Larimar Therapeutics Reports Preliminary Top-line Data from Phase 2 Trial's 25 mg Cohort Showing Increases in Frataxin Levels in Patients with Friedreich's Ataxia and First Quarter 2023 Financial Results

May 15, 2023

- *Safety data indicate that repeated subcutaneous injections of 25 mg CTI-1601 were generally well tolerated when administered daily for 14 days and then every-other-day thereafter until day 28*
- *Daily subcutaneous injections of 25 mg CTI-1601 for 14 days led to increases in frataxin levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells)*
- *Median placebo-adjusted increases from baseline of 3.5 pg/µg and 0.9 pg/µg in frataxin levels observed in skin and buccal cells, respectively, with 14 days of daily dosing of CTI-1601*
- *Larimar has submitted data to FDA and will meet with the Agency to discuss next steps; update expected in Q3 2023*
- *Company management to host webcast and conference call today at 8:00 a.m. ET*

BALA CYNWYD, Pa., May 15, 2023 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced preliminary top-line data from the 25 mg cohort of its Phase 2, four-week, placebo-controlled, dose exploration trial of CTI-1601 in participants with Friedreich's ataxia (FA). Participants in the trial's 25 mg cohort (n=13) were randomized to receive subcutaneous injections of 25 mg CTI-1601 (n=9) or placebo (n=4) daily for 14 days and then every-other-day thereafter until day 28. Data from the cohort indicate CTI-1601 was generally well tolerated and showed increases in frataxin (FXN) levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14.

In skin, a median placebo-adjusted increase from baseline of 3.5 pg/µg in frataxin levels was observed on day 14 (frataxin concentration normalized to total protein). Of the seven CTI-1601-treated participants with quantifiable levels of frataxin in skin at both baseline and day 14, all seven had increases in skin frataxin concentrations, compared to none of the four placebo participants with quantifiable levels of frataxin in skin at both baseline and day 14. In buccal cells, a median placebo-adjusted increase from baseline of 0.9 pg/µg in frataxin levels was observed on day 14 (frataxin concentration normalized to total protein). Of the seven CTI-1601-treated participants with quantifiable levels of frataxin in buccal cells at both baseline and day 14, five had increases in buccal cell frataxin concentrations, compared to neither of the two placebo participants with quantifiable levels of frataxin in buccal cells at both baseline and day 14.

In a non-interventional study that used the same sampling technique and assay as Larimar's Phase 2 trial to measure frataxin levels in 60 homozygous healthy volunteers, median frataxin concentrations observed in skin and buccal cells were 16 pg/µg and 8 pg/µg, respectively (frataxin concentration normalized to total protein). Larimar therefore estimates phenotypically healthy heterozygous carriers of the FA-causing gene to have median frataxin concentrations of approximately 8 pg/µg and 4 pg/µg in skin and buccal cells, respectively, based on published literature indicating heterozygous carriers have frataxin levels that are approximately 50% of those of homozygous healthy people.

Larimar's Phase 2 data and non-interventional study results follow Phase 1 data that showed dose-dependent increases in frataxin levels in peripheral tissue with daily dosing of 50 and 100 mg of CTI-1601 for at least 7 days, and no detectable increase in FXN levels with daily dosing of 25 mg of CTI-1601 for only 4 days.

Larimar has submitted the data from the trial's 25 mg cohort to FDA and has a meeting scheduled with the Agency for later this quarter to discuss the information needed to gain clearance to initiate a 50 mg cohort in the Phase 2 trial.

"Our preliminary Phase 2 data provide the first clinical indication that a 25 mg dose of CTI-1601 can increase frataxin levels in peripheral tissues, building upon our proof-of-concept Phase 1 results," said Carole Ben-Maimon**,** MD, President and Chief Executive Officer of Larimar. "Importantly, the frataxin increases achieved with a relatively low 25 mg dose in our Phase 2 trial suggest a continuous daily dosing regimen is preferred for maintaining increases achieved with 25 mg CTI-1601. I would like to thank all those who participated in our trials and look forward to our upcoming meeting with the FDA later this quarter."

The median change from baseline in FXN levels at day 14 (last day of daily dosing) and day 28 (end of treatment and after switching to every other day dosing) in the Phase 2 trial are shown in the table below.

TABLE 1: FXN Change from Baseline in Skin Biopsies[#]
Units: pg FXN / µg total protein
Data presented as: median (25th percentile, 75th percentile) (n)

Dose Group	**Day 14**			**Day 28**		
Placebo	-0.66	(-1.08, -0.37)	(n=4)	-0.30	(-0.90, 0.77)	(n=4)
25 mg CTI-1601	2.81	(2.16, 3.32)	(n=7)	2.28	(-0.03, 2.71)	(n=7)

#Day 14 and 28 skin biopsies were not collected from one CTI-1601 treated participant who discontinued treatment and one CTI-1601 treated participant had FXN levels below quantifiable levels at day 14 and day 28

TABLE 2: FXN Change from Baseline in Buccal Cells#
Units: pg FXN / µg total protein
Data presented as: median (25th percentile, 75th percentile) (n)

Dose Group	Day 14			Day 28		
Placebo	-0.35	(-0.58, -0.13)	(n=2)	0.01	(-0.52, 0.53)	(n=2)
25 mg CTI-1601	0.56	(-0.28, 0.64)	(n=7)	0.03	(-0.66, 0.86)	(n=6)

For the placebo group, one participant had buccal cell FXN levels below quantifiable levels at baseline and one participant had buccal cell FXN levels below quantifiable levels at day 14 and day 28
#For 25 mg group, day 28 buccal FXN were not collected from one participant who discontinued treatment and two participants had buccal FXN levels below quantifiable levels at baseline

Pharmacokinetic data suggest that steady state was achieved by day 14 in the Phase 2 trial's 25 mg cohort, which was the last day of daily dosing. Safety data indicate that CTI-1601 was generally well tolerated in the Phase 2 trial's 25 mg cohort. A summary of safety data from the cohort is shown below:

- No serious adverse events were reported
- No important medical events were reported
- One severe adverse event was reported, which was an allergic reaction to study drug that resolved with standard treatment
- Of the nine participants dosed with CTI-1601, eight completed the trial, with one participant withdrawing due to the aforementioned allergic reaction that resolved with standard treatment
- The most common adverse events were mild and moderate injection site reactions. At least one injection site reaction was seen in two of four placebo treated participants and in all CTI-1601 treated participants.

Dr. Ben-Maimon added, "Our Phase 2 results add to our safety database indicating that CTI-1601 is generally well tolerated. Thirty-seven adults with FA have been dosed with CTI-1601 across our Phase 1 and 2 trials, with 35 completing treatment, one withdrawing due to an allergic reaction, and another withdrawing after a single 50 mg dose in the multiple ascending dose trial due to mild to moderate nausea and vomiting. We believe the safety, pharmacokinetic, and pharmacodynamic data generated to date support evaluation of a 50 mg dose of CTI-1601 in our Phase 2 trial and look forward to discussing our findings with the U.S. Food and Drug Administration (FDA) at our meeting later this quarter."

The initiation of additional cohorts in the Phase 2 trial and/or the initiation of other clinical trials of CTI-1601 are contingent on a review of data and analyses from the Phase 2 trial's 25 mg cohort by the FDA, in accordance with a partial clinical hold on the CTI-1601 program first put into place after a full clinical hold was lifted in September 2022. Larimar has submitted the data from the trial's 25 mg cohort to FDA and has a meeting scheduled with the Agency for later this quarter to discuss the information needed to gain clearance to initiate a 50 mg cohort in the Phase 2 trial. Larimar expects to provide an update on the next steps for the CTI-1601 program in the third quarter of 2023, after it has received feedback from the upcoming FDA meeting.

First Quarter 2023 Financial Results

In addition to preliminary top-line data from its Phase 2 trial's 25 mg cohort, Larimar today announced financial results for the first quarter of 2023.

As of March 31, 2023, the Company had cash and cash equivalents totaling $111.5 million.

The Company reported a net loss for the first quarter of 2023 of $6.5 million, or $0.15 per share, compared to a net loss of $8.9 million, or $0.49 per share, for the first quarter of 2022.

Research and development expenses for the first quarter of 2023 were $4.6 million compared to $5.8 million for the first quarter of 2022. The decrease in research and development expenses compared to the prior year period was primarily driven by a decrease of $0.7 million in nonclinical development costs a decrease of $0.4 million in drug manufacturing costs, and a decrease of $0.1 million in clinical trial expense.

General and administrative expenses were $3.1 million in both the first quarter of 2023 and the first quarter of 2022. Decreases in recruiting and professional service fees were offset by increases in stock-based compensation expense and other personnel-related expenses.

Conference Call and Webcast
Larimar will host a conference call and webcast today, May 15, 2023 at 8:00 a.m. ET. To access the webcast, please visit this link to the event. To participate by phone, please dial 1-877-407-9716 (domestic) or 1-201-493-6779 (international) and refer to conference ID 13738708, or click on this link and request a return call. Following the live event, an archived webcast will be available on the "Events & Presentations" page of the Larimar website.

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 partial 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, 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, Larimar's ability to successfully engage with the FDA and satisfactorily respond to requests from the FDA for further information and data regarding the CTI-1601 clinical trial including the FDA review of data from cohort one from the Phase 2 dose exploration trial and FDA's agreement to escalate the dosing in cohort two, the timing and outcomes of Larimar's interactions with the FDA concerning the partial clinical hold, the success, cost and timing of Larimar's product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones; that preliminary and top-line 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; 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 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.

Investor Contact:
Joyce Allaire
LifeSci Advisors
jallaire@lifesciadvisors.com
(212) 915-2569

Company Contact:
Michael Celano
Chief Financial Officer
mcelano@larimartx.com
(484) 414-2715

Larimar Therapeutics, Inc.
Consolidated Balance Sheet
(unaudited)

	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 111,524	$ 26,825
Marketable securities	—	91,603
Prepaid expenses and other current assets	1,940	2,311
Total current assets	113,464	120,739
Property and equipment, net	753	831
Operating lease right-of-use assets	2,719	2,858
Restricted cash	1,339	1,339
Other assets	632	638
Total assets	$ 118,907	$ 126,405
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 695	$ 1,686

Accrued expenses	6,726	8,408
Operating lease liabilities, current	589	611
Total current liabilities	8,010	10,705
Operating lease liabilities	4,656	4,797
Total liabilities	12,666	15,502
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of March 31, 2023 and December 31, 2022; no shares issued and outstanding as of March 31, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of March 31, 2023 and December 31, 2022; 43,269,200 shares issued and outstanding as of March 31, 2023 and December 31, 2022	43	43
Additional paid-in capital	264,329	262,496
Accumulated deficit	(158,131)	(151,605)
Accumulated other comprehensive loss	—	(31)
Total stockholders' equity	106,241	110,903
Total liabilities and stockholders' equity	$ 118,907	$ 126,405

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

	Three Months Ended March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 4,562	$ 5,806
General and administrative	3,075	3,081
Total operating expenses	7,637	8,887
Loss from operations	(7,637)	(8,887)
Other income (expense), net	1,111	(56)
Net loss	$ (6,526)	$ (8,943)
Net loss per share, basic and diluted	$ (0.15)	$ (0.49)
Weighted average common shares outstanding, basic and diluted	43,897,603	18,338,853
Comprehensive loss:		
Net loss	$ (6,526)	$ (8,943)
Other comprehensive gain:		
Unrealized gain on marketable securities	31	—
Total other comprehensive gain	31	—
Total comprehensive loss	$ (6,495)	$ (8,943)

Source: Larimar Therapeutics