Larimar Therapeutics Reports Third Quarter 2023 Operating and Financial Results

November 14, 2023

- *Completed full enrollment and dosing of the 50 mg cohort in Phase 2 Friedreich's ataxia (FA) dose exploration trial; based on blinded observations during the dosing period, there were no serious adverse events in either the CTI-1601 (nomlabofusp) or placebo groups.*
- *Top-line safety, pharmacokinetic, and frataxin data from the Phase 2 trial now expected in Q1 2024, refined from H1 2024*
- *Initiation of open label extension (OLE) trial with 25 mg daily dosing of nomlabofusp remains on track for Q1 2024; interim data expected in Q4 2024*
- *Cash, cash equivalents and marketable securities of $95.6 million as of September 30, 2023, provides projected cash runway into Q1 2025*

BALA CYNWYD, Pa., Nov. 14, 2023 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. (“Larimar”) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its third quarter operating and financial results.

“We are pleased with the execution and pace at which our nomlabofusp program is advancing. The 50 mg cohort in our Phase 2 dose exploration trial is fully enrolled and all 15 participants have completed dosing and continue in the blinded follow up period. Based on blinded observations during the dosing period, there were no serious adverse events in either the nomlabofusp or placebo groups. We expect top-line safety, pharmacokinetic, and frataxin data now in the first quarter of 2024, refined from the first half of 2024. As our next major catalyst, clinical findings from the 50 mg cohort should provide additional data to inform the dose and dose regimen for our dose exploration trial, potential registrational trial and any dosing updates to our soon to be initiated OLE trial,” said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. “For the OLE trial, initiation remains on track for the first quarter of 2024, and we expect to report interim data later that year in the fourth quarter. We believe the OLE trial is a foundational step for the nomlabofusp program. Importantly, it will provide real-life experience for daily subcutaneous injections of nomlabofusp at home directly by patients or caregivers, as well as further characterize the long-term safety and pharmacokinetic profiles of nomlabofusp and the effect of nomlabofusp on frataxin levels.”

“We continue to have ongoing interactions with global regulatory health authorities regarding manufacturing, regulatory pathways, and clinical development with a focus on initiating the pediatric clinical development program and planning our global clinical studies. The addition of Dr. Jeffrey Sherman to our Board of Directors, an industry executive with invaluable insight in global regulatory and clinical strategy for rare diseases will further complement our current efforts to broaden the nomlabofusp clinical program. As we look ahead, we are energized and focused on executing across our key near term milestones over the next six months, and bringing nomlabofusp, a novel therapy designed to increase frataxin levels and address the underlying deficiency causing Friedreich’s ataxia, to more patients as quickly as possible,” Dr. Ben-Maimon concluded.

Third Quarter and Subsequent Highlights

- In November 2023, Larimar completed enrollment and dosing of the 50 mg cohort of its Phase 2 double-blind dose exploration trial evaluating CTI-1601 (nomlabofusp) for the treatment of Friedreich’s ataxia. Treatment assignment of the fully enrolled cohort of 15 participants remains blinded as they complete the follow up period. Participants were dosed daily with nomlabofusp or placebo for the first 14 days, and then every other day until Day 28. Based on blinded Phase 2 observations during the dosing period, there were no serious adverse events for either the nomlabofusp or placebo groups. Top-line Phase 2 safety, pharmacokinetic, and frataxin data from skin and buccal cells from both the 25 mg and 50 mg cohorts is now expected in the first quarter of 2024, refined from the first half of 2024. Initiation of additional U.S. clinical trials or potential further dose escalation in these trials is contingent on FDA review of Phase 2 data from the 50 mg cohort due to the partial clinical hold.

- In November 2023, Larimar reaffirmed guidance for initiation of the OLE trial evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. Participants who complete treatment in the Phase 2 dose exploration trial, or who previously completed a prior clinical trial of nomlabofusp are potentially eligible for the OLE. The OLE will evaluate the safety and tolerability, pharmacokinetics, and measures of frataxin levels in peripheral tissues as well as other exploratory pharmacodynamic markers (lipid profiles and gene expression data)following long-term subcutaneous administration of nomlabofusp. Clinical measures collected during the trial will be compared to data from a synthetic control arm derived from participants in the Friedreich’s Ataxia Clinical Outcome Measures Study (FACOMS) database. The OLE trial is expected to begin in Q1 2024 with interim data expected in Q4 2024.

- In October 2023, Larimar appointed Jeffrey W. Sherman, M.D., F.A.C.P. to the Company’s Board of Directors. Dr. Sherman, Executive Vice President, Chief Medical Officer (CMO) at Horizon Therapeutics Public Limited Company

(recently acquired by Amgen), brings more than 25 years of pharmaceutical experience, specializing in regulatory and clinical strategy, and therapeutic development for rare diseases.

- As of October 2023, "nomlabofusp" was published as the INN (International Nonproprietary Name) and USAN (United States Adopted Name) for CTI-1601.
- In July 2023, Larimar received FDA clearance to initiate both a 50 mg cohort in the Phase 2 dose exploration trial evaluating nomlabofusp for FA and an OLE trial following FDA review of unblinded safety, pharmacokinetic, and frataxin data from the Phase 2 trial's 25 mg cohort.

Third Quarter 2023 Financial Results

As of September 30, 2023, the Company had cash, cash equivalents and marketable securities totaling $95.6 million, which provides projected cash runway into the first quarter of 2025.

The Company reported a net loss for the third quarter of 2023 of $9.1 million, or $0.21 per share, compared to a net loss of $8.3 million, or $0.37 per share, for the third quarter of 2022.

Research and development expenses for the third quarter of 2023 were $6.6 million compared to $5.6 million for the third quarter of 2022. The increase in research and development expenses was driven by an increase of $0.9 million in clinical trial costs primarily associated with the Phase 2 dose exploration study, an increase of $0.7 million in personnel related costs, an increase of $0.4 million in professional fees primarily associated with an increase in legal IP costs and consulting fees, partially offset by a decrease of $1.2 million in clinical supply manufacturing costs.

General and administrative expenses for the third quarter of 2023 were $3.8 million compared to $2.9 million for the third quarter of 2022. The increase in general and administrative expense was driven by an increase of $0.3 million of professional fees related to increased legal expense, an increase of $0.2 million in operational expense primarily related to recruiting costs, and an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2023 and prior periods.

Other income (expense), net was $1.3 million of income in the third quarter of 2023 compared to $0.2 million in the third quarter of 2022. The increase primarily relates to interest income on a higher investment base and higher investment yields on that base during the current period.

The Company reported a net loss for the 9-month period ending September 30, 2023 of $24.0 million, or $0.55 per share, compared to a net loss of $25.9 million, or $1.32 per share, for the 9-month period ending September 30, 2022.

Research and development expenses for the 9-month period ending September 30, 2023 were $17.0 million compared to $17.0 million for the 9-month period ending September 30, 2022. A decrease of $3.2 million in clinical supply manufacturing costs was offset by an increase of $1.2 million in personnel related costs, an increase of $0.9 million in clinical trial costs primarily associated with the Phase 2 dose exploration study, an increase of $0.4 million in professional fees primarily associated with an increase in legal IP costs and consulting fees, and an increase of $0.4 million in test method development and optimization and an increase of $0.3 million in stock-based compensation expense associated with stock option grants made in 2023 and prior periods.

General and administrative expenses for the 9-month period ending September 30, 2023 were $10.6 million compared to $9.1 million for the 9-month period ending September 30, 2022. The increase in general and administrative expense was driven by an increase of $0.5 million of professional fees primarily related to increased legal expense, an increase of $0.5 million in stock-based compensation expense associated with stock option grants made in 2023 and prior periods, an increase of $0.4 million in operational expense primarily related to recruiting costs, and an increase of $0.4 million in personnel related costs related to increases in headcount, partially offset by a decrease of $0.3 million in insurance expense.

Other income (expense), net was $3.6 million of income in the 9-months ended September 30, 2023 compared to $0.2 million of net expense in the 9-months ended September 30, 2022. The increase primarily relates to interest income on a higher investment base and higher investment yields on that base during the current period as compared to the prior period.

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 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 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 regarding the partial clinical hold; 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; 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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Larimar Therapeutics, Inc.
Condensed Consolidated Balance Sheet
(unaudited)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 38,721	$ 26,825
Marketable securities	56,869	91,603
Prepaid expenses and other current assets	2,890	2,311
Total current assets	98,480	120,739
Property and equipment, net	601	831
Operating lease right-of-use assets	2,898	2,858
Restricted cash	1,339	1,339
Other assets	634	638
Total assets	$ 103,952	$ 126,405
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 756	$ 1,686
Accrued expenses	5,094	8,408
Operating lease liabilities, current	708	611
Total current liabilities	6,558	10,705
Operating lease liabilities	4,682	4,797
Total liabilities	11,240	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 September 30, 2023 and December 31, 2022; no shares issued and outstanding as of September 30, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of September 30, 2023 and December 31, 2022; 43,905,903 and 43,269,200 shares issued and outstanding as of September 30, 2023 and December 31, 2022, respectively	43	43
Additional paid-in capital	268,223	262,496
Accumulated deficit	(175,561)	(151,605)
Accumulated other comprehensive gain (loss)	7	(31)
Total stockholders' equity	92,712	110,903
Total liabilities and stockholders' equity	$ 103,952	$ 126,405

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

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 6,585	$ 5,582	$ 17,022	$ 17,032
General and administrative	3,754	2,931	10,574	9,055
Total operating expenses	10,339	8,513	27,596	26,087
Loss from operations	(10,339)	(8,513)	(27,596)	(26,087)
Other income, net	1,275	193	3,640	157
Net loss	$ (9,064)	$ (8,320)	$ (23,956)	$ (25,930)
Net loss per share, basic and diluted	$ (0.21)	$ (0.37)	$ (0.55)	$ (1.32)
Weighted average common shares outstanding, basic and diluted	43,903,738	22,228,228	43,899,670	19,649,558
Comprehensive loss:				
Net loss	$ (9,064)	$ (8,320)	$ (23,956)	$ (25,930)
Other comprehensive gain (loss):				
Unrealized gain (loss) on marketable securities	(5)	17	38	(40)
Total other comprehensive gain (loss)	(5)	17	38	(40)
Total comprehensive loss	$ (9,069)	$ (8,303)	$ (23,918)	$ (25,970)

Source: Larimar Therapeutics