

**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): January 12, 2026

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 January 12, 2026, Larimar Therapeutics, Inc. (the “*Company*”) disclosed in an updated slide presentation, posted on its website, that, as of December 31, 2025, the Company had approximately \$136.9 million of cash, cash equivalents and marketable securities. The Company has not yet completed its quarter-end or year-end financial close processes for the quarter and year ended December 31, 2025. This unaudited, preliminary amount has been prepared by and is the responsibility of management. This amount is based upon information available to management as of the date of this Current Report on Form 8-K and subject to completion of financial closing procedures that could result in changes to the amount. Furthermore, this amount does not present all information necessary for an understanding of the Company’s financial condition as of December 31, 2025. The Company’s independent registered public accounting firm, PricewaterhouseCoopers LLP, has not audited, reviewed, compiled or performed any procedures with respect to this preliminary financial data and, accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. The Company’s actual results for the year ended December 31, 2025 will be included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2025 and may differ materially from the above estimate.

The information furnished pursuant to this Item 2.02 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 January 12, 2026, the Company posted on its website an updated slide presentation, which is attached as Exhibit 99.1 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.

<u>Exhibit No.</u>	<u>Document</u>
99.1	Larimar Therapeutics, Inc. Corporate Presentation, dated January 12, 2026*
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: January 12, 2026

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



Larimar Therapeutics

Corporate Deck

January 2026

Forward-Looking Statements

This presentation 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 presentation 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 and any other planned product candidates, Larimar's planned research and development efforts, including the timing of its nomlabofusp clinical trials, interactions and filings with the FDA, expectations regarding the timing of the BLA submission, the expectations of the timing of, and potential for, accelerated approval or accelerated access, time to launch and market and overall development plans 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, and Larimar's ability to timely implement the revised dosing regimen in its clinical program for nomlabofusp; 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 non-clinical or clinical trials, and assessments; delays in patient recruitment, including as a result of changes in clinical protocols and adverse events; 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.

Friedreich's ataxia (FA): A rare, debilitating and progressive disease

Affects ~20,000 patients globally

~5,000 patients in the U.S., with a concentration of patients in Europe
~70% of patients present before age 14

Caused by a genetic defect that lowers frataxin levels

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

Heterozygous carriers

Asymptomatic with FXN levels of 50-75%* of normal frataxin levels

Progressive, debilitating disease with early mortality

Characterized by loss of coordination, slurred speech, difficulty swallowing, scoliosis, diabetes, and cardiovascular disease
Life expectancy 30-50 years, with early death usually caused by heart disease

High unmet medical need

The only currently approved treatment for FA does not address frataxin deficiency



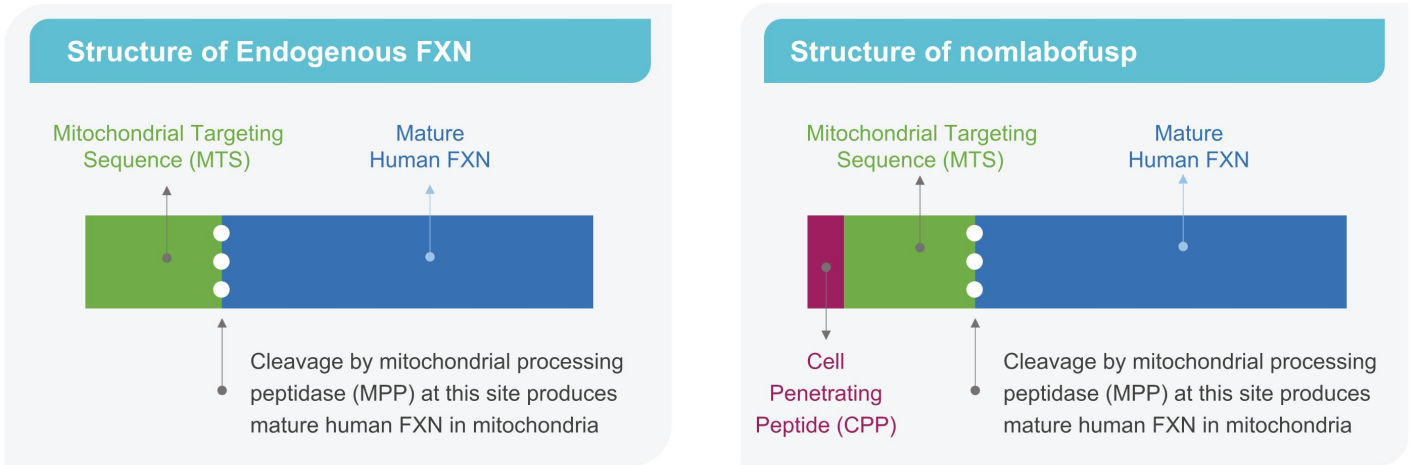
Larimar is developing nomlabofusp as the first potential disease modifying therapy for FA. Designed to potentially save patients from enormous suffering and deterioration of quality of life.



* 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

FA-Treating Clinicians' Perspectives on Importance of FXN Replacement

A recent national survey* of clinicians treating patients with FA (n = 50)

Reinforces the ongoing need for additional therapeutic options for the FA patient

98%

believe there is a need for a treatment that **targets frataxin levels directly**

94%

agree that despite recent advances in the management of FA, there **remain unmet patient needs** in FA treatment

98%

concur that **addressing the root cause of FA** is the next step in advancing treatment



*Larimar Therapeutics-sponsored survey (April 2025); data on file.

FXN Levels Clearly 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.

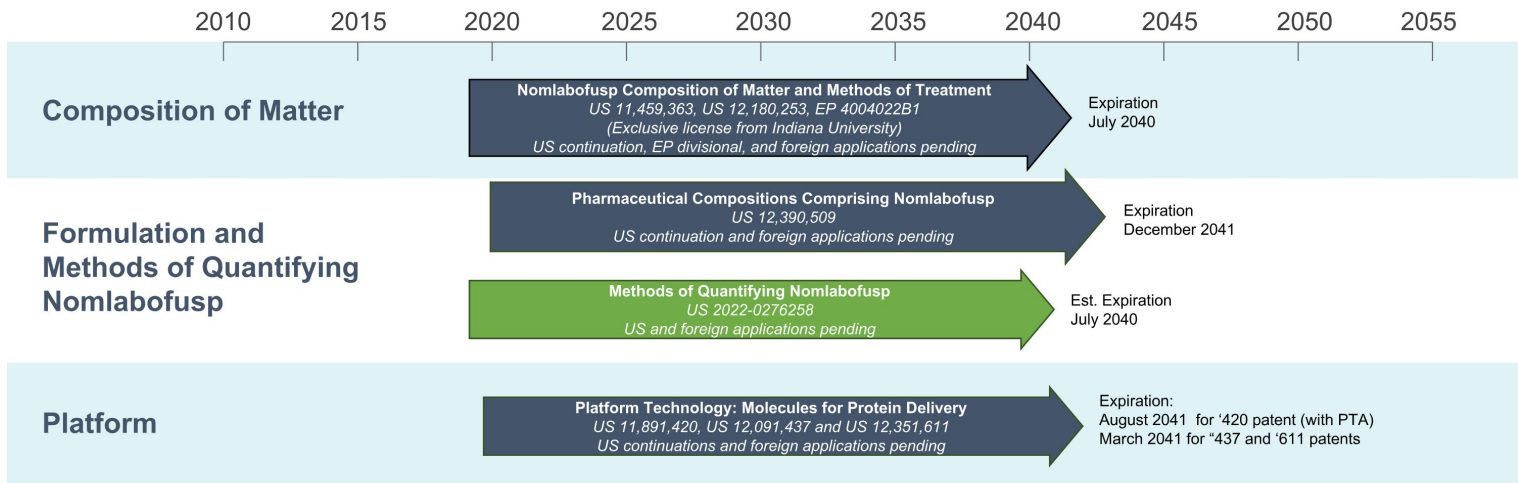
**FARS: Friedreich's ataxia rating score, measures disease progression with a higher score indicating a greater level of disability.

Advancing Nomlabofusp Towards Registration as the First Potential Disease Modifying Therapy for Friedreich's Ataxia

Targeting the Root Cause of Disease	Nomlabofusp is a first-in-class mitochondrial protein replacement therapy designed to directly address systemic frataxin deficiency in patients with FA, a rare neurodegenerative disease
Strong and Consistent Data Package	Data from 4 successfully completed studies (Phase 1 SAD and MAD, Phase 2 dose-exploration, and adolescent PK); ongoing long-term open label study supports dose-dependent increases in tissue FXN
Clear FDA Expectations	Pursuing an accelerated approval using FXN levels as a novel surrogate endpoint with written FDA recommendations on key elements for BLA submission
Regulatory Designations	Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), PRIME (EU) and ILAP (UK) designations. Selected by FDA to participate in its START pilot program
Registrational Near-Term Catalysts	Regulatory update and timeline confirmation expected in Q1 2026; BLA submission seeking potential accelerated approval targeted in Q2 2026 with a U.S. launch targeted for early 2027

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 and patents 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



Nomlabofusp Long-term Open Label Study

Expanded Open Label Study*: Now Includes Adolescents and Participants not in Prior Nomlabofusp Studies

Patient Population

Initially, adult participation in a prior Phase 1 or Phase 2 trial required

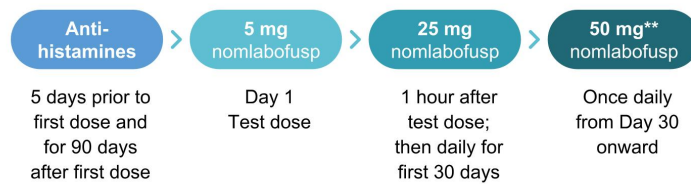
Expanded study criteria to include:

- Adolescents (12-17 yrs) from the PK run-in study
- Adult and adolescent participants not in prior studies

Plan to enroll children (2 to 11 yrs) directly in study

Dosing and Administration

Current Dose Regimen



5 days prior to first dose and for 90 days after first dose

Day 1
Test dose

1 hour after test dose; then daily for first 30 days

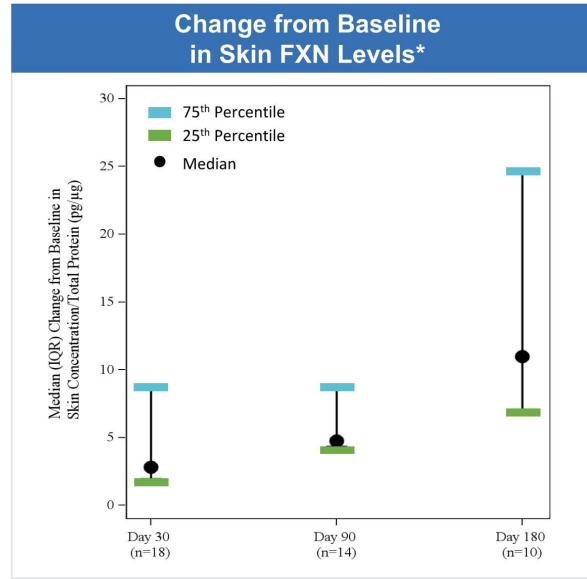
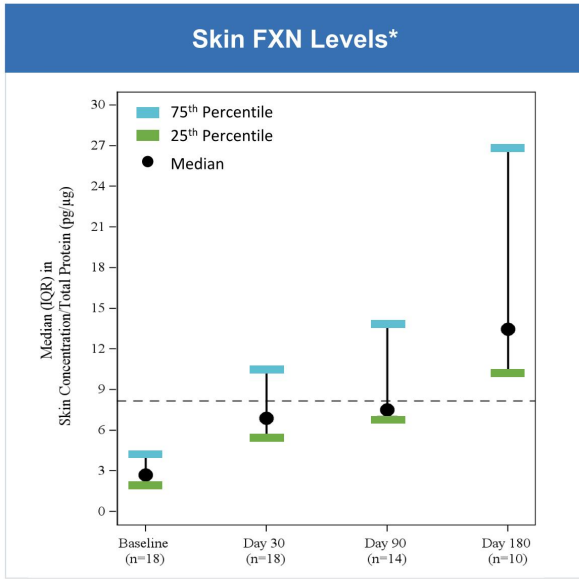
Once daily from Day 30 onward

Key Study Objectives

- Skin FXN concentrations
- Safety and tolerability
- Long-term PK
- Clinical efficacy measures relative to reference population from Friedreich's Ataxia Clinical Outcome Measures Study (FACOMS) database

Increases in Skin FXN Levels* were Sustained Over Time

100% of Participants at Day 180 had Skin FXN Levels >50% of Healthy Volunteers



Dotted Line indicates 50% of the average FXN concentrations of healthy volunteers.

*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data include all participants with quantifiable FXN levels at baseline and at least 1 post-baseline FXN level. Data presented is based on the September 2025 data release.

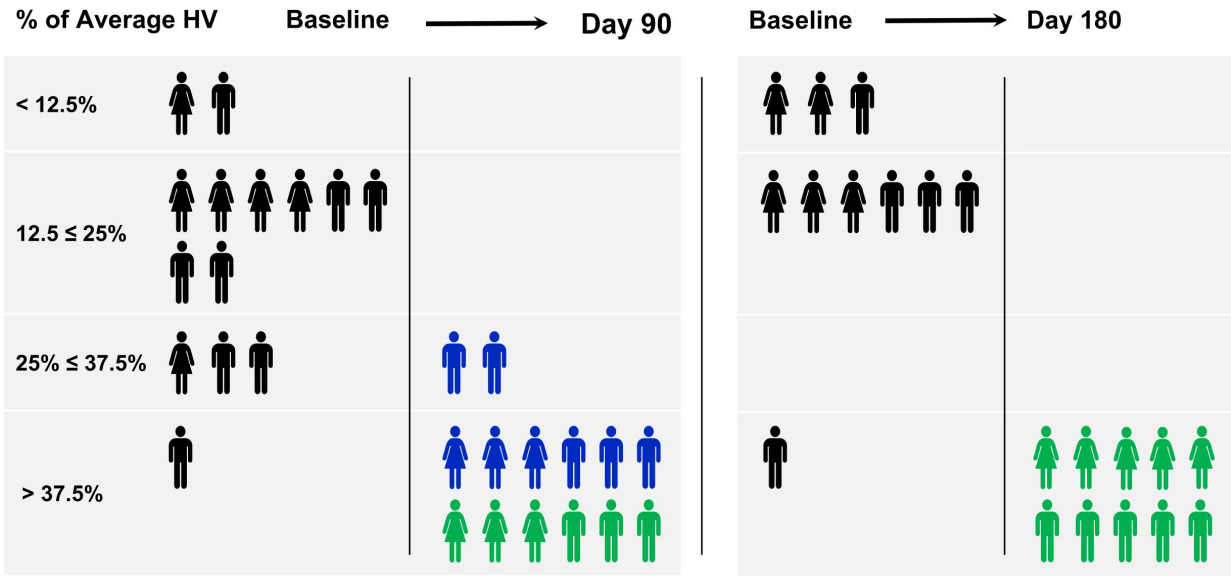
Nomlabofusp Increased FXN to Levels Similar to Asymptomatic Carriers Over Time in the Open Label Study

% of Participants* with Skin FXN Levels > 8.2 pg/μg** (50% of the average FXN concentration levels of healthy volunteers which is similar to levels in asymptomatic carriers)				Absolute Skin FXN Levels* Increase Over Time with Nomlabofusp Treatment				
Baseline	Day 30	Day 90	Day 180	Statistic	Baseline	Day 30	Day 90	Day 180
0% 0/18	33% 6/18	43% 6/14	100% 10/10	N	18	18	14	10
				Median (IQR)	2.70 (2.14, 4.13)	6.87 (5.34, 10.37)	7.50 (6.66, 13.73)	13.44 (10.10, 26.71)
				(Min, Max)	(1.5, 6.3)	(1.5, 76.4)	(5.6, 37.1)	(8.7, 92.9)



*Data include all participants with quantifiable levels at each measurement point who had received 25 mg, 50 mg or had the dose increased from 25 mg to 50 mg.
 **8.2 pg/μg represents 50% of the average FXN concentration average FXN concentration of healthy volunteers.
 Note: Data presented is based on the September 2025 data release.

Skin FXN Levels Achieved Higher % of Healthy Volunteers' FXN Levels* Following Daily Nomlabofusp



*% of average FXN level in healthy volunteers (HV); 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).

Data include all participants with quantifiable FXN levels at baseline and Day 90/Day 180.

Note: Data presented is based on the September 2025 data release.



Baseline as a percentage of average FXN level in HV



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



FXN levels increased from baseline and reached > 50% of average FXN level in HV

Nomlabofusp Safety Observations* with Long-term Treatment

- 65 total participants received at least 1 dose of nomlabofusp across all studies with 39 participants in OL study
 - Most common AEs were mild/moderate local ISRs and did not lead to any withdrawals
- 7 of 39 participants in OL study experienced anaphylaxis
 - Standard treatment with epinephrine resulted in reversal of symptoms and no late phase response or complications were observed
 - All affected participants returned to usual state of health with no further sequelae
- 10 participants in OL Study did not have prior exposure to nomlabofusp; 1 of these 10 experienced anaphylaxis
- Long-term daily dosing was generally well tolerated, including 14 on treatment for at least 6 months and 8 for over 1 year

Education and Management Measures for Risk Mitigation

Education and Awareness

Information on anaphylaxis and potential risks is included in the investigator brochure, study protocols, and informed consent forms

Investigators' guide informs how to recognize, manage, and treat anaphylaxis

Participants' guide informs how to recognize anaphylaxis and promptly administer epinephrine if needed

Management

Histamine blockers (famotidine and cetirizine) administered for 5 days prior to the first dose and for the first 90 days of treatment

Current dosing* regimen modified to potentially reduce the severity of anaphylaxis if it occurs, and may facilitate desensitization to nomlabofusp

Epinephrine auto-injectors supplied to treat anaphylaxis in the event it occurs

OL Study: Most Withdrawals Occurred in the First 90 Days of Treatment*

Reason for Withdraw	Total	Study Day 1	Study Day 2- 90	Study Day 91-180	Study Day 181-360	Study Day 360+
Anaphylaxis	7	4	3	0	0	0
Generalized Urticaria	3	0	2	1	0	0
Other Adverse Event**	3	0	1	1	0	1
Other***	2	0	2	0	0	0
Total Withdrawals	15	4	8	2	0	1

*Data presented is based on the September 2025 data release.

**Three subjects withdrawn by investigator after a report of adverse event (two subjects after experiencing pre-syncope; one subject after experiencing seizure).

***Other includes participants that withdrew upon discovery of an exclusion criterion and due to personal reasons.

Disease Characteristics – OL Study & FACOMS Reference Population

	Nomlabofusp*	FACOMS
Age of screening (years)		
n	38	370
Mean (SD)	30.2 (10.94)	27.5 (9.30)
Min, Max	12, 55	12, 54
Age of symptom onset (years)		
n	38	370
Mean (SD)	12.7 (6.09)	13.8 (5.50)
Min, Max	5, 30	5,30
Baseline mFARS Total Score		
n	38	370
Mean (SD)	55.7 (17.05)	49.7 (14.5)
Min, Max	23.3, 85.5	23.3, 80.5

	Nomlabofusp*	FACOMS
Baseline FARS-ADL Overall Score		
n	38	370
Mean (SD)	17.5 (6.84)	14.2 (5.70)
Min, Max	2, 27	2, 27
Baseline 9-HPT Average Time of Dominant Hand(s)		
n	34	370
Mean (SD)	95.4 (67.65)	124.8 (51.90)
Min, Max	35.8, 277.3	36.7, 276.5
Baseline MFIS Overall Score		
n	38	370
Mean (SD)	33.2 (15.05)	31.8 (15.60)
Min, Max	2, 79	2, 78

FACOMS longitudinal natural history study (N = 955) includes participants with confirmed FA diagnosis

Larimar identified participants from the FACOMS dataset with similar range of baseline characteristics of participants in the OL study using data recorded over the last 4 years for each participant

Improvements Across Clinical Outcomes with Nomlabofusp Relative to Worsening in FACOMS Reference Group Supports Potential Clinical Benefits

		mFARS [0- 93]		FARS-ADL [0- 36]		9-HPT Dominant Hand [Seconds]		MFIS [0- 84]	
	Statistic	Nomlabofusp	FACOMS ¹	Nomlabofusp	FACOMS ¹	Nomlabofusp	FACOMS ¹	Nomlabofusp	FACOMS ¹
Baseline	Median (IQR)	54.75 (41.2, 71.0)	50.00 (37.0, 61.0)	17.75 (13.0, 24.5)	14.50 (10.0, 18.5)	71.95 (49.6, 114.8)	113.50 (86.5, 148.5)	34.00 (20.0, 34.0)	32.00 (21.0, 42.0)
	n	38	370	38	370	34	370	38	370
Change from Baseline at 1 year	Median (IQR)	-2.25 (-3.75, -0.25)	1.00 (-1.5, 4.0)	-0.50 (-2.0, 1.0)	0.50 (-1.0, 2.5)	-7.40 (-38.8, -2.5)	3.40 (-4.5, 18.0)	-6.50 (-17.5, 4.0)	1.50² (-9.5, 11.0)
	n	8	185	8	237	7	219	8	136

IQR = interquartile range

¹ Based on the range of baseline characteristics of participants in the OL study, Larimar identified patients from the FACOMS dataset with similar characteristics using data recorded over the last 4 years for each patient.

² Modified Fatigue scale presented here is at Month 24 because it was not assessed at Month 12.

Note: Data presented is based on the September 2025 data release.

Benefit-Risk Profile Underpins the Potential of Nomlabofusp

Based on FDA October 2023 Guidance on Benefit-Risk Assessment Framework for Biological Products

- 1 Potentially addresses unmet need by increasing tissue frataxin levels to treat the underlying cause of FA in both adults and children with this progressive and debilitating disease
- 2 Clinical data suggests increases in frataxin levels may result in slowing or halting disease progression
- 3 Anaphylaxis occurred in some patients and was treated with epinephrine, resulting in reversal of symptoms and return to usual state of health with no further sequelae
- 4 Generally well-tolerated long-term safety profile in complex patients; most common AEs were mild/moderate local ISRs and did not lead to any withdrawals

Path to Planned BLA Submission in Q2 2026 Seeking Accelerated Approval

Expected Elements of BLA Submission	
Use of Skin FXN Levels as a Surrogate Endpoint	<ul style="list-style-type: none"> Justification for using increases in skin FXN concentrations from participants in the OL study as a reasonably likely surrogate endpoint (RLSE) FDA has stated that acceptability of increases in skin FXN for accelerated approval will be decided during future BLA review
Safety Database	<ul style="list-style-type: none"> At least 30 participants with continuous exposure for 6-months A subset of at least 10 participants with continuous exposure for 1-year A large majority of the exposure should be on the 50 mg dose
Clinical Data Package & Global Phase 3 Study	<ul style="list-style-type: none"> Data from 4 successfully completed studies (Phase 1 SAD and MAD, Phase 2 dose-exploration, and adolescent PK) and an ongoing long-term OL study OL study data <ul style="list-style-type: none"> Results to date: 10/10 participants in OL study with data at 6 months achieved skin FXN levels over 50% of median levels in healthy volunteers (which is similar to levels in asymptomatic carriers) Consistent directional improvement in OL study across 4 key clinical outcomes (mFARS, FARS-ADL, 9-HPT, MFIS) observed after 1 year of nomlabofusp treatment relative to a worsening in a FACOMS natural history study reference population could suggest potential for clinical benefit Global Phase 3 study, intended as the confirmatory study, to evaluate clinical outcomes including upright stability and mFARS expected to be underway at the time of BLA submission
Pharmacology & Toxicology	<ul style="list-style-type: none"> Nonclinical data supporting a relationship between increased skin FXN and relevant tissues such as the heart, dorsal root ganglion, and skeletal muscle Complete toxicology package including juvenile toxicology study
Chemistry Manufacturing & Controls	<ul style="list-style-type: none"> Data supporting the lyophilized drug product with stability at room temperature Data on batches manufactured at a commercial scale Analytical methods and proposed specifications



FXN: Frataxin; OL: Open label study; BLA: Biologics License Application; FDA: Food and Drug Administration; RLSE: reasonably likely surrogate endpoint; SAD: Single ascending-dose ; MAD: Multiple ascending dose

Global Phase 3 Double-blind Placebo-controlled Study

Patient Population

- Ambulatory participants
- 2 – 40** years of age (~2/3 under 21 years of age)
- n = 100 – 150

Placebo

50 mg nomlabofusp*

Daily subcutaneous injections self-administered or by a caregiver

← 18 months of treatment →

Key Study Objectives

- Safety and tolerability
- Upright stability (U.S.) and mFARS (Europe) as primary outcome measures

Qualifying sites in U.S., Europe, U.K., Canada, and Australia



*5 mg nomlabofusp test dose, then 25 mg 1 hour later and daily for the first 30 days followed by 50 mg daily.

**Study will initiate with participants 12-40 yrs of age and will change to 2-40 yrs when dose is confirmed in children 2-11 yrs of age.

Nomlabofusp Advancing Towards BLA Submission for FA

First potential disease modifying therapy

Designed to systemically address FXN deficiency in FA

FDA clarity on key BLA elements

BLA based on skin FXN levels as potential novel surrogate endpoint

Positive long-term data

Increased skin FXN levels similar to levels expected in asymptomatic carriers and consistent directional improvement across 4 key clinical outcome measures

BLA submission seeking accelerated approval targeted Q2 2026

U.S. launch targeted for early 2027

\$136.9 million* in estimated cash and investments at 12/31/25 with projected runway into Q4 2026



*Estimate is unaudited and preliminary and actual results may differ due to the completion of our December 31, 2025 closing procedures and issuance of our December 31, 2025 financial statements prepared in accordance with U.S. generally accepted accounting principles.



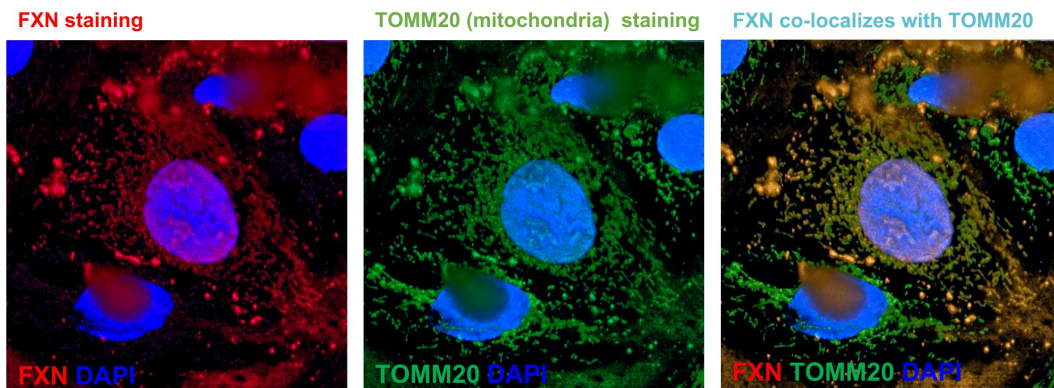
Larimar Therapeutics

Appendix



Mitochondrial Localization and Preclinical Data

Nomlabofusp Cell Transduction In Vitro Leads to hFXN 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

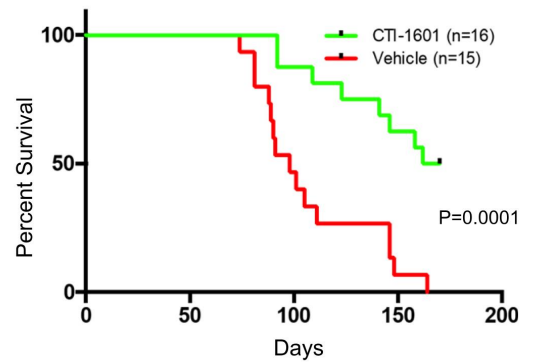
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

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

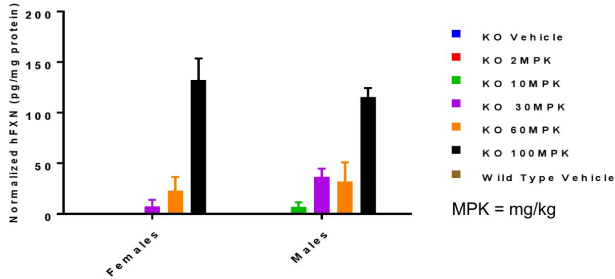
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

- ☑ 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.

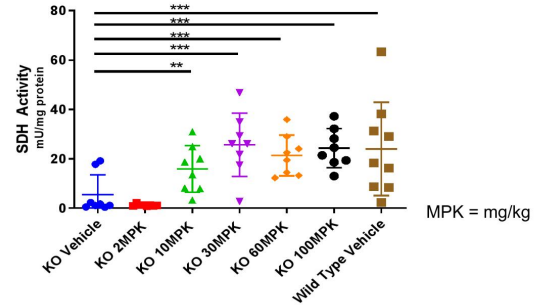
Mitochondrial FXN (Heart)



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



SDH Activity (Muscle)

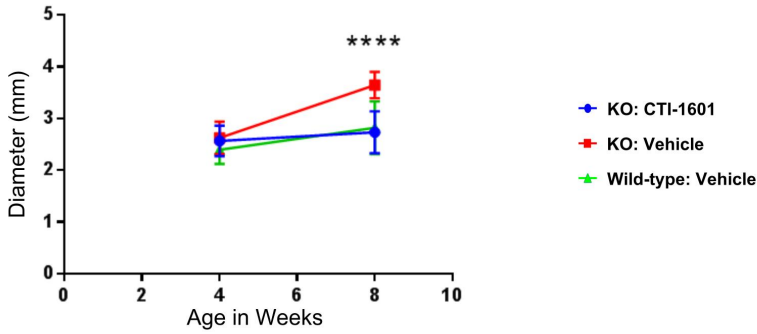


Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type

Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

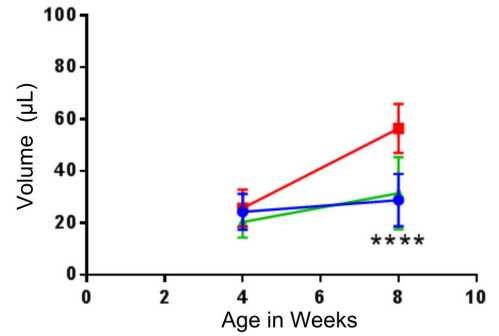
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

Left Ventricle Internal Diameter (Systole)



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

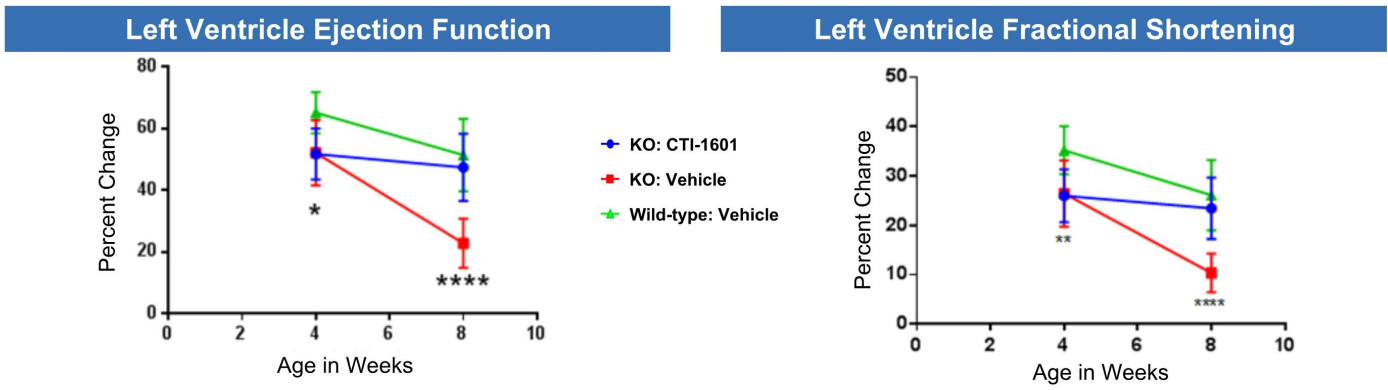
Left Ventricle Volume (Systole)



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

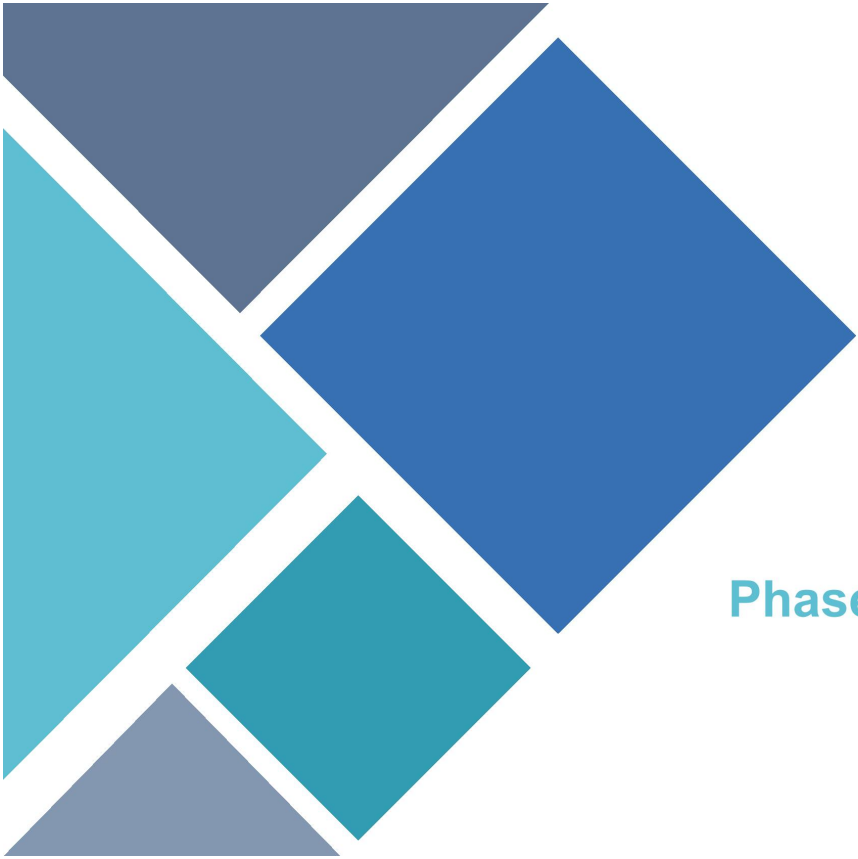
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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 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



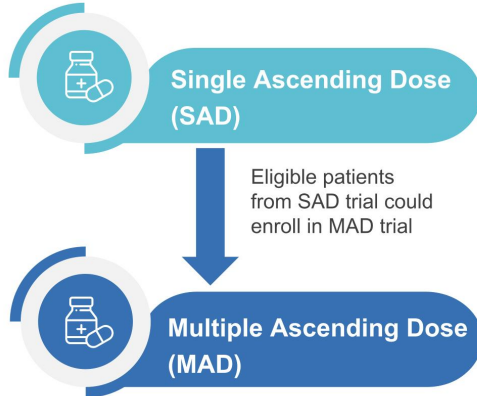
Phase 1 Clinical Data

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



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

Number of Subjects: 27

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

Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days

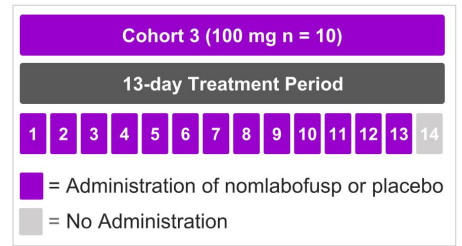
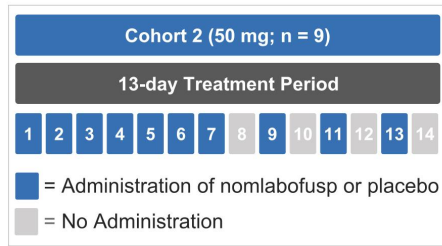
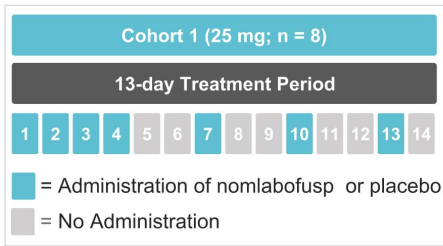
1° Endpoint: Safety and tolerability

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

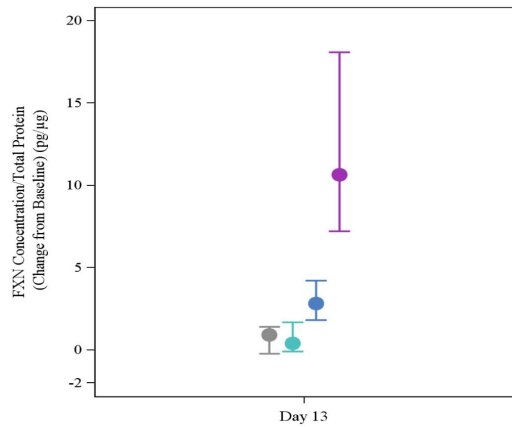
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Cohort 3 Sampling Days

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

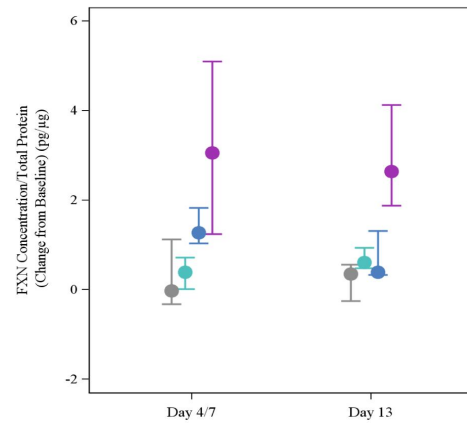
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

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)

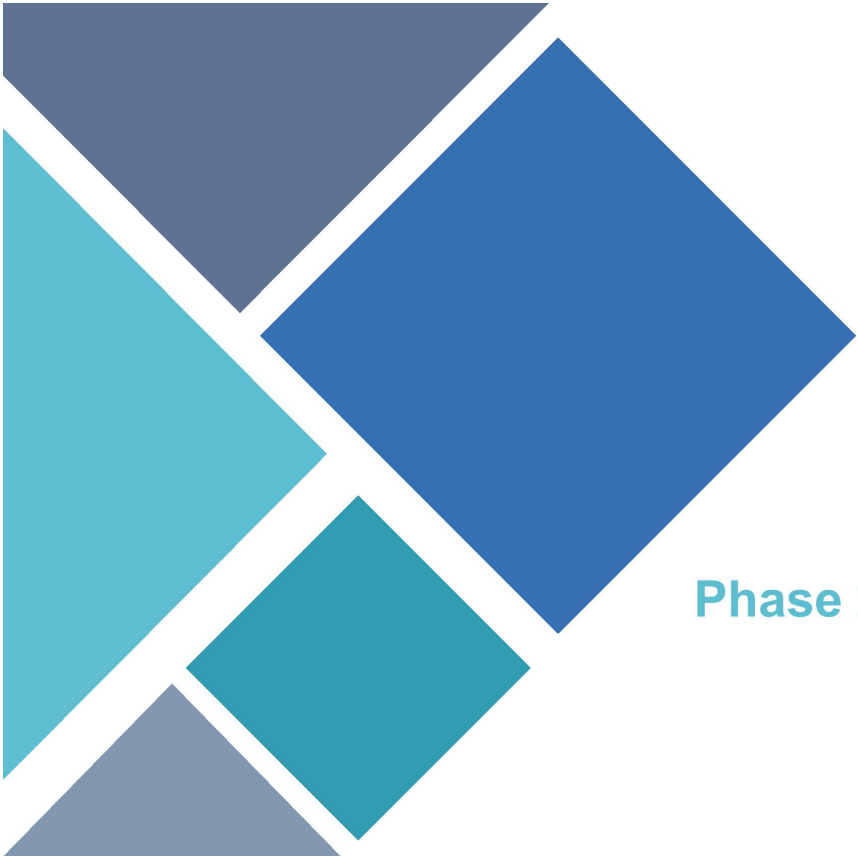
MAD Trial Patient Disease Characteristics

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 Dose Exploration Data



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

28-day Treatment Period - nomlabofusp (CTI-1601) or placebo



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

 = No Administration

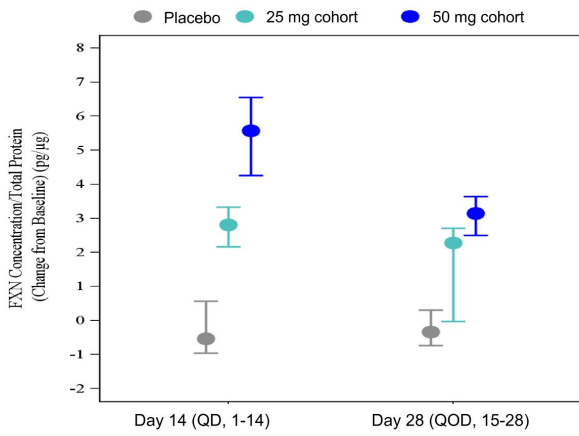
Study Details

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)
Key Results	Generally well tolerated; most common adverse events were mild and moderate injection site reactions Dose dependent increases of frataxin levels in tissues tested (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% of the average of the healthy volunteers

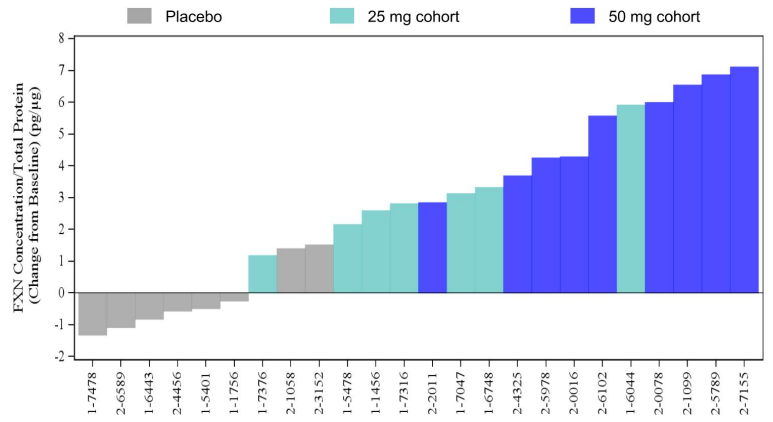
Dose-Dependent Increase in FXN Levels in Skin Cells

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

**FXN Levels* in Skin Cells
Change from Baseline****



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

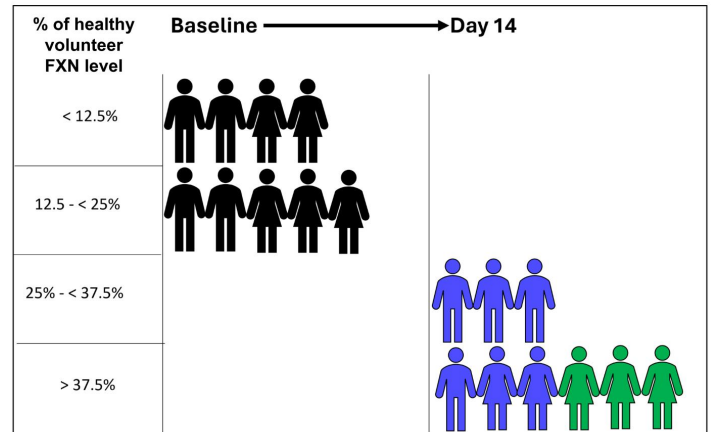
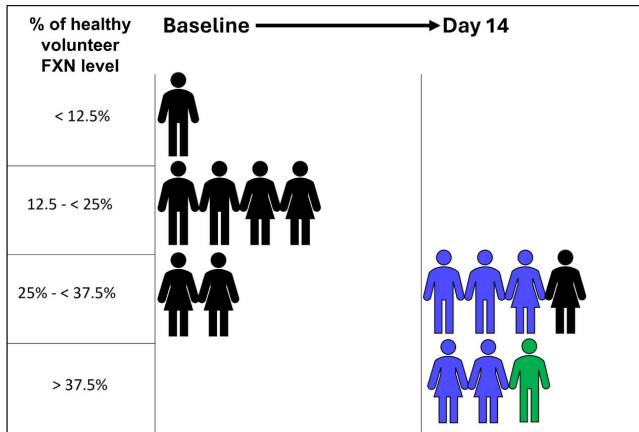


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.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

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).

Nomlabofusp PK Profile Consistent Across Studies

Long-term PK Profile Consistent with Phase 1 and Phase 2 Studies

- Rapid absorption after subcutaneous administration
- Steady state reached by Day 30 at both the 25 mg and 50 mg doses with no further accumulation
- Pharmacokinetic profile consistent with Phase 1 and Phase 2 studies

Adolescent PK Profile Consistent with Adult

- Adolescents 12 to 17 years of age received a weight-based equivalent of 50 mg for 7 days
- Exposure and PK in 9 adolescents 12 to 17 years of age on nomlabofusp was similar to adults on 50 mg of nomlabofusp

Elevated TGs in FA Decreased with Nomlabofusp and Correlated with FXN Increases

In patients with FA from Phase 2 dose exploration study after treatment with nomlabofusp

Plasma lipids at baseline were compared to Day 28 after nomlabofusp treatment

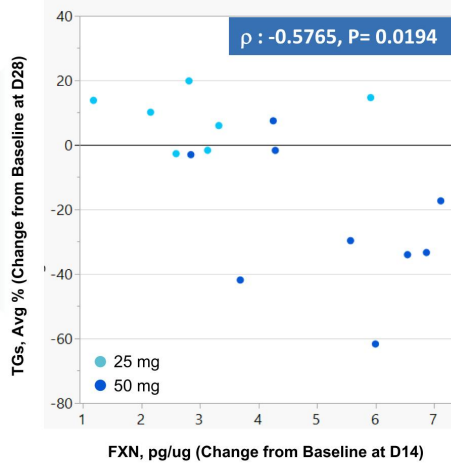
↓

27 TGs* were identified as nomlabofusp-responsive and were typically elevated at baseline in patients with FA

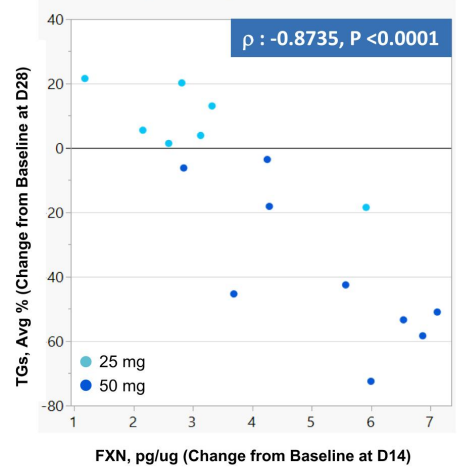
↓

Baseline elevated TGs decreased towards levels in healthy volunteers and correlated with FXN levels after nomlabofusp treatment

27 elevated TGs correlated with skin FXN levels
Representing ~28% of total TGs



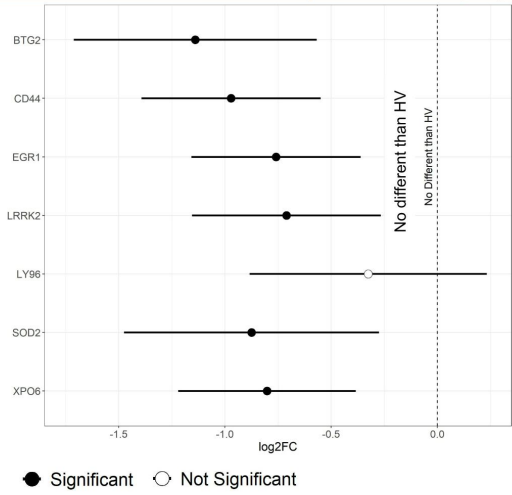
Subset of 4 TGs were highly correlated with skin FXN levels
Representing ~22% of total TGs



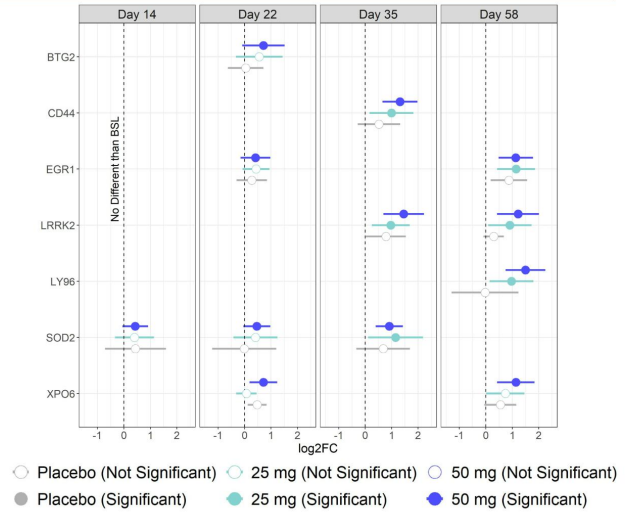
Plasma samples were collected before, during, and after treatment for lipid profiling from the Phase 2 dose exploration study evaluating nomlabofusp 25 mg and 50 mg or placebo daily for 14 days followed by alternate day administration for 14 days
*Triglycerides (TG) were selected with a median fold-change (≥ 1.25 fold) post-treatment vs. baseline, a correlation (r value ≥ 0.4) between baseline and post-treatment results in the 50 mg group with consistent directionality in the 25 mg group and no changes in the placebo group

Increase Towards Normal Gene Expression in Adults with FA* Observed After Nomlabofusp Treatment

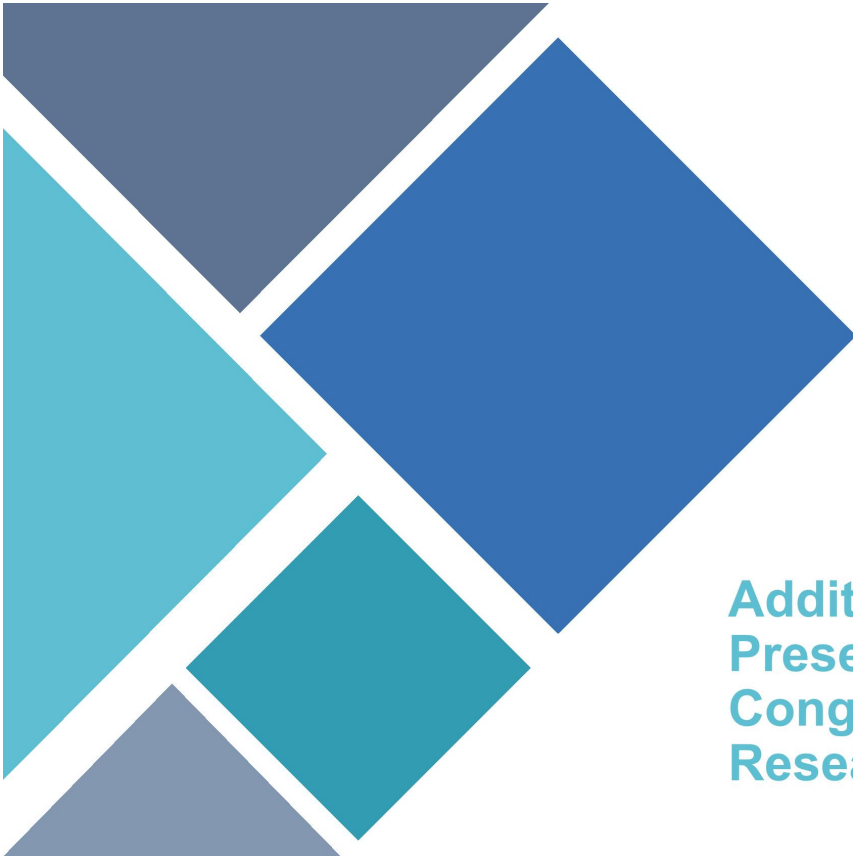
Select Baseline Gene Expression Patients with FA* vs. Healthy Volunteers (HV)**



Post-treatment Changes in Gene Expression From Baseline



Data presented at the International Congress for Ataxia Research, November 2024
 *Samples from Phase 2 dose exploration study evaluating nomlabofusp 25 mg (Cohort 1) and 50 mg (Cohort 2) or placebo via subcutaneous injection daily for 14 days followed by alternate day administration for 14 days. Buccal samples were collected before, during, and after treatment for gene expression profiling
 **Data from Larimar's non-interventional healthy volunteer study



**Additional Phase 1 and 2 Data
Presented at the International
Congress for Ataxia
Research, November 2024**

Nomlabofusp Clinical Studies Included a Broad, Representative Population of Adults with FA

Broad population of adults with FA included in Phase 1 and 2 Studies

Age of onset between 5 - 60 years with a median age of onset of 15 yrs

81% of participants had FXN levels at baseline less than 30% of healthy controls and 37% of participants had less than 20%

Over 50% of participants were non-ambulatory at baseline

**18 subjects participated in more than 1 study*

***Quantifiable buccal cell FXN levels relative to the median of healthy controls*

****Ambulatory status is based on the gait score (E7=5 vs. <5) of the upright stability subscore of the mFARS*



*****Data presented at the International Congress for Ataxia Research, November 2024*

Demographics and Baseline Disease Characteristics from Nomlabofusp Phase 1 and 2 Interventional Studies****

	N*	Median	Mean	Min	Max
Age	61	28.0	31.9	19	69
Age of Onset	61	15.0	15.9	5	60
Age of Diagnosis	61	19.0	21.0	5	64
Shorter GAA (GAA₁)	60	550.0	555.8	99	1000
Longer GAA (GAA₂)	60	900.0	890.2	265	1300
Fratxin, % of Control**	57	24.4	23.9	8.7	61.9
mFARS Score	61	52.0	49.5	13.2	74.5
Upright Stability Score	61	32.0	26.9	7.0	35.0
Dominant hand 9-hole peg test	61	71.0	84.8	26.0	229.2
T25-FW Test Score	51	9.9	13.4	4.3	48.5
Left Ventricular Mass (g)	61	163.4	168.0	73.7	398.8
LVEF %	61	63.0	63.5	52	76
Ambulatory Status***					
No	36				
Yes	25				

Pooled Data from Completed Phase 1 & 2 Studies Confirms Disease & FXN Relationships are Consistent with Literature

Disease Characteristics by Quartiles Based on Buccal Cell FXN Levels at Baseline

Quartile	FXN Concentration* (pg/mcg)	Age at Symptom Onset**	Age at Diagnosis**	GAA ₁ **	GAA ₂ **
Q1 (N=14)	< 1.31	10.5	14.5	616.5	899.5
Q2 (N=14)	1.31 - <1.95	13.5	23.0	486.0	866.0
Q3 (N=14)	1.95 - <2.30	16.0	19.0	555.0	871.5
Q4 (N=15)	≥ 2.30	19.0	27.0	400.0	933.0

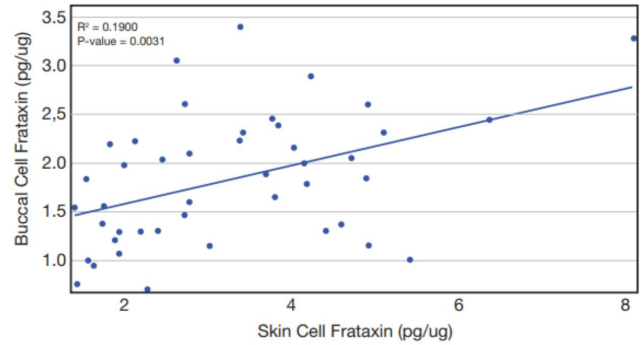
*Quantifiable buccal cell frataxin levels

**Median values

Median buccal cell FXN concentration in healthy controls = 8.1 ng/mcg

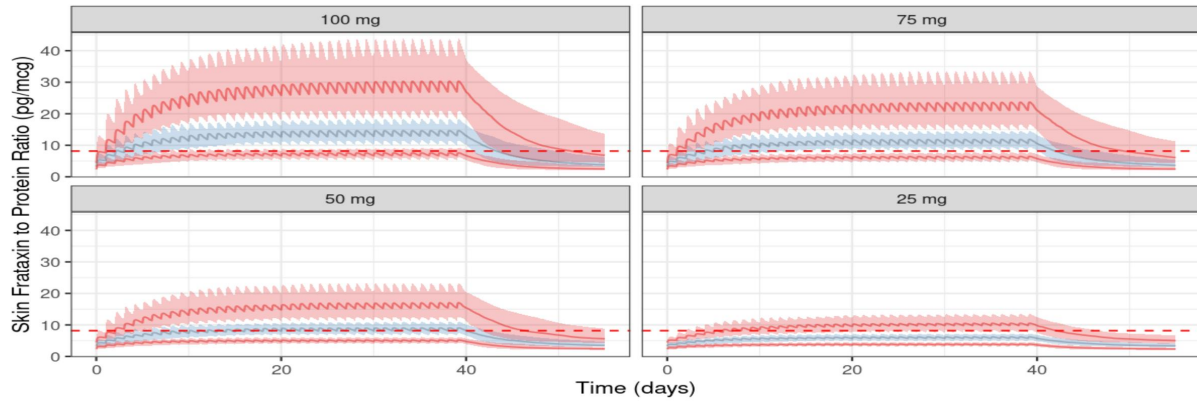
Buccal cell FXN levels correlated with age of onset and inversely correlated with the number of GAA repeats and rate of disease progression

Baseline Buccal and Skin Cell FXN Levels



Buccal cell FXN levels correlated with skin cell FXN levels

Modeling/Simulation Predicts* 50mg Daily Can Achieve Skin FXN Levels $\geq 50\%$ of Healthy Controls in Most Patients



Dashed red line – 50% the average skin FXN/protein ratio (8.17 pg/ug) in a non-interventional study in healthy controls (HC)
Blue line – median of simulated values across trials
Red lines – 10th and 90th percentiles
Shaded regions – 95% confidence intervals of the corresponding percentiles (10th, 50th, and 90th).

Data presented at the International Congress for Ataxia Research, November 2024

50 mg nomlabofusp daily was predicted to lead to:
 A median increase of 5.64 (2.3 – 13.5) pg/ μ g in FXN levels from baseline

Increase in skin FXN levels in 59% of simulated patients with FA to levels $\geq 50\%$ of average skin FXN levels in HC



*PK/PD model was developed with data collected from 3 completed studies in adults with FA. A population of virtual FA patients (n = 100, 100 trials) receiving subcutaneous daily doses of 25, 50, 75, or 100 mg nomlabofusp for 40 days was simulated

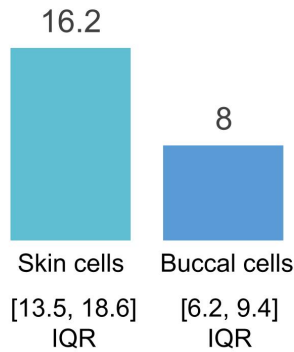


Non-Interventional Study Data

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



FDA START Pilot Program

START Pilot Program Continues to Expedite the Clinical and Regulatory Development of Nomlabofusp

START Pilot Program

Support for Clinical Trials Advancing Rare Disease Therapeutics

1 of 7 novel drugs development programs selected by FDA

A new milestone-driven program launched by the FDA in September 2023

Designed to accelerate the development of novel therapies for rare diseases

Sponsors selected can benefit from:

- more frequent and rapid ad-hoc FDA interactions
- help facilitating the development of programs to pre-BLA meeting stage
- guidance on generating high-quality and reliable data intended to support a BLA

CDER Selection Based On

Demonstrated development **program readiness**

Potential to address serious and unmet medical need in a **rare neurodegenerative condition**

Alignment of CMC development timelines with clinical development plans

Proposed plan where **enhanced communication can improve efficiency of product development**



FDA: Food and Drug Administration; CDER: Center for Drug Evaluation and Research; CMC: Chemistry, Manufacturing, and Controls



FARA

Strong Relationship with FARA – Joined FARA’s TRACK-FA Neuroimaging Consortium as an Industry Partner

TRACK-FA collects natural history data to establish disease specific neuroimaging biomarkers for potential use in clinical trials. Larimar will have access to all study data for use in regulatory filings, as appropriate

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”



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