Larimar Therapeutics Selected by FDA to Participate in START Pilot Program for Nomlabofusp in Friedreich's Ataxia

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- *START is a new milestone-driven program designed to accelerate development of novel therapies intended to address an unmet medical need for rare diseases*
- *Nomlabofusp was selected based on potential for clinical benefit in a rare neurodegenerative disease and demonstrated development program readiness*
- *START pilot program is intended to improve development efficiency through enhanced communication with the FDA*
- *Nomlabofusp is expected to be one of three CDER programs and one of six total programs selected by the FDA*
- *BLA submission continues to be targeted for 2H 2025*

BALA CYNWYD, Pa., May 30, 2024 (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 United States Food and Drug Administration (FDA) has selected nomlabofusp to participate in the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program. Nomlabofusp is a novel protein replacement therapy designed to address the root cause of Friedreich's ataxia (FA) by delivering frataxin to mitochondria. START selection was based on demonstrated development program readiness, including the potential of nomlabofusp to address the serious and unmet medical needs in a rare neurodegenerative condition, alignment of chemistry, manufacturing, and controls (CMC) development timelines with clinical development plans, and a proposed communications plan where enhanced communication could accelerate pivotal study initiation and path to potential Biologics License Application (BLA) submission.

The START pilot program was launched by the FDA in September 2023 to further accelerate the pace of development, with an initial selection of up to six novel drugs, three by the Center for Drug Evaluation and Research (CDER) and three by the Center for Biologics Evaluation and Research (CBER), intended to treat a rare disease or other serious condition with high unmet medical need through an enhanced mechanism for communication with the FDA. Sponsors selected can benefit from more frequent and rapid ad-hoc interactions with the FDA to help facilitate the development of programs to the pivotal clinical study or pre-BLA meeting stage, and to generate high-quality and reliable data intended to support a BLA or New Drug Application (NDA).

"We are thrilled nomlabofusp, the first potential therapy to increase frataxin levels in patients with FA, has been selected by the FDA to participate in the START pilot program. This selection highlights the commitment of the FDA to augment currently available formal meeting communications with more rapid, ad-hoc communication mechanisms with the goal of accelerating the pace of development of nomlabofusp," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "We look forward to participating in this pilot program and working with the FDA to further the development of nomlabofusp which has the potential to address the root cause of Friedreich's Ataxia by increasing frataxin levels."

Dr. Russell Clayton, Chief Medical Officer of Larimar added, "Participating in the START program enables increased communication with the FDA to help expedite the progression of our nomlabofusp development program to the pre-BLA meeting stage. This is important to patients who are continuing to live with this devastating disease."

Nomlabofusp is currently being evaluated in an ongoing open label extension (OLE) study to assess the long-term safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues in patients with FA. 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, expectations with respect to the START program, 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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