Larimar Therapeutics Reports First Quarter 2024 Operating and Financial Results

May 9, 2024

- *First patient dosed in open label extension (OLE) study with 25 mg daily dosing of nomlabofusp; interim data on track for Q4 2024*
- *Positive top-line Phase 2 dose exploration study data demonstrated nomlabofusp was generally well-tolerated with dose-dependent increases in tissue frataxin levels, reinforcing therapeutic potential*
- *Biologics License Application ("BLA") submission targeted for 2H 2025; discussions initiated with Food and Drug Administration ("FDA") on potential to pursue accelerated approval pathway*
- *Successful $161.8 million financing strengthens cash, cash equivalents, and marketable securities to $239 million as of March 31, 2024, extending projected cash runway into 2026*

BALA CYNWYD, Pa., May 09, 2024 (GLOBE NEWSWIRE) -- Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its first quarter 2024 operating and financial results.

"We have started 2024 off strong, achieving critical milestones that support late-stage advancement of our nomlabofusp clinical program. Positive Phase 2 dose exploration study data demonstrated that nomlabofusp appears to be generally well-tolerated, and observed dose dependent increases in tissue frataxin levels that have the potential to address the underlying frataxin deficiency that is the root cause of Friedreich's ataxia (FA). In March, we dosed the first patient in our OLE study and continue to enroll patients and activate additional sites. We are on track to report interim data in the fourth quarter of the year which will inform on the long-term safety and tissue frataxin levels of nomlabofusp," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Together, these datasets will help support our BLA submission which we are targeting for the second half of 2025. We are continuing our regulatory discussions with the FDA on the potential use of frataxin as a novel surrogate endpoint to support accelerated approval and are planning for a global double-blind placebo-controlled confirmatory study which we expect to initiate prior to BLA submission. With our recent capital infusion and runway extended through key registrational catalysts, we are well positioned to further advance the first potential therapy to increase frataxin levels in patients with FA."

Recent Highlights

- In February 2024, Larimar announced positive top-line data and successful completion of its four-week, placebo-controlled Phase 2 dose exploration study of nomlabofusp (CTI-1601) in patients with FA. Nomlabofusp was generally well-tolerated throughout the four-week treatment periods, had a predictable pharmacokinetic profile, and led to dose-dependent increases in frataxin in skin and buccal cells after daily dosing for 14 days followed by every other day dosing until day 28 in the 25 mg and 50 mg cohorts. Increases in frataxin levels in skin cells were seen in all treated patients, and in buccal cells for the majority of patients. At Day 14, all patients (with quantifiable levels at baseline and Day 14) treated with 50 mg of nomlabofusp achieved frataxin levels in skin cells greater than 33% of the average level observed in healthy volunteers, with 3 patients achieving levels greater than 50% of the average healthy volunteer level.
- In February 2024, Larimar announced the Company had initiated discussions with the FDA on use of tissue frataxin levels as a potential novel surrogate endpoint. Larimar received FDA acknowledgement that frataxin deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. The Company intends to pursue an accelerated approval using FXN levels, supportive pharmacodynamics and clinical information, and safety data from the OLE study, along with additional nonclinical pharmacology information needed to support the novel surrogate endpoint approach, with a BLA submission targeted for the second half of 2025.
- In February 2024, Larimar raised net proceeds of approximately $161.8 million through a public offering of common stock.
- In March 2024, the first patient was dosed in the OLE study evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. Participants who completed treatment in the Phase 2 dose exploration study, or who previously completed a prior clinical trial of nomlabofusp, are potentially eligible to screen for the OLE study. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and 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. Dose escalation in the OLE study is contingent on the FDA's review of data from the 50 mg cohort of the Phase 2 study and available data from the OLE study, due to the continued

partial clinical hold. Interim data is expected in the fourth quarter of 2024. In addition, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcomes Measures Study (FACOMS) database.

- In March 2024, Larimar began to build its commercial team with the appointment of Frank Nazzario, RPh, as Vice President of Commercial. Mr. Nazzario brings nearly 30 years of leadership experience in drug launches for rare diseases. Most recently, he served as Senior Vice President of Sales at BioCryst Pharmaceuticals. Previously, he held commercial leadership roles at Spark Therapeutics where he led the commercialization of Luxturna®, the first FDA-approved gene therapy for an inherited retinal disorder, and at ViroPharma, Inc., where he led the launch of Cinryze®, the first approved biologic for Hereditary Angioedema.

First Quarter 2024 Financial Results

As of March 31, 2024, the Company had cash, cash equivalents and marketable securities totaling $239 million. In February 2024, we raised approximately $161.8 million in net proceeds through a public offering of common stock.

The Company reported a net loss for the first quarter of 2024 of $14.7 million, or $0.27 per share, compared to a net loss of $6.5 million, or $0.15 per share, for the first quarter of 2023.

Research and development expenses for the first quarter of 2024 were $12.9 million, compared to $4.6 million for the first quarter of 2023. The increase in research and development expenses was primarily driven by an increase of $5.7 million in nomlabofusp manufacturing costs, an increase of $1.0 million in clinical costs primarily associated with the initiation of the OLE study, an increase of $1.0 million in personnel expense driven by increasing headcount, an increase of $0.3 million in consulting fees and an increase of $0.2 million in stock compensation expense.

General and administrative expenses were $3.8 million in the first quarter of 2024, compared to $3.1 million in the first quarter of 2023. The increase in general and administrative expenses was primarily driven by an increase of $0.2 million in personnel expense, an increase of $0.2 million in legal fees, and an increase of $0.1 million in stock compensation expense.

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, 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 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, interactions with the FDA 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; 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; that the FDA may not ultimately agree with Larimar's nomlabofusp development strategy; 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)

	March 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 110,125	$ 26,749
Short-term marketable securities	117,171	60,041
Prepaid expenses and other current assets	3,657	3,385
Total current assets	230,953	90,175
Long-term marketable securities	11,711	—
Property and equipment, net	604	684
Operating lease right-of-use assets	2,920	3,078
Restricted cash	1,339	1,339
Other assets	678	659
Total assets	$ 248,205	$ 95,935
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,918	$ 1,283
Accrued expenses	10,098	7,386
Operating lease liabilities, current	825	837
Total current liabilities	12,841	9,506
Operating lease liabilities	4,520	4,709
Total liabilities	17,361	14,215
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, 2024 and December 31, 2023; no shares issued and outstanding as of March 31, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of March 31, 2024 and December 31, 2023; 63,800,017 and 43,909,069 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively	64	43
Additional paid-in capital	434,013	270,150
Accumulated deficit	(203,208)	(188,554)
Accumulated other comprehensive gain (loss)	(25)	81
Total stockholders' equity	230,844	81,720
Total liabilities and stockholders' equity	$ 248,205	$ 95,935

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

	Three Months Ended March 31,	
	2024	2023
Operating expenses:		
Research and development	$ 12,939	$ 4,562
General and administrative	3,795	3,075
Total operating expenses	16,734	7,637
Loss from operations	(16,734)	(7,637)
Other income, net	2,080	1,111
Net loss	(14,654)	(6,526)
Net loss per share, basic and diluted	$ (0.27)	$ (0.15)
Weighted average common shares outstanding, basic and diluted	53,553,707	43,897,603
Comprehensive loss:		
Net loss	$ (14,654)	$ (6,526)
Other comprehensive gain (loss):		
Unrealized gain (loss) on marketable securities	(106)	31

Total other comprehensive gain (loss)	(106)	31
Total comprehensive loss	$ (14,760)	$ (6,495)

Source: Larimar Therapeutics