Larimar Therapeutics Reports Second Quarter 2021 Operating and Financial Results

August 12, 2021

- Reported positive proof-of-concept and dose response data from Phase 1 program evaluating CTI-1601 in patients with Friedreich's ataxia (FA)

- Subcutaneous injections of CTI-1601 at doses of 50 mg or 100 mg resulted in frataxin levels in buccal cells of FA patients that were at or in excess of those that would be expected in phenotypically normal heterozygous carriers

- Phase 1 safety data indicate that repeated subcutaneous injections of CTI-1601 were generally well tolerated at doses up to 100 mg administered daily for 13 days

- Completed dosing in July in 180-day non-human primate toxicology study designed to support extended dosing of CTI-1601

- Cash and investments of $70.6 million as of June 30, 2021

- Closed $20 million equity financing on July 2, 2021

BALA CYNWYD, Pa., Aug. 12, 2021 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its second quarter and year to date June 30, 2021 operating and financial results.

"We finished the second quarter in a strong financial position and with a compelling clinical data set that demonstrates proof-of-concept for CTI-1601, which to our knowledge is the only clinical-stage candidate designed to address the root cause of Friedreich's ataxia," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar. "These positive Phase 1 data along with non-clinical pharmacology data demonstrate proof-of-concept, and CTI-1601's differentiated mechanism of action helped us to earn a PRIME designation from the European Medicines Agency, providing us with valuable regulatory benefits and important external validation. Looking forward, we continue to collect and analyze data from our 180-day non-human primate toxicology study and remain confident that there is a path forward through the resolution of the CTI-1601 clinical hold and towards the initiation of our Jive open-label extension and pediatric multiple ascending dose trials."

Second Quarter 2021 Highlights

- In May 2021, Larimar reported positive topline data from its Phase 1 Friedreich's ataxia (FA) program after completing dosing of the single ascending dose (SAD) trial in December, 2020 and of the multiple ascending dose (MAD) trial in March, 2021. Data from these trials demonstrate proof-of-concept by showing that daily subcutaneous injections of CTI-1601 for up to 13 days resulted in dose-dependent increases in frataxin levels from baseline compared to placebo in all evaluated tissues (buccal cells, skin, and platelets). Frataxin levels achieved in peripheral tissues (buccal cells) following daily 50 mg and 100 mg subcutaneous injections of CTI-1601 were at or in excess of frataxin levels that would be expected in phenotypically normal heterozygous carriers. There were no serious adverse events (SAEs), associated with either the MAD or SAD trials.

- In May 2021, Larimar received European Medicines Agency (EMA) Priority Medicines (PRIME) designation for CTI-1601 in FA. Through PRIME, the EMA offers early and proactive support to medicine developers to optimize the generation of robust data on a medicine's benefits and risks and enables accelerated assessment of medicines applications so that these medicines can reach patients earlier. Larimar's PRIME designation was based on pre-clinical data as well as tolerability data from the CTI-1601 Phase 1 program in patients with FA.

- On May 25, 2021 the United States Food and Drug Administration (FDA) placed a clinical hold on the CTI-1601 clinical program following the Company's notification 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 that 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 FDA that additional clinical trials may commence. At the time of the notice, the Company had no interventional clinical trials with patients enrolled or enrolling.

- In July 2021, the Company completed dosing in the 180-day NHP toxicology study discussed above. The Company is currently collecting and analyzing data from the study. While there is no way to predict the FDA's response or whether they will require additional data or testing before lifting the clinical hold on CTI-1601 in full or in part, the Company expects to initiate its Jive open-label extension and pediatric MAD trials in the first half of next year,

Recent Developments
Under an Equity Distribution Agreement with an investment bank, the Company may sell up to an aggregate of $50,000,000 of shares of common stock from time to time in connection with an "at the market" program. In July 2021, the Company sold an additional 2,342,720 shares under the agreement for net proceeds of $19.9 million. The Company can raise an additional $29.2 million under this program.

Anticipated Milestones

- Obtain FDA clearance to initiate future clinical trials
- Initiate a non-interventional healthy volunteer study this year to generate data for comparison to FA patients
- Initiate Jive open-label extension clinical trial in the first half of 2022
- Initiate MAD trial in patients under 18 years of age in the first half of 2022

Second Quarter 2021 Financial Results

As of June 30, 2021, the Company had cash and cash equivalents totaling $70.6 million.

The Company reported a net loss for the second quarter of 2021 of $12.6 million, or $0.79 per share, compared to a net loss of $11.3 million, or $1.21 per share, for the second quarter of 2020.

Research and development expenses for the second quarter of 2021 were $9.1 million compared to $8.9 million for the second quarter of 2020. The increase in research and development expenses compared to the prior year period was primarily driven by higher non-clinical costs associated with assay development and toxicology studies, an increase in clinical trial costs, an increase in personnel related costs due to headcount additions in our research and development functions, an increase in stock compensation expense associated with stock option grants made in the second half of 2020 and thus far in 2021, partially offset by a decrease of clinical supply manufacturing costs.

General and administrative expenses for the second quarter of 2021 were $3.4 million, compared to $2.5 million for the second quarter of 2020. The increase in general and administrative expenses as compared to the prior year period was primarily driven by an increase in personnel related costs due to increased headcount, an increase in stock-based compensation associated with stock option grants made in the second half of 2020 and thus far in 2021, an increase in professional fees primarily associated with insurance costs, legal and consulting fees as a result of operating as a public company.

For the first half of 2021, the Company reported a net loss of $24.7 million, or $1.54 per share, compared to a net loss of $18.1 million, or $2.33 per share for the same period in 2020.

Research and development expenses for the first half of 2021 were $18.1 million compared to $13.9 million for the first half of 2020. The increase in research and development expenses compared to the prior year period was primarily driven by an increase in clinical trial costs, higher non-clinical costs associated with the assay and toxicology studies, an increase in personnel related costs due to headcount additions in our research and development functions, an increase in stock compensation expense associated with stock option grants made in the second half of 2020 and thus far in 2021, partially offset by a decrease of clinical supply manufacturing costs.

General and administrative expenses for the first half of 2021 were $6.6 million, compared to $4.2 million for the first half of 2020. The increase in general and administrative expenses as compared to the prior year period was primarily driven by an increase in personnel related costs due to increased headcount, an increase in stock-based compensation associated with stock option grants made in the second half of 2020 and thus far in 2021, an increase in professional fees primarily associated with insurance costs, recruiting expenses, legal and consulting fees as a result of operating as a public company, partially offset by a decrease in accounting and audit costs related to additional years under audit in the first quarter 2020.

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 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 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 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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Larimar Therapeutics, Inc.
Consolidated Balance Sheet
(Unaudited)

	June 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 70,630	$ 68,148
Marketable debt securities	—	24,490
Prepaid expenses and other current assets	3,406	5,314
Total current assets	74,036	97,952
Property and equipment, net	1,211	1,040
Operating lease right-of-use assets	3,673	3,936
Restricted cash	1,339	1,339
Other assets	672	419
Total assets	$ 80,931	$ 104,686
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,463	$ 2,634
Accrued expenses	5,675	5,843
Operating lease liabilities, current	553	515
Total current liabilities	7,691	8,992
Operating lease liabilities	5,715	6,002
Total liabilities	13,406	14,994
Commitments and contingencies		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of December 31, 2020 and December 31, 2019; no shares issued and outstanding as of December 31, 2020 and December 31, 2019	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2021 and December 31, 2020; 15,367,730 and 6,091,250 shares issued and outstanding as of June 30, 2021 and December 31, 2020, respectively	15	15
Additional paid-in capital	157,820	155,290
Accumulated deficit	(90,311)	(65,614)
Accumulated other comprehensive gain	1	1
Total stockholders' equity	67,525	89,692
Total liabilities and stockholders' equity	$ 80,931	$ 104,686

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

	Three Months Ended June 30,	Six Months Ended June 30,

	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 9,102	$ 8,907	$ 18,076	$ 13,914
General and administrative	3,441	2,492	6,573	4,159
Total operating expenses	12,543	11,399	24,649	18,073
Loss from operations	(12,543)	(11,399)	(24,649)	(18,073)
Other income, net	(66)	69	(48)	69
Net loss	$ (12,609)	$ (11,330)	$ (24,697)	$ (18,004)
Net loss per share, basic and diluted	$ (0.79)	$ (1.21)	$ (1.54)	$ (2.33)
Weighted average common shares outstanding, basic and diluted	15,996,133	9,381,412	15,996,133	7,736,331

Source: Larimar Therapeutics