Larimar Therapeutics Provides Updates on CTI-1601 Clinical Program Following a Type C Meeting with the U.S. Food and Drug Administration and Reports Second Quarter 2022 Operating and Financial Results

August 11, 2022

- *Larimar plans to submit a complete response to CTI-1601's clinical hold in the third quarter of 2022*
- *In conjunction with the complete response, Larimar is proposing a Phase 2, four-week dose exploration study in Friedreich's ataxia (FA) patients as CTI-1601's next clinical trial*
- *Cash and marketable debt securities at June 30, 2022 of $54.9 million provides projected cash runway through the third quarter of 2023*

BALA CYNWYD, Pa., Aug. 11, 2022 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced that it has received meeting minutes from the U.S. Food and Drug Administration (FDA) following a recent Type C Meeting with the Agency and reported its second quarter 2022 operating and financial results.

The purpose of the Type C Meeting was to obtain FDA feedback on the information needed to resolve CTI-1601's current clinical hold in full or in part, as well as to discuss a proposed change in CTI-1601's clinical development plan to introduce a Phase 2 dose exploration study to precede initiation of an open label extension study.

The Company plans to submit a complete response to the CTI-1601 clinical hold in the third quarter of 2022. In conjunction with the complete response, Larimar is proposing as CTI-1601's next clinical trial a Phase 2, four-week dose exploration study in FA patients starting at the lower dose levels tested in the Company's Phase 1 multiple-ascending dose clinical trial. This study will provide data on extended dosing of lower doses of CTI-1601, including additional data on safety and tolerability as well as whether lower doses for longer periods of time can increase frataxin levels while maintaining acceptable exposures.

Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar, commented, "We would like to thank the FDA for a productive Type C Meeting, which informed the preparation and planned submission of a complete response that we believe will enable CTI-1601's return to the clinic. We look forward to receiving FDA feedback on the complete response and our proposed Phase 2 dose exploration study. The proposed study has been designed to provide additional information on CTI-1601's safety profile as well as pharmacokinetic and pharmacodynamic profiles, which we believe will allow us to select appropriate doses for future long-term dosing. It will also enable us to build on our prior Phase 1 results, which provided clinical proof-of-concept for CTI-1601 by demonstrating its ability to increase frataxin levels in peripheral tissues. Given FA's root cause is the inability of patients to produce sufficient amounts of frataxin, we believe these data highlight CTI-1601's potential to address the urgent need for disease-modifying treatments that go beyond just symptom management."

The CTI-1601 program was placed on clinical hold by the FDA in May 2021 following the Company's notification to the Agency of mortalities which occurred at the highest dose levels in a 26-week non-human primate (NHP) toxicology study that was designed to support extended dosing of patients with CTI-1601. At the time of the notice, Larimar had no interventional clinical trials with patients enrolled or enrolling.

Data from Phase 1 single- and multiple-ascending dose (MAD) clinical trials indicated that repeated subcutaneous injections of CTI-1601 were generally well tolerated at doses up to 100 mg administered daily for up to 13 days. No serious adverse events, important medical events, or treatment-related severe adverse events were reported in the trials 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 cohorts 2 and 3 of the MAD trial also showed that subcutaneous injections of 50 or 100 mg of CTI-1601, administered daily for at least seven days, 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. Cohort 1 of the MAD trial, which evaluated a 25 mg dose, explored a daily dosing regimen for only four days.

Second Quarter 2022 Financial Results

As of June 30, 2022, the Company had cash and marketable debt securities totaling $54.9 million which provides projected cash runway through the third quarter of 2023.

The Company reported a net loss for the second quarter of 2022 of $8.7 million, or $0.47 per share, compared to a net loss of $12.6 million, or $0.79 per share, for the second quarter of 2021.

Research and development expenses for the second quarter of 2022 were $5.6 million compared to $9.1 million for the second quarter of 2021. The decrease in research and development expenses compared to the prior year period was primarily driven by a decrease in nonclinical costs of $2.5 million and a decrease of $1.1 million in clinical trial costs partially offset by an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

General and administrative expenses for the second quarter of 2022 were $3.0 million compared to $3.4 million for the second quarter of 2021. The decrease in general and administrative expenses compared to the prior year period was primarily driven by a decrease in professional fees associated

with legal and accounting services of $0.5 million partially offset by an increase of $0.1 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

The Company reported a net loss for the first half of 2022 of $17.6 million, or $0.96 per share, compared to a net loss of $24.7 million, or $1.54 per share, for the first half of 2021.

Research and development expenses for the first half of 2022 were $11.5 million compared to $18.1 million for the first half of 2021. The decrease in research and development expenses compared to the prior year period was primarily driven by a decrease of $3.0 million in clinical trial costs, a decrease in clinical supply manufacturing costs of $2.3 million, and a decrease in nonclinical costs of $2.3 million partially offset by an increase of $0.4 million in personnel related costs due to annual compensation increases and an increase of $0.4 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

General and administrative expenses for the first half of 2022 were $6.1 million compared to $6.6 million for the first half of 2021. The decrease in general and administrative expenses compared to the prior year period was primarily driven by a decrease in professional fees associated with legal and accounting services of $0.6 million, a decrease in operational expenses of $0.3 million and partially offset by an increase of $0.4 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

About CTI-1601
CTI-1601 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. 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 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, 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 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, including the timing of its CTI-1601 clinical development plan 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, FDA's acceptance of our proposed four-week dose exploration study and any modifications thereto, and any requests for additional toxicology studies, the timing and outcome of Larimar's planned interactions with the FDA concerning 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 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 ongoing impact of the COVID-19 pandemic on Larimar's future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and the potential impact of the Russian invasion of Ukraine on Larimar's ability to raise additional capital 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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Larimar Therapeutics, Inc.
Condensed Consolidated Balance Sheet
(Unaudited)

	June 30, 2022	December 31, 2021

Assets		
Current assets:		
Cash and cash equivalents	$ 19,736	$ 70,097
Marketable debt securities	35,188	—
Prepaid expenses and other current assets	1,820	2,107
Total current assets	56,744	72,204
Property and equipment, net	986	1,049
Operating lease right-of-use assets	3,134	3,406
Restricted cash	1,339	1,339
Other assets	649	669
Total assets	$ 62,852	$ 78,667
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 425	$ 1,660
Accrued expenses	6,656	6,592
Operating lease liabilities, current	638	594
Total current liabilities	7,719	8,846
Operating lease liabilities	5,077	5,408
Total liabilities	12,796	14,254
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of June 30, 2022 and December 31, 2021; no shares issued and outstanding as of June 30, 2022 and December 31, 2021	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2022 and December 31, 2021; 17,710,450 shares issued and outstanding as of June 30, 2022 and December 31, 2021	18	18
Additional paid-in capital	183,955	180,645
Accumulated deficit	(133,860)	(116,250)
Accumulated other comprehensive loss	(57)	—
Total stockholders' equity	50,056	64,413
Total liabilities and stockholders' equity	$ 62,852	$ 78,667

Larimar Therapeutics, Inc.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	$ 5,644	$ 9,102	$ 11,450	$ 18,076
General and administrative	3,043	3,441	6,124	6,573
Total operating expenses	8,687	12,543	17,574	24,649
Loss from operations	(8,687)	(12,543)	(17,574)	(24,649)
Other income (expense), net	20	(66)	(36)	(48)
Net loss and Total comprehensive loss	$ (8,667)	$ (12,609)	$ (17,610)	$ (24,697)
Net loss per share, basic and diluted	$ (0.47)	$ (0.79)	$ (0.96)	$ (1.54)
Weighted average common shares outstanding, basic and diluted	18,338,853	15,996,133	18,338,853	15,996,133
Comprehensive loss:				
Net loss and Total comprehensive loss	$ (8,667)	$ (12,609)	$ (17,610)	$ (24,697)
Other comprehensive loss:				
Unrealized gain (loss) on marketable debt securities	(57)	—	(57)	—
Total other comprehensive loss	(57)	—	(57)	—
Total comprehensive loss	$ (8,724)	$ (12,609)	$ (17,667)	$ (24,697)

Source: Larimar Therapeutics