Larimar Therapeutics Reports Fourth Quarter and Full Year 2021 Operating and Financial Results

March 25, 2022

BALA CYNWYD, Pa., March 25, 2022 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (“Larimar”) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its full year 2021 operating and financial results.

“The past year was highlighted by our first clinical data readouts, which demonstrated the potential of CTI-1601 to address the root cause of Friedreich’s ataxia by increasing frataxin levels in patients,” said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar. “With these important proof-of-concept data in hand, we continue to work expeditiously to identify the best path forward through the resolution of CTI-1601’s clinical hold. Our commitment to furthering CTI-1601’s development remains steadfast, and the urgent unmet needs of patients with Friedreich’s ataxia continue to inspire us. We look forward to our continued progress and would like to thank all those who played a role in the advancement of our Phase 1 program over the past year including our clinical trial participants, their families, and members of the Friedreich’s Ataxia Research Alliance.”

2021 and Subsequent Highlights

- In May 2021, Larimar reported positive topline data from its Phase 1 Friedreich’s ataxia (FA) program. These data, which were from single- and multiple ascending dose trials in FA patients, demonstrated 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 similar to or exceeded frataxin levels that would be expected in phenotypically normal heterozygous carriers. The data also show that CTI-1601 was generally well tolerated at doses up to 100 mg administered daily for up to 13 days, as there were no serious adverse events (SAEs) associated with either of the Phase 1 trials.

- In May 2021, Larimar received a 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. It also enables accelerated assessment of medicine applications so that these medicines can reach patients earlier. Larimar’s PRIME designation was based on tolerability data from the Phase 1 program of CTI-1601 in patients with FA as well as pre-clinical results.

- In August 2021, Larimar initiated a non-interventional healthy volunteer study designed to generate data for comparison to patients with FA. Enrollment in this study was completed in early 2022.

- In February 2022, Larimar received feedback from the U.S. Food and Drug Administration (FDA) regarding the May 2021 clinical hold placed on the CTI-1601 program. The May 2021 hold followed 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 hold was placed, Larimar had no interventional clinical trials with patients enrolled or enrolling. In the feedback provided in February 2022, the FDA stated it was maintaining the clinical hold and that additional data are needed to resolve the clinical hold. Larimar is evaluating how to best provide these data to FDA and is also reassessing the timing of its planned open label extension and the pediatric MAD studies.

Fourth Quarter and Full Year 2021 Financial Results

As of December 31, 2021, the Company had cash and cash equivalents totaling $70.1 million.

The Company reported a net loss for the fourth quarter of 2021 of $9.1 million, or $0.50 per share, compared to a net loss of $14.2 million, or $0.89 per share, for the fourth quarter of 2020.

Research and development expenses for the fourth quarter of 2021 were $6.3 million compared to $10.6 million for the fourth quarter of 2020. The decrease in research and development expenses compared to the prior year period was primarily driven by lower clinical supply manufacturing costs of $4.1 million and a decrease of $1.5 million in clinical trial costs, partially offset by higher non-clinical and internal laboratory costs of $0.4 million, an increase of $0.3 million in personnel related costs due to headcount additions in our research and development functions, an increase of $0.3 million in professional fees primarily related to consulting services and an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2021.

General and administrative expenses for the fourth quarter of 2021 were $2.8 million compared to $3.8 million for the fourth quarter of 2020. The decrease in general and administrative expenses as compared to the prior year period was primarily driven by a decrease of $0.7 million in professional fees primarily associated with accounting, legal, communication and consulting fees and a decrease of $0.3 million in facility costs, partially offset by an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2021.

For the full year 2021, the Company reported a net loss of $50.6 million, or $2.95 per share, compared to a net loss of $42.5 million, or $3.57 per share for the same period in 2020.

Research and development expenses for the full year 2021 were $38.4 million compared to $31.4 million for the same period in 2020. The increase in research and development expenses compared to the prior year period was primarily driven by higher non-clinical and internal laboratory costs of $3.2 million, an increase of $2.0 million in personnel related costs due to increased headcount in our research and development functions, an increase of $1.3 million in stock-based compensation expense associated with stock option grants made in the second half of 2020 and in 2021, higher clinical supply manufacturing costs of $0.4 million, an increase of $0.4 million in professional fees primarily related to consulting services, partly offset by a decrease of $0.6 million in clinical trial costs.

General and administrative expenses for the full year 2021 were $12.1 million compared to $11.4 million for the same period in 2020. The increase in general and administrative expenses as compared to the prior year period was primarily driven by an increase of $2.0 million in stock-based compensation expense associated with stock option grants made in the second half of 2020 and in 2021, an increase of $1.1 million in other operating expenses including insurance, recruiting and IT costs required to function as a public company, an increase of $0.3 million in personnel related costs due to increased headcount, partially offset by a decrease of $2.1 million in professional fees primarily associated with accounting, legal, communication and consulting fees and a decrease of $0.6 million in facility costs associated with the sublease agreement entered in late 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 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 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, 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, the ongoing impact of the COVID-19 pandemic 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 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 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.
Consolidated Balance Sheet
(unaudited)

	December 31, 2021	December 31, 2020
Assets		
Current assets:		

Cash and cash equivalents	$ 70,097	$ 68,148
Marketable debt securities	—	24,490
Prepaid expenses and other current assets	2,107	5,314
Total current assets	72,204	97,952
Property and equipment, net	1,049	1,040
Operating lease right-of-use assets	3,406	3,936
Restricted cash	1,339	1,339
Other assets	669	419
Total assets	$ 78,667	$ 104,686
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,660	$ 2,634
Accrued expenses	6,592	5,843
Operating lease liabilities, current	594	515
Total current liabilities	8,846	8,992
Operating lease liabilities	5,408	6,002
Total liabilities	14,254	14,994
Commitments and contingencies		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of December 31, 2021 and December 31, 2020; no shares issued and outstanding as of December 31, 2021 and December 31, 2020	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of December 31, 2021 and December 31, 2020; 17,710,450 and 15,367,730 shares issued and outstanding as of December 31, 2021 and December 31, 2020, respectively	18	15
Additional paid-in capital	180,645	155,290
Accumulated deficit	(116,250)	(65,614)
Accumulated other comprehensive loss	—	1
Total stockholders' equity	64,413	89,692
Total liabilities and stockholders' equity	$ 78,667	$ 104,686

Larimar Therapeutics, Inc.

Consolidated Statements of Operations

(In thousands, except share and per share data)

(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 6,292	$ 10,563	$ 38,396	$ 31,407
General and administrative	2,794	3,832	12,069	11,397
Total operating expenses	9,086	14,395	50,465	42,804
Loss from operations	(9,086)	(14,395)	(50,465)	(42,804)
Other income (loss), net	(48)	192	(171)	322
Net loss	$ (9,134)	$ (14,203)	$ (50,636)	$ (42,482)
Net loss per share, basic and diluted	$ (0.50)	$ (0.89)	$ (2.95)	$ (3.57)
Weighted average common shares outstanding, basic and diluted	18,338,853	15,985,199	17,164,284	11,883,106

Source: Larimar Therapeutics