# Larimar Therapeutics Announces Positive Opinion on Orphan Drug Designation Received from the European Medicines Agency for CTI-1601 for the Treatment of Friedreich's Ataxia

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BALA CYNWYD, Pa., July 28, 2020 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (Nasdaq:LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on the company's application for orphan drug designation for CTI-1601, a potential treatment for Friedreich's ataxia (FA), a rare, progressive, multi-symptom genetic disease that affects the functioning of multiple organs and systems. 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 U.S. Food and Drug Administration (FDA) previously granted Orphan Drug, Fast Track and Rare Pediatric Disease designations to CTI-1601 for the treatment of FA. Larimar expects that the European Commission, based on this positive opinion of the COMP, will formally grant the orphan drug designation for the European Union (EU) this year.

"The positive opinion for orphan drug designation from the EMA COMP is an important milestone to bring a much-needed potential therapy to patients with FA, a devastating disease that currently has no approved medical treatments," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar Therapeutics. "We look forward to working closely with EMA and continuing our U.S. Phase 1 trial of CTI-1601, which has the potential to become the first frataxin replacement therapy for patients with FA. We remain on track to report topline data in the first half of 2021."

Orphan drug designation in the EU is granted by the European Commission based on a positive opinion issued by the EMA COMP. To qualify, an investigational medicine must be intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient non-clinical or clinical data to suggest the investigational medicine may produce clinically relevant outcomes. EMA orphan drug designation provides companies with certain benefits and incentives, including clinical protocol assistance, differentiated evaluation procedures for Health Technology Assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees and 10 years of market exclusivity.

**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 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). 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 approximately 25,000 in the European Economic Area and United Kingdom. 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 in the U.S. 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 receipt of Orphan Drug Designation for the EU for CTI-1601 in FA from the European Commission, the expected timing for receiving Orphan Drug Designation from the European Commission, the expected incentives associated with receiving Orphan Drug Designation for 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, the that European Commission may not grant Orphan Drug Designation for the EU for CTI-1601

in FA or may do so in a longer than anticipated timeframe, 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 the Company 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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