

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): February 24, 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 8.01 Other Events.*****Press Release***

On February 24, 2026, Larimar Therapeutics, Inc. (the “Company”) issued a press release announcing that the U.S. Food and Drug Administration has granted Breakthrough Therapy Designation to nomlabofusp for the treatment of adults and children with Friedreich’s ataxia. A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

***Corporate Presentation***

On February 24, 2026, the Company posted on its website an updated slide presentation, which is attached as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. Representatives of the Company will use the presentation in various meetings with investors, analysts and other parties from time to time.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

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

Exhibit No.	Document
99.1	<a href="#">Press release of Larimar Therapeutics, Inc., dated February 24, 2026*</a>
99.2	<a href="#">Larimar Therapeutics, Inc. Corporate Presentation, dated February 24, 2026*</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)
*	Filed 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: February 24, 2026

By: /s/ Carole S. Ben-Maimon

*Name: Carole S. Ben-Maimon*

*Title: President and Chief Executive Officer*

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## Larimar Therapeutics Announces FDA Breakthrough Therapy Designation for Nomlabofusp in FA and Reiterates Planned BLA Submission in June 2026

- *Nomlabofusp program granted Breakthrough Therapy Designation for the treatment of adults and children with FA based on FDA's review of available clinical data from open label study*
- *FDA written communications after recent START meeting continue to align with use of skin FXN to support BLA submission seeking accelerated approval*
- *Topline open label study data to support BLA submission expected in Q2 2026*
- *Planned BLA submission seeking accelerated approval on track for June 2026; U.S. launch targeted for first-half 2027, if approved*

**Bala Cynwyd, PA**, February 24, 2026 – Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) to nomlabofusp, a frataxin (FXN) protein replacement therapy with disease modifying potential, for the treatment of adults and children with Friedreich's ataxia (FA). Additionally, after a recent Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program meeting with FDA, the Company announced continued alignment with the FDA to consider the use of skin FXN as a novel surrogate endpoint reasonably likely to predict clinical benefit to support a planned Biologics License Application (BLA) submission seeking accelerated approval. There was also agreement on the relevant clinical outcomes with consistent directional improvement in all four clinical outcomes assessed and the type of analyses required to support the exposure response relationships for the nomlabofusp program. The FDA stated that the adequacy of the safety database will be a matter of review at the time of BLA submission. The planned BLA submission is targeted for June 2026.

Breakthrough Therapy Designation and FDA feedback on BLA submission were based on the FDA's review of available clinical data from the Company's ongoing open label (OL) study evaluating nomlabofusp in adult and pediatric patients with FA.

### **Breakthrough Therapy Designation Granted by FDA for Nomlabofusp for the Treatment of FA**

Breakthrough Therapy Designation is intended to expedite the development and regulatory review of a drug intended to treat a serious condition. A drug is eligible for BTD if preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available treatments in one or more clinically significant endpoints.

The Breakthrough Therapy Designation request for nomlabofusp included preliminary clinical data from the OL study demonstrating increases in skin FXN to levels expected in asymptomatic carriers and consistent directional improvement across four key clinical outcomes including modified Friedreich Ataxia Rating Scale (mFARS) score, FARS-Activities of Daily Living (ADL), 9 Hole Peg Test (9-HPT), and Modified Fatigue Impact Scale (MFIS) after 1-year on treatment. These findings reinforce the potential of nomlabofusp to improve FA's disease course relative to a worsening observed in a reference group from the Friedreich's Ataxia Clinical Outcomes Measure Study (FACOMS) natural history study.

"There continues to be a substantial burden of disease affecting the estimated 5,000 children and adults in the U.S. living with FA. Receiving Breakthrough Therapy Designation underscores the FDA's recognition of the high unmet medical needs and the potential for nomlabofusp to demonstrate a substantial improvement over available therapy on clinically significant endpoints," said Dr. Rusty Clayton, Chief Medical Officer of Larimar. "As part of the BTD request, the FDA reviewed preliminary nomlabofusp data demonstrating improvements in mFARS score, ADL, 9-HPT performance, as well as decreased fatigue, in the context of increased tissue FXN to levels similar to those observed in asymptomatic carriers with no signs of disease. We are encouraged by the increasing body of clinical data supporting the potential of

nomlabofusp to modify disease progression by targeting the root cause of FA, FXN deficiency. We look forward to continued collaboration with the FDA as we proceed toward potential registration.”

### FDA Meeting Comments Support a Planned BLA Submission for Nomlabofusp in June 2026

Dr. Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar added, “We are pleased to have continued engagement with the FDA on our planned BLA submission for nomlabofusp and we appreciate FDA’s thorough review of the preliminary clinical data. This regulatory progress supports our BLA readiness seeking accelerated approval and allows us to focus on continued execution. We are committed to ensuring a robust and comprehensive data package that captures the favorable benefit-risk profile of nomlabofusp and its potential to meaningfully improve outcomes for patients with FA. We continue to plan for a June 2026 BLA submission seeking accelerated approval and are excited to initiate our confirmatory Phase 3 study in the U.S., E.U., U.K., Canada and Australia. We are proud to have clinical trial applications related to our Phase 3 study currently under review in France and Canada, with submission to U.K. regulatory authorities soon to follow.”

In connection with a recent START meeting, the FDA reviewed preliminary clinical data for the nomlabofusp program and continued to provide encouraging feedback on BLA content:

- **FXN as Surrogate Endpoint:** FDA reaffirmed willingness to consider use of FXN as novel surrogate endpoint and confirmed Larimar’s exposure-response analysis exploring the relationship between nomlabofusp exposures and clinical outcome measures is the type that could support the future BLA submission.
- **Reference Population:** FDA confirmed the process proposed for selecting a reference population based on matched subjects from the FACOMS database for the natural history comparisons of clinical endpoints to be used for BLA submission and offered to provide advance review and comment on the statistical plan.
- **Safety Dataset:** FDA stated that the adequacy of the safety dataset will be a matter of review at the time of BLA submission.
- **Global Phase 3 Study:** FDA is aligned with plans to have the global confirmatory Phase 3 study underway at the time of BLA submission and confirmed that change from baseline in the Upright Stability Score (USS) (a subscale of mFARS) is a reasonable and clinically relevant primary endpoint for the planned Phase 3 study.

Dr. Marshall Summar, Chief Executive Officer (CEO) of Uncommon Cures, past Founding Director of the Rare Disease Institute and Margaret O’Malley Chair of Genetic Medicine at Children’s National Hospital added, “As the CEO of a key clinical site in the OL study and a career Medical Geneticist, I have seen firsthand the significant burden that FA places on patients and their families. The data generated to date suggest that nomlabofusp has the potential to meaningfully impact the underlying biology of the disease and translate into clinically relevant benefits. The clinical improvements observed so far are promising and mark a meaningful step toward what could become the first disease-modifying therapy for a patient population with significant unmet medical needs.”

### Expected Near-term Milestones

- Topline OL study data to support BLA submission expected in Q2 2026
- Plan to initiate screening in global confirmatory Phase 3 study in Q2 2026; dosing of first patient expected mid-2026
- BLA seeking accelerated approval planned to be submitted in June 2026
- U.S. launch targeted for first-half 2027, if approved

### 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

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platform to design other fusion proteins to target additional rare diseases characterized by deficiencies in intracellular bioactive compounds. For more information, please visit: <https://larimartx.com>.

### **Forward-Looking Statements**

This press release contains forward-looking statements that are based on Larimar's management's beliefs and assumptions and on information currently available to management. All statements contained in this release other than statements of historical fact are forward-looking statements, including but not limited to statements regarding Larimar's ability to develop and commercialize nomlabofusp and 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 potential for accelerated approval or accelerated access and time to market 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", "target" 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 and regulatory 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; Larimar's ability to realize the benefits of Breakthrough Therapy Designation; 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.

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# Larimar Therapeutics

## Corporate Deck

February 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.

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# Nomlabofusp Program Granted FDA Breakthrough Therapy Designation for the Treatment of Adults and Children with Friedreich's Ataxia

## FDA Breakthrough Designation

Designed to expedite the development and regulatory review of a drug for a serious condition

Eligibility requires preliminary clinical evidence which indicates that the drug may demonstrate substantial improvement over available treatments for clinically significant endpoints

## Clinical evidence from open label study supporting BTB included:

In participants with 6-months of data and daily administration of nomlabofusp for the full 6-months, **100% (10/10)** of participants achieved FXN levels similar to asymptomatic carriers

Consistent directional improvement across key clinical outcomes including mFARS score, ADL, 9-HPT, and MFIS after 1-year of treatment

**Reinforces the potential of nomlabofusp to be disease modifying & improve FA's disease course**

# Nomlabofusp Awarded Multiple Global Regulatory Designations Intended to Expedite and Incentivize the Development Program

## US Designations

### Breakthrough Therapy Designation

START Pilot Program

Orphan Drug Designation

Fast Track Designation

### Rare Pediatric Disease Designation

*Priority review voucher program extended to 2029*

## Global Designations

Orphan Drug Designation (EU)

PRIME Designation (EU)

Innovative Licensing and Access

Pathway (ILAP) (UK)

# FDA Engagement on Planned BLA Submission for Nomlabofusp Program

Submission continues to be targeted for June 2026

FXN as Surrogate Endpoint	Safety Database	Reference Population	Global Phase 3 Study
<p>Continued alignment with FDA to consider the use of skin FXN as novel surrogate endpoint reasonably likely to predict clinical benefit</p> <p>FDA confirmed Larimar's exposure-response analysis exploring the relationship between nomlabofusp exposures and clinical outcome measures is the type that could support future BLA submission</p>	<p>As part of the recent START meeting and BTD application, Larimar submitted available safety and exposure data to FDA for review</p> <p>FDA stated that the adequacy of the safety database will be a matter of review at the time of BLA submission</p>	<p>FDA confirmed the process proposed for selecting a reference population and will review and comment on statistical analysis plan</p> <p>Analysis will identify subjects in the FACOMS database who have baseline characteristics that match those of participants who have data after 1-year of treatment with nomlabofusp in the OL study</p>	<p>FDA alignment to have global Phase 3 study underway at time of BLA submission</p> <p>Confirmed that change from baseline in Upright Stability Score (a subscale of mFARS) is a reasonable and clinically relevant primary endpoint</p>



Note: Data is based on the September 2025 data release with appropriate safety updates.

## Multiple Planned Near-term Milestones for Potential Registration



**\$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.

# 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



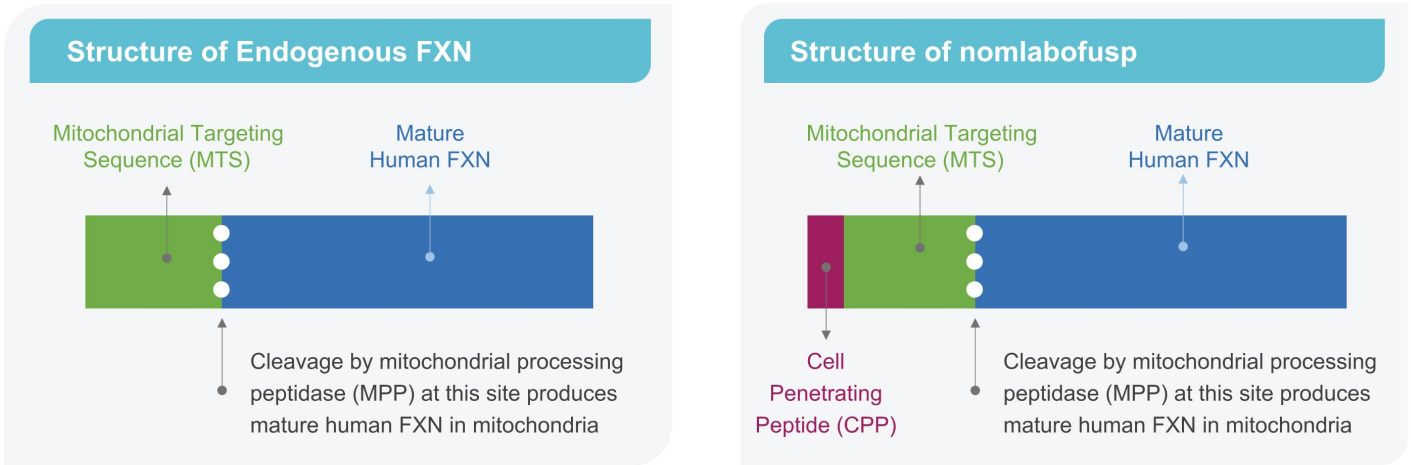
**Larimar is developing nomlabofusp as the first potential disease modifying therapy for FA. Designed to potentially save patients from enormous suffering and deterioration of quality of life.**



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

# Nomlabofusp is Designed to Target the Root Cause of FA, FXN Deficiency

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 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.

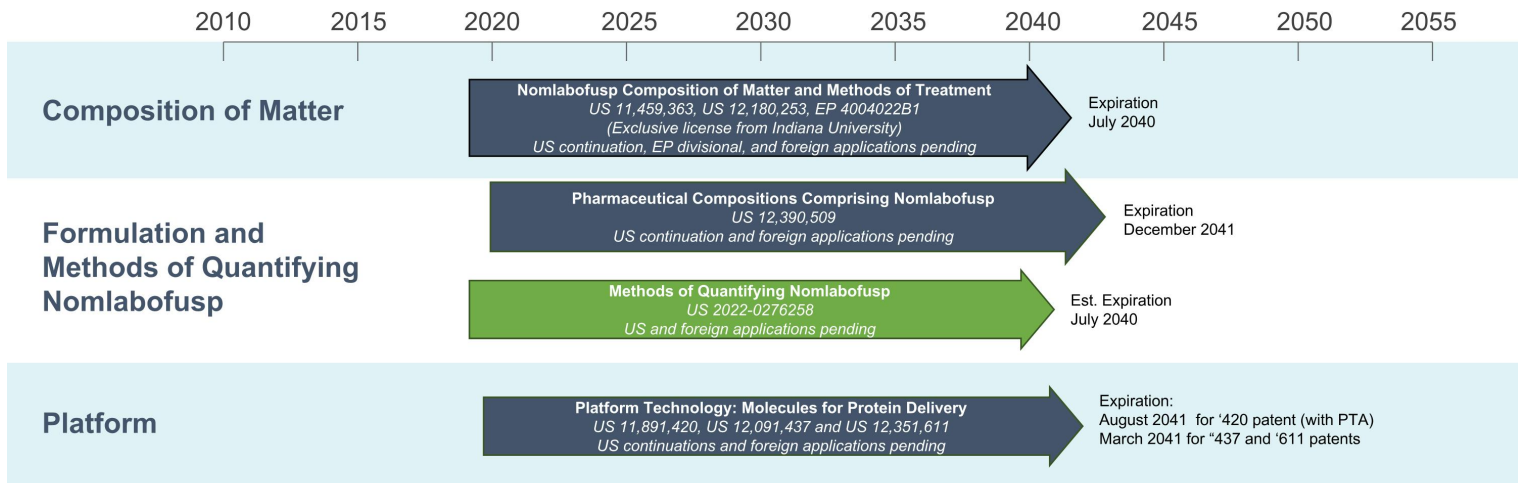
# Robust Clinical Development Program to Support Nomlabofusp Planned BLA Submission Seeking Accelerated Approval

Consistent, dose-dependent increases in FXN levels in skin and buccal cells across the development program

 <b>Phase 1 SAD</b> Ph 1 single ascending dose study	Assessed the safety, tolerability, PK and PD of 25, 50, 75, and 100 mg dose levels of nomlabofusp vs placebo in 28 participants with FA
 <b>Phase 1 MAD</b> Ph 1 multiple ascending dose study	Assessed the safety, tolerability, PK, and PD of nomlabofusp vs placebo in 27 participants with FA. 25 mg (Days 1-4, 7, 10, 13), 50 mg (Days 1-7, 9, 11, 13), and 100 mg doses (Days 1-13) were evaluated
 <b>Phase 2 Dose Exploration Study</b>	Randomized, double-blind, placebo-controlled study assessing nomlabofusp in 28 adult participants with FA treated for 28 days with 25 mg or 50 mg of nomlabofusp or placebo
 <b>Adolescent PK</b> Phase 1 study	Assessed the safety, tolerability, PK, and PD of nomlabofusp in 14 adolescents ages 12 to < 18 years with FA; participants were treated for 7 days with 0.8 mg/kg (maximum 50 mg) of nomlabofusp or placebo
<b>Ongoing OL Study</b> Open label study	Assessing the long-term safety, efficacy, PK, PD, and tolerability of nomlabofusp in participants with FA

# 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

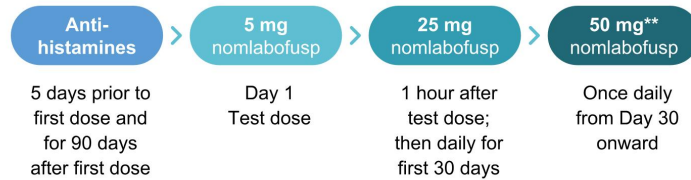
Expanded study criteria to include:

- 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

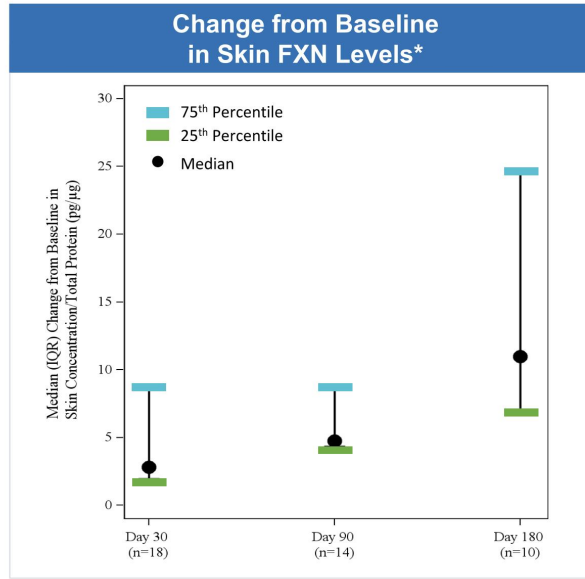
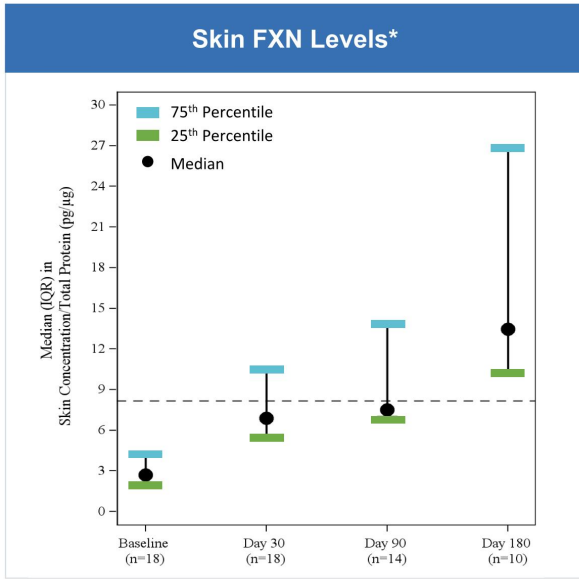


## Key Study Objectives

- 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.

## Nomlabofusp Safety Summary\* with Long-term Treatment

- ~8,000 doses of nomlabofusp have been administered throughout the clinical development program
  - Most common AEs were mild/moderate local ISRs and did not lead to any discontinuations
- 65 total participants received at least 1 dose of nomlabofusp across all studies including 39 participants in OL study
- 7 of 39 participants in OL study experienced anaphylaxis
  - 6 of the 7 cases occurred in participants who had been exposed to nomlabofusp in at least one prior study
  - 10 of 39 participants were exposed to nomlabofusp only in the OL study; 1 of these 10 experienced anaphylaxis
  - Standard treatment with epinephrine autoinjector 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
- Long-term daily dosing was generally well tolerated, including 14 on treatment for at least 6 months and 8 for over 1 year in the OL study

## Disease Characteristics – OL Study & FACOMS Reference Population

	Nomlabofusp*	FACOMS
<b>Age of screening (years)</b>		
n	38	370
Mean (SD)	30.2 (10.94)	27.5 (9.30)
Min, Max	12, 55	12, 54
<b>Age of symptom onset (years)</b>		
n	38	370
Mean (SD)	12.7 (6.09)	13.8 (5.50)
Min, Max	5, 30	5,30
<b>Baseline mFARS Total Score</b>		
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
<b>Baseline FARS-ADL Overall Score</b>		
n	38	370
Mean (SD)	17.5 (6.84)	14.2 (5.70)
Min, Max	2, 27	2, 27
<b>Baseline 9-HPT Average Time of Dominant Hand(s)</b>		
n	34	370
Mean (SD)	95.4 (67.65)	124.8 (51.90)
Min, Max	35.8, 277.3	36.7, 276.5
<b>Baseline MFIS Overall Score</b>		
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 <sup>1</sup>	Nomlabofusp	FACOMS <sup>1</sup>	Nomlabofusp	FACOMS <sup>1</sup>	Nomlabofusp	FACOMS <sup>1</sup>
Baseline	Median (IQR)	<b>54.75</b> (41.2, 71.0)	<b>50.00</b> (37.0, 61.0)	<b>17.75</b> (13.0, 24.5)	<b>14.50</b> (10.0, 18.5)	<b>71.95</b> (49.6, 114.8)	<b>113.50</b> (86.5, 148.5)	<b>34.00</b> (20.0, 34.0)	<b>32.00</b> (21.0, 42.0)
	n	38	370	38	370	34	370	38	370
Change from Baseline at 1 year	Median (IQR)	<b>-2.25</b> (-3.75, -0.25)	<b>1.00</b> (-1.5, 4.0)	<b>-0.50</b> (-2.0, 1.0)	<b>0.50</b> (-1.0, 2.5)	<b>-7.40</b> (-38.8, -2.5)	<b>3.40</b> (-4.5, 18.0)	<b>-6.50</b> (-17.5, 4.0)	<b>1.50<sup>2</sup></b> (-9.5, 11.0)
	n	8	185	8	237	7	219	8	136

IQR = interquartile range

<sup>1</sup> 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.

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

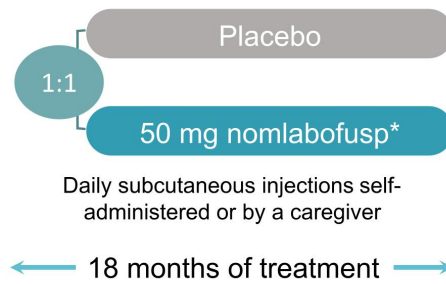
Note: Data presented is based on the September 2025 data release.

# Global Confirmatory Phase 3 Double-blind Placebo-controlled Study

Plan to initiate screening in Q2 2026; dosing of first patient expected mid-2026

## Patient Population

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



## Key Study Objectives

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

## Qualifying sites in U.S., E.U., U.K., Canada, and Australia

Clinical trial application submissions are underway in the E.U. and Canada with the U.K. soon to follow



\*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 Program for the Treatment of Adults and Children with FA Advances Towards BLA Submission and Potential Approval

## Breakthrough Therapy Designation

is awarded to drugs that may demonstrate substantial improvement over available treatments on clinically significant endpoints

## Continued FDA Alignment

willingness to consider FXN as novel surrogate endpoint to support accelerated approval

## Safety and Exposure Dataset

will be a matter of review at time of BLA submission

## Anticipated Near-term Registrational Milestones

- Topline OL data in Q2 2026
- Submission of BLA seeking accelerated approval in June 2026
- Initiate screening in global Phase 3 study in Q2 2026
- U.S. launch in first-half 2027, if approved



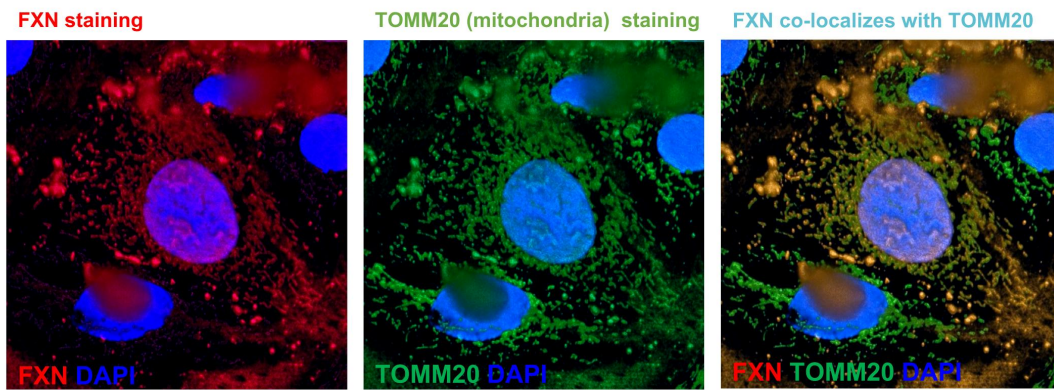
# Larimar Therapeutics

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

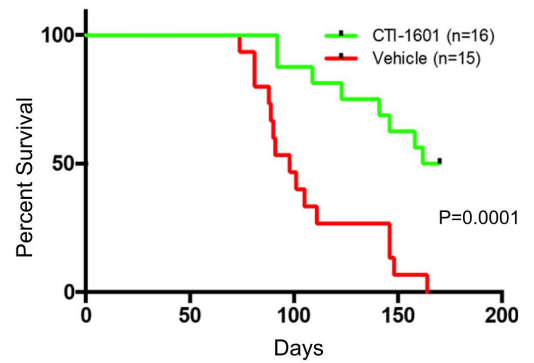
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

## 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

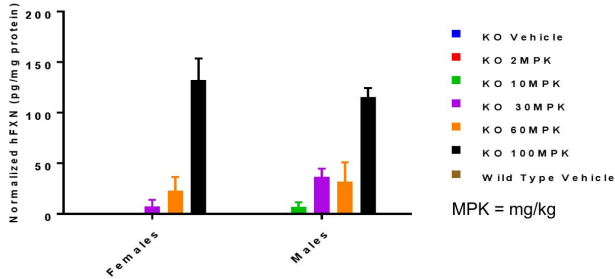
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

- ☑ 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.

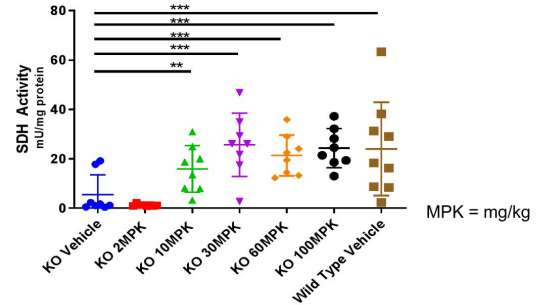
## Mitochondrial FXN (Heart)



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



## SDH Activity (Muscle)

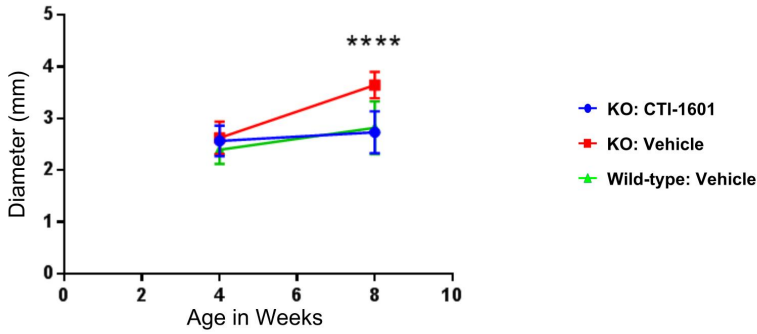


Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type

## Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

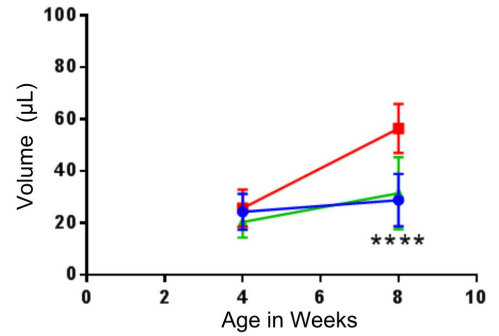
**Study Design** – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

### 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)

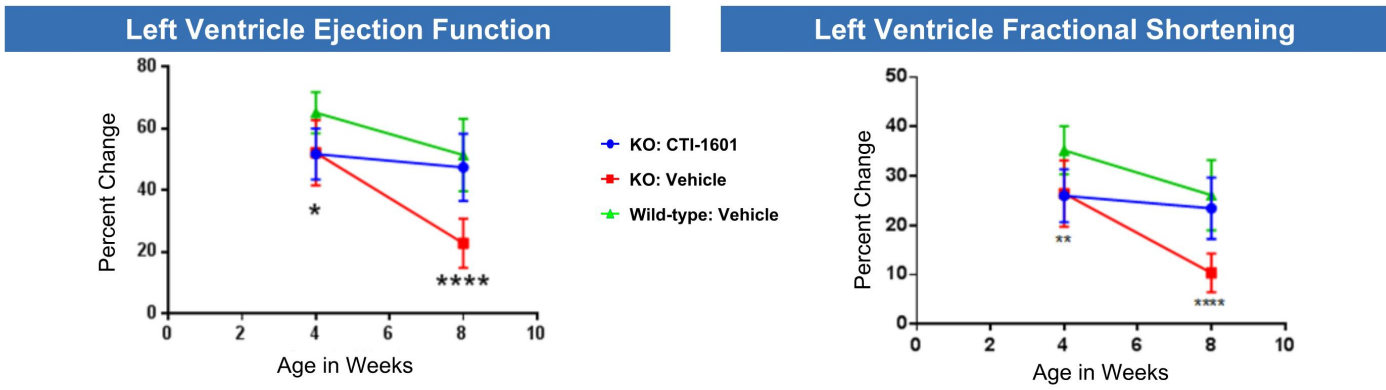
### Left Ventricle Volume (Systole)



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

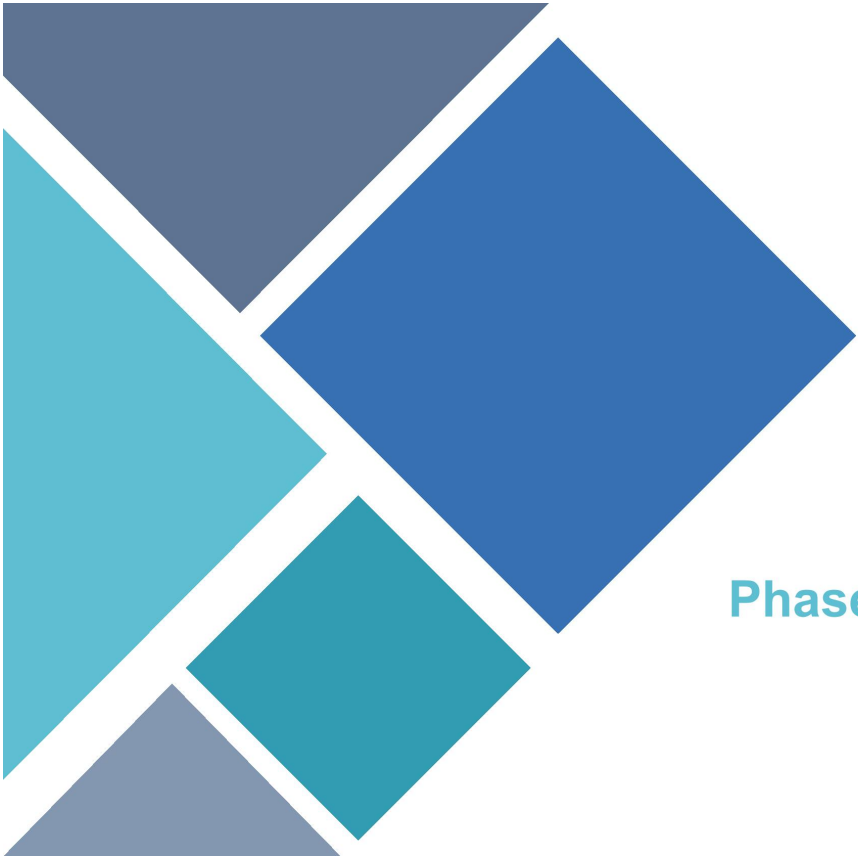
# Nomlabofusp Preserves Left Ventricle Function in KO Mice

**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



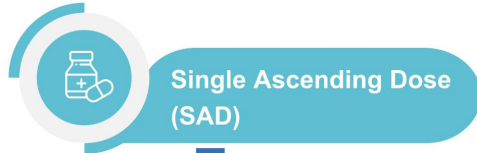
## Phase 1 Clinical Data

# 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



### Single Ascending Dose (SAD)

Eligible patients from SAD trial could enroll in MAD trial

**Number of subjects:** 28

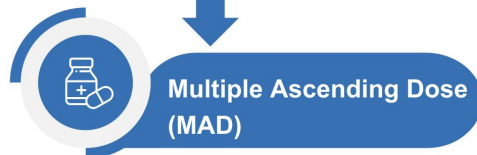
**Dose levels:** 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)

**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

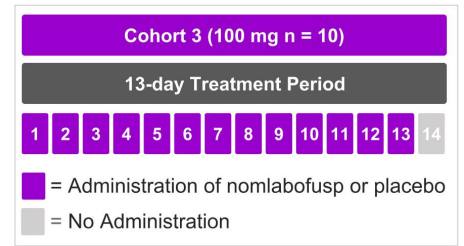
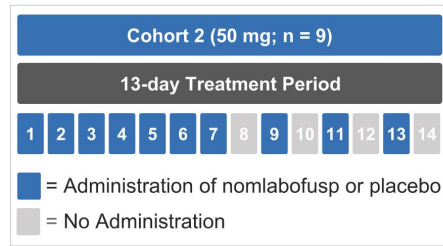
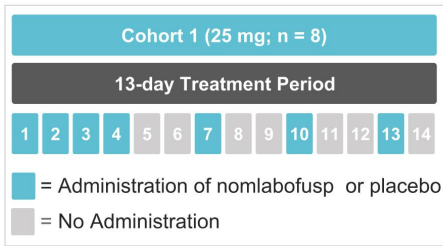
**1° Endpoint:** Safety and tolerability

**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**

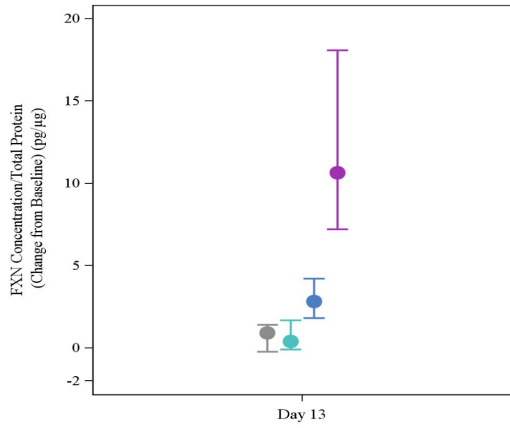
<b>Buccal Cells</b>	Baseline, Day 7, Day 13
<b>Skin</b>	Baseline, Day 13
<b>Platelets</b>	Baseline, Day 7, Day 13

**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

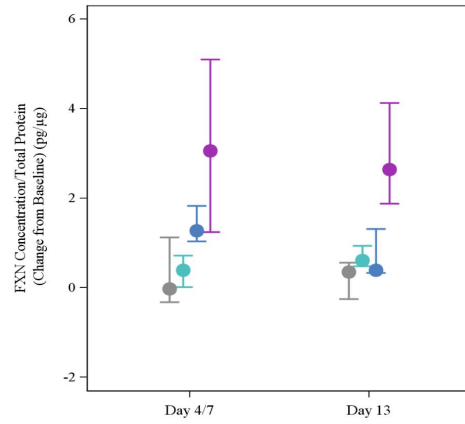
# Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

**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)

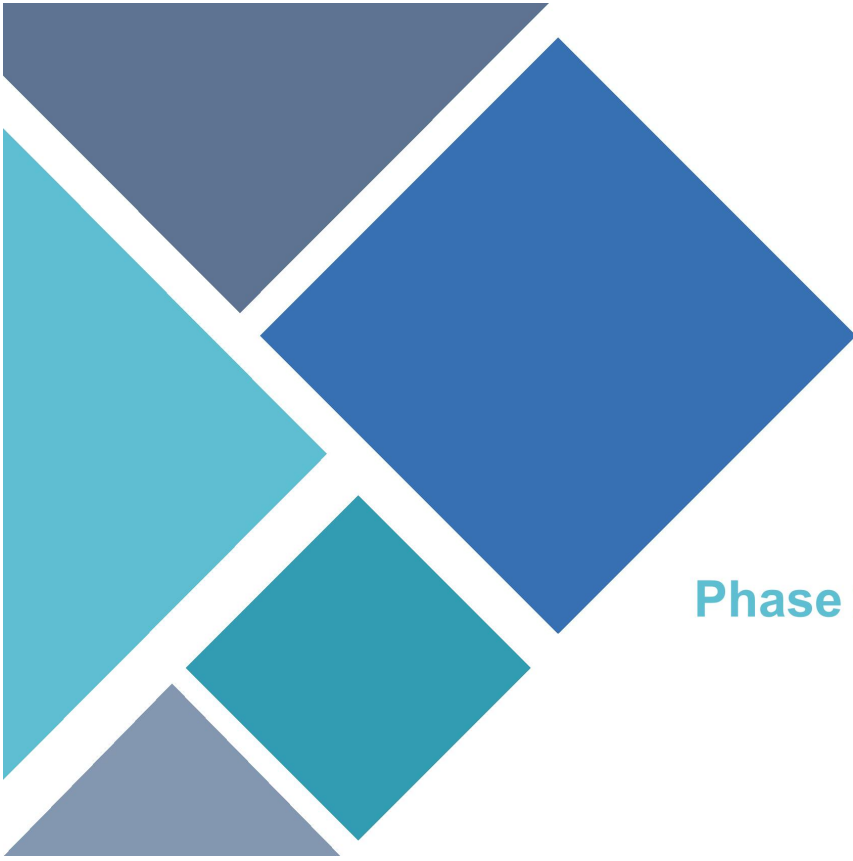
## MAD Trial Patient Disease Characteristics

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 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

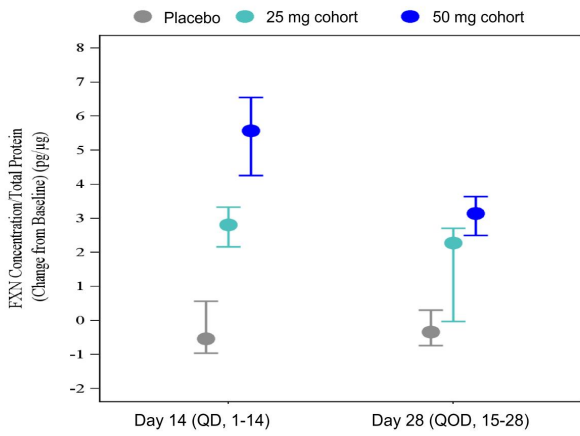
### Study Details

<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)
<b>Key Results</b>	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

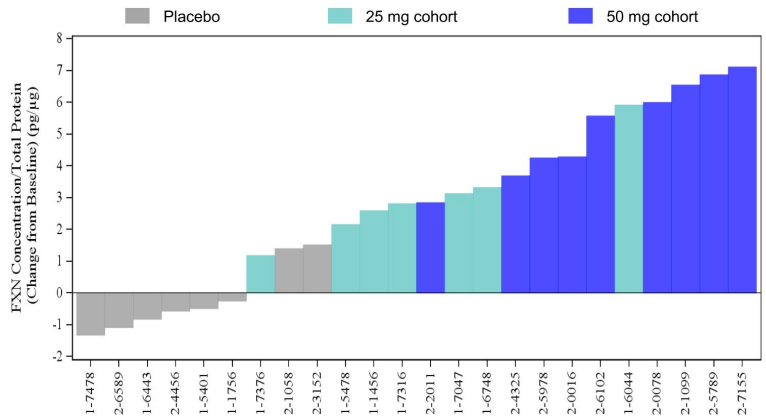
# Dose-Dependent Increase in FXN Levels in Skin Cells

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

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



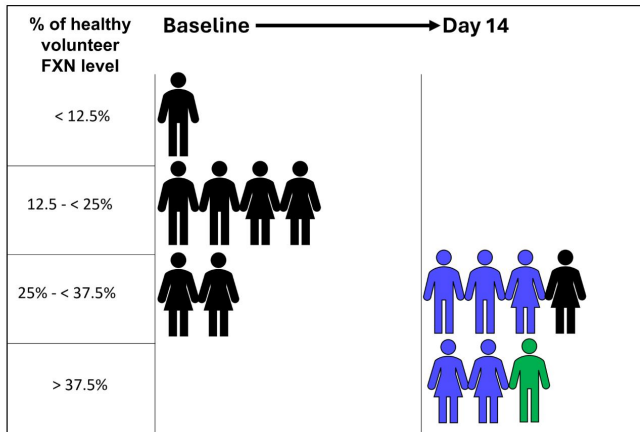
## FXN Levels\* in Skin Cells Change from Baseline at Day 14



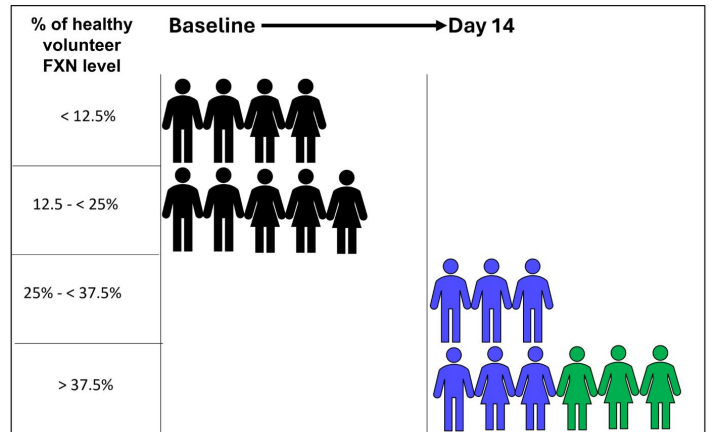
\*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25<sup>th</sup> and 75<sup>th</sup> percentiles. Only participants with quantifiable levels at both baseline and Day 14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

# 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

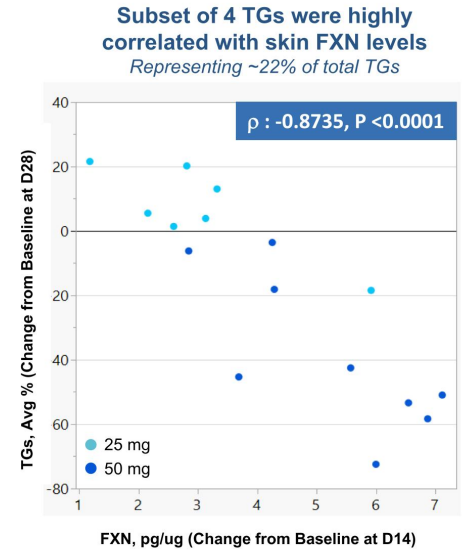
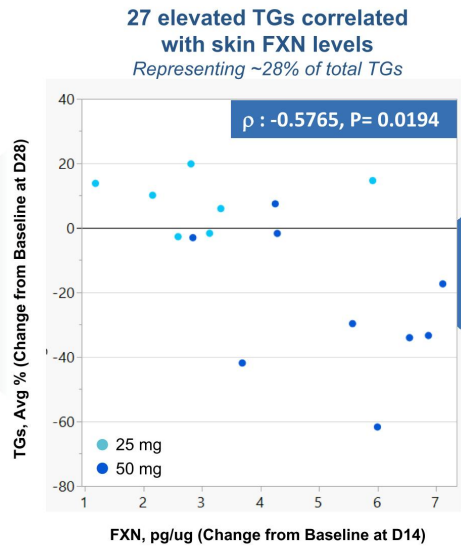
Plasma lipids at baseline were compared to Day 28 after nomlabofusp treatment

↓

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**

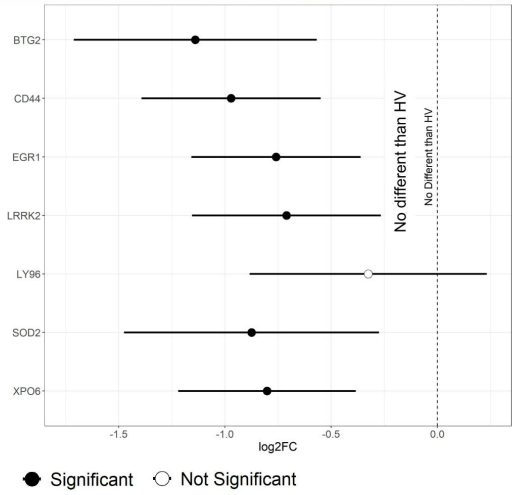


Plasma samples were collected before, during, and after treatment for lipid profiling from the Phase 2 dose exploration study evaluating nomlabofusp 25 mg and 50 mg or placebo daily for 14 days followed by alternate day administration for 14 days

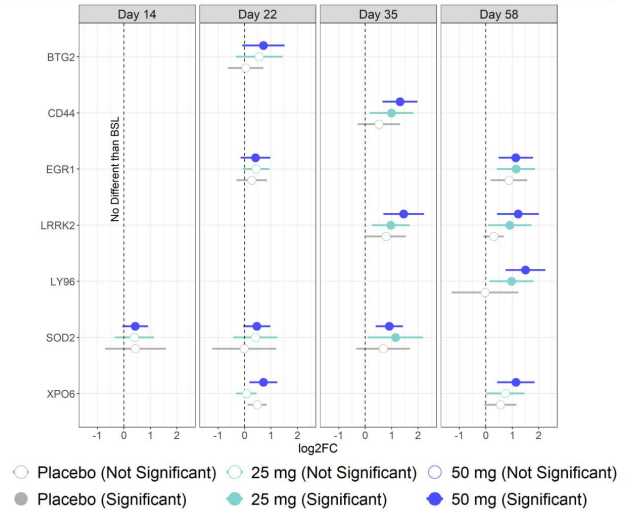
\*Triglycerides (TG) were selected with a median fold-change ( $\geq 1.25$  fold) post-treatment vs. baseline, a correlation ( $r$  value  $\geq 0.4$ ) between baseline and post-treatment results in the 50 mg group with consistent directionality in the 25 mg group and no changes in the placebo group

# 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

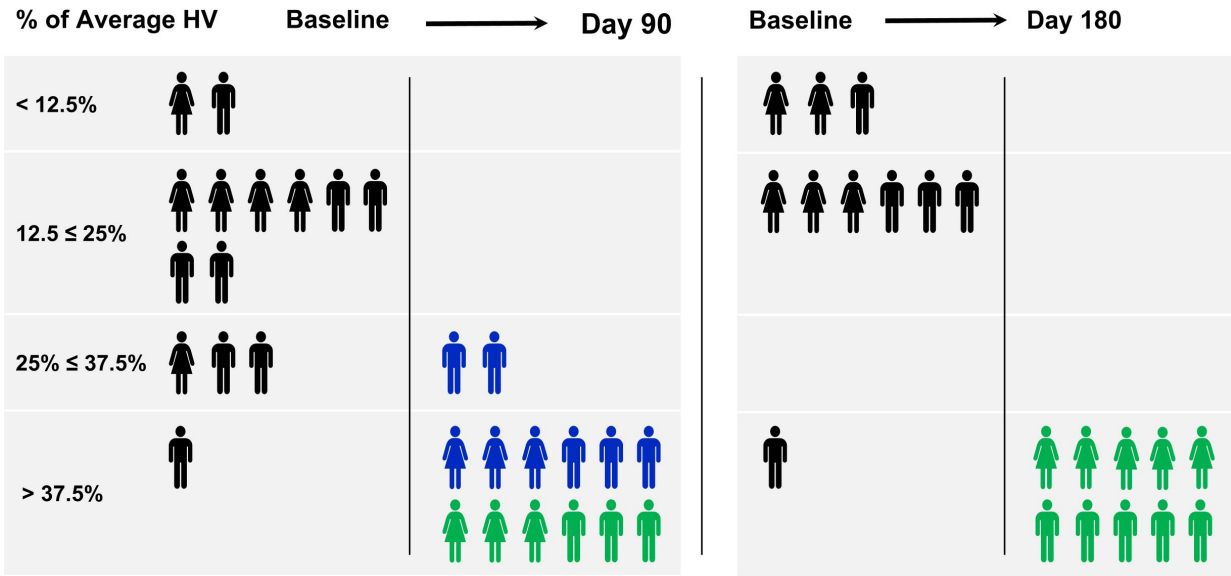


Data presented at the International Congress for Ataxia Research, November 2024  
 \*Samples from Phase 2 dose exploration study evaluating nomlabofusp 25 mg (Cohort 1) and 50 mg (Cohort 2) or placebo via subcutaneous injection daily for 14 days followed by alternate day administration for 14 days. Buccal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



## Additional Open Label Data

# 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.



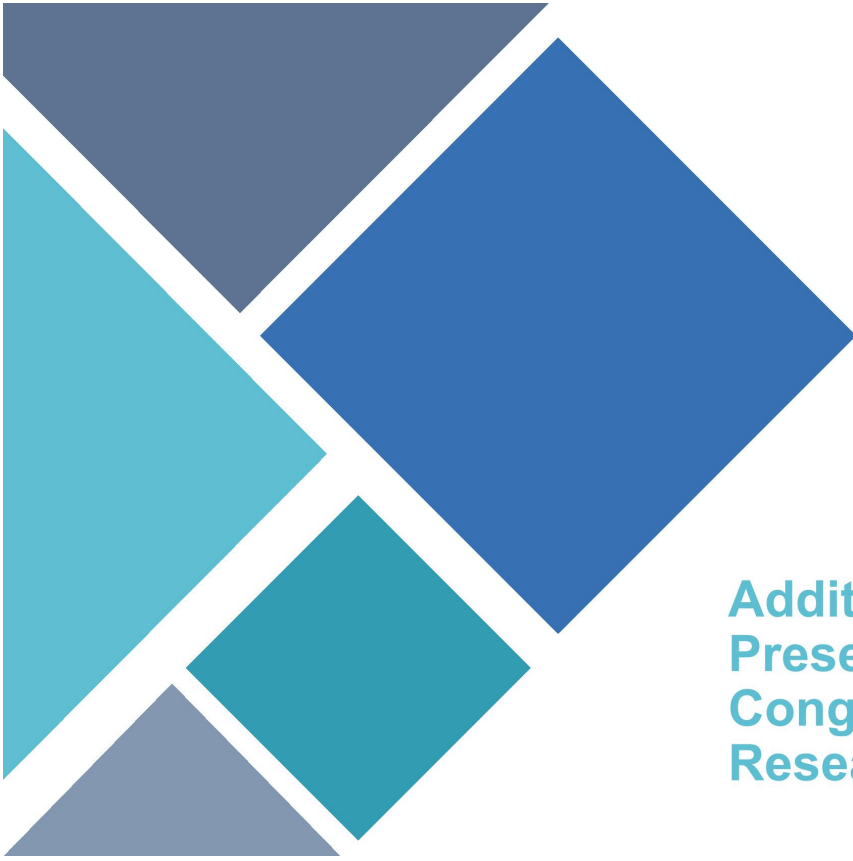
Baseline as a percentage of average FXN level in HV



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



**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
<b>Age</b>	61	28.0	31.9	19	69
<b>Age of Onset</b>	61	15.0	15.9	5	60
<b>Age of Diagnosis</b>	61	19.0	21.0	5	64
<b>Shorter GAA (GAA<sub>1</sub>)</b>	60	550.0	555.8	99	1000
<b>Longer GAA (GAA<sub>2</sub>)</b>	60	900.0	890.2	265	1300
<b>Fratxin, % of Control**</b>	57	24.4	23.9	8.7	61.9
<b>mFARS Score</b>	61	52.0	49.5	13.2	74.5
<b>Upright Stability Score</b>	61	32.0	26.9	7.0	35.0
<b>Dominant hand 9-hole peg test</b>	61	71.0	84.8	26.0	229.2
<b>T25-FW Test Score</b>	51	9.9	13.4	4.3	48.5
<b>Left Ventricular Mass (g)</b>	61	163.4	168.0	73.7	398.8
<b>LVEF %</b>	61	63.0	63.5	52	76
<b>Ambulatory Status***</b>					
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 <sub>1</sub> **	GAA <sub>2</sub> **
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

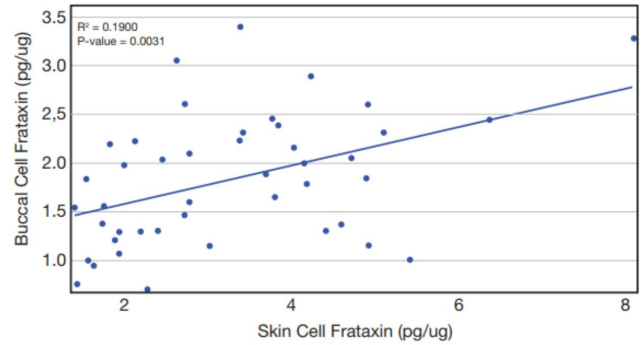
\*Quantifiable buccal cell frataxin levels

\*\*Median values

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

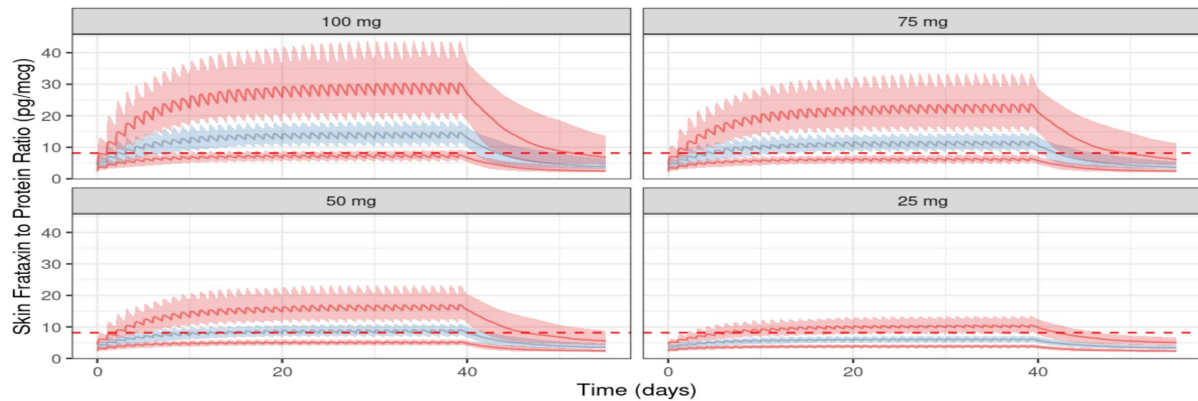
Buccal cell FXN levels correlated with age of onset and inversely correlated with the number of GAA repeats and rate of disease progression

## 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/ $\mu$ 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

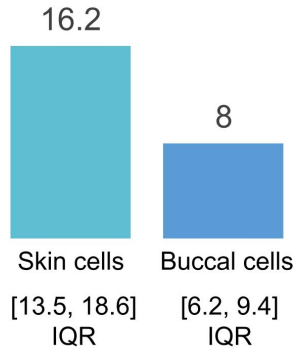


## Non-Interventional Study Data

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

Non-interventional study measured FXN in homozygous healthy volunteers

**Median Frataxin Concentration (pg/ $\mu$ 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>



## 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