

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): May 20, 2024

Larimar Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-36510
(Commission File Number)

20-3857670
(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 8.01 Other Events.**Press Release**

On May 20, 2024, Larimar Therapeutics, Inc. (the “*Company*”) issued a press release announcing that the U.S. Food and Drug Administration has removed the partial clinical hold previously placed on the Company’s nomlabofusp (CTI-1601) clinical program. A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Investor Presentation

On May 20, 2024, 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.

<u>Exhibit No.</u>	<u>Document</u>
99.1	Press Release issued by Larimar Therapeutics, Inc. on May 20, 2024*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated May 20, 2024*
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed 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: May 20, 2024

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

Larimar Therapeutics Announces FDA has Removed Partial Clinical Hold for Nomlabofusp Program in Friedreich's Ataxia

- *Food and Drug Administration (FDA) removed partial clinical hold following review of Phase 2 dose exploration study data*
- *Ongoing open label extension (OLE) study initially evaluating 25 mg; Larimar plans to dose escalate to 50 mg following further characterization of frataxin pharmacodynamics (PD) at the 25 mg dose*
- *Interim data from OLE study remains on track for Q4 2024*
- *Biologics License Application (BLA) submission targeted for 2H 2025*

Bala Cynwyd, PA, May 20, 2024 – Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced the U.S. FDA has removed the partial clinical hold previously placed on the company's nomlabofusp (CTI-1601) clinical program. Nomlabofusp is currently in development for the treatment of patients with Friedreich's Ataxia (FA). Nomlabofusp is a novel protein replacement therapy designed to address the root cause of FA by delivering frataxin to mitochondria. The FDA removed the partial clinical hold after a review of data from the Company's recently completed four-week, placebo-controlled Phase 2 dose exploration study. The review included data from both the 25 mg and 50 mg cohorts in patients who received daily dosing of nomlabofusp for 14 days followed by every other day dosing until day 28.

"We are very excited the FDA has removed the partial clinical hold on our nomlabofusp program following review of our Phase 2 data. Helping patients with FA is our top priority and we appreciate the attention and thorough review by the FDA of all submitted data," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Importantly, we are now cleared to dose escalate to the 50 mg dose in our ongoing OLE study which we plan to do following further characterization of frataxin PD at the 25 mg dose. The OLE study is evaluating the long-term safety as well as frataxin levels following daily administration of nomlabofusp and we look forward to interim data in the fourth quarter of the year."

In the Phase 2 dose exploration study, nomlabofusp was generally well-tolerated throughout the four-week treatment period. Nomlabofusp had a predictable pharmacokinetic profile and demonstrated dose-dependent increases in frataxin levels in skin and buccal cells. All patients with quantifiable levels at baseline and Day 14 in the 50 mg cohort achieved frataxin levels in skin cells over 33% of the average level observed in healthy volunteers at Day 14, and 3 patients achieved levels greater than 50% of the average healthy volunteer level.

The long-term safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues following nomlabofusp are currently being evaluated in the ongoing OLE study in patients with FA. The OLE study will initially evaluate daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. Larimar plans to dose escalate to 50 mg in the OLE study following additional characterization of frataxin PD at the 25 mg dose. Further dose escalation above 50 mg, if necessary, would require submission of additional data for FDA review to support the increased dose. Interim data from the OLE study is expected in the fourth quarter of 2024.

About Nomlabofusp (CTI-1601)

Nomlabofusp is a recombinant fusion protein intended to deliver human frataxin to the mitochondria of patients with Friedreich's ataxia who are unable to produce enough of this essential protein. Nomlabofusp has been granted Rare Pediatric Disease designation, Fast Track designation and Orphan Drug designation by the U.S. Food and Drug Administration (FDA), Orphan Drug Designation by the European Commission, and a PRIME designation by the European Medicines Agency.

About Larimar Therapeutics

Larimar Therapeutics, Inc. (Nasdaq: LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. Larimar's lead compound, 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 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

Corporate Deck

May 20 2024

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 (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; 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 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.

Clinical-Stage Novel Protein Replacement Therapy Platform

Potential first therapy to increase frataxin levels

Lead candidate nomlabofusp (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering the protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations

Consistent Phase 1 and Phase 2 findings

Nomlabofusp was generally well tolerated and demonstrated dose-dependent increases in frataxin (FXN) levels from baseline in skin and buccal cells in a completed 4-week placebo-controlled Phase 2 study and a completed multiple ascending dose Phase 1 study

Intend to pursue accelerated approval with FDA

FDA acknowledgement that FXN 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. BLA submission targeted for 2H 2025

OLE study with near-term catalysts

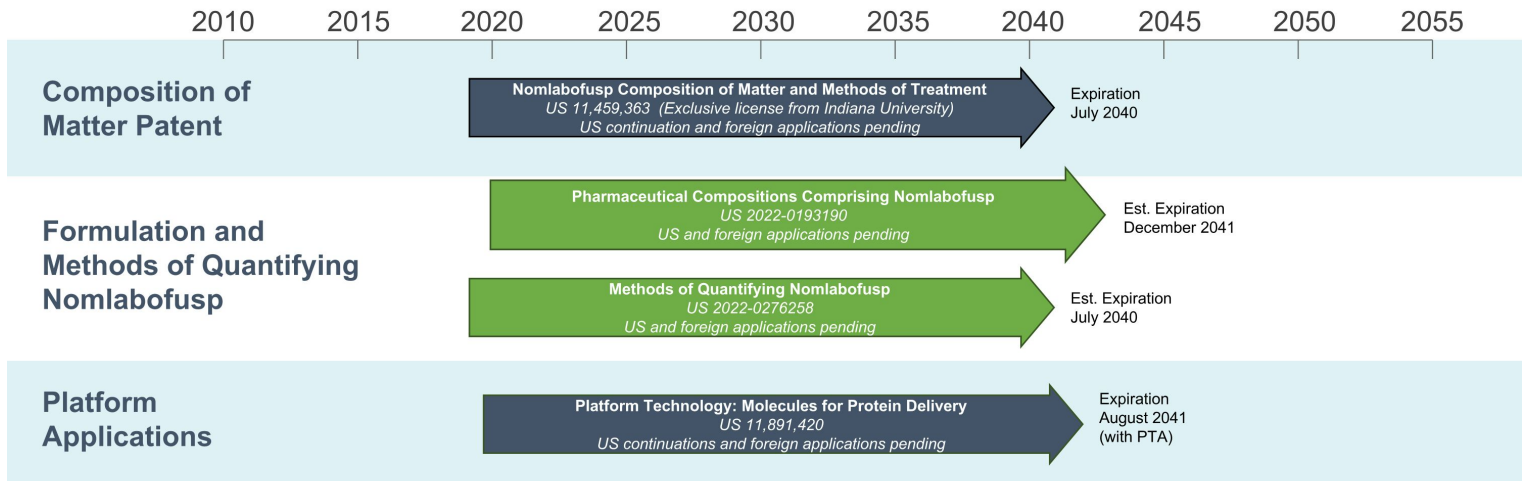
Dosed first patient in OLE study with 25 mg daily dosing in Q1 2024 with **interim data expected in Q4 2024**
Continuing to enroll patients and activate additional sites
In May 2024, the FDA removed the partial clinical hold
Dose escalation to 50 mg planned following further characterization of FXN pharmacodynamics at the 25 mg dose

Strong financial foundation

Approximately \$239 million in cash and investments at 3/31/24 which includes \$161.8 million in net proceeds raised from a Feb 24 public offering
Provides projected cash runway into 2026

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): A rare and progressive disease



Genetic defect on both alleles lowers frataxin levels

Most patients with FA only produce ~20-40% of normal frataxin levels depending on the tissue, sampling technique, and assay considered*

Affects ~20,000 patients globally

~5,000 patients in the U.S., with most remaining patients in the EU
~70% of patients present before age 14

Progressive disease

Initial symptoms include unsteady posture and frequent falling, and patients are eventually confined to a wheelchair
Life expectancy of 30-50 years with an early death usually caused by heart disease

No approved therapies increase frataxin levels

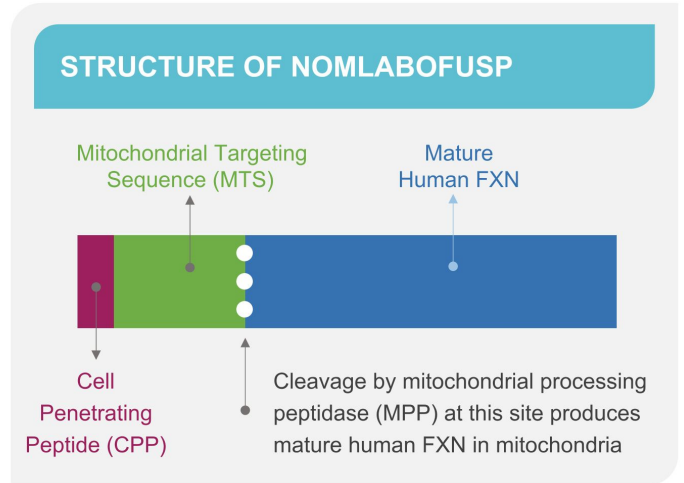
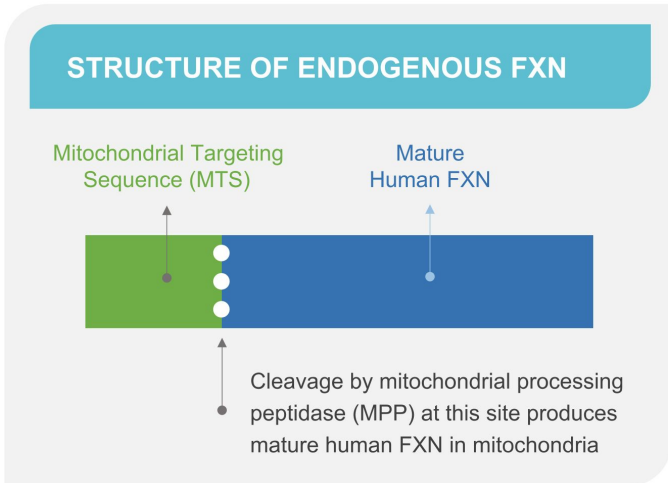
Only treatment approved for FA does not address frataxin deficiency



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

Nomlabofusp is Designed to Deliver Additional Frataxin

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



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

FXN Levels Predict 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

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

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



*FXN levels measured in peripheral blood mononuclear cells (PBMCs). FXN levels as measured by % of normal demonstrated to be equivalent in PBMCs, buccal cells, and whole blood.

**FARS: Friedreich's ataxia rating score, measures disease progression with a higher score indicating a greater level of disability.

Completed Ph 2 Dose Exploration Study (25 & 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



 = Subcutaneous administration of nomlabofusp (CTI-1601) or placebo

 = No Administration

Study Details

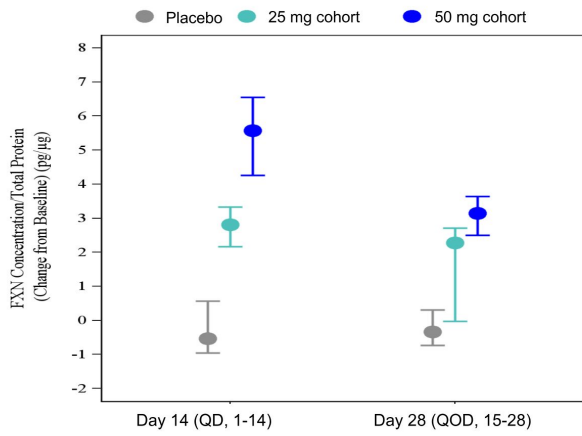
Population	Ambulatory and non-ambulatory Friedreich's ataxia patients ≥18 years of age Nomlabofusp (CTI-1601) treatment naïve or participated (if eligible) in a previous Larimar study
Dose	Cohort 1: 25 mg Cohort 2: 50 mg
Key Endpoints	Frataxin levels in peripheral tissue, PK, safety and tolerability; other exploratory endpoints include lipids and gene expression levels
Number of Patients	Cohort 1: Enrolled 13 participants (9 on nomlabofusp; 4 on placebo) Cohort 2: Enrolled 15 participants (10 on nomlabofusp; 5 on placebo)



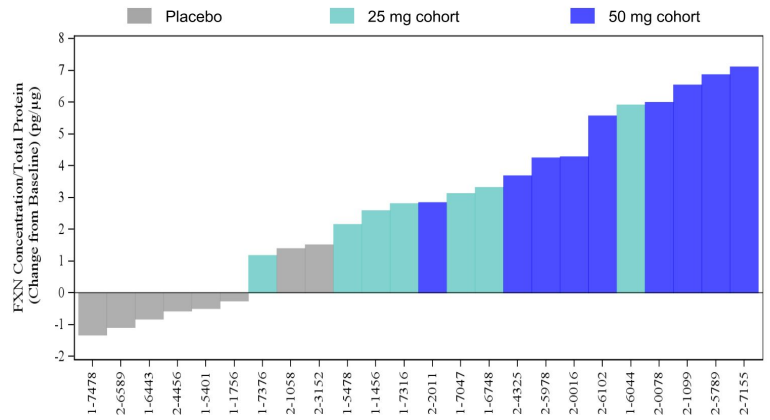
Dose-Dependent Increase in FXN Levels in Skin Cells

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

Skin Cells FXN Levels* Change from Baseline**



FXN Levels* in Skin Cells Change from Baseline at Day 14

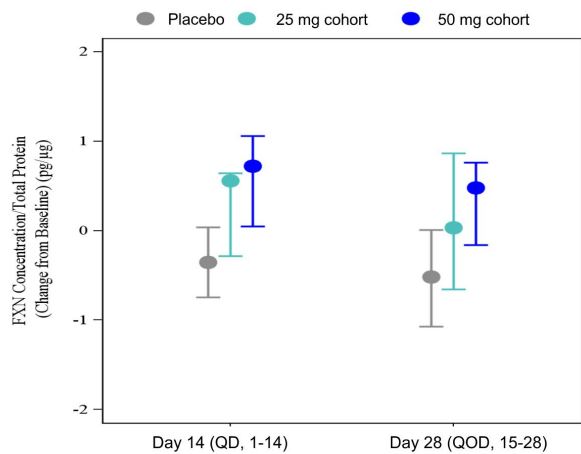


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.
 **Median baseline FXN levels in patients were 3.5 pg/μg for the placebo, 3.7 pg/μg for the 25 mg cohort and 2.1 pg/μg for the 50 mg cohort.

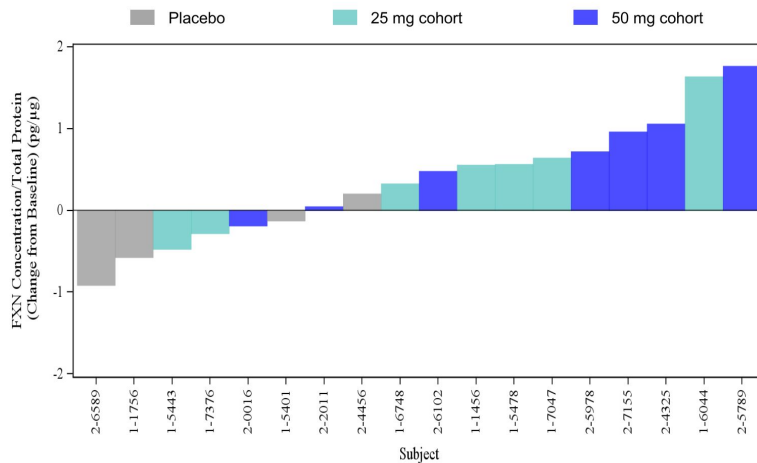
Dose-Dependent Increase in FXN Levels in Buccal Cells

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

Buccal Cells FXN Levels* Change from Baseline**



FXN Levels* in Buccal Cells Change from Baseline at Day 14



*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. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.
**Median baseline FXN level in patients were 2.1 pg/μg for the placebo, 1.8 pg/μg for the 25 mg cohort and 1.6 pg/μg for the 50 mg cohort.

Absolute Increases in Skin FXN Levels

Dose response in tissue FXN concentrations and increases from baseline after dosing

Day 14 Skin FXN Levels			
Dose	Visit	Absolute Values (pg/μg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 14	5.53	6.40
	Change from Baseline	2.81	3.02
50 mg	Baseline	2.12	2.08
	Day 14	7.40	7.32
	Change from Baseline	5.57	5.24

Day 28 Skin FXN Levels			
Dose	Visit	Absolute Values (pg/μg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 28	4.39	4.80
	Change from Baseline	2.28	1.41
50 mg	Baseline	2.12	2.08
	Day 28	5.23	5.24
	Change from Baseline	3.14	3.17



Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

Absolute Increases in Buccal FXN Levels

Dose response in tissue FXN concentrations and increases from baseline after dosing

Day 14 Buccal FXN Levels			
Dose	Visit	Absolute Values (pg/μg)	
		Median	Mean
25 mg	Baseline	1.78	1.80
	Day 14	2.24	2.22
	Change from Baseline	0.56	0.42
50 mg	Baseline	1.61	1.69
	Day 14	2.44	2.38
	Change from Baseline	0.72	0.69

Day 28 Buccal FXN Levels			
Dose	Visit	Absolute Values (pg/μg)	
		Median	Mean
25 mg	Baseline	1.70	1.65
	Day 28	1.73	1.76
	Change from Baseline	0.03	0.11
50 mg	Baseline	1.76	1.77
	Day 28	2.15	2.15
	Change from Baseline	0.48	0.38

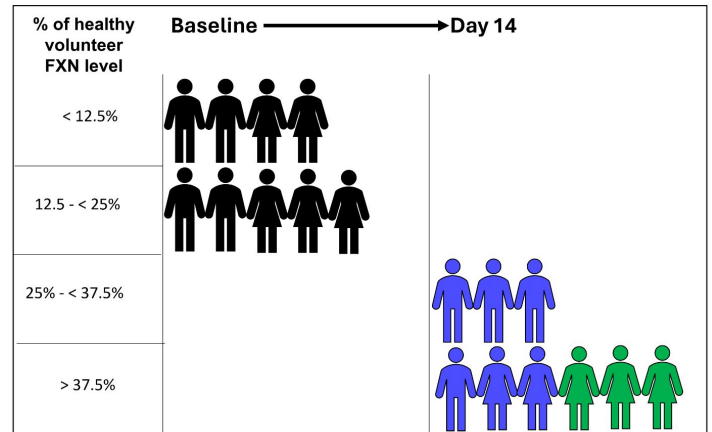
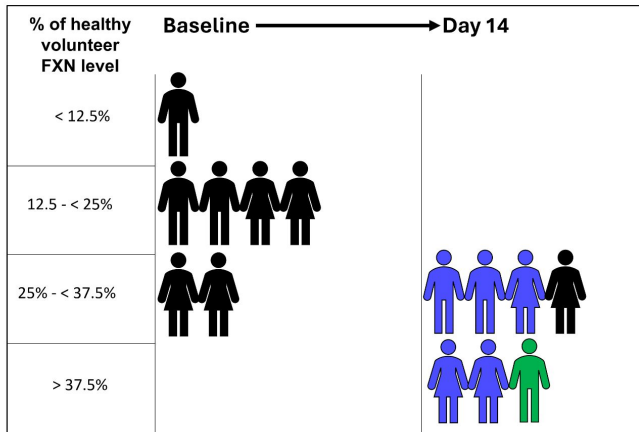


Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

Skin Cell FXN Levels Achieve Higher % of Healthy Volunteers* Following 14 days of Daily Nomlabofusp

25 mg of Nomlabofusp

50 mg of Nomlabofusp



■ Baseline FXN levels as a % of average FXN level in healthy volunteers

■ FXN levels increased from baseline and reached 25% to < 50% of average FXN level in healthy volunteers

■ FXN levels increased from baseline and reached > 50% of average FXN level in healthy volunteers

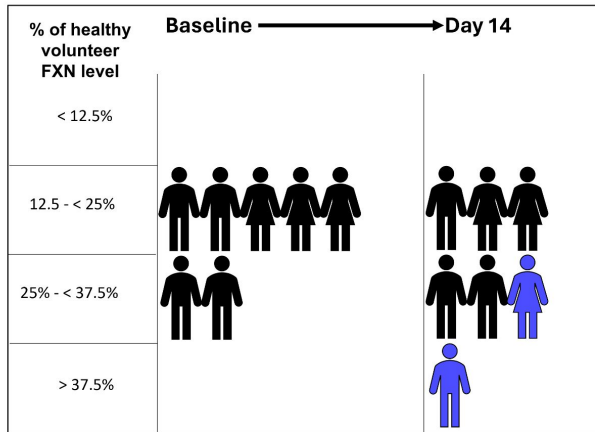


Only participants with quantifiable levels at baseline and day 14 are included in the figures.

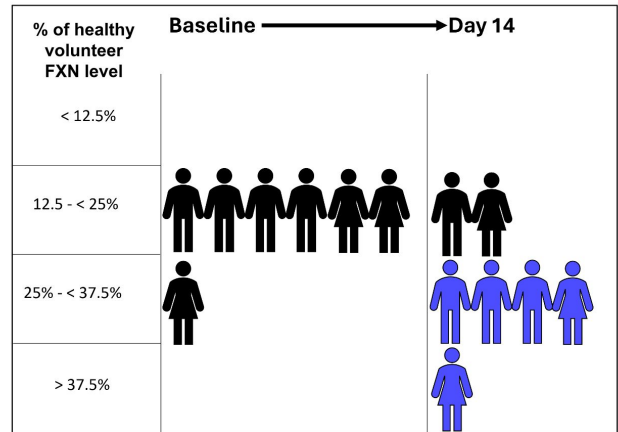
*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/μg) from the noninterventional healthy volunteer study (N=60).

Buccal Cell FXN Levels Achieve Higher % of Healthy Volunteers* Following 14 days of Daily Nomlabofusp

25 mg of Nomlabofusp



50 mg of Nomlabofusp



■ Baseline FXN levels as a % of average FXN level in healthy volunteers

■ FXN levels increased from baseline and reached 25% to < 50% of average FXN level in healthy volunteers



Only participants with quantifiable levels at baseline and day 14 are included in the figures.

*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (8.24 pg/μg) from Larimar's noninterventional healthy volunteer study (N=60).

Nomlabofusp: Predictable Pharmacokinetics

1

Quick absorption after subcutaneous administration

2

Dose-proportional increases in exposure observed

3

Pharmacokinetic profile consistent with Phase 1 studies

Ph1 & Ph2 Data: Nomlabofusp is Generally Well Tolerated



61 patients have participated in our Phase 1 and Phase 2 studies with no serious adverse events in any nomlabofusp clinical study. One severe adverse event occurred, an allergic reaction that resolved with standard treatment referenced below.



44 of 46 clinical trial participants dosed with nomlabofusp completed their respective study

One Phase 2 participant in the 25 mg cohort withdrew due to 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



Most common adverse events (AEs) were mild and moderate injection site reactions (ISRs)

No study discontinuations due to ISRs and all resolved

Open-label Extension Study: Dosed first patient in Q1 2024

Preliminary interim data expected in Q4 2024

Key Eligibility Criteria

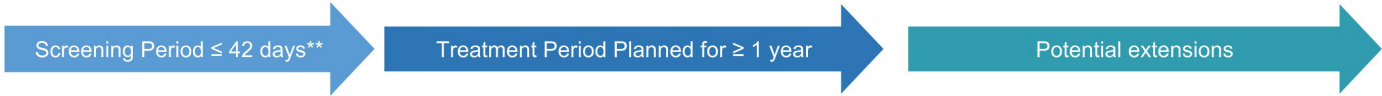
Previous participation in Phase 1 or Phase 2 trials

Daily subcutaneous injection of 25 mg nomlabofusp; self-administered or by a caregiver

- First site initiated
- First patient dosed in March 2024
- Continuing to enroll patients and activate additional sites

Key Study Objectives

- Safety and tolerability
- Long-term PK
- Dose escalation to 50 mg planned following further characterization of FXN pharmacodynamics at 25 mg dose
- Tissue FXN concentrations and potential use as surrogate endpoint to support accelerated approval
- Clinical efficacy measures compared to the matched set of untreated patients from FACOMS* database



*FACOMS: Friedreich's Ataxia Clinical Outcome Measures Study.

**Estimated screening period may be extended for those study participants who have not been on a stable regimen of omaveloxolone for at least six months.

Nomlabofusp Clinical Development Plan

In May 2024, the FDA removed the partial clinical hold

Intend to pursue accelerated approval pathway with potential BLA submission targeted for 2H 2025



Ongoing open-label extension study with 25 mg daily dosing for eligible patients who participated in SAD, MAD, and/or four-week dose exploration studies

Interim data expected Q4 2024



Plan to include pediatric patients 2 to 17 years of age in clinical development*

Participants eligible to participate in long term studies



Planned global double-blind placebo-controlled registration/confirmatory study**

BLA submission targeted for 2H 2025



*Company is discussing with FDA as to what additional clinical trial data in adults would inform inclusion of pediatric patients ages 2 to 17 in our studies.
**Company initiated discussions with FDA on the potential use of FXN levels to support accelerated approval. Also, the Company is planning discussions with regulators and investigators outside the U.S. to expand clinical program to international geographies.
Larimar plans to dose escalate to 50 mg following further characterization of FXN pharmacodynamics at the 25 mg dose.

Nomlabofusp is a Competitively Differentiated Treatment Approach*

\$7.3B

Acquisition supports the **robust market potential** for FA treatments



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 (US and EU)
	Vatiquinone	PTC Therapeutics	15-Lipoxygenase Inhibitor	Phase III
Gene Expression Regulator	DT-216P2 (new formulation)	Design Therapeutics	GeneTAC	Pre-clinical
Gene Therapy	LX2006	Lexeo Therapeutics	Frataxin Gene Replacement	Phase I/II



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

Positive Topline 50 mg & 25 mg Ph 2 Data and Dosed First Patient in OLE

Consistent Ph 1 and Ph 2 Findings

Nomlabofusp is generally well tolerated at doses tested up to 4 weeks
Dose-dependent increases in FXN levels from baseline in evaluated tissues (skin and buccal cells)
Baseline FXN levels in skin cells in the 50 mg cohort were < 17% of the average of healthy volunteers. After daily dosing for 14 days, FXN levels increased to 33% to 59%

Regulatory Updates

Initiated discussions with FDA regarding use of FXN as a surrogate endpoint to support accelerated approval
In May 2024, the FDA removed the partial clinical hold
Intend to pursue accelerated approval with potential BLA submission for 2H 2025
Beginning preparations to expand nomlabofusp clinical program to ex-U.S. geographies

2024/2025 Milestones

Q1 2024: Dosed first patient in OLE study
Q4 2024: Interim data from OLE study
2H 2024: Final Phase 2 data planned to be presented at a conference
2H 2025: BLA submission

Clinical-Stage Novel Protein Replacement Therapy Platform

Potential first therapy to increase frataxin levels

Lead candidate nomlabofusp (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering the protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations

Consistent Phase 1 and Phase 2 findings

Nomlabofusp was generally well tolerated and demonstrated dose-dependent increases in frataxin (FXN) levels from baseline in skin and buccal cells in a completed 4-week placebo-controlled Phase 2 study and a completed multiple ascending dose Phase 1 study

Intend to pursue accelerated approval with FDA

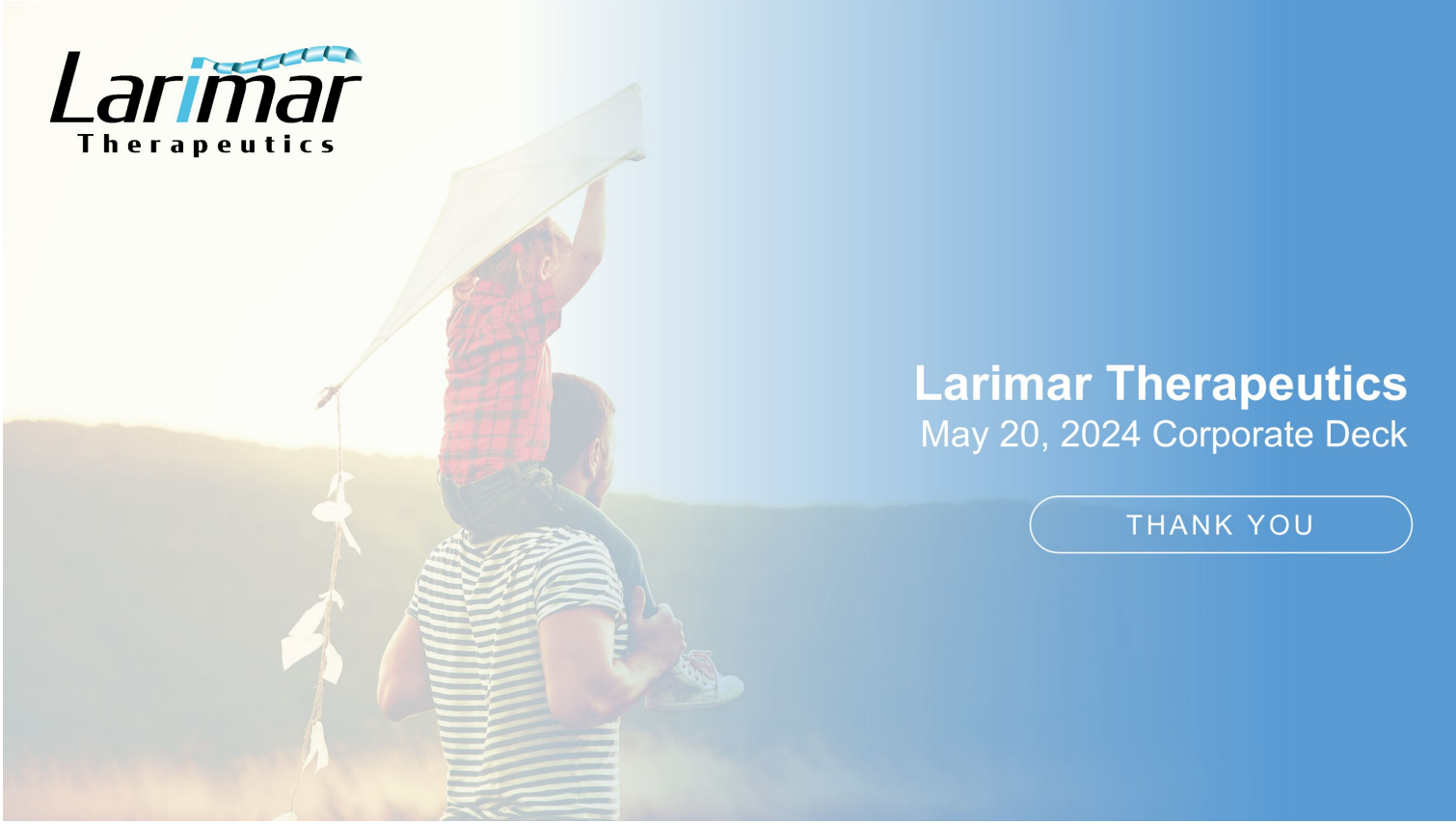
FDA acknowledgement that FXN deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. Discussions to support an accelerated approval are ongoing. BLA submission targeted for 2H 2025

OLE study with near-term catalysts

Dosed first patient in OLE study with 25 mg daily dosing in Q1 2024 with **interim data expected in Q4 2024**
Continuing to enroll patients and activate additional sites
In May 2024, the FDA removed the partial clinical hold
Dose escalation to 50 mg planned following further characterization of FXN pharmacodynamics at the 25 mg dose

Strong financial foundation

Approximately \$239 million in cash and investments at 3/31/24 which includes \$161.8 million in net proceeds raised from a Feb 24 public offering
Provides projected cash runway into 2026



Larimar Therapeutics

May 20, 2024 Corporate Deck

THANK YOU



Larimar Therapeutics

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

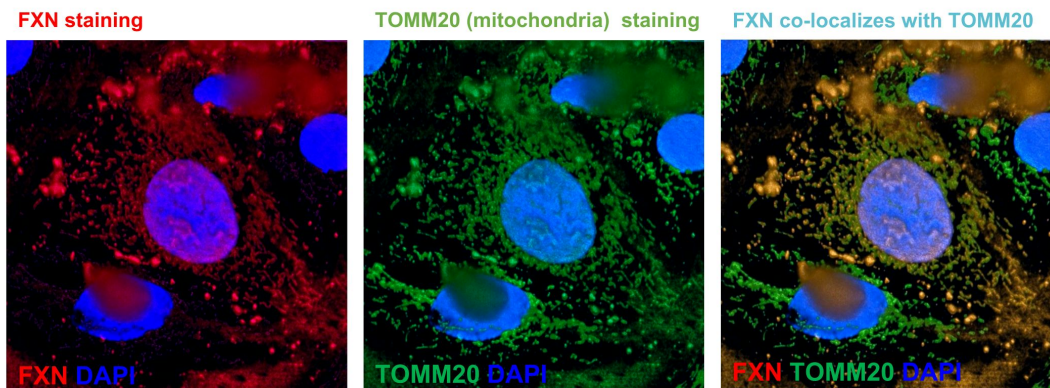
- Assistance with patient recruitment and education
- Access to Global Patient Registry with demographic and clinical information on more than 1,000 FA patients
- Sponsored a Patient-Focused Drug Development Meeting in 2017 resulting in a publication titled "The Voice of the Patient"





Mitochondrial Localization and Preclinical Data

Nomlabofusp Transduction of Cells In Vitro Leads to hFXN Located in Mitochondria



- Rat cardiomyocytes (H9C2) were transduced with nomlabofusp
- Cells were fixed and analyzed by immunofluorescence microscopy to detect the presence of human frataxin (hFXN) and TOMM20 (a mitochondrial outer membrane protein)
- Nuclei were stained with DAPI

Nomlabofusp Extends Survival in FXN-deficient KO Mice

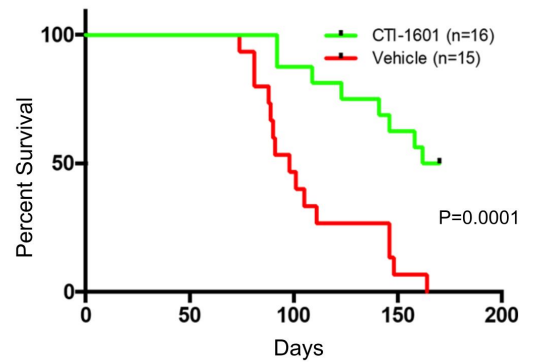
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

Median survival of MCK-Cre FXN-KO mice

- 166 days (nomlabofusp) vs. 98 days (Vehicle)
- Nomlabofusp administered 10 mg/kg SC every other day

Survival beyond vehicle mean (107.5 days)

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



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

Nomlabofusp Prevents Development of Ataxic Gait in Neurologic KO Mouse Model

In-Vivo Efficacy Data in Pvalb-Cre FXN-KO Mouse Model

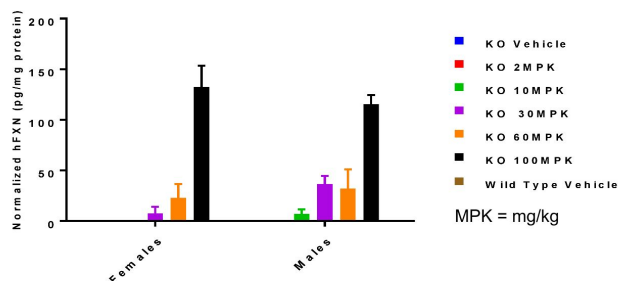
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

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

Nomlabofusp Delivers hFXN to Mitochondria and Restores SDH Activity in KO Mice

Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at varying SQ doses of nomlabofusp every other day for two weeks at Jackson Laboratories (Bar Harbor, ME). After dosing, animals were sacrificed, and heart and skeletal muscle were evaluated for hFXN concentration in mitochondrial extracts and SDH activity was assessed.

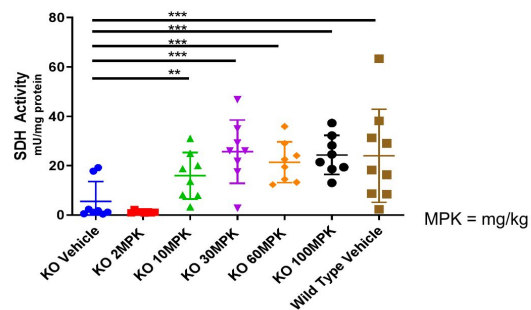
Mitochondrial FXN (Heart)



Mitochondria hFXN concentration increases dose-dependently
Given subcutaneously, nomlabofusp functionally replaces hFXN in mitochondria of KO mice



SDH Activity (Muscle)

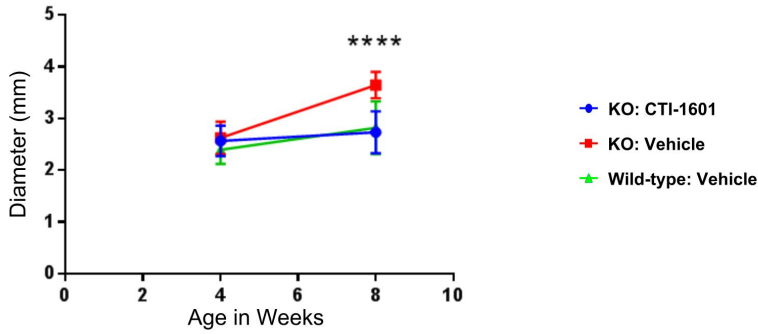


Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type

Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

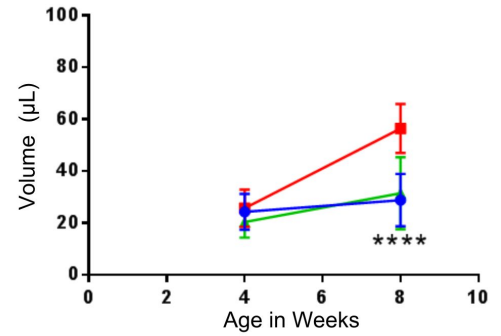
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

Left Ventricle Internal Diameter (Systole)



Left ventricular (LV) diameter increases in systole in untreated mice by 8 weeks (after 4 weeks of dosing with vehicle), but remains similar to wildtype when treated with nomlabofusp (10 mg/kg every other day)

Left Ventricle Volume (Systole)

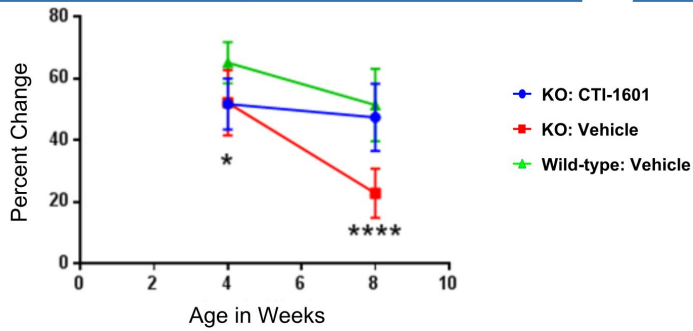


Nomlabofusp-treated mice have similar LV volume as wild type; echocardiogram shows significant differences between vehicle and nomlabofusp treated (10 mg/kg every other day) KO mice

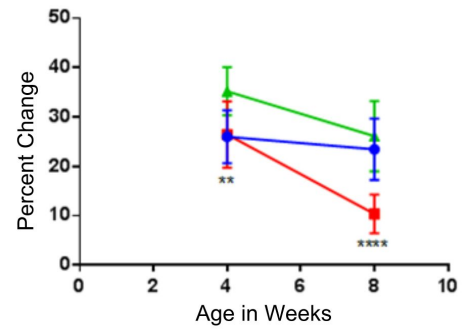
Nomlabofusp Preserves Left Ventricle Function in KO Mice

Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

Left Ventricle Ejection Function

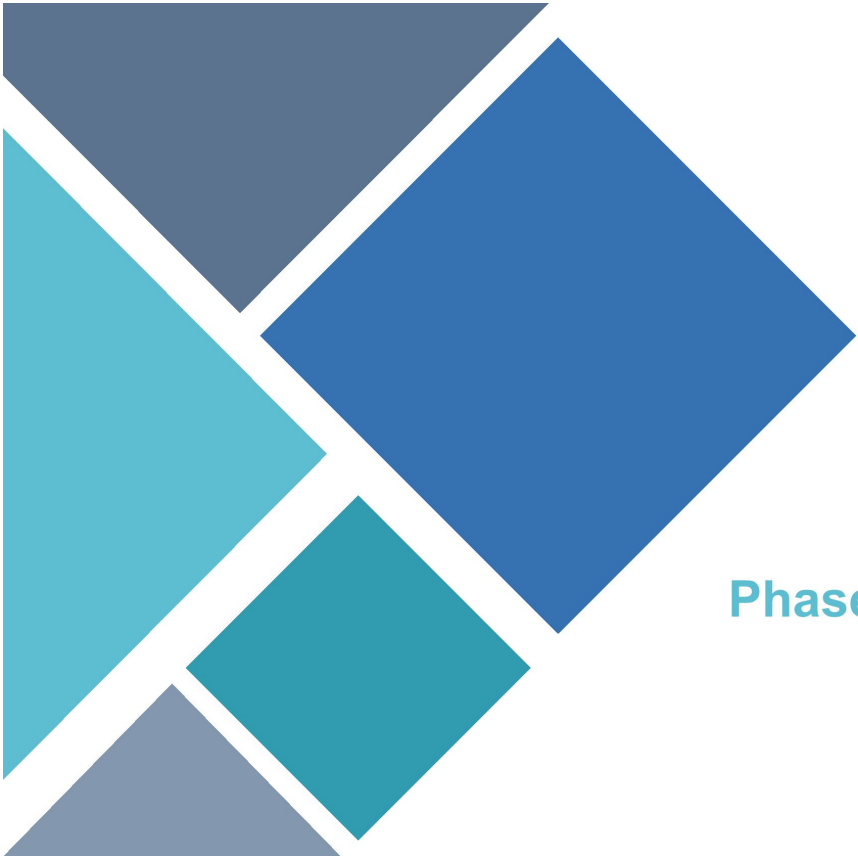


Left Ventricle Fractional Shortening



Left ventricular (LV) function drops significantly in vehicle treated mice by Week 8

Nomlabofusp-treated (10 mg/kg every other day) mice have similar LV function as wildtype; echocardiogram shows significant differences between vehicle and nomlabofusp treated KO mice



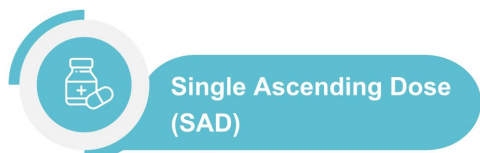
Phase 1 Clinical Data

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



Single Ascending Dose (SAD)

Eligible patients from SAD trial could enroll in MAD trial

Number of subjects: 28

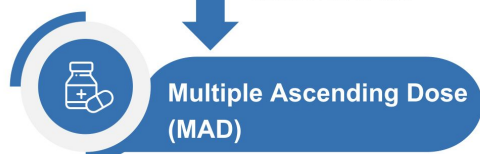
Dose levels: 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)

Treatment Duration: 1 day

1° Endpoint: Safety and tolerability

2° Endpoints: PK; PD; FXN levels; multiple exploratory

Status: Complete



Multiple Ascending Dose (MAD)

Number of Subjects: 27

Dose Range: 25 mg, 50 mg, 100 mg (subcutaneous administration)

Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days

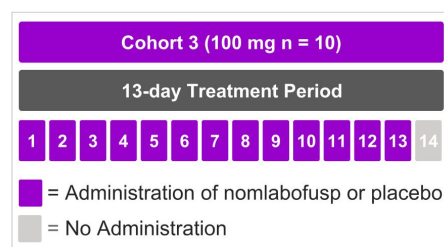
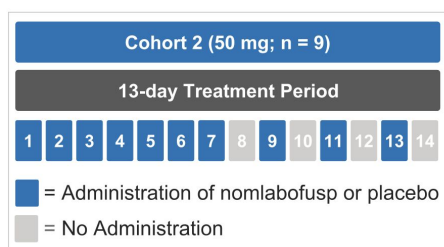
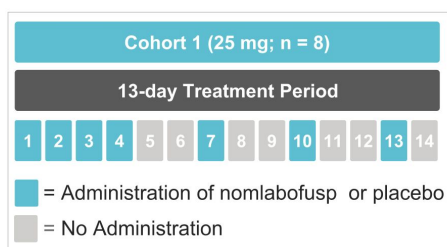
1° Endpoint: Safety and tolerability

2° Endpoints: PK; PD; FXN levels (buccal cells, platelets, optional skin biopsies); multiple exploratory

Status: Complete

Completed Phase 1 Multiple Ascending Dose Study

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



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

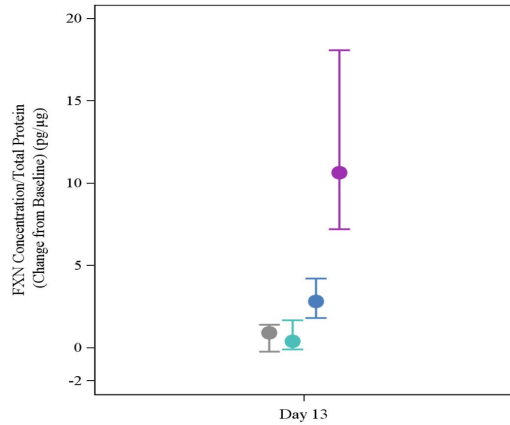
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Cohort 3 Sampling Days

Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

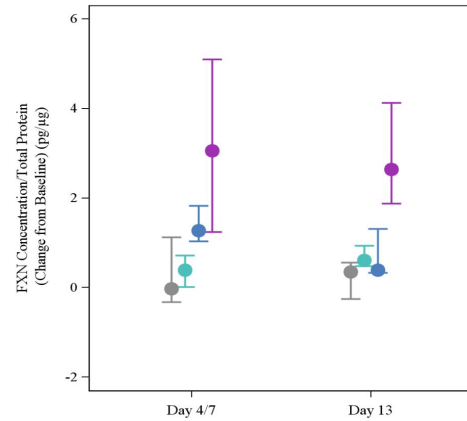
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

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



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

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



50 mg: Dosed daily for 7 days, every other day thereafter
 100 mg: Dosed daily for 13 days



*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;

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)

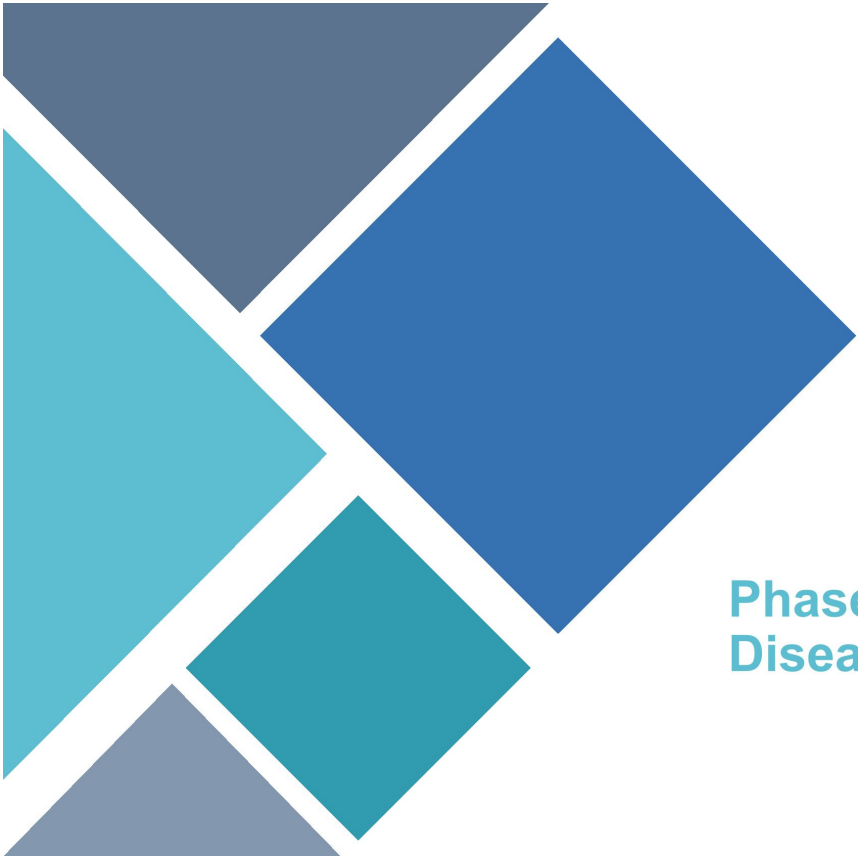
MAD Trial Patient Disease Characteristics

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)

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



Phase 2 Demographic/ Disease Characteristics Data



Demographics – Phase 2 Trial

	25 mg Cohort			50 mg Cohort		
	Placebo N = 4	Nomlabofusp N = 9	Overall N = 13	Placebo N = 5	Nomlabofusp N = 10	Overall N = 15

Age at Screening (Years)

Mean (SD)	34.0 (9.20)	37.8 (14.93)	36.6 (13.16)	28.6 (4.67)	28.1 (11.00)	28.3 (9.17)
Median	33	31	31	27	24	26
Q1, Q3	27, 42	27, 42	27, 42	26, 30	21, 32	21, 32
Min, Max	25, 45	25, 69	25, 69	24, 36	19, 54	19, 54

Sex n (%)

Male	2 (50.0)	5 (55.6)	7 (53.8)	1 (20.0)	4 (40.0)	5 (33.3)
Female	2 (50.0)	4 (44.4)	6 (46.2)	4 (80.0)	6 (60.0)	10 (66.7)

Previously Treated with Nomlabofusp n (%)

Yes	1 (25.0)	3 (33.3)	4 (30.8)	0	1 (10.0)	1 (6.7)
No	3 (75.0)	6 (66.7)	9 (69.2)	5 (100.0)	9 (90.0)	14 (93.3)

Disease Characteristics – Phase 2 Study

	25 mg Cohort			50 mg Cohort		
	Placebo N = 4	Nomlabofusp N = 9	Overall N = 13	Placebo N = 5	Nomlabofusp N = 10	Overall N = 15
Age at Symptom Onset (Years)						
Mean (SD)	14.5 (4.93)	13.0 (10.47)	13.5 (8.77)	15.2 (7.26)	13.7 (8.37)	14.2 (7.78)
Median	14.5	10	11	14	12.5	14
Q1, Q3	11, 19	8, 13	9, 15	11, 16	7, 18	7, 18
Min, Max	9, 20	5, 38	5, 38	8, 27	5, 30	5, 30
Age at Diagnosis (Years)						
Mean (SD)	17.5 (5.57)	18.6 (11.20)	18.2 (9.58)	18.6 (6.80)	16.6 (8.03)	17.3 (7.46)
Median	16.5	16	16	19	13.5	14
Q1, Q3	14, 22	14, 20	14, 20	13, 20	10, 21	12, 21
Min, Max	12, 25	5, 42	5, 42	12, 29	9, 30	9, 30
Time Since Diagnosis (Years)						
Mean (SD)	16.1 (5.97)	18.5 (11.52)	17.8 (9.94)	9.5 (3.72)	11.9 (7.05)	11.1 (6.10)
Median	13.42	14.32	13.5	11	11.26	11
Q1, Q3	12.9, 19.3	12.8, 21.6	12.8, 21.6	5.8, 11.3	7.4, 15.3	5.8, 15.2
Min, Max	12.5, 25.0	5.4, 45.0	5.4, 45.0	5.6, 14.0	2.3, 25.1	2.3, 25.1

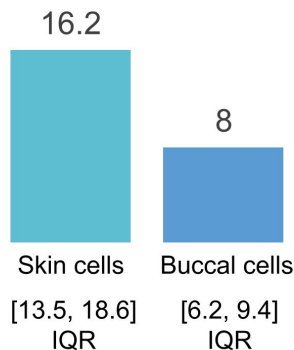


Non-Interventional Study Data

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

Non-interventional study measured FXN in homozygous healthy volunteers

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



Most 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 onset² (5 to 15 years of age)

Higher FXN levels seen with late onset² (after 25 years of age)

Heterozygous carriers who show no signs of disease have buccal cell FXN levels of ~50% of unaffected healthy persons¹