UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 14, 2023

Larimar Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-36510**	**20-3857670**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

Three Bala Plaza East **Bala Cynwyd, Pennsylvania** **(Address of Principal Executive Offices)**	**19004** **(Zip Code)**

Registrant's Telephone Number, Including Area Code: (844) 511-9056

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, par value $0.001 per share	lrmr	Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On November 14, 2023, Larimar Therapeutics, Inc. (the "***Company***") announced its financial results and operational highlights for the third quarter ended September 30, 2023. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information furnished pursuant to this Item 2.02, including Exhibit 99.1 attached hereto, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "***Exchange Act***"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On November 14, 2023, the Company posted on its website an updated slide presentation, which is attached as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. Representatives of the Company will use the presentation in various meetings with investors, analysts and other parties from time to time.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Below is a list of exhibits included with this Current Report on Form 8-K.

Exhibit No.	Document
99.1	Press Release issued by Larimar Therapeutics, Inc. on November 14, 2023*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated November 14, 2023
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Furnished herewith

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Larimar Therapeutics, Inc.

Date: November 14, 2023

By: /s/ Carole S. Ben-Maimon, M.D.

Name: Carole S. Ben-Maimon, M.D.
Title: President and Chief Executive Officer

Larimar Therapeutics Reports Third Quarter 2023 Operating and Financial Results

- *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, November 14, 2023 – 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)

Exhibit 99.2

Larimar Therapeutics

Corporate Presentation

November 2023

Forward-Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions of Larimar Therapeutics, Inc. ("Company") and on information currently available to management. All statements contained in this presentation other than statements of historical fact are forward-looking statements, including but not limited to Larimar's ability to develop and commercialize nomlabofusp (nomlabofusp is the new Internatioional Nonproprietary Name and United States Adopted Name for CTI-1601) and other planned product candidates, Larimar's planned research and development efforts, including the timing of its CTI-1601 (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 presentation 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.

Investment Highlights

Novel protein replacement therapy platform	Clinical-stage biotechnology company focused on addressing unmet needs in Friedreich's ataxia (FA) and potentially other complex rare diseases based on a platform technology backed by a strong intellectual property portfolio
Potential first-ever therapy to increase frataxin levels	Lead candidate nomlabofusp* (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency by delivering the protein to mitochondria. Nomlabofusp has received Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations
Completed Phase 1 proof-of-concept	Two double-blind, placebo-controlled Phase 1 trials in FA demonstrating nomlabofusp was **generally well tolerated** when dosed daily for up to 13 days; **dose-dependent increases in frataxin (FXN) levels** from baseline vs. placebo were observed in all evaluated tissues
Phase 2 and OLE studies with near-term catalysts	Ongoing Phase 2, placebo-controlled, 4-week dose exploration study in FA; 25 mg cohort data show nomlabofuspis generally well tolerated, increasing FXN levels from baseline vs. placebo in skin and buccal cells; **top-line data for 50 mg and 25 mg cohorts expected in Q1 2024; OLE trial with 25 mg daily dosing cleared for initiation in Q1 2024.** To potentially further escalate dose in Phase 2 study or the OLE study, Phase 2 data from 50 mg cohort will be submitted to FDA due to continued partial clinical hold.
Strong financial foundation	$95.6 Million cash balance (September 30, 2023) with projected cash runway into Q1 2025

Larimar Therapeutics

*As of October 2023, nomlabofusp was published as the INN (International Proprietary Name) and USAN (United States Adopted Name) for CTI-1601

Phase 2 Dose Exploration Trial Advances and Initiation of Long-Term Open Label Extension Trial Remains on Track

Phase 2 dose exploration trial evaluating 25 mg and 50 mg dosing cohorts

Completed full enrollment and dosing of the 50 mg cohort (15 trial participants)*

Based on blinded observations during the dosing period, there were no reported serious adverse events in the nomlabofusp (CTI-1601) or placebo groups

Safety, PK and frataxin levels will inform on potential for additional cohorts

Top-line data expected in Q1 2024

Open label extension (OLE) trial with 25 mg daily dosing to be initiated in Q1 2024

Assesses long-term safety, PK, frataxin levels, efficacy outcomes and self-administration

Enables the comparison of clinical efficacy measures with a matched set of untreated patients from the Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS) database

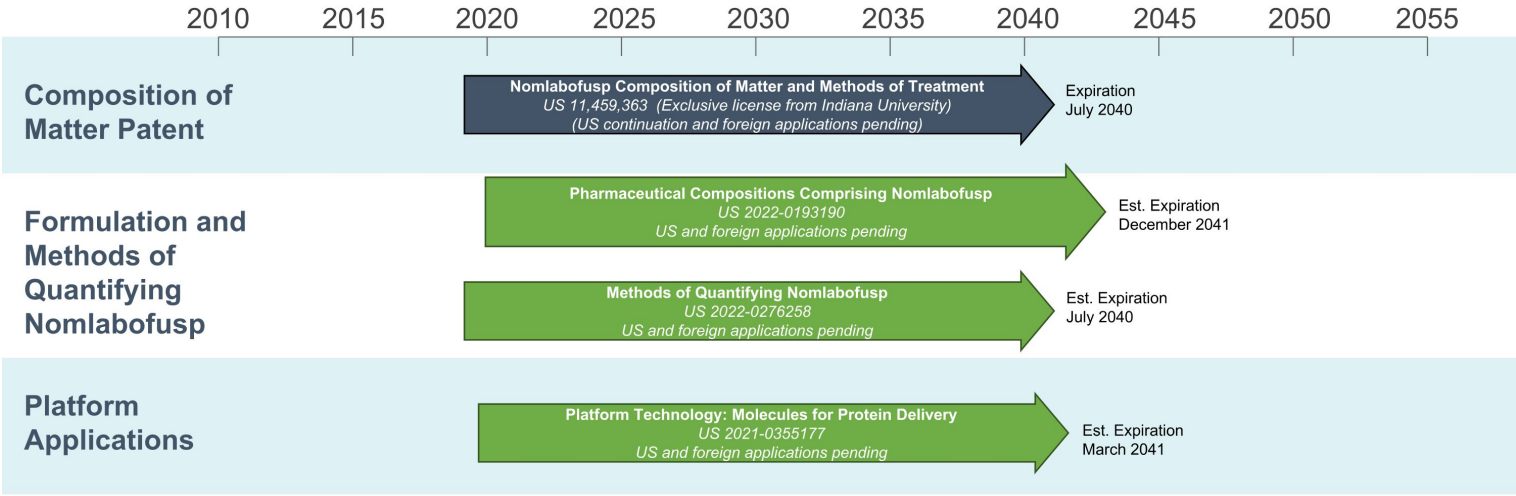
Initial interim data expected in Q4 2024

Larimar Therapeutics

*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 partial clinical hold

Larimar Technology is Supported by a Strong IP Portfolio

Granted nomlabofusp (CTI-1601) composition of matter patent extends into 2040



Additional nomlabofusp IP protection

- US and foreign pending applications cover key biomarkers, analytical tools and methods of treatment for additional disease indications for nomlabofusp
- Nomlabofusp should be eligible for **12 years of market exclusivity** upon approval in the US (independent of patents) and at least **10 years of market exclusivity** upon approval in EU (independent of patents)

Granted Pending

Friedreich's Ataxia (FA)

Rare and Progressive Disease

Caused by genetic defect resulting in low levels of frataxin

- Patients with FA only produce ~20-40% of normal frataxin levels depending on the tissue, sampling technique, and assay considered[1]
- Affects ~20,000 patients globally, with ~5,000 patients in the U.S. and majority of the remaining patients in the EU

Approximately 70% of patients present before age 14

- Initial symptoms may include unsteady posture, frequent falling and progressive difficulty in walking. By the time symptoms occur, heart damage may have already occurred. Progressive disease: symptoms worsen and patients are eventually confined to a wheelchair with speech becoming hesitant and jerky (often referred to as "scanning speech")

Life expectancy of 30-50 years

- Early death usually caused by heart disease

No available therapies increase frataxin levels

- Only treatment approved for FA does not address frataxin deficiency

LRMR continues to have a strong relationship with Friedreich's Ataxia Research Alliance

- Dedicated FA patient advocacy group focused on treatments for FA

Larimar Therapeutics

1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245

FXN Levels Determine Disease Progression in FA

Lower FXN levels are associated with earlier onset of disease, faster rate of disease progression, and shorter time to loss of ambulation

Median Age of Onset and Rate of Disease Progression in Relation to FXN Levels

Age of Onset (Years)	**FARS*** (Change/Year)	**FXN Level**** (% of Normal Level)
7	2.9	11.2
11	2.1	22.0
16	2.0	31.0
19	1.6	48.7

Adapted from H.L.Plasterer et al. PLoS ONE 2013 8(5):e63958

Median Age of Onset Predicts Time to Loss of Ambulation

Age of Onset (Years)	**Median Time to Loss of Ambulation** (Years)
< 15	11.5
15 to 24	18.3
> 24	23.5

Adapted from C. Rummey et al. EClinicalMedicine. 2020 18:100213

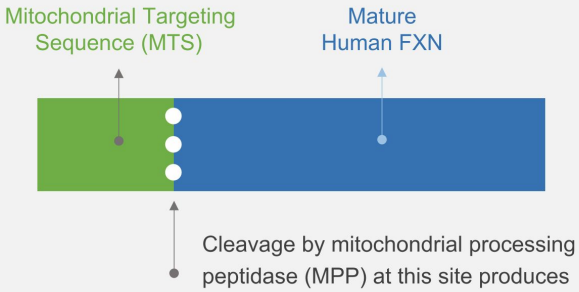
Larimar Therapeutics

*FARS: Friedreich's ataxia rating scale, measures disease progression with a higher score indicating a greater level of disability; **FXN levels measured in peripheral blood mononuclear cells (PBMCs). FXN levels demonstrated to be equivalent in PBMCs, buccal cells, and whole blood.

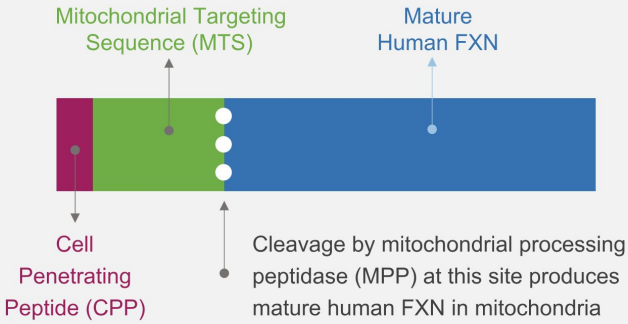
Nomlabofusp is Designed to Deliver Additional Frataxin

Nomlabofusp (CTI-1601) maintains the cleavage site between the MTS and mature human frataxin (FXN)

STRUCTURE OF ENDOGENOUS FXN



STRUCTURE OF NOMLABOFUSP



The presence of the cleavage site allows the CPP and MTS to be removed by mitochondrial processing peptidase to produce mature human FXN in the mitochondria

Larimar Therapeutics

Completed Phase 1 Multiple Ascending Dose Study

Treatment Schedules for Each Cohort- nomlabofusp (CTI-1601) or placebo

Cohort 1 (25 mg; n = 8)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of nomlabofusp or placebo

= No Administration

Cohort 2 (50 mg; n = 9)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of nomlabofusp or placebo

= No Administration

Cohort 3 (100 mg n = 10)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of nomlabofusp or placebo

= No Administration

FXN Level Sampling Days Presented for Each Cohort

Cohort 1 Sampling Days	
Buccal Cells	Baseline, Day 4, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 4, Day 13

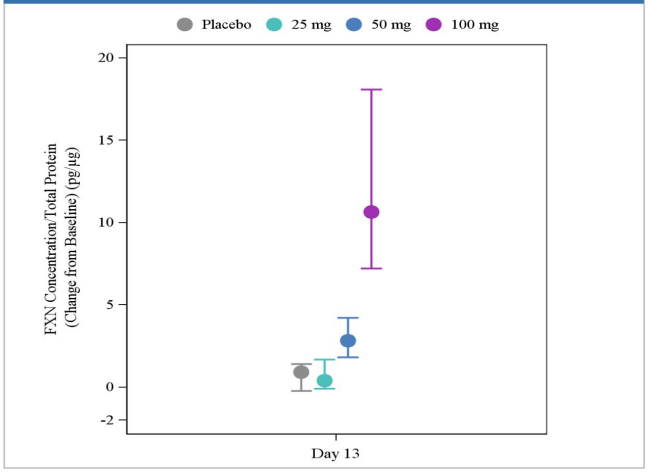
Cohort 2 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Cohort 3 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

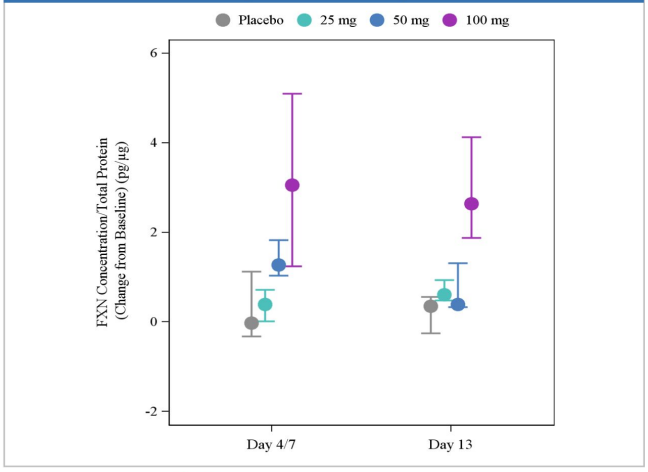
Larimar Therapeutics

Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

FXN* Change from Baseline By Dose Group (Skin Cells)



FXN* Change from Baseline By Dose Group (Buccal Cells)



Placebo: Participants randomized to placebo in each cohort
25 mg: Dosed daily for 4 days, every third day thereafter
50 mg: Dosed daily for 7 days, every other day thereafter
100 mg: Dosed daily for 13 days

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*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from Day 7 & Day 13 measurements are shown for data derived from the 50 & 100 mg cohorts; Sample collection days varied in each cohort per the trial protocol

Phase 2 Dose Exploration Study for 25 and 50 mg Cohorts

Goal: Further characterize PK/PD and assess safety to inform long-term dose and dose regimen

Treatment Schedule- nomlabofusp (CTI-1601) or placebo

28-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28

= Administration of nomlabofusp (CTI-1601) or placebo

= No Administration

Study Details

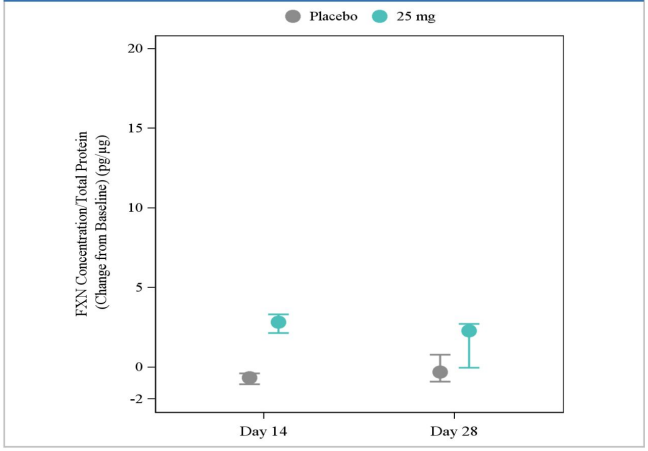
Population	Ambulatory and non-ambulatory Friedreich's ataxia patients ≥18 years of age. Nomlabofusp (CTI-1601) treatment naïve or participated (if eligible) in a previous Larimar study.
Dose	**Cohort 1:** 25 mg **Cohort 2:** 50 mg
Key Endpoints	Frataxin levels in peripheral tissue, PK, safety and tolerability. Other endpoints include lipid profiles and gene expression data.
Number of Patients	**Cohort 1:** Enrolled 13 participants (9 on nomlabofusp; 4 on placebo). **Cohort 2:** Enrolled 15 participants (randomized 2:1 to receive nomlabofusp or placebo.
Timing	Dosing in Cohort 1 is complete. Cohort 2 enrollment dosing complete; Participants remain blinded as they complete follow up period. Top-line data from Cohort 2 expected in Q1 2024.

Increases in FXN Levels in Phase 2 Trial's 25 mg Cohort

Participants dosed daily for 14 days, then every other day until day 28

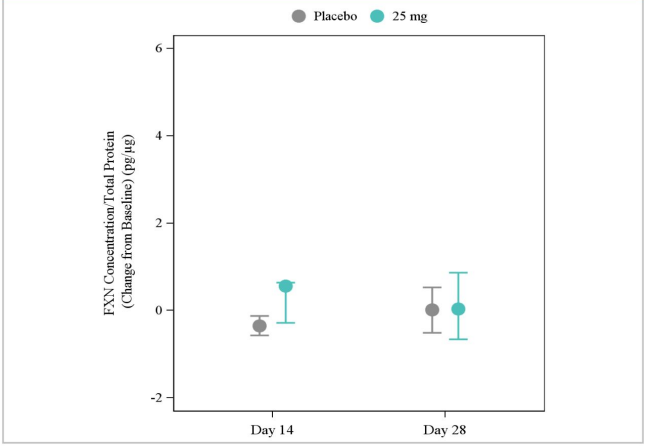
Skin Cells FXN* Change from Baseline**

Median placebo-adjusted increase from baseline of 3.5 pg/µg with 14 days daily dosing



Buccal Cells FXN* Change from Baseline**

Median placebo-adjusted increase from baseline of 0.9 pg/µg with 14 days daily dosing



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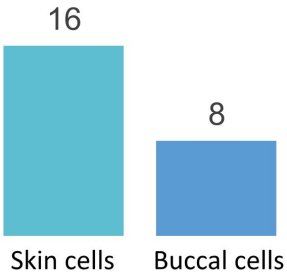
*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles.

** Median baseline from all of the patients were approximately 3.7 pg/µg for skin cells, and 1.9 pg/µg for buccal cells

CLIN-1601-002: Top-line Non-interventional Study Results

Recently completed non-interventional study measured FXN in homozygous healthy volunteers

Median Frataxin Concentration (pg/µg)
in Homozygous Healthy Volunteers (n = 60)



Patients with FA only produce ~20-40%[1] of normal frataxin levels depending on the tissue, sampling technique, and assay considered

Lower FXN levels seen with typical onset[2] (5 to 15 years of age)

Higher FXN levels seen with late onset[2] (after 25 years of age)

FXN concentrations were measured in skin and buccal cells from 60 homozygous healthy volunteers utilizing the same sampling technique and assay as clinical trials of nomlabofusp; FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations normalized to total cellular protein content in each sample. 1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245. 2. Friedreich's Ataxia Research Alliance

Ph1 & Ph2 Data: Nomlabofusp is Generally Well Tolerated*

Nomlabofusp (CTI-1601) administered to 37 different adults with FA in multiple studies

35 of 37 clinical trial participants dosed with nomlabofusp completed their respective study
One Phase 2 participant withdrew due to an allergic reaction that resolved with standard treatment
One Phase 1 participant in the 50 mg cohort withdrew due to mild-to-moderate nausea and vomiting

No serious adverse events or important medical events in any nomlabofusp clinical trial
One severe adverse event (allergic reaction that resolved with standard treatment referenced above)

Most common adverse events (AEs) were mild and moderate injection site reactions (ISRs)
No study discontinuations due to ISRs and all resolved
ISRs in 100% of nomlabofusp-treated participants and 40% of placebo-treated participants across all trials

In MAD study, except for ISRs, number & severity of AEs did not increase with increasing dose

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*Based on Phase 1 study data and Phase 2 study 25 mg cohort data. Data excludes Phase 2 study 50 mg cohort data; data remains blinded as participants are completing the follow up period. Phase 2 study 50 mg cohort consisted of 15 participants and based on blinded observations during the dosing period, there were no serious adverse events in the CTI-1601 or placebo groups. Top-line data is expected in Q1 2024.

Open-label Extension Trial: Initiation Expected in Q1 2024

Preliminary interim data expected in Q4 2024

Key Eligibility Criteria

Previous participation in Phase 1 or Phase 2 trials

Subcutaneous Injections of 25 mg nomlabofusp (CTI-1601)

Dosing: 25 mg daily with subcutaneous injections self-administered or administered by a caregiver

Matched Set of Untreated Patients from FACOMS Database

Screening Period ≤ 42 days

Treatment Period: Planned for ≥ 1 year with any necessary extensions

Safety and Pharmacodynamic Objectives

- Evaluate safety and tolerability
- Evaluate long-term PK
- Evaluate tissue frataxin concentrations
- Evaluate lipid profiles and gene expression data

Other Objectives

- Assess clinical efficacy measures

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FACOMS: Friedreich's Ataxia Clinical Outcome Measures Study

Nomlabofusp (CTI-1601) Clinical Development Plan

Top-line data from 50 mg and 25 mg cohorts of Phase 2 dose exploration trial expected in Q1 2024

Ongoing and Planned Trials* Include:

Phase 2, four-week dose exploration study intended to identify dose and dose regimen for long-term studies.
50 mg cohort enrollment and dosing recently competed.
Blinded follow-up on going.

Open-label extension trial with 25 mg daily dosing for eligible patients who participated in SAD, MAD, and/or four-week dose exploration studies.
Initiation expected Q1 2024.

MAD trial in patients 2 to 17 years of age**. Participants eligible to screen for OLE trial.

Global double-blind placebo-controlled pivotal trial***

*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 partial clinical hold.
**Company is planning discussions with FDA on how to best include patients 2 to 17 years of age in clinical development
***Company plans to initiate discussions with FDA on appropriate pivotal trial endpoints, including value of FXN levels. Also, the Company is planning discussions with regulators & investigators outside the U.S. to expand clinical program to international geographies.

Nomlabofusp is a Competitively Differentiated Treatment Approach*

$7.3B Pending acquisition supports the **robust market potential** for FA treatments

REATA PHARMACEUTICALS Biogen

Nomlabofusp is a potential **first-and-only protein replacement therapy** designed to address the underlying cause of FA

Approach	Product	Company	Mechanism of Action	Clinical Status
Protein replacement	**Nomlabofusp (CTI-1601)**	**Larimar**	**Recombinant frataxin protein**	**Phase II**
Mitochondrial Oxidative Stress Modifier	Omaveloxolone (SKYCLARYS™)	Reata Pharma/Biogen	Nrf2 Activator	Approved
	Vatiquinone	PTC Therapeutics	15-Lipoxygenase Inhibitor	Phase III
	Epicatechin	Epirium Bio	Acetylcholinesterase Agonist	Phase II
	MIB-626	MetroBiotech	NAD+ Precursor	Phase II
Gene Expression Regulator	Resveratrol	Jupiter Neurosciences	P450 Enzyme Inhibitor	Phase II
	Etravirine	Frategene	Frataxin Pathway Modifier	Phase II
	DT-216	Design Therapeutics	GeneTAC	Phase I
Pathway Modifier	Leriglitazone	Minoryx	PPAR Gamma Agonist	Phase II
	Dimethyl Fumarate	Ixchel Pharma	Fumaric Acid Derivative	Phase I
Gene Therapy	LX2006	Lexeo Therapeutics	Frataxin Gene Replacement	Phase I/II

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*Competitive landscape focuses on clinical-stage, industry-sponsored programs

Top-line Phase 2 50 mg & 25 mg Cohort Data & OLE Initiation in Q1 2024

Nomlabofusp (CTI-1601)	Generally well tolerated in Phase 1 and Phase 2 cohort 1 Dose-dependent increases in FXN levels in all evaluated tissues in Phase 1 Increases in FXN from baseline compared to placebo in all evaluated tissues (skin and buccal cells) in Phase 2 cohort 1
Regulatory Updates	Phase 2 dose exploration trial of nomlabofusp (CTI-1601) in FA cleared to proceed to 50 mg cohort Open label extension trial with 25 mg daily dosing cleared for initiation Beginning preparations to expand nomlabofusp (CTI-1601) clinical program to ex-U.S. geographies
Expected Milestones	**Q4 2023**: Completed full enrollment (15 participants) and dosing of the 50 mg cohort in Phase 2 dose exploration trial **Q1 2024:** Top-line Phase 2 data from 50 mg and 25 mg cohorts **Q1 2024:** Initiation of open-label extension (OLE) trial **Q4 2024:** Interim data from OLE trial

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Investment Highlights

Novel protein replacement therapy platform	Clinical-stage biotechnology company focused on addressing unmet needs in Friedreich's ataxia (FA) and potentially other complex rare diseases based on a platform technology backed by a strong intellectual property portfolio
Potential first-ever therapy to increase frataxin levels	Lead candidate nomlabofusp* (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency by delivering the protein to mitochondria. Nomlabofusp has received Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations
Completed Phase 1 proof-of-concept	Two double-blind, placebo-controlled Phase 1 trials in FA demonstrating nomlabofusp was **generally well tolerated** when dosed daily for up to 13 days; **dose-dependent increases in frataxin (FXN) levels** from baseline vs. placebo were observed in all evaluated tissues
Phase 2 and OLE studies with near-term catalysts	Ongoing Phase 2, placebo-controlled, 4-week dose exploration study in FA; 25 mg cohort data show nomlabofuspis generally well tolerated, increasing FXN levels from baseline vs. placebo in skin and buccal cells; **top-line data for 50 mg and 25 mg cohorts expected in Q1 2024; OLE trial with 25 mg daily dosing cleared for initiation in Q1 2024.** To potentially further escalate dose in Phase 2 study or the OLE study, Phase 2 data from 50 mg cohort will be submitted to FDA due to continued partial clinical hold.
Strong financial foundation	$95.6 Million cash balance (September 30, 2023) with projected cash runway into Q1 2025

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*As of October 2023, nomlabofusp was published as the INN (International Proprietary Name) and USAN (United Staes Adopted Name) for CTI-1601


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November 2023 Corporate Update
THANK YOU

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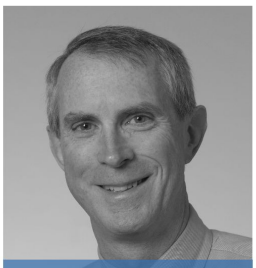
Appendix

Scientific Advisory Board

Giovanni Manfredi, MD, PhD

Finbar and Marianne Kenny Professor in Clinical and Research Neurology at Weill Cornell Medicine.

Professor of Neuroscience at Weill Cornell Medicine.



Mark Payne, MD

Co-founder of Chondrial Therapeutics, which became Larimar Therapeutics, Inc.

Professor of Pediatrics at Indiana University School of Medicine

Marni J. Falk, MD

Executive Director of the Mitochondrial Medicine Frontier Program at The Children's Hospital of Philadelphia (CHOP)

Professor in the Division of Human Genetics, Department of Pediatrics at University of Pennsylvania Perelman School of Medicine

Jill Ostrem, MD

Medical Director and Division Chief of the University of California San Francisco (UCSF) Movement Disorders and Neuromodulation Center.

Carlin and Ellen Wiegner Endowed Professor of Neurology

Strong Relationship with FARA

Company has strong relationship with Friedreich's Ataxia Research Alliance (FARA)

- National, non-profit organization dedicated to the pursuit of scientific research leading to treatments and a cure for FA

FARA provides industry with several key items

- Assistance with patient recruitment and education
- Access to Global Patient Registry with demographic and clinical information on more than 1,000 FA patients
- Sponsored a Patient-Focused Drug Development Meeting in 2017 resulting in a publication titled "The Voice of the Patient"

FARA Friedreich's Ataxia Research Alliance

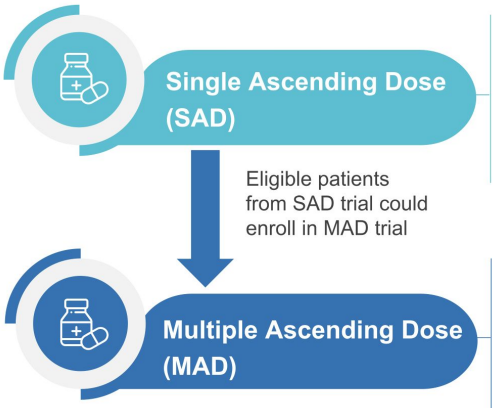
Additional Phase 1 Data

CTI-1601: Phase 1 Clinical Program in Patients with FA

Program consisted of double-blind, placebo controlled single- and multiple-ascending dose trials

Phase 1 Development Plan

- Two double-blind, placebo-controlled dosing trials in patients with FA
- Patient dosing began December 2019
- Safety Review Committee assessed all blinded data between each cohort to ensure patient safety



Number of subjects: 28
Dose levels: 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)
Treatment Duration: 1 day
1º Endpoint: Safety and tolerability
2º Endpoints: PK; PD; FXN levels; multiple exploratory
Status: Complete

Number of Subjects: 27
Dose Range: 25 mg, 50 mg, 100 mg (subcutaneous administration)
Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days
1º Endpoint: Safety and tolerability
2º Endpoints: PK; PD; FXN levels (buccal cells, platelets, optional skin biopsies); multiple exploratory
Status: Complete

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Completed Phase 1 Multiple Ascending Dose Study

Treatment Schedules for Each Cohort

Cohort 1 (25 mg; n = 8)

13-day Treatment Period

1 2 3 4 5 6 7 8 9 10 11 12 13 14

= Administration of CTI-1601 or placebo

= No Administration

Cohort 2 (50 mg; n = 9)

13-day Treatment Period

1 2 3 4 5 6 7 8 9 10 11 12 13 14

= Administration of CTI-1601 or placebo

= No Administration

Cohort 3 (100 mg n = 10)

13-day Treatment Period

1 2 3 4 5 6 7 8 9 10 11 12 13 14

= Administration of CTI-1601 or placebo

= No Administration

FXN Level Sampling Days Presented for Each Cohort

Cohort 1 Sampling Days	
Buccal Cells	Baseline, Day 4, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 4, Day 13

Cohort 2 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Cohort 3 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

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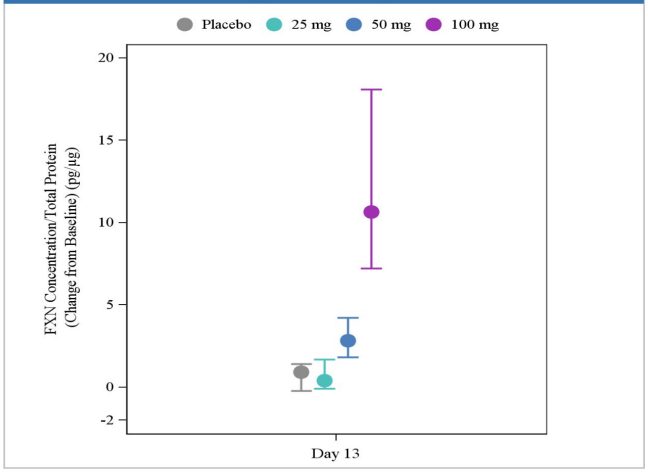
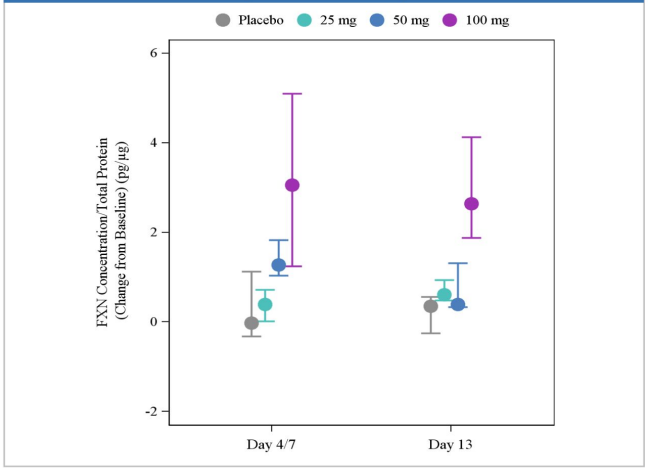
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

FXN* Change from Baseline By Dose Group (Skin Cells)



FXN* Change from Baseline By Dose Group (Buccal Cells)



Placebo: Participants randomized to placebo in each cohort
25 mg: Dosed daily for 4 days, every third day thereafter
50 mg: Dosed daily for 7 days, every other day thereafter
100 mg: Dosed daily for 13 days

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*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from baseline, Day 7 & Day 13 measurements are shown for data derived from the 50 & 100 mg cohorts; Sample collection days varied in each cohort per the trial protocol

MAD Trial Patient Demographics

Parameter	Statistic	All placebo (n=7)	25 mg CTI-1601 (n=6)	50 mg CTI-1601 (n=7)	100 mg CTI-1601 (n=7)	All CTI-1601 (n=20)	Overall (n=27)
Sex							
Male	n (%)	5 (71.4)	3 (50.0)	4 (57.1)	3 (42.9)	10 (50.0)	15 (55.6)
Female	n (%)	2 (28.6)	3 (50.0)	3 (42.9)	4 (57.1)	10 (50.0)	12 (44.4)
Age (years)							
	Mean	25.7	39.7	34.7	28.0	33.9	31.7
	SD	6.37	16.59	9.03	8.96	12.13	11.40
	Median	23	37	36	24	34	28
	Min, Max	20,36	21,65	19,47	20,44	19,65	19,65
Race							
White	n (%)	6 (85.7)	6 (100.0)	6 (85.7)	6 (85.7)	18 (90.0)	24 (88.9)
Asian	n (%)	0	0	1 (14.3)	1 (14.3)	2 (10.0)	2 (7.4)
American Indian	n (%)	1 (14.3)	0	0	0	0	1 (3.7)
Ethnicity							
Hispanic/Latino	n (%)	2 (28.6)	0	0	0	0	2 (7.4)
Not Hispanic/Latino	n (%)	5 (71.4)	6 (100.0)	7 (100.0)	7 (100.0)	20 (100.0)	25 (92.6)

SD: Standard deviation

MAD Trial Patient Disease Characteristics

Parameter	Statistic	All placebo (n=7)	25 mg CTI-1601 (n=6)	50 mg CTI-1601 (n=7)	100 mg CTI-1601 (n=7)	All CTI-1601 (n=20)	Overall (n=27)
Age at Symptom Onset							
	Mean	14.1	24.0	19.3	11.9	18.1	17.1
	SD	5.34	14.48	6.21	6.72	10.37	9.39
	Median	15.0	18.0	19.0	10.0	18.0	16.0
	Min, Max	8,23	12,44	8,28	5,22	5,44	5,44
Age at Diagnosis							
	Mean	18.3	31.5	26.4	15.9	24.3	22.7
	SD	7.87	19.88	4.28	8.21	13.24	12.23
	Median	20.0	25.5	28.0	13.0	27.0	21.0
	Min, Max	9,32	14,64	17,30	5,27	5,64	5,64
Assistive Device							
Walker	n (%)	0	2 (33.3)	3 (42.9)	0	5 (25.0)	5 (18.5)
Wheelchair	n (%)	4 (57.1)	3 (50.0)	1 (14.3)	6 (85.7)	10 (50.0)	14 (51.9)
Other	n (%)	1 (14.3)	0	1(14.3)	0	1 (5.0)	2 (7.4)
None	n (%)	2 (28.6)	1 (16.7)	2 (28.6)	1 (14.3)	4 (20.0)	6 (22.2)

SD: Standard deviation

Repeated subcutaneous injections of CTI-1601 were generally well tolerated in Phase 1 MAD trial

Summary of MAD trial safety data:

Repeated doses (25 mg, 50 mg, and 100 mg) of CTI-1601 or placebo were administered subcutaneously.

- No serious adverse events (SAEs), important medical events, or treatment-related severe adverse events were observed.
- Most common adverse events (AEs) were mild and moderate injection site reactions (ISR). At least one ISR was seen in 43% of patients receiving placebo, and all patients receiving CTI-1601 experienced ISRs.
- Most ISRs resolved within an hour after injection and there were no study discontinuations due to ISRs.
- Except for ISRs, the number & severity of AEs did not increase with increasing dose.

PK analyses support evaluating once-daily and every-other-day dosing regimens for CTI-1601

Summary of MAD Trial PK Analyses

- CTI-1601 was quickly absorbed after subcutaneous administration
- Dose-proportional increases in exposure observed with increasing doses of CTI-1601
- Mean half life of CTI-1601 in plasma was approximately 11 hours
- CTI-1601 appeared to be at or close to steady state exposure after 13 days of dosing 100 mg once daily

Additional Phase 2 Cohort 1 Data

Phase 2 Dose Exploration Study for 25 and 50 mg Cohorts

Goal: Further characterize PK/PD and assess safety to inform long-term dose and dose regimen

Treatment Schedule- nomlabofusp (CTI-1601) or placebo

28-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28

= Administration of nomlabofusp (CTI-1601) or placebo

= No Administration

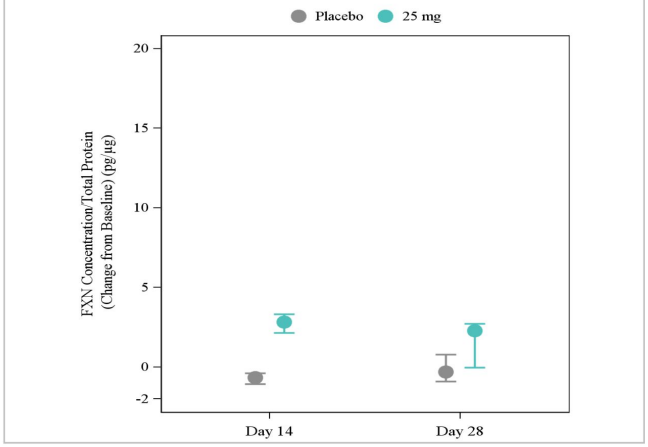
Study Details

Population	Ambulatory and non-ambulatory Friedreich's ataxia patients ≥18 years of age. Nomlabofusp (CTI-1601) treatment naïve or participated (if eligible) in a previous Larimar study.
Dose	**Cohort 1:** 25 mg **Cohort 2:** 50 mg
Key Endpoints	Frataxin levels in peripheral tissue, PK, safety and tolerability. Other endpoints include lipid profiles and gene expression data.
Number of Patients	**Cohort 1:** Enrolled 13 participants (9 on nomlabofusp; 4 on placebo). **Cohort 2:** Enrolled 15 participants (randomized 2:1 to receive nomlabofusp or placebo.
Timing	Dosing in Cohort 1 is complete. Cohort 2 enrollment dosing complete; Participants remain blinded as they complete follow up period. Top-line data from Cohort 2 expected in Q1 2024.

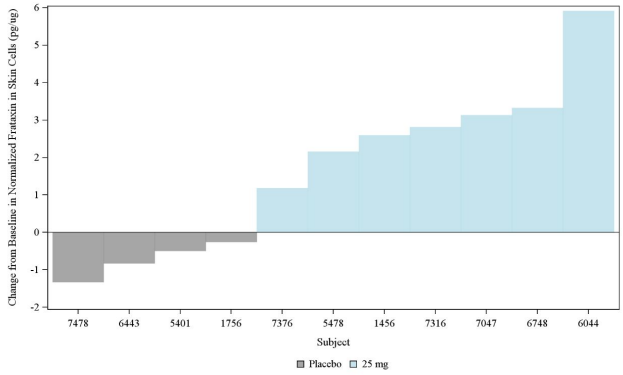
Increases in FXN Levels Observed in Skin Cells (Ph 2 Cohort 1)

Median placebo-adjusted increase from baseline of 3.5 pg/µg in skin with 14 days daily dosing

FXN* Change from Baseline (Skin Cells)



Day 14 FXN* Change from Baseline (Skin Cells)#

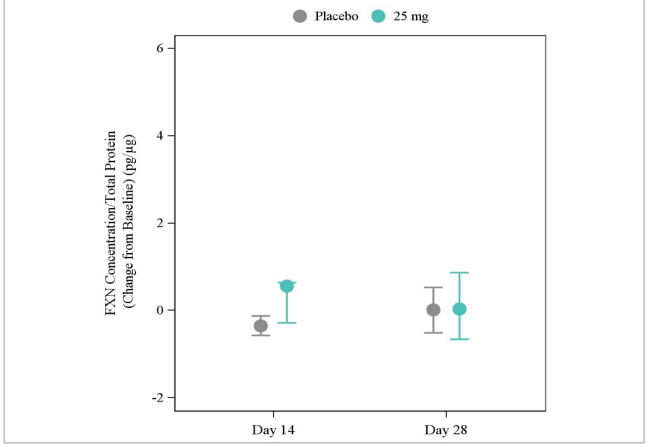


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data for left graph represent median and 25th and 75th percentiles.
#: One participant treated with CTI-1601 discontinued from study at Day 14 (Day 14 sample was not collected) and another treated with CTI-1601 had a FXN concentration value < lower limit of quantitation (LLOQ) at Day 14.

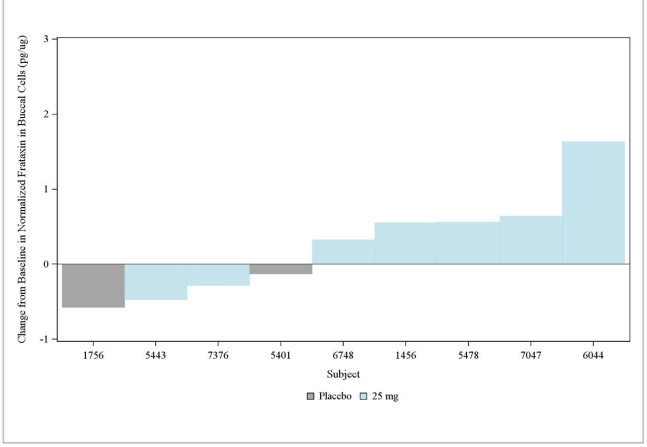
Increases in FXN Levels Observed in Buccal Cells (Ph 2 Cohort 1)

Median placebo-adjusted increase from baseline of 0.9 pg/µg in buccal cells with 14 days daily dosing

FXN* Change from Baseline (Buccal Cells)



Day 14 FXN* Change from Baseline (Buccal Cells)#



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data for left graph represent median and 25th and 75th percentiles.

#:One participant treated with CTI-1601 had a baseline value < lower limit of quantitation (LLOQ) and another participant treated with CTI-1601 had a baseline and Day 14 value < lower limit of quantitation (LLOQ); One participant treated with placebo had a FXN concentration value <LLOQ at baseline and another participant treated with placebo had a FXN concentration value <LLOQ at Day 14.

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Demographics of Phase 2 (Cohort 1)

Demographics similar between Phase 1 and Phase 2 trials of CTI-1601

Parameter, n (%)	Placebo (N=4)	CTI-1601 25 mg (N=9)	Overall (N=13)
Mean Age (SD) (Years)	34.0 (9.20)	37.8 (14.93)	36.6 (13.16)
Male	2 (50.0%)	5 (55.6%)	7 (53.8%)
White	4 (100.0%)	8 (88.9%)	12 (92.3%)
Other	0	1 (11.1%)	1 (7.7%)
Not Hispanic or Latino	3 (75.0%)	8 (88.9%)	11 (84.6%)
Hispanic or Latino	1 (25.0%)	1 (11.1%)	2 (15.4%)
Mean BMI (SD) (kg/m^2)	23.66 (3.235)	25.26 (6.262)	24.77 (5.417%)
Previously participated in a CTI-1601 trial	1 (25.0%)	4 (44.4%)	5 (38.5%)

Disease Characteristics (Phase 2 Cohort 1)

Parameter	Statistic	Placebo (N=4)	CTI-1601 (n=9)	Overall (n=13)
Age at Symptom Onset (years)				
	n	4	8	12
	Mean (SD)	14.5 (4.93)	13.0 (10.47)	13.5 (8.77)
	Median	14.5	10.0	11.0
	Q1, Q3	11, 19	8, 13	9, 15
	Min, Max	9, 20	5, 38	5, 38
Age at Diagnosis (years)				
	n	4	9	13
	Mean (SD)	17.5 (5.57)	18.6 (11.20)	18.2 (9.58)
	Median	16.5	16.0	16.0
	Q1, Q3	14, 22	14, 20	14, 20
	Min, Max	12, 25	5, 42	5, 42
Time Since Diagnosis (years)				
	n	4	9	13
	Mean (SD)	16.08 (5.965)	18.49 (11.523)	17.75 (9.938)
	Median	13.42	14.32	13.50
	Q1, Q3	12.9, 19.3	12.8, 21.6	12.8, 21.6
	Min, Max	12.5, 25.0	5.4, 45.0	5.4, 45.0

CTI-1601 appeared to be generally well tolerated in Phase 2 trial's 25 mg cohort

Summary of Phase 2 trial safety data (25 mg cohort):

25 mg CTI-1601 or placebo were administered subcutaneously daily for 14 days and then every other day until day 28. 13 participants were dosed in the trial (9 active, 4 placebo). Of the 9 CTI-1601-treated participants, 8 completed the trial with 1 withdrawing due to an allergic reaction to study drug, which resolved with standard treatment

- Data indicate CTI-1601 is generally well tolerated
- No serious adverse events. No important medical events. 1 severe adverse event (allergic reaction that resolved with standard treatment as referenced above).
- The most common adverse events were mild and moderate injection site reactions (at least one injection site reaction was seen in 50% of placebo participants and in 100% of CTI-1601 participants)

Pharmacokinetic Data

- Suggest steady state achieved by day 14

Preclinical Data

CTI-1601 Extends Survival in FXN-deficient KO Mice

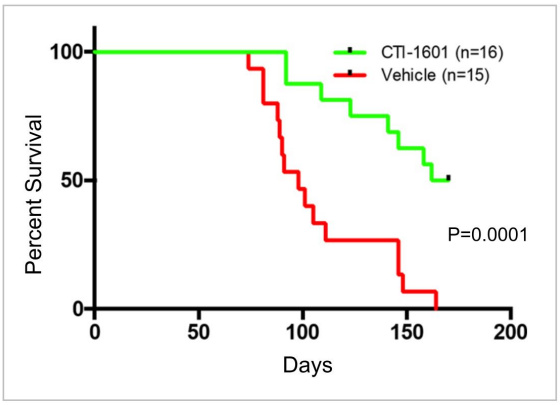
Initial Proof-of-Concept for FXN Replacement Therapy in Cardiac Mouse Model of FA

Median Survival of MCK-Cre FXN-KO Mice

- 166 days (CTI-1601) vs. 98 days (Vehicle)
- CTI-1601 was administered 10 mg/kg SC every other day

Survival beyond vehicle mean (107.5 days)

- 87.5% (CTI-1601) vs. 33% (Vehicle)
- Demonstrates that CTI-1601 is capable of delivering sufficient amounts of FXN to mitochondria



CTI-1601 rescues a severe disease phenotype in a well-characterized cardiac mouse model of FA

Larimar Therapeutics

CTI-1601 Prevents The Development of Ataxic Gait in KO mice

In-Vivo Efficacy Data in Neurologic KO Mouse Model

Pvalb-Cre FXN-KO mouse

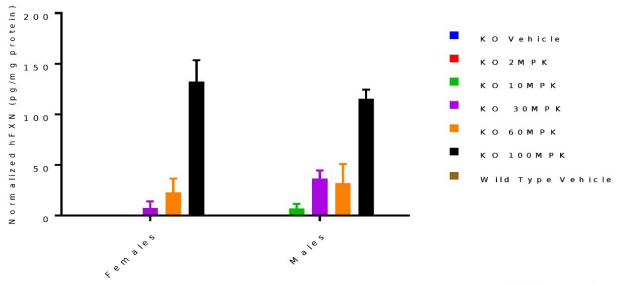
Single dose level: 10 mg/kg CTI-1601 or vehicle given intraperitoneally three times per week

- hFXN replacement with CTI-1601 **prevents the development of ataxic gait**
- CTI-1601-treated mice **survive longer** than untreated mice
- Human frataxin **present in brain, dorsal root ganglia and spinal cord** demonstrating central nervous system penetration

CTI-1601 Delivers hFXN to Mitochondria in KO Mice

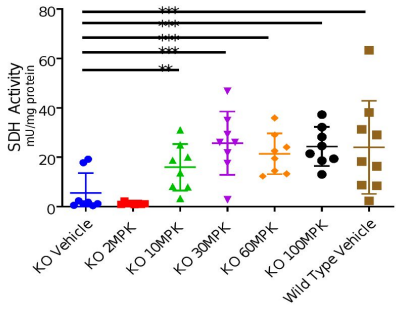
- hFXN concentration within mitochondria increases in a dose-dependent manner
- Given subcutaneously, CTI-1601 functionally replaces hFXN in mitochondria of KO mice
- Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of CTI-1601; activity plateaus at 30 mg/kg and is equivalent to activity in wild type animals
- Demonstrated normalization of gene expression in cardiac tissue

Normalized Mitochondrial FXN (Heart)



MPK = mg/kg

Normalized SDH Activity (Muscle)



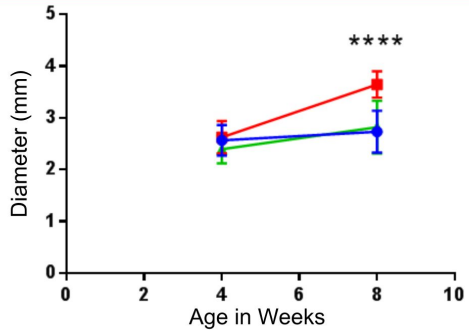
MPK = mg/kg

Larimar Therapeutics

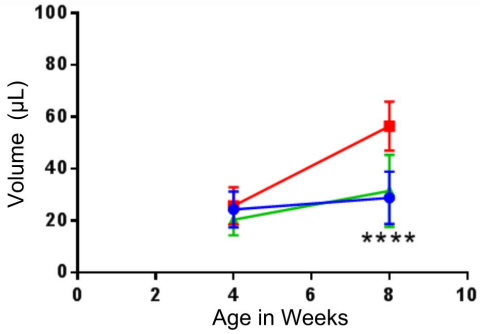
CTI-1601 Prevents Left Ventricle Dilation in KO Mice

- Left ventricular (LV) volume increases in systole in untreated mice by 8 weeks (after 4 weeks of dosing with vehicle), but remains similar to wildtype when treated with CTI-1601 (10 mg/kg every other day)
- CTI-1601-treated mice have similar LV volume as healthy controls; echocardiogram shows significant differences between vehicle and CTI-1601 treated (10 mg/kg every other day) KO mice

Left Ventricle Internal Diameter (Systole)



Left Ventricle Volume (Systole)



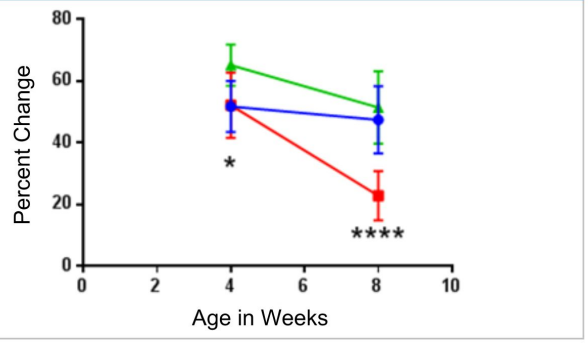
KO: CTI-1601 KO: Vehicle Wild-type: Vehicle

Larimar Therapeutics

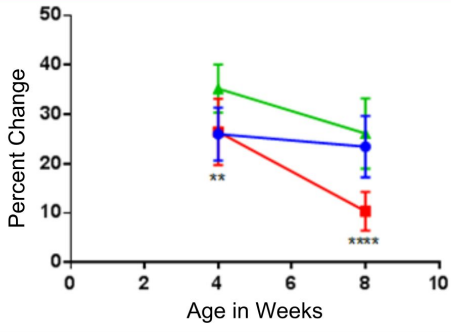
CTI-1601 Preserves Left Ventricle Function in KO Mice

- Left ventricular (LV) function drops significantly in vehicle treated mice by Week 8
- CTI-1601-treated (10 mg/kg every other day) mice have similar LV as healthy controls; echocardiogram shows significant differences between vehicle and CTI-1601 treated KO mice

Left Ventricle Ejection Function



Left Ventricle Fractional Shortening



KO: CTI-1601 KO: Vehicle Wild-type: Vehicle