Topline results from the Phase 1 clinical program are planned for the first half of 2021.

About Friedreich's ataxia
Friedreich's ataxia (FA) is a rare, progressive, multi-symptom genetic disease that typically presents in mid-childhood and affects the functioning of multiple organs and systems. The most common inherited ataxia, FA is a debilitating neurodegenerative disease resulting in multiple symptoms including progressive neurologic and cardiac dysfunction – poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing and speech. FA affects an estimated 4,000-5,000 individuals living in the United States and between 18,000 and 20,000 patients in the European Union. FA results from a deficiency of the mitochondrial protein, frataxin (FXN), which is found in cells throughout the body. To date, there are no medical treatment options approved for patients with FA.

About Larimar Therapeutics
Larimar Therapeutics, Inc. (Nasdaq:LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. The company's lead compound, CTI-1601, is currently being evaluated in a Phase 1 clinical program as a potential treatment for Friedreich's ataxia, a rare and progressive genetic disease. 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 the 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, the success, cost and timing of Larimar's product development activities, studies and clinical trials; the ongoing impact of the COVID-19 pandemic on Larimar's clinical trial 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 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 Zafgen 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 Securities and Exchange Commission 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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