

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 09, 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 2.02 Results of Operations and Financial Condition.**

On May 9, 2024, Larimar Therapeutics, Inc. (the “*Company*”) announced its financial results and operational highlights for the first quarter ended March 31, 2024. 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 May 9, 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	<a href="#">Press Release issued by Larimar Therapeutics, Inc. on May 9, 2024*</a>
99.2	<a href="#">Larimar Therapeutics, Inc. Corporate Presentation, dated May 9, 2024</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

\* Furnished herewith

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**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 9, 2024

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

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## Larimar Therapeutics Reports First Quarter 2024 Operating and Financial Results

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

**Bala Cynwyd, PA**, May 9, 2024 – Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its first quarter 2024 operating and financial results.

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

### Recent Highlights

- In February 2024, Larimar announced positive top-line data and successful completion of its four-week, placebo-controlled Phase 2 dose exploration study of nomlabofusp (CTI-1601) in patients with FA. Nomlabofusp was generally well-tolerated throughout the four-week treatment periods, had a predictable pharmacokinetic profile, and led to dose-dependent increases in frataxin in skin and buccal cells after daily dosing for 14 days followed by every other day dosing until day 28 in the 25 mg and 50 mg cohorts. Increases in frataxin levels in skin cells were seen in all treated patients, and in buccal cells for the majority of patients. At Day 14, all patients (with quantifiable levels at baseline and Day 14) treated with 50 mg of nomlabofusp achieved frataxin levels in skin cells greater than 33% of the average level observed in healthy volunteers, with 3 patients achieving levels greater than 50% of the average healthy volunteer level.
- In February 2024, Larimar announced the Company had initiated discussions with the FDA on use of tissue frataxin levels as a potential novel surrogate endpoint. Larimar received FDA acknowledgement that frataxin deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. The Company intends to pursue an accelerated approval using FXN levels, supportive pharmacodynamics and clinical information, and safety data from the OLE study,

along with additional nonclinical pharmacology information needed to support the novel surrogate endpoint approach, with a BLA submission targeted for the second half of 2025.

- In February 2024, Larimar raised net proceeds of approximately \$161.8 million through a public offering of common stock.
- In March 2024, the first patient was dosed in the OLE study evaluating daily subcutaneous injections of 25 mg of nomlabofusp self-administered or administered by a caregiver. Participants who completed treatment in the Phase 2 dose exploration study, or who previously completed a prior clinical trial of nomlabofusp, are potentially eligible to screen for the OLE study. The OLE study will evaluate the safety and tolerability, pharmacokinetics, and frataxin levels in peripheral tissues as well as other exploratory pharmacodynamic markers (lipid profiles and gene expression data) following long-term subcutaneous administration of nomlabofusp. Dose escalation in the OLE study is contingent on the FDA's review of data from the 50 mg cohort of the Phase 2 study and available data from the OLE study, due to the continued partial clinical hold. Interim data is expected in the fourth quarter of 2024. In addition, clinical assessments collected during the study will be compared to data from a matched control arm derived from participants in the Friedreich's Ataxia Clinical Outcomes Measures Study (FACOMS) database.
- In March 2024, Larimar began to build its commercial team with the appointment of Frank Nazzario, RPh, as Vice President of Commercial. Mr. Nazzario brings nearly 30 years of leadership experience in drug launches for rare diseases. Most recently, he served as Senior Vice President of Sales at BioCryst Pharmaceuticals. Previously, he held commercial leadership roles at Spark Therapeutics where he led the commercialization of Luxturna®, the first FDA-approved gene therapy for an inherited retinal disorder, and at ViroPharma, Inc., where he led the launch of Cinryze®, the first approved biologic for Hereditary Angioedema.

### First Quarter 2024 Financial Results

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

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

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

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

### About Larimar Therapeutics

Larimar Therapeutics, Inc. (Nasdaq: LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. Larimar's lead compound, nomlabofusp, is being developed as a potential treatment for Friedreich's ataxia. Larimar also plans to use its intracellular delivery platform to design other fusion proteins to target additional rare diseases characterized by deficiencies in intracellular bioactive compounds. For more information, please visit: <https://larimartx.com>.

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## **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](http://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.

## **Investor Contact:**

Joyce Allaire  
LifeSci Advisors  
[jallaire@lifesciadvisors.com](mailto:jallaire@lifesciadvisors.com)  
(212) 915-2569

## **Company Contact:**

Michael Celano  
Chief Financial Officer  
[mcelano@larimartx.com](mailto:mcelano@larimartx.com)  
(484) 414-2715

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**Larimar Therapeutics, Inc.**  
Condensed Consolidated Balance Sheet  
(unaudited)

	March 31, 2024	December 31, 2023
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 110,125	\$ 26,749
Short-term marketable securities	117,171	60,041
Prepaid expenses and other current assets	3,657	3,385
Total current assets	230,953	90,175
Long-term marketable securities	11,711	—
Property and equipment, net	604	684
Operating lease right-of-use assets	2,920	3,078
Restricted cash	1,339	1,339
Other assets	678	659
Total assets	\$ 248,205	\$ 95,935
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,918	\$ 1,283
Accrued expenses	10,098	7,386
Operating lease liabilities, current	825	837
Total current liabilities	12,841	9,506
Operating lease liabilities	4,520	4,709
Total liabilities	17,361	14,215
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; \$0.001 par value per share; 5,000,000 shares authorized as of March 31, 2024 and December 31, 2023; no shares issued and outstanding as of March 31, 2024 and December 31, 2023	—	—
Common stock, \$0.001 par value per share; 115,000,000 shares authorized as of March 31, 2024 and December 31, 2023; 63,800,017 and 43,909,069 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively	64	43
Additional paid-in capital	434,013	270,150
Accumulated deficit	(203,208)	(188,554)
Accumulated other comprehensive gain (loss)	(25)	81
Total stockholders' equity	230,844	81,720
Total liabilities and stockholders' equity	\$ 248,205	\$ 95,935

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

	Three Months Ended March 31,	
	2024	2023
Operating expenses:		
Research and development	\$ 12,939	\$ 4,562
General and administrative	3,795	3,075
Total operating expenses	16,734	7,637
Loss from operations	(16,734)	(7,637)
Other income, net	2,080	1,111
Net loss	(14,654)	(6,526)
Net loss per share, basic and diluted	\$ (0.27)	\$ (0.15)
Weighted average common shares outstanding, basic and diluted	53,553,707	43,897,603
Comprehensive loss:		
Net loss	\$ (14,654)	\$ (6,526)
Other comprehensive gain (loss):		
Unrealized gain (loss) on marketable securities	(106)	31
Total other comprehensive gain (loss)	(106)	31
Total comprehensive loss	\$ (14,760)	\$ (6,495)





**Larimar Therapeutics**  
Corporate Deck

May 2024

# Forward-Looking Statements

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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](http://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.

# Positive Topline Phase 2 Data for Nomlabofusp (CTI-1601)

Successful completion of 4-week, placebo-controlled dose exploration study (25 mg and 50 mg) in FA

## Dose-dependent increases in tissue frataxin (FXN) levels in skin and buccal cells

Participants treated with 50 mg daily for 14 days and then every other day for an additional 14 days until day 28

Nomlabofusp was generally well-tolerated following repeated subcutaneous injections up to 28 days

- Baseline FXN levels in skin cells < 17% of average FXN levels of healthy volunteers
- After 14 days of daily dosing, FXN levels in skin cells increased to 33% to 59% of average FXN level of healthy volunteers
- After switching to every other day dosing on day 15, continued to observe dose dependent increases in FXN levels with reduced magnitude

All treated patients in the 50 mg dose group had at least a 100% increase over baseline in FXN levels in skin cells at day 14

Across all studies to date, higher variability in FXN levels was observed in buccal vs. skin cells

## OLE trial initiated for 25 mg daily dosing

High patient interest in study participation  
First patient dosed in March 2024; Continuing to enroll patients and activate additional sites  
Interim data expected Q4 2024

## Intend to Pursue Accelerated Approval with FDA

Discussions initiated on FXN as surrogate endpoint  
Potential BLA submission targeted for 2H 2025



FA: Friedreich's ataxia; OLE: Open-label extension; BLA: Biologics License Application

# 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 4-week placebo-controlled Phase 2 study and a 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

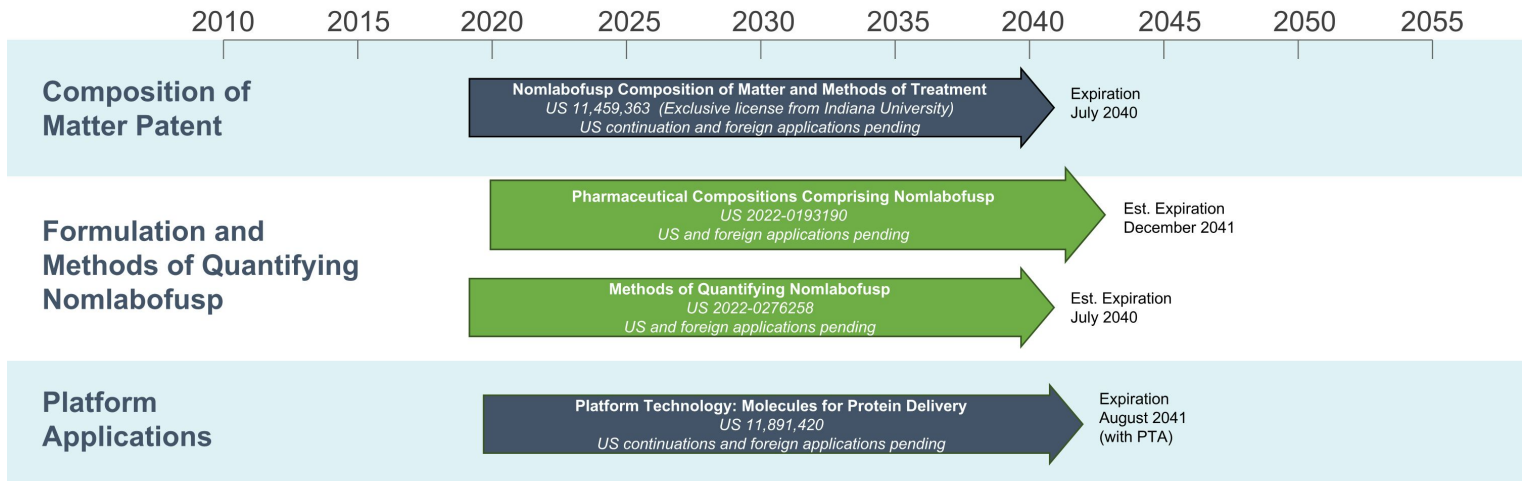
Dosed first patient in OLE study with 25 mg daily dosing in Q1 2024 with **interim data expected in Q4 2024**  
Continuing to enroll patients and activate additional sites  
Dose escalation in the OLE study is contingent on the FDA's review of data from the 50 mg cohort of the Phase 2 study and available data from the OLE study due to continued partial clinical hold

## 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

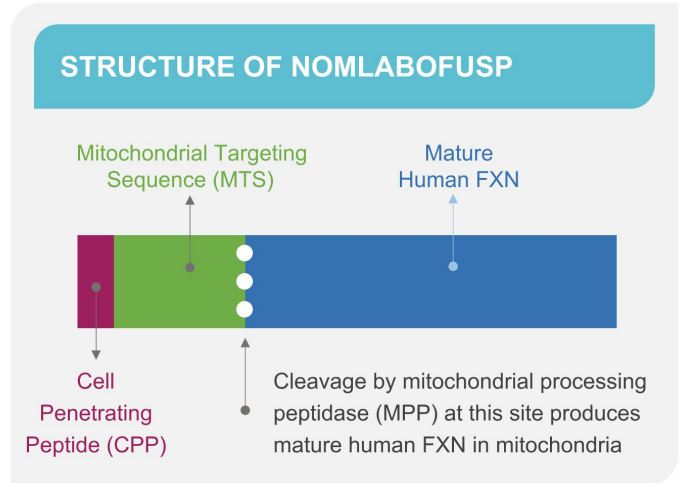
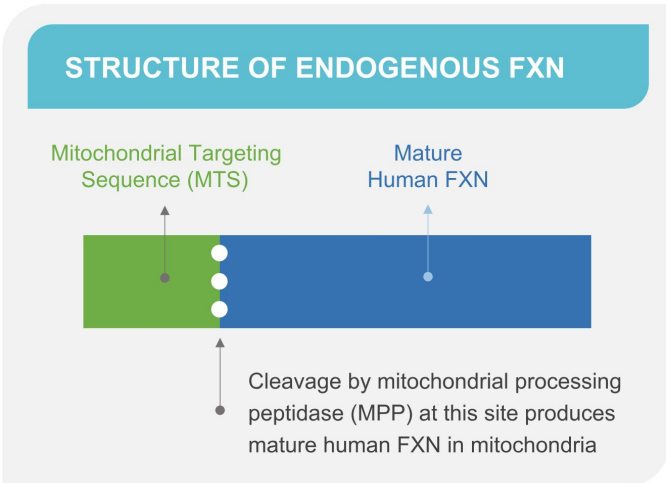
**No approved therapies increase frataxin levels**

Only treatment approved for FA does not address frataxin deficiency



# 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.



# 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



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

■ = No Administration

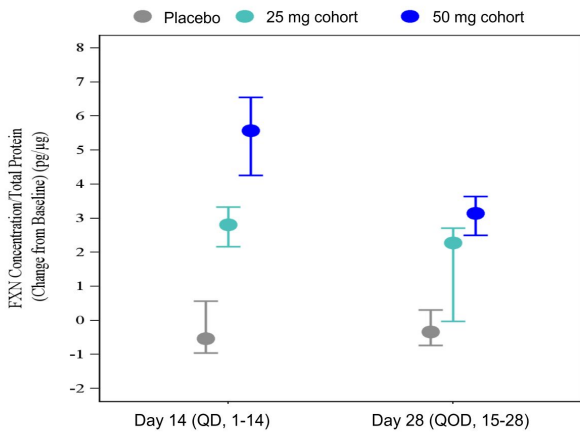
## Study Details

<b>Population</b>	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
<b>Dose</b>	<b>Cohort 1:</b> 25 mg <b>Cohort 2:</b> 50 mg
<b>Key Endpoints</b>	Frataxin levels in peripheral tissue, PK, safety and tolerability; other exploratory endpoints include lipids and gene expression levels
<b>Number of Patients</b>	<b>Cohort 1:</b> Enrolled 13 participants (9 on nomlabofusp; 4 on placebo) <b>Cohort 2:</b> Enrolled 15 participants (10 on nomlabofusp; 5 on placebo)

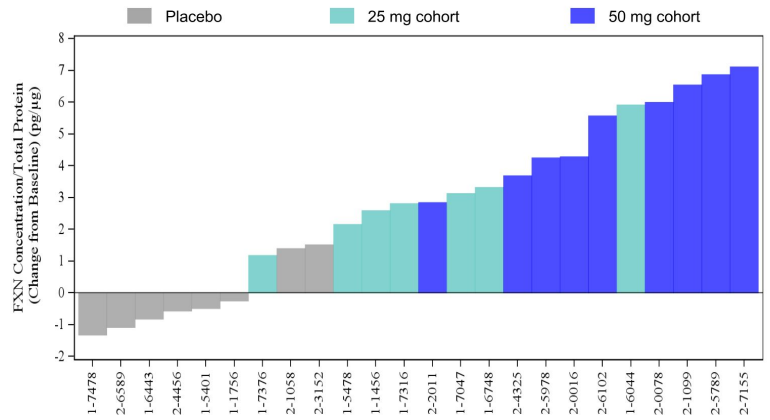
# Dose-Dependent Increase in FXN Levels in Skin Cells

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

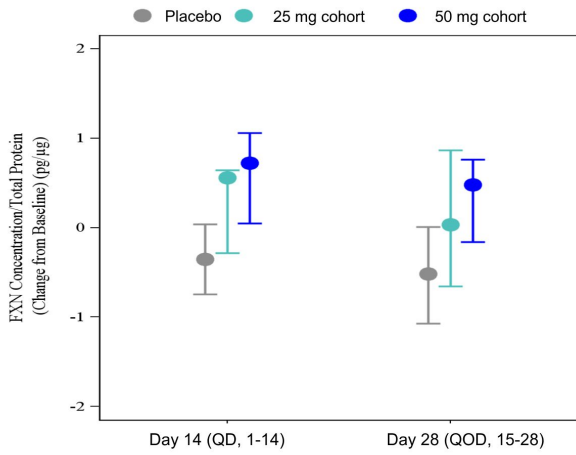


\*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25<sup>th</sup> and 75<sup>th</sup> percentiles. Only participants with quantifiable levels at both baseline and Day 14 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.

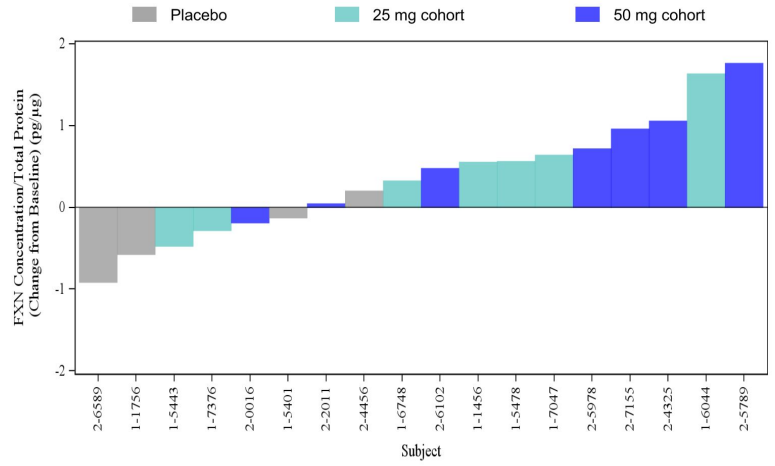
# Dose-Dependent Increase in FXN Levels in Buccal Cells

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 25<sup>th</sup> and 75<sup>th</sup> 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
	<b>Change from Baseline</b>	<b>2.81</b>	<b>3.02</b>
50 mg	Baseline	2.12	2.08
	Day 14	7.40	7.32
	<b>Change from Baseline</b>	<b>5.57</b>	<b>5.24</b>

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
	<b>Change from Baseline</b>	<b>2.28</b>	<b>1.41</b>
50 mg	Baseline	2.12	2.08
	Day 28	5.23	5.24
	<b>Change from Baseline</b>	<b>3.14</b>	<b>3.17</b>



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
	<b>Change from Baseline</b>	<b>0.56</b>	<b>0.42</b>
50 mg	Baseline	1.61	1.69
	Day 14	2.44	2.38
	<b>Change from Baseline</b>	<b>0.72</b>	<b>0.69</b>

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
	<b>Change from Baseline</b>	<b>0.03</b>	<b>0.11</b>
50 mg	Baseline	1.76	1.77
	Day 28	2.15	2.15
	<b>Change from Baseline</b>	<b>0.48</b>	<b>0.38</b>

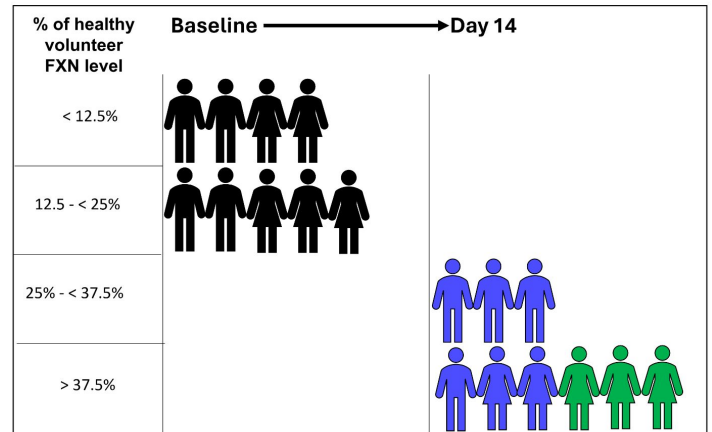
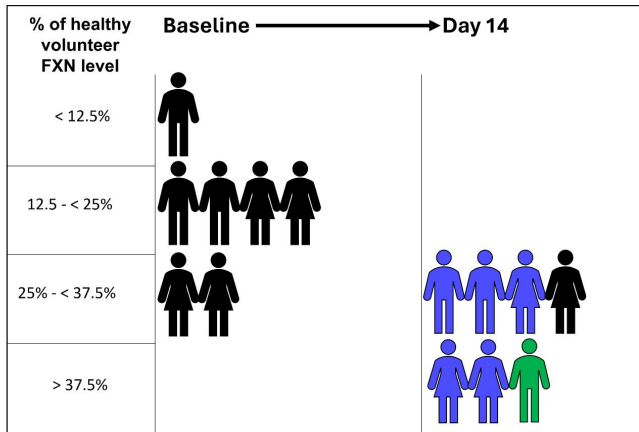


Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

# 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

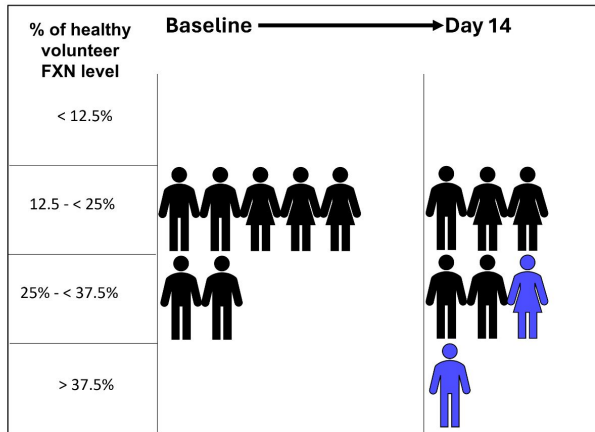


Only participants with quantifiable levels at baseline and day 14 are included in the figures.

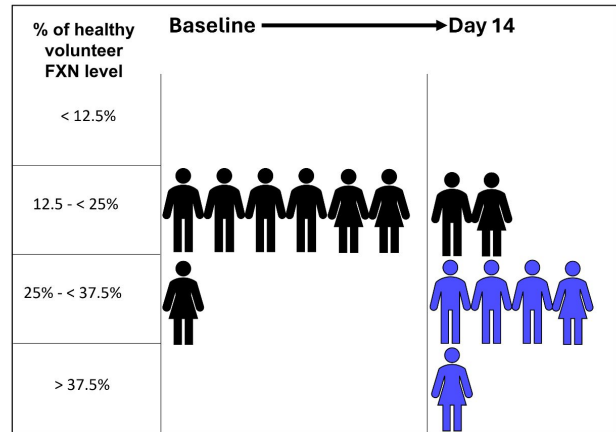
\*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/μg) from the noninterventional healthy volunteer study (N=60).

# 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).

# Increasing FXN Levels May Slow Disease Progression

## Disease Characteristics\* Based on Literature\*\*

### Patients with FXN levels 11% of average healthy volunteers

- Median age of onset at 7 years
- Deteriorate by 2.9 points/year as measured by FARS
- Lose ambulation at a median of 11.5 years

### Patients with FXN levels > 30% of average healthy volunteers

- Median age of onset at 16 years
- Deteriorate by 2.0 points/year as measured by FARS
- Lose ambulation at a median of 18.3 years

## Nomlabofusp Administration in Phase 2 Study

### 25 mg daily for 14 days shifted FXN levels in

- All but one patient to > 25% of average healthy volunteers in skin cells with a median value of 33.9%

### 50 mg daily for 14 days shifted FXN levels in

- All patients from < 25% of average healthy volunteers to 33% to 59% (3 patients > 50%) in skin cells with a median value of 45%

H.L.Plasterer et al. PLoS ONE 2013 8(5):e63958; C. Rummey et al. EClinicalMedicine. 2020 18:100213



\*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 as measured by % of normal demonstrated to be equivalent in PBMCs, buccal cells, and whole blood.



# 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

Screening Period  $\leq$  42 days\*\*

Treatment Period Planned for  $\geq$  1 year

Potential extensions

## Key Study Objectives

- Safety and tolerability
- Long-term PK
- 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

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. Initiation of additional U.S. clinical trials is contingent on FDA review of clinical data due to partial clinical hold.

# 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
Gene Therapy	TBD	Voyager/Neurocrine	Frataxin Gene Replacement	Phase 1 planned in 2025



\*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

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 4-week placebo-controlled Phase 2 study and a multiple ascending dose Phase 1 study

## Intend to pursue accelerated approval with FDA

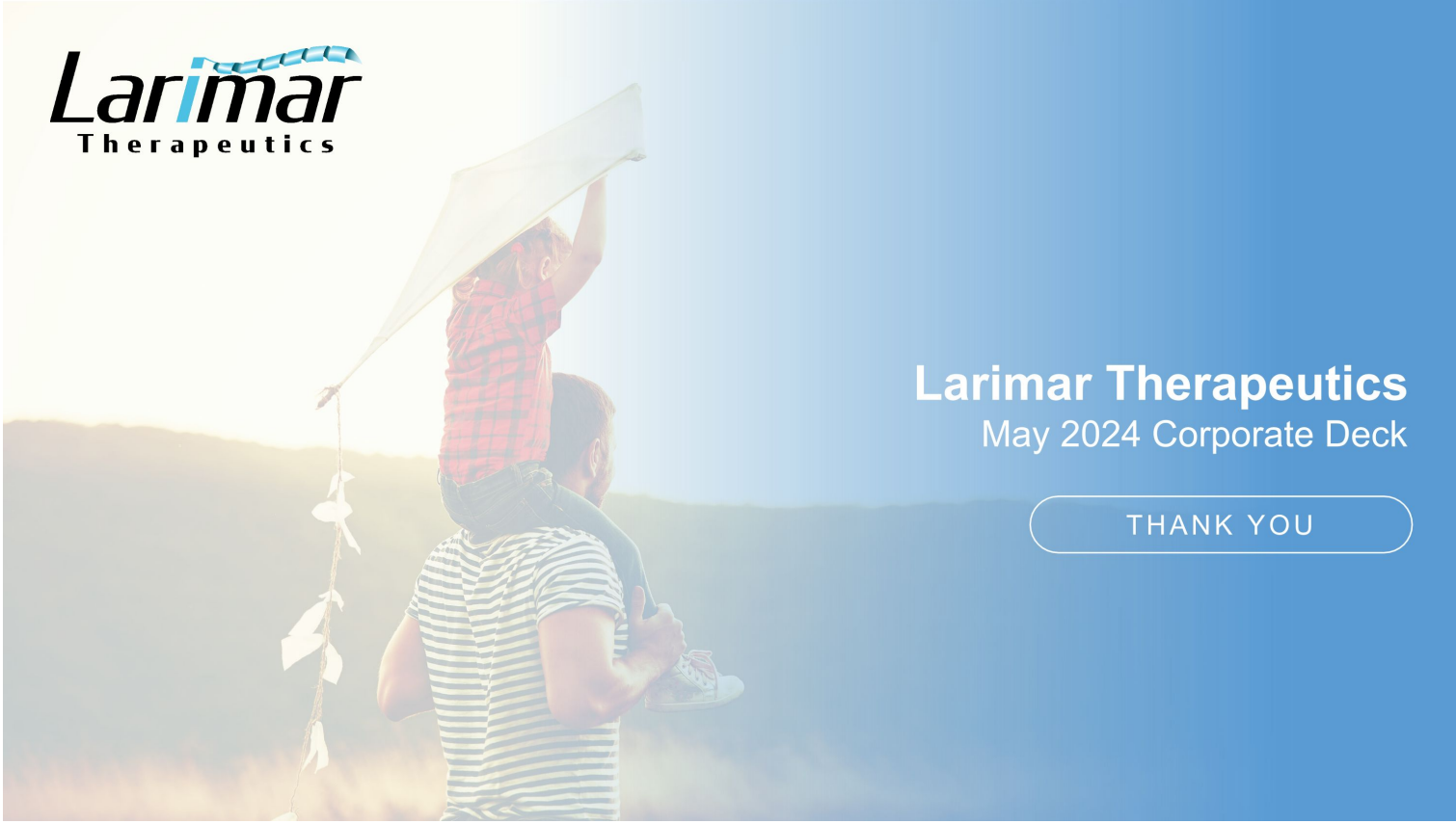
FDA acknowledgement that FXN deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. Discussions to support an accelerated approval are ongoing. BLA submission targeted for 2H 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  
Dose escalation in the OLE study is contingent on the FDA's review of data from the 50 mg cohort of the Phase 2 study and available data from the OLE study due to continued partial clinical hold

## 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 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

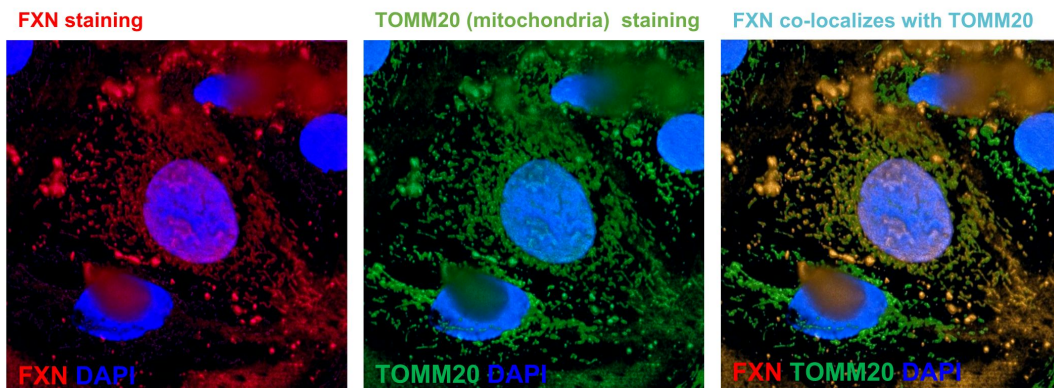
- Assistance with patient recruitment and education
- Access to Global Patient Registry with demographic and clinical information on more than 1,000 FA patients
- Sponsored a Patient-Focused Drug Development Meeting in 2017 resulting in a publication titled "The Voice of the Patient"





## 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

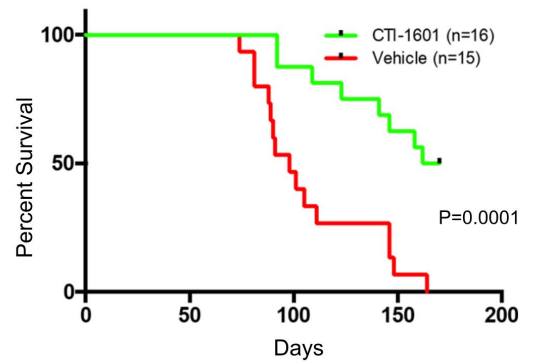
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

## 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

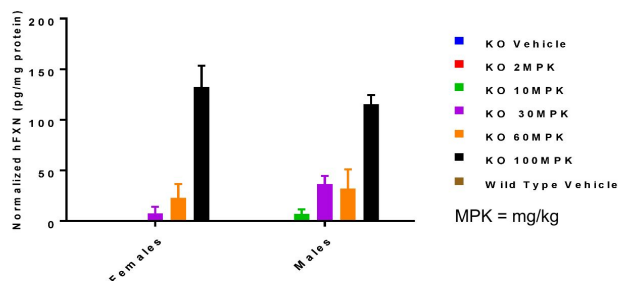
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

- ✓ 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.

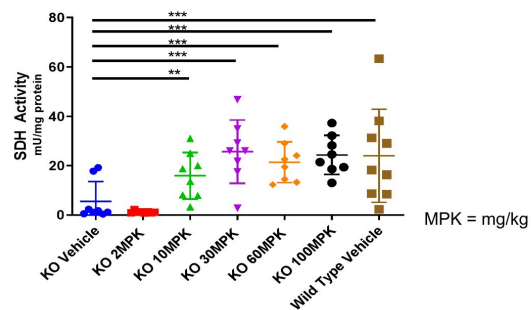
## Mitochondrial FXN (Heart)



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



## SDH Activity (Muscle)



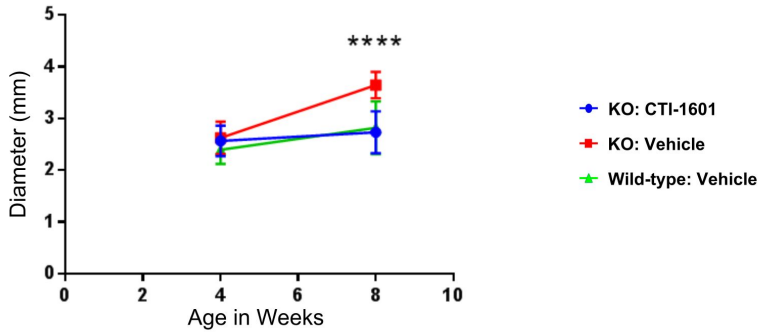
Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type



# Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

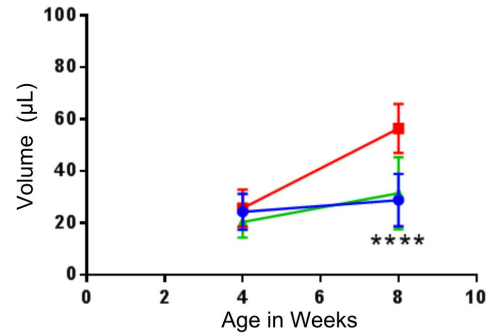
**Study Design** – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

## 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)

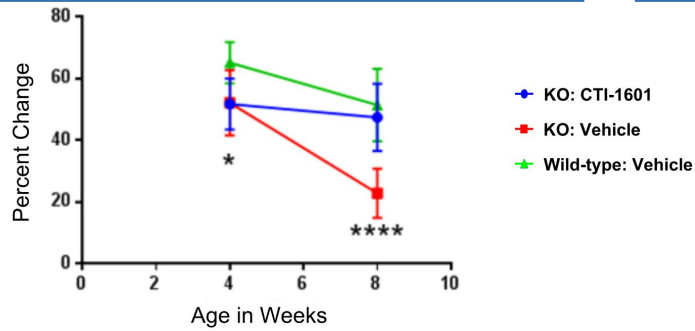


Nomlabofusp-treated mice have similar LV volume as wild type; echocardiogram shows significant differences between vehicle and nomlabofusp treated (10 mg/kg every other day) KO mice

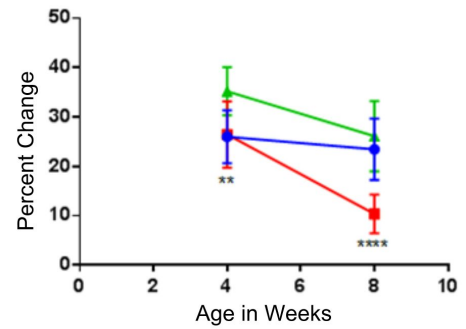
# Nomlabofusp Preserves Left Ventricle Function in KO Mice

**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

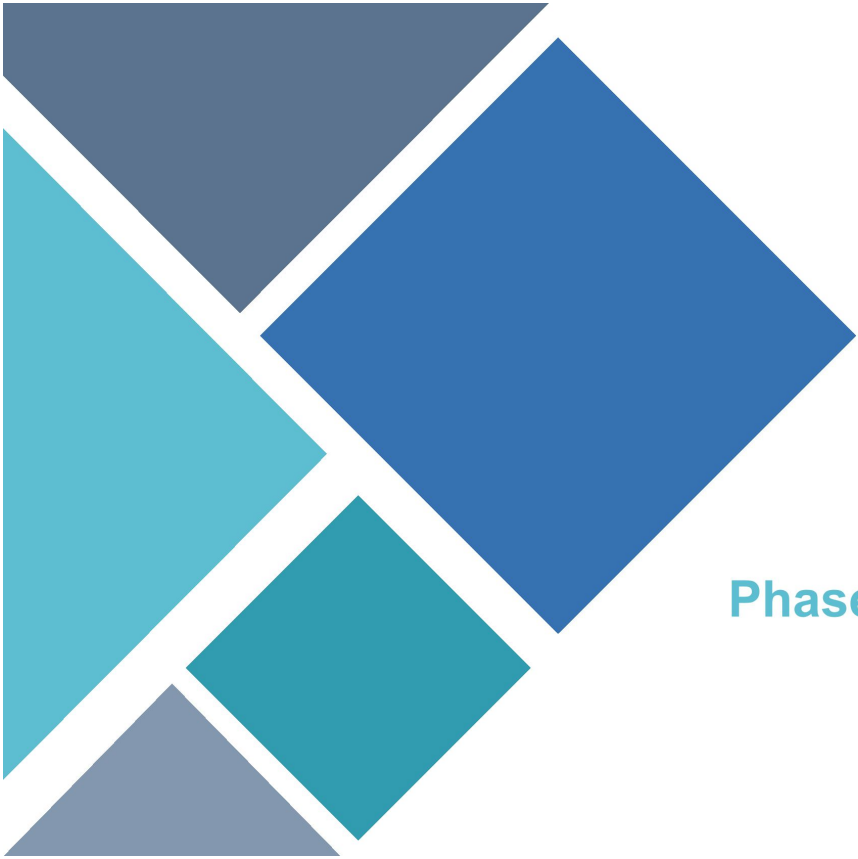


## Left Ventricle Fractional Shortening



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



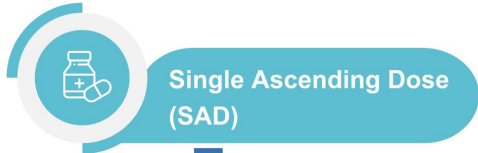
## Phase 1 Clinical Data

# 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

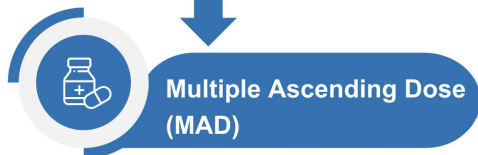
**Dose levels:** 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)

**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

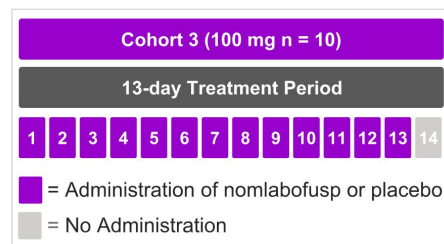
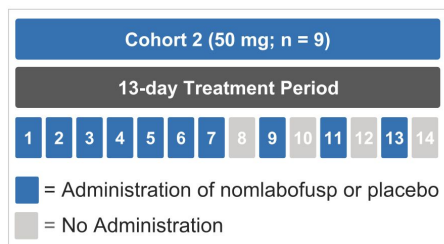
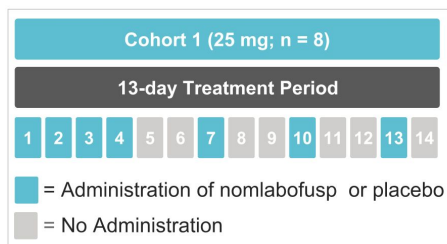
**1° Endpoint:** Safety and tolerability

**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**

<b>Buccal Cells</b>	Baseline, Day 4, Day 13
<b>Skin</b>	Baseline, Day 13
<b>Platelets</b>	Baseline, Day 4, Day 13

**Cohort 2 Sampling Days**

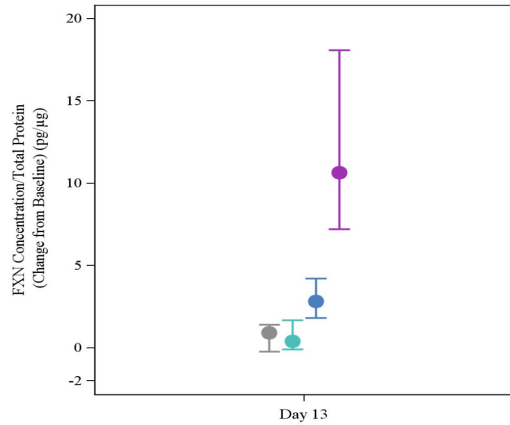
<b>Buccal Cells</b>	Baseline, Day 7, Day 13
<b>Skin</b>	Baseline, Day 13
<b>Platelets</b>	Baseline, Day 7, Day 13

**Cohort 3 Sampling Days**

<b>Buccal Cells</b>	Baseline, Day 7, Day 13
<b>Skin</b>	Baseline, Day 13
<b>Platelets</b>	Baseline, Day 7, Day 13

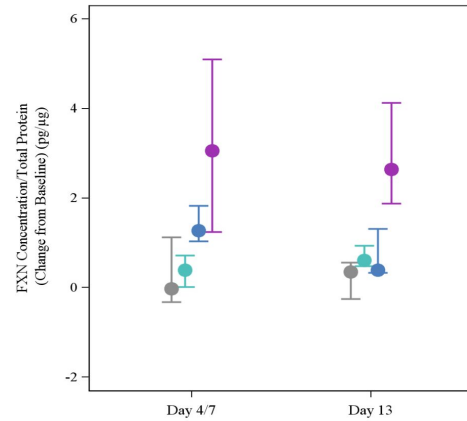
# Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

**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 25<sup>th</sup> and 75<sup>th</sup> 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)
<b>Sex</b>							
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)
<b>Age (years)</b>							
	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
<b>Race</b>							
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)
<b>Ethnicity</b>							
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

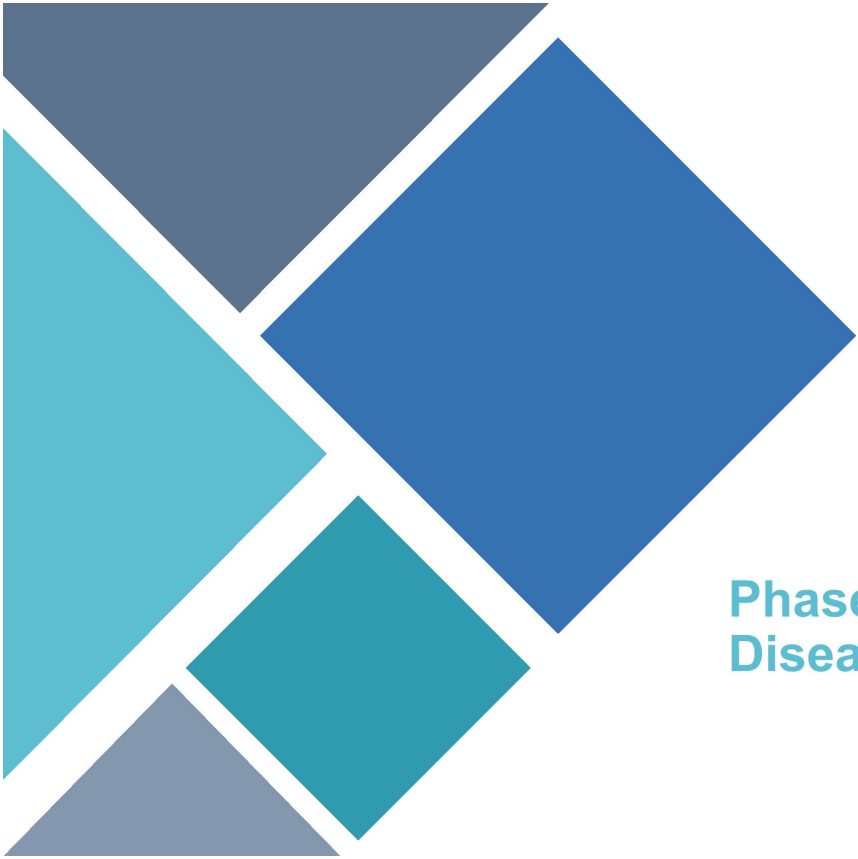
Parameter	Statistic	All placebo (n=7)	25 mg CTI-1601 (n=6)	50 mg CTI-1601 (n=7)	100 mg CTI-1601 (n=7)	All CTI-1601 (n=20)	Overall (n=27)
<b>Age at Symptom Onset</b>							
	Mean	14.1	24.0	19.3	11.9	18.1	17.1
	SD	5.34	14.48	6.21	6.72	10.37	9.39
	Median	15.0	18.0	19.0	10.0	18.0	16.0
	Min, Max	8,23	12,44	8,28	5,22	5,44	5,44
<b>Age at Diagnosis</b>							
	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
<b>Assistive Device</b>							
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
<b>Age at Symptom Onset (Years)</b>						
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
<b>Age at Diagnosis (Years)</b>						
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
<b>Time Since Diagnosis (Years)</b>						
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

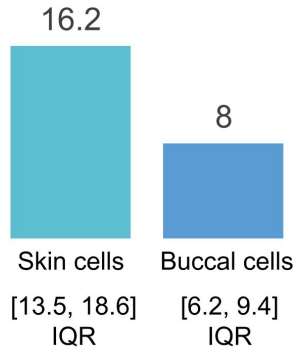


## Non-Interventional Study Data

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

Non-interventional study measured FXN in homozygous healthy volunteers

## Median Frataxin Concentration (pg/ $\mu$ g) in Homozygous Healthy Volunteers (n = 60)



Most patients with FA only produce ~20-40%<sup>1</sup> of normal frataxin levels depending on the tissue, sampling technique, and assay considered

Lower FXN levels seen with typical onset<sup>2</sup> (5 to 15 years of age)

Higher FXN levels seen with late onset<sup>2</sup> (after 25 years of age)

Heterozygous carriers who show no signs of disease have buccal cell FXN levels of ~50% of unaffected healthy persons<sup>1</sup>