Larimar Therapeutics Reports Fourth Quarter and Full Year 2023 Operating and Financial Results and Provides Update on Nomlabofusp Development

March 14, 2024

- *Positive top-line data from Phase 2 dose exploration study of nomlabofusp, which was generally well-tolerated, with dose-dependent increases in tissue frataxin levels observed*
- *Initiated discussions with the Food and Drug Administration (FDA) on the potential use of tissue frataxin levels as a novel surrogate endpoint to support a Biologics License Application ("BLA") submission for accelerated approval targeted for 2H 2025*
- *In January 2024, initiated open label extension (OLE) study with 25 mg daily dosing of nomlabofusp, with first patient dosed in March 2024; interim data expected in Q4 2024*
- *Recent successful financing that raised $161.6 million extends expected operating runway into 2026.*

BALA CYNWYD, Pa., March 14, 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 fourth quarter and full year 2023 operating and financial results.

"This year we made tremendous progress across key clinical and regulatory milestones for our nomlabofusp program. We were thrilled to recently report positive top-line data and successful completion of our Phase 2 dose exploration study. Nomlabofusp was generally well-tolerated and demonstrated dose-dependent increases in skin and buccal cell frataxin levels. Importantly, skin frataxin levels as a percentage of levels in healthy volunteers more than doubled in all patients after 14 days of daily treatment with 50 mg. The clear dose-response and the magnitude of increase in tissue frataxin levels further reinforces the therapeutic potential of nomlabofusp to address frataxin deficiency, the known root cause of disease in patients with Friedreich's ataxia (FA)," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Additionally, the first patient in our OLE study has been dosed. The OLE study will inform on the long-term safety profile and long-term tissue frataxin levels. We remain on track to report interim data from the OLE study in the fourth quarter of 2024."

Dr. Ben-Maimon continued, "On the regulatory front, we are continuing discussions with the FDA on the potential use of frataxin as a novel surrogate endpoint to support accelerated approval. We are also beginning to plan for a global double-blind placebo-controlled confirmatory study expected to be initiated prior to a potential BLA submission. The BLA submission is targeted for the second half of 2025. We believe our clinical datasets supporting nomlabofusp's differentiated mechanism of action, coupled with our strengthened balance sheet and expected runway into 2026, strongly position us for execution of our upcoming pivotal milestones designed to help address the urgent unmet needs of the FA community."

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 up to 4-weeks, had a predictable pharmacokinetic profile, and led to dose-dependent increases in frataxin in skin and buccal cells after daily dosing of 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 found in healthy volunteers, with 3 patients achieving levels greater than 50% of the average healthy volunteer level.

- In February 2024, Larimar announced that it 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. Discussions to support an accelerated approval are ongoing with a BLA submission targeted for 2H 2025.

- In February 2024, Larimar raised net proceeds of approximately $161.6 million through a public offering of common stock.

- In March 2024, the first patient in the OLE study evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver was dosed. Participants who completed treatment in the Phase 2 dose exploration trial, 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. Clinical assessments collected during the trial will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS) database. Interim data is expected in Q4 2024. To potentially escalate dose in the OLE study, data from the 50 mg cohort of the Phase 2 study, as well as available data from the 25 mg dose in the OLE study will be submitted for FDA review due to continued partial clinical hold.

Fourth Quarter and Full Year 2023 Financial Results

As of December 31, 2023, the Company had cash, cash equivalents and marketable securities totaling $86.8 million. As noted above, in February 2024, we raised approximately $161.6 million in net proceeds through a public offering of common stock.

The Company reported a net loss for the fourth quarter of 2023 of $13.0 million, or $0.30 per share, compared to a net loss of $9.4 million, or $0.21 per share, for the fourth quarter of 2022.

Research and development expenses for the fourth quarter of 2023 were $10.6 million compared to $7.2 million for the fourth quarter of 2022. The increase in research and development expenses compared to the prior year period was primarily driven by an increase of $1.1 million in drug manufacturing costs, an increase of $0.8 million in consulting expenditures, an increase of $0.7 million in personnel expense, an increase of $0.4 million in clinical costs related to an increase in the OLE study partially offset by a decrease in clinical costs associated with the Phase 2 nomlabofusp trial discussed above.

General and administrative expenses for the fourth quarter of 2023 were $3.5 million compared to $3.2 million for the fourth quarter of 2022. The increase in general and administrative expense was primarily driven by an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2023, an increase of $0.1 million in personnel expense and an increase of $0.1 million in operational costs primarily related to technology, partially offset by a decrease of $0.2 million in legal fees.

For the full year 2023, the Company reported a net loss of $36.9 million, or $0.84 per share, compared to a net loss of $35.4 million, or $1.37 per share for the same period in 2022.

Research and development expenses for the full year 2023 were $27.7 million compared to $24.3 million for the same period in 2022. The increase in research and development expenses compared to the prior year period was primarily driven by an increase of $1.9 million in personnel expense, an increase of $1.3 million in clinical expense, an increase of $1.1 million in consulting expenditures, an increase of $0.5 million in nonclinical development costs, an increase of $0.5 million in internal lab costs, and an increase of $0.3 million in stock-based compensation expense associated with stock option grants made in 2022 and 2023, partially offset by a decrease of $2.0 million in drug manufacturing costs and a decrease of $0.2 million in royalty fees associated with the milestone achieved in 2022.

General and administrative expenses for the full year 2023 were $14.1 million compared to $12.3 million for the same period in 2022. The increase in general and administrative expense was primarily driven by an increase of $0.7 million in stock-based compensation expense associated with stock option grants made in 2022 and 2023, an increase of $0.5 million in personnel expense, an increase of $0.5 million in operational costs primarily related to technology and an increase of $0.4 million in professional fees primarily related to legal, accounting and consulting services, partially offset by a decrease of $0.3 million in insurance 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.
Consolidated Balance Sheet
(dollars in thousands except share and per share data)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 26,749	$ 26,825
Marketable securities	60,041	91,603
Prepaid expenses and other current assets	3,385	2,311
Total current assets	90,175	120,739
Property and equipment, net	684	831
Operating lease right-of-use assets	3,078	2,858
Restricted cash	1,339	1,339
Other assets	659	638
Total assets	$ 95,935	$ 126,405
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,283	$ 1,686
Accrued expenses	7,386	8,408
Operating lease liabilities, current	837	611
Total current liabilities	9,506	10,705
Operating lease liabilities	4,709	4,797
Total liabilities	14,215	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 December 31, 2023 and December 31, 2022; no shares issued and outstanding as of December 31, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of December 31, 2023 and December 31, 2022; 43,909,069 and 43,269,200 shares issued and outstanding as of December 31, 2023 and December 31, 2022, respectively	43	43
Additional paid-in capital	270,150	262,496
Accumulated deficit	(188,554)	(151,605)
Accumulated other comprehensive loss	81	(31)
Total stockholders' equity	81,720	110,903
Total liabilities and stockholders' equity	$ 95,935	$ 126,405

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

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 10,648	$ 7,218	$ 27,670	$ 24,250

General and administrative	3,514	3,221	14,088	12,276
Total operating expenses	14,162	10,439	41,758	36,526
Loss from operations	(14,162)	(10,439)	(41,758)	(36,526)
Other income, net	1,169	1,014	4,809	1,171
Net loss	$ (12,993)	$ (9,425)	$ (36,949)	$ (35,355)
Net loss per share, basic and diluted	$ (0.30)	$ (0.21)	$ (0.84)	$ (1.37)
Weighted average common shares outstanding, basic and diluted	43,905,903	43,897,603	43,901,241	25,761,394

Source: Larimar Therapeutics