Larimar Therapeutics

Corporate Deck

May 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," target," "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; that the FDA may not ultimately agree with Larimar's rolling BLA submission strategy; Larimar's ability to submit BLA modules on the intended timelines; 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; the timing of any potential launch of nomlabofusp, if approved; 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.

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 BTD 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

Intend to initiate rolling BLA* seeking accelerated approval with submission of nonclinical and clinical modules in June 2026; submission of the final modules, including the CMC module, expected in 2H 2026

FXN as Surrogate Endpoint	**Safety Database**	**Reference Population**	**Global Phase 3 Study**
Continued alignment with FDA to consider the use of skin FXN as novel surrogate endpoint reasonably likely to predict clinical benefit	As part of the recent START meeting and BTD application, Larimar submitted available safety and exposure data to FDA for review	FDA will review and comment on the selection of a reference population from a natural history study and the statistical analysis plan	FDA alignment to have global Phase 3 study underway at time of BLA submission
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	FDA stated that the adequacy of the safety database will be a matter of review at the time of BLA submission	The planned 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	Confirmed that change from baseline in Upright Stability Score (a subscale of mFARS) is a reasonable and clinically relevant primary endpoint

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

*Type B meeting with FDA scheduled in Q2 2026 to align on the overall BLA data package readiness. Pending FDA feedback, Larimar intends to initiate a rolling BLA seeking accelerated approval.

Multiple Planned Near-term Milestones for Potential Registration

Open Label Study

Topline data
Q2 2026

Global Phase 3

Initiate dosing of first patient
Mid-2026

***Rolling BLA Submission**

Nonclinical and clinical modules
June 2026

Final modules including CMC
2H 2026

U.S. Approval/Launch

1H 2027

$200.4 M in cash & investments as of March 31, 2026, with projected cash runway into Q2 2027

*Type B meeting with FDA scheduled in Q2 2026 to align on the overall BLA data package readiness. Pending FDA feedback, Larimar intends to initiate a rolling BLA seeking accelerated approval.

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.

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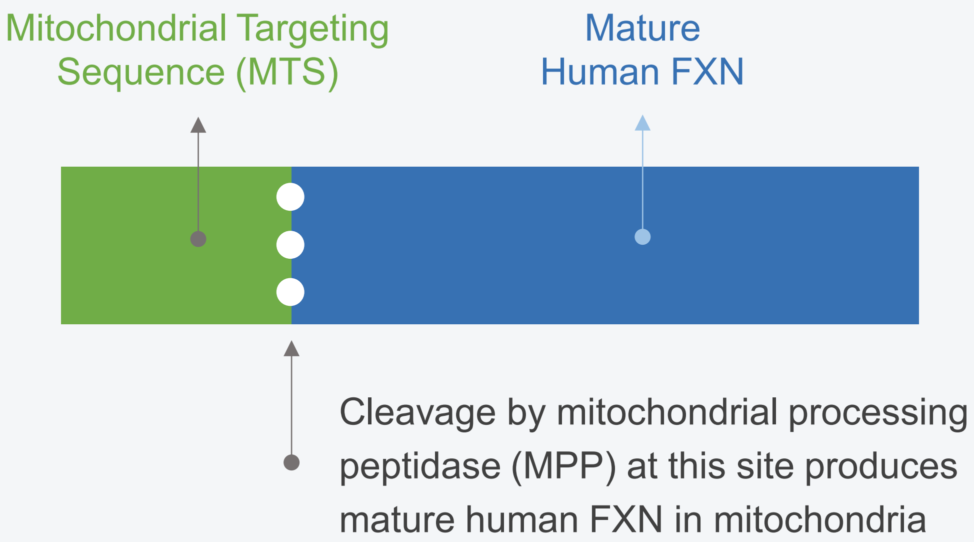
* E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245.

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)

Structure of Endogenous FXN

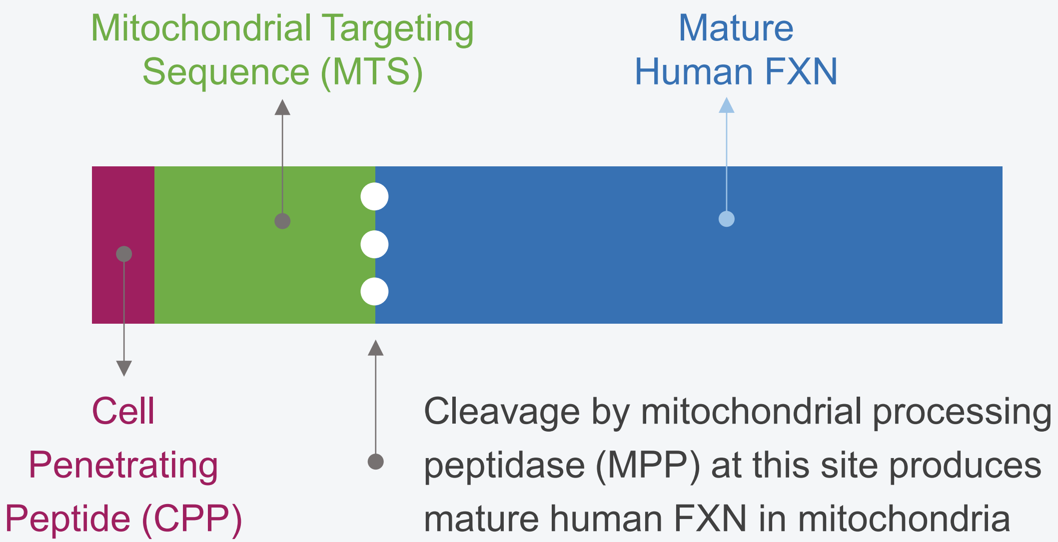


Mitochondrial Targeting Sequence (MTS)

Mature Human FXN

Cleavage by mitochondrial processing peptidase (MPP) at this site produces mature human FXN in mitochondria

Structure of nomlabofusp



Mitochondrial Targeting Sequence (MTS)

Mature Human FXN

Cell Penetrating Peptide (CPP)

Cleavage by mitochondrial processing peptidase (MPP) at this site produces mature human FXN in mitochondria

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

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*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

✓ **Phase 1 SAD** 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
✓ **Phase 1 MAD** 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
✓ **Phase 2 Dose Exploration Study**	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
✓ **Adolescent PK** 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
Ongoing OL Study Open label study	Assessing the long-term safety, efficacy, PK, PD, and tolerability of nomlabofusp in participants with FA

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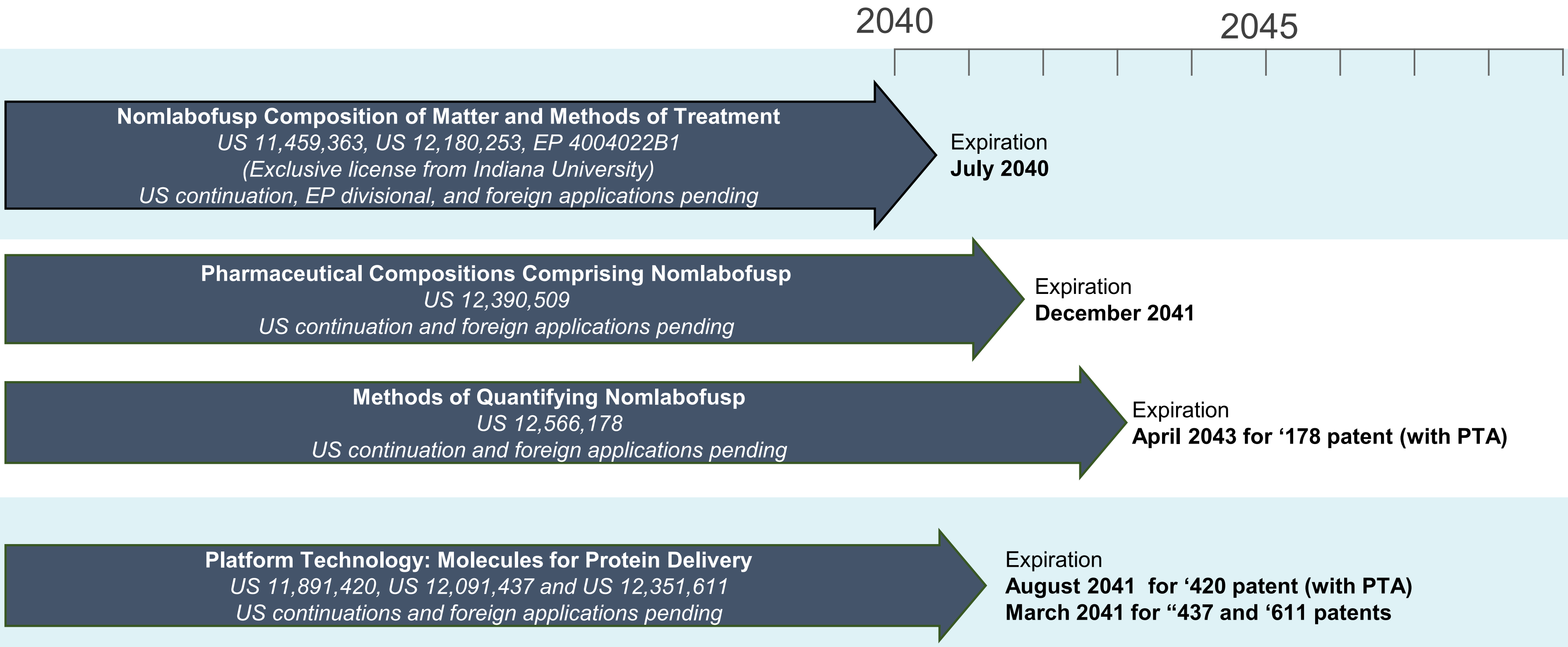
Larimar Technology is Supported by a Strong IP Portfolio

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



Composition of Matter

Nomlabofusp Composition of Matter and Methods of Treatment
US 11,459,363, US 12,180,253, EP 4004022B1
(Exclusive license from Indiana University)
US continuation, EP divisional, and foreign applications pending

Expiration
July 2040

Formulation and Methods of Quantifying Nomlabofusp

Pharmaceutical Compositions Comprising Nomlabofusp
US 12,390,509
US continuation and foreign applications pending

Expiration
December 2041

Methods of Quantifying Nomlabofusp
US 12,566,178
US continuation and foreign applications pending

Expiration
April 2043 for '178 patent (with PTA)

Platform

Platform Technology: Molecules for Protein Delivery
US 11,891,420, US 12,091,437 and US 12,351,611
US continuations and foreign applications pending

Expiration
August 2041 for '420 patent (with PTA)
March 2041 for "437 and '611 patents

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)

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

Anti-histamines ›	**5 mg** nomlabofusp ›	**25 mg** nomlabofusp ›	**50 mg**** nomlabofusp
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

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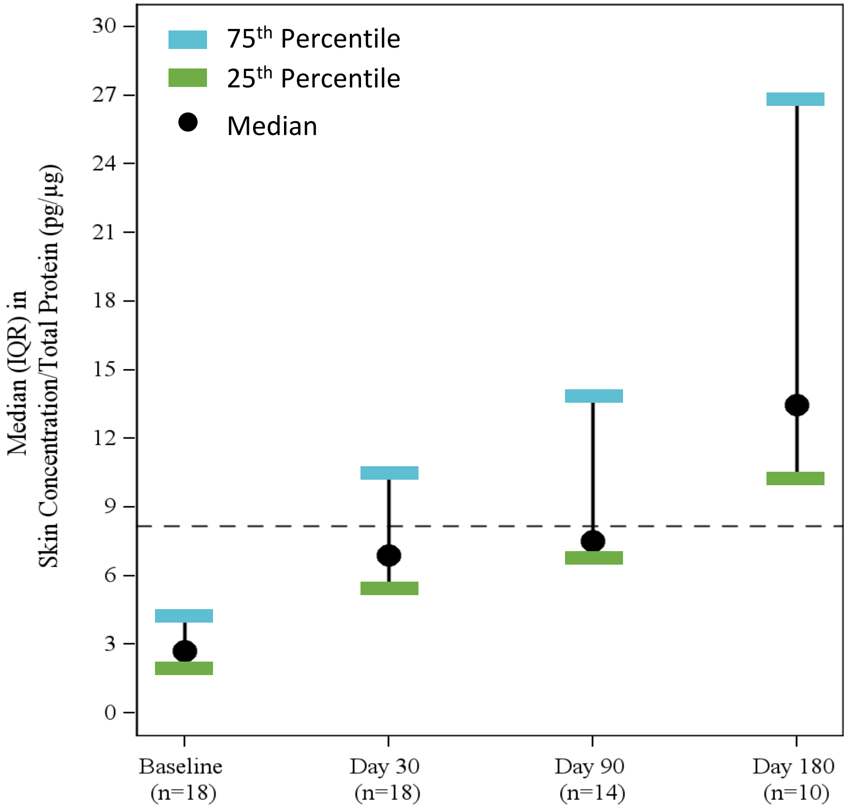
*Open Label Extension study is now referred to as Open Label study following inclusion of participants who were not part of a prior nomlabofusp clinical study.

**Participants under 18 years of age receive a weight-based dose equivalent.

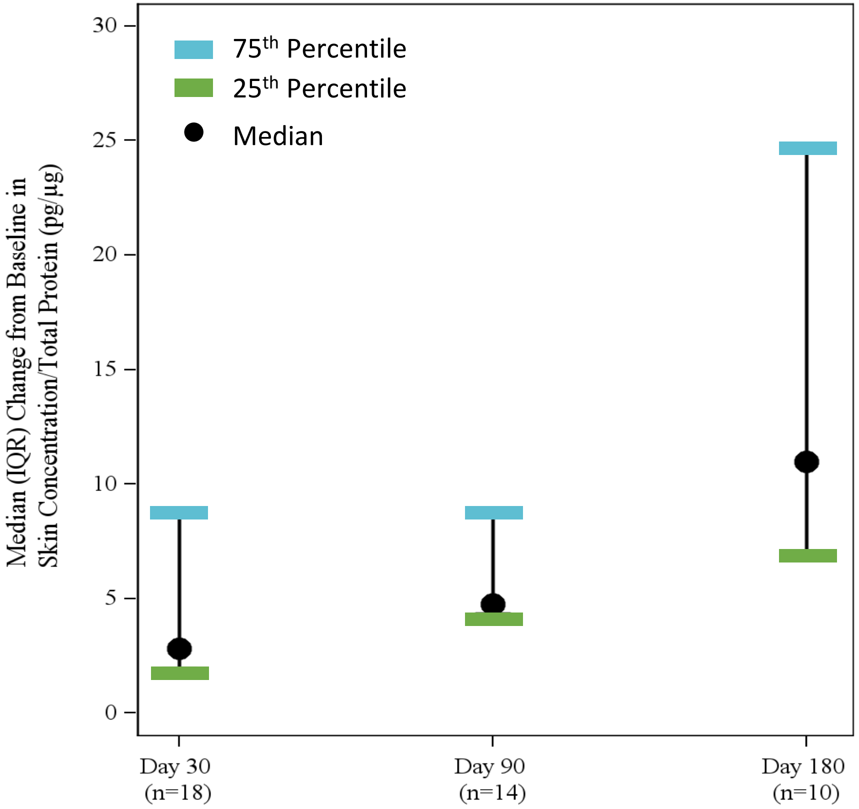
Increases in Skin FXN Levels* were Sustained Over Time

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

Skin FXN Levels*



Change from Baseline in Skin FXN Levels*



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)

Baseline	Day 30	Day 90	Day 180
0% 0/18	**33%** 6/18	**43%** 6/14	**100%** 10/10

Absolute Skin FXN Levels* Increase Over Time with Nomlabofusp Treatment

Statistic	Baseline	Day 30	Day 90	Day 180
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

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* Data presented is based on the September 2025 data release except for total doses administered.
AE: Adverse events; ISR: Injection site reaction

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

*Participants in open label study included 58% female, 53% with exposure to omaveloxolone and 53% non-ambulatory.

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)	**1.50[2]** (-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.

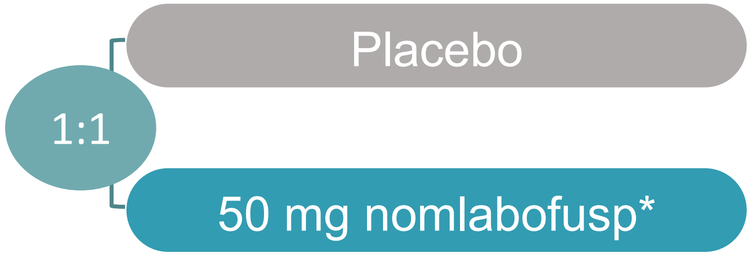
Note: Data presented is based on the September 2025 data release.

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



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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 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

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*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 Seeking Accelerated Approval

Granted Breakthrough Therapy Designation

supporting potential of nomlabofusp to 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
- FDA Type B meeting in Q2 2026
- Plan to initiate rolling BLA submission* in June 2026
- Submission of final modules, including CMC module, expected in 2H 2026
- U.S. launch in 1H 2027, if approved

*Pending FDA feedback, Larimar intends to initiate a rolling BLA seeking accelerated approval.

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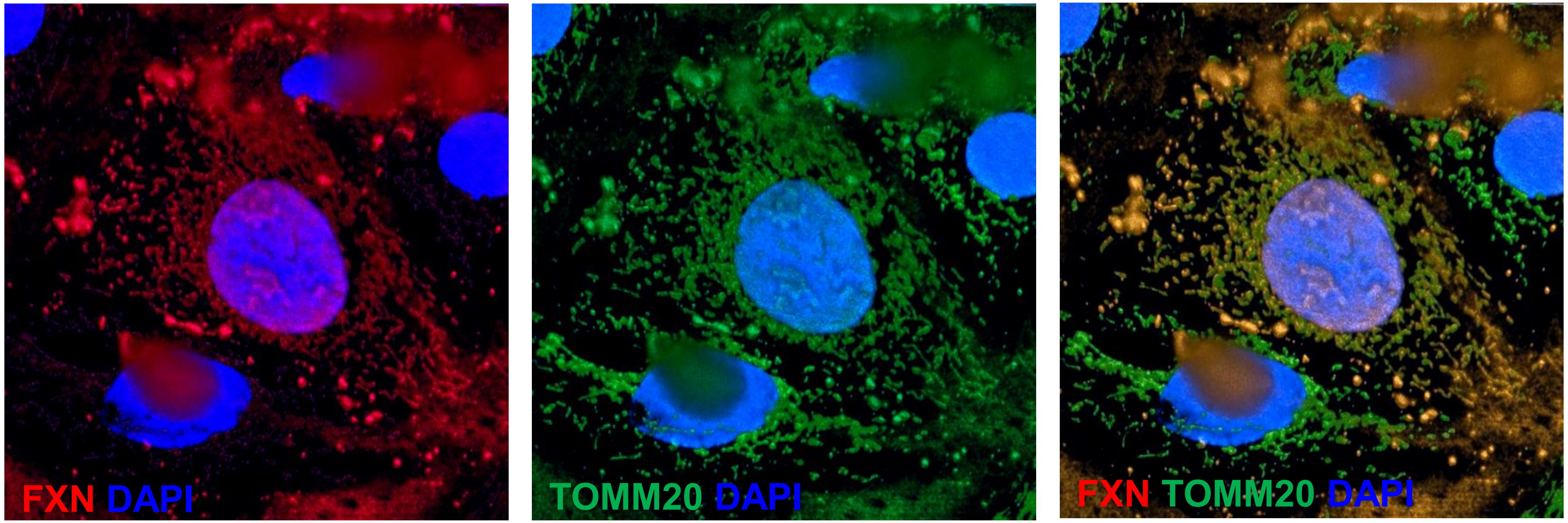
Appendix

Mitochondrial Localization and Preclinical Data

Nomlabofusp Cell Transduction In Vitro Leads to hFXN in Mitochondria



FXN staining

TOMM20 (mitochondria) staining

FXN co-localizes with TOMM20

- 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

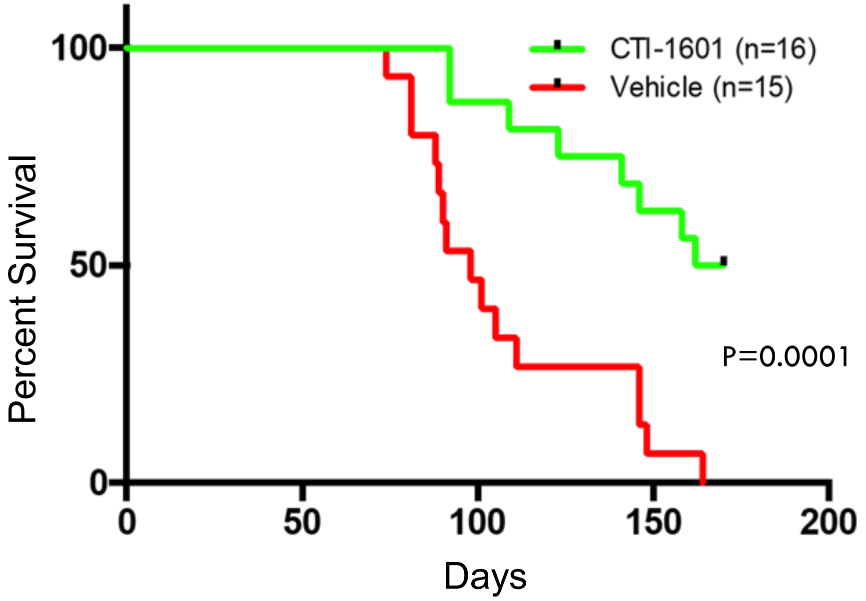
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

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

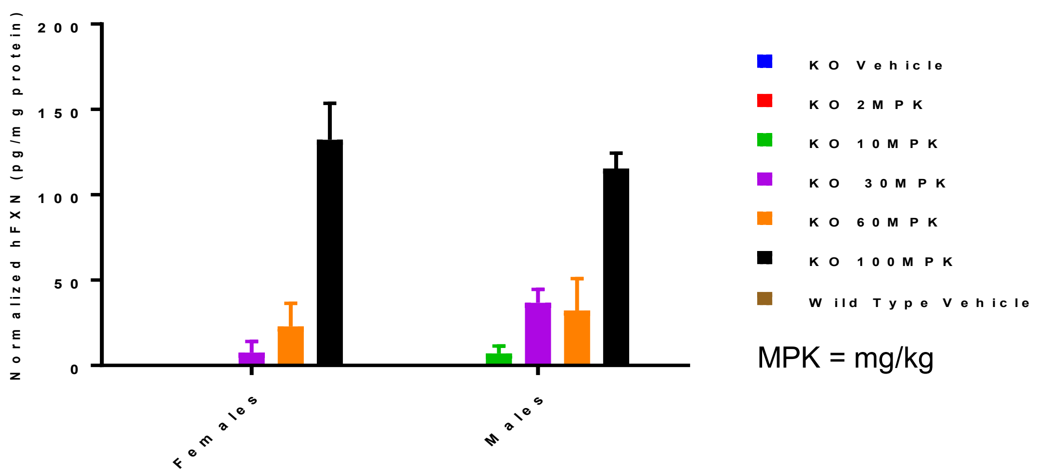
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

- ✓ 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)

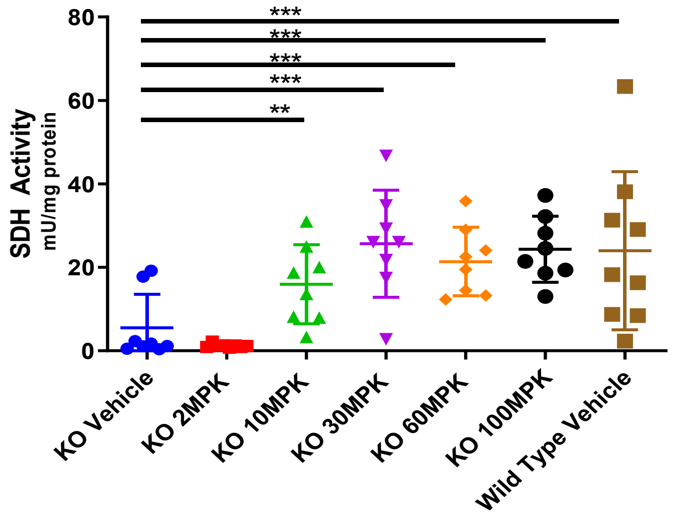


MPK = mg/kg

Mitochondria hFXN concentration increases dose-dependently

Given subcutaneously, nomlabofusp functionally replaces hFXN in mitochondria of KO mice

SDH Activity (Muscle)



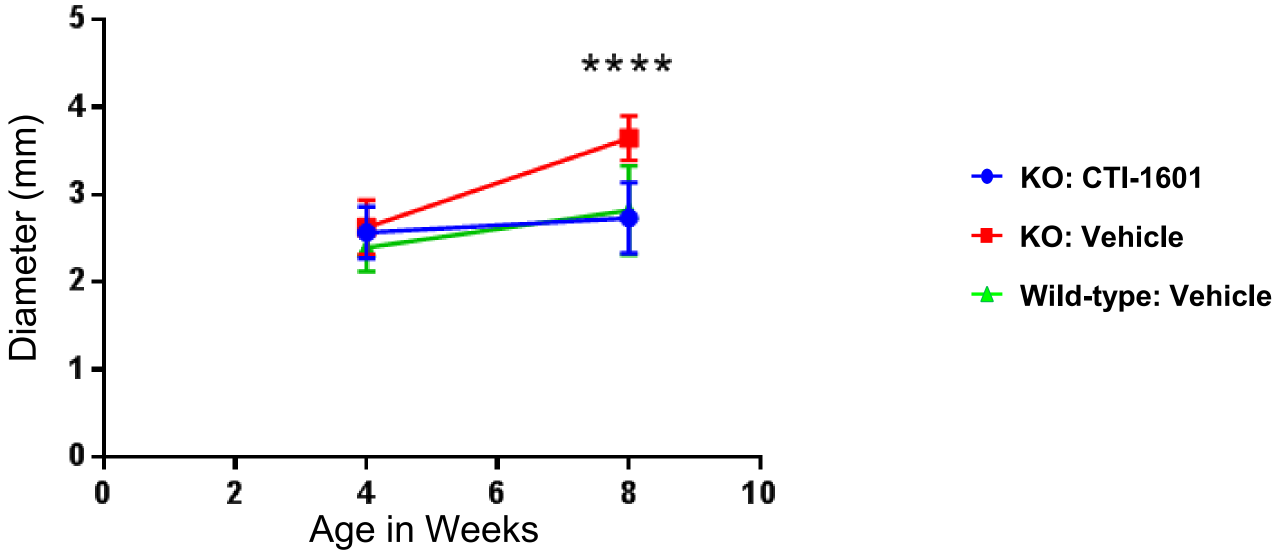
MPK = mg/kg

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

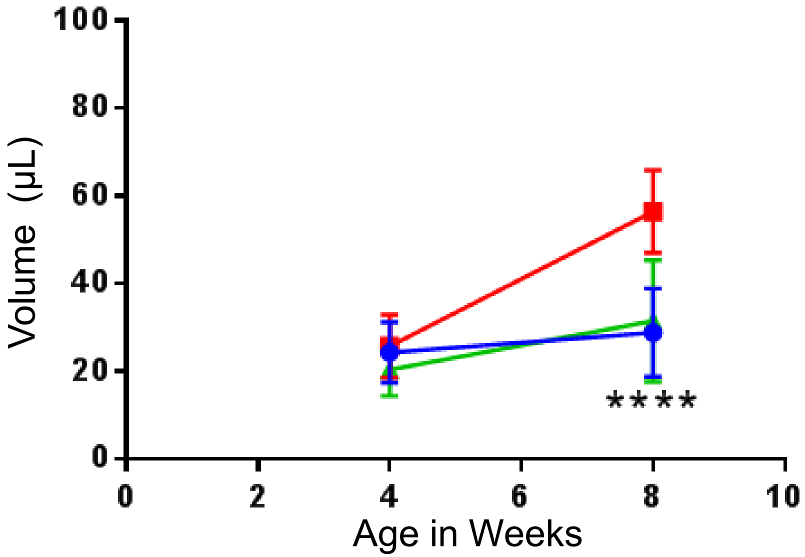
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

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)

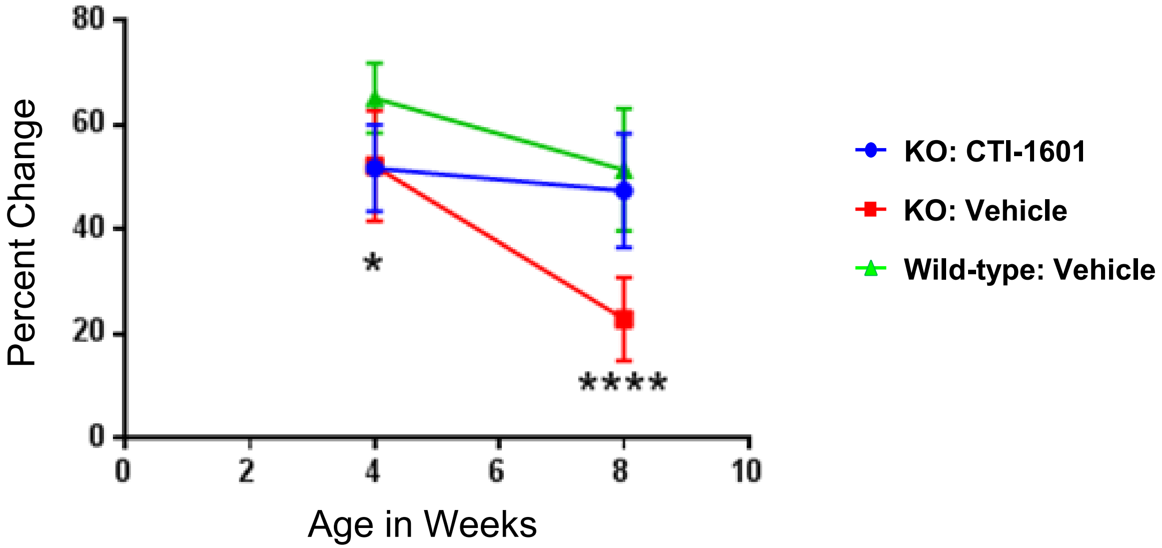


Nomlabofusp-treated mice have similar LV volume as wild type; echocardiogram shows significant differences between vehicle and nomlabofusp treated (10 mg/kg every other day) KO mice

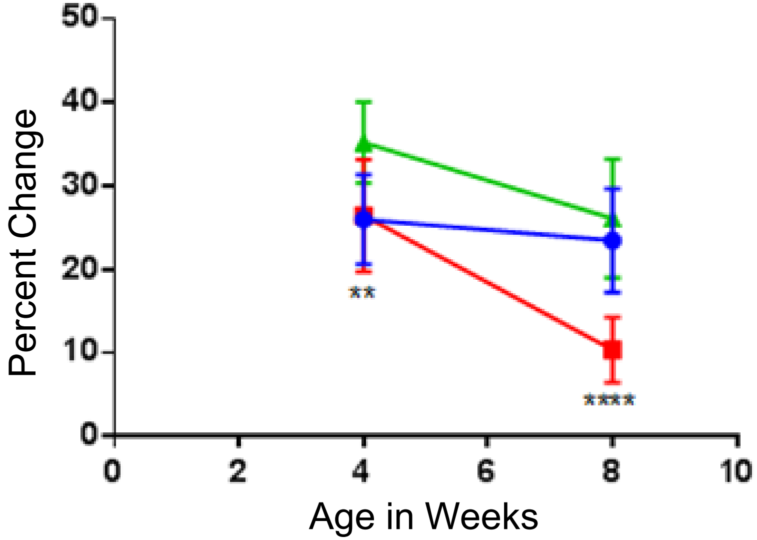
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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 Ventricle Ejection Function



Left Ventricle Fractional Shortening



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

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

Phase 1 Clinical Data

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

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

Phase 1 Development Plan

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

Single Ascending Dose (SAD)

Number of subjects: 28

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

Treatment Duration: 1 day

1º Endpoint: Safety and tolerability

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

Status: Complete

Eligible patients from SAD trial could enroll in MAD trial

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

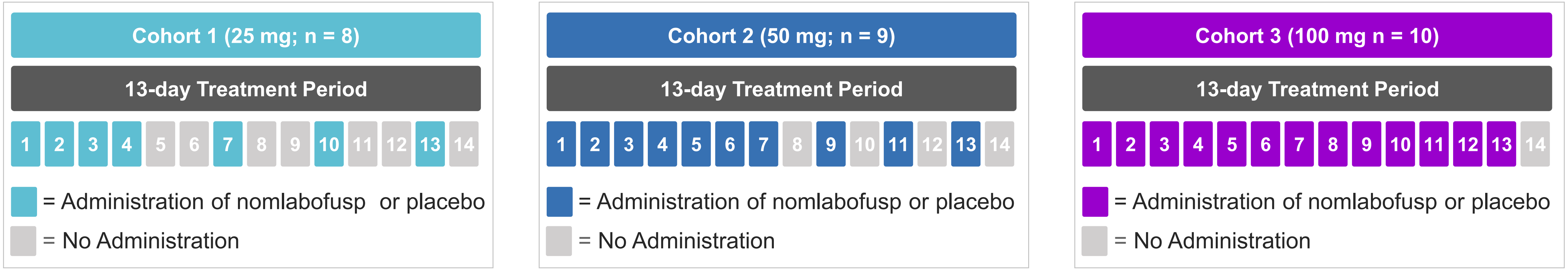
1º Endpoint: Safety and tolerability

2º 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



Cohort 1 (25 mg; n = 8)

13-day Treatment Period

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

🟦 = Administration of nomlabofusp or placebo

⬜ = No Administration

Cohort 2 (50 mg; n = 9)

13-day Treatment Period

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

🟦 = Administration of nomlabofusp or placebo

⬜ = No Administration

Cohort 3 (100 mg n = 10)

13-day Treatment Period

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

🟪 = Administration of nomlabofusp or placebo

⬜ = No Administration

FXN Level Sampling Days Presented for Each Cohort

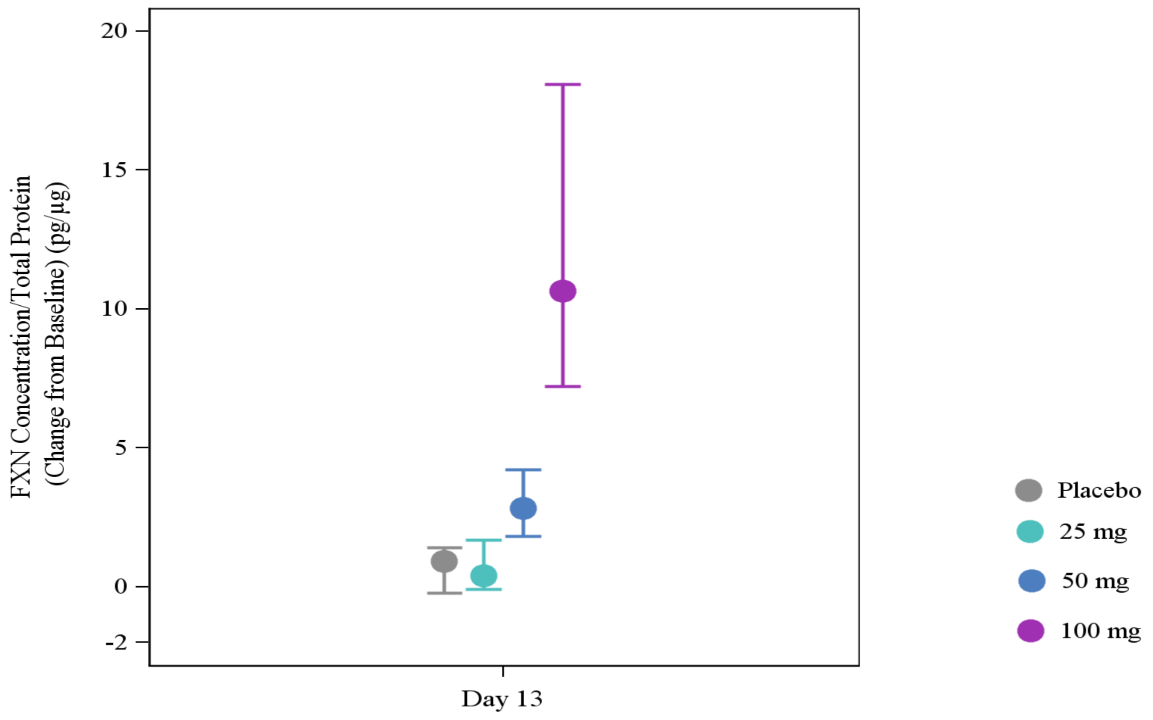
Cohort 1 Sampling Days	
Buccal Cells	Baseline, Day 4, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 4, Day 13

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

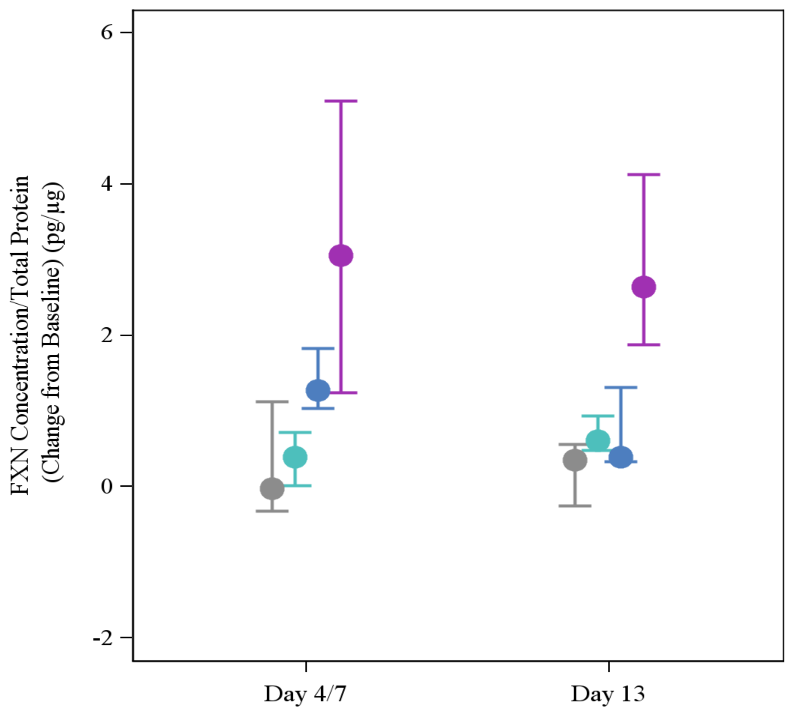
Cohort 3 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

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

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



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



Placebo: Participants randomized to placebo in each cohort

25 mg: Dosed daily for 4 days, every third day thereafter

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

100 mg: Dosed daily for 13 days

*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)

SD: Standard deviation

MAD Trial Patient Disease Characteristics

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

SD: Standard deviation

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

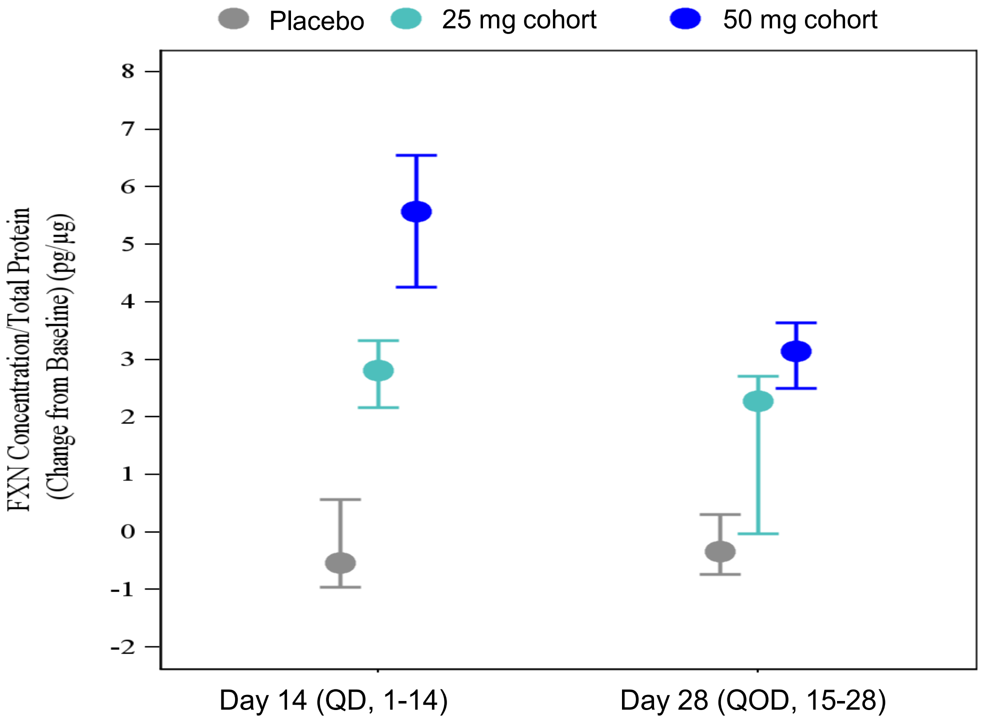
⬜ = No Administration

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

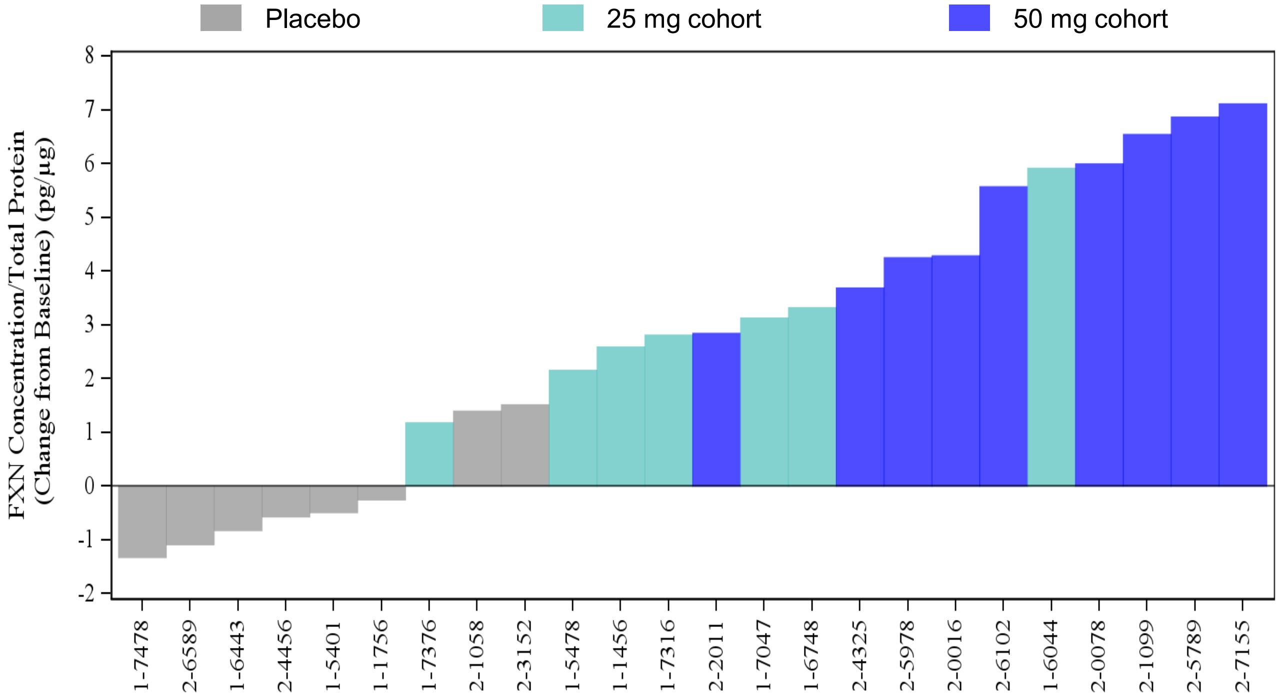
Dose-Dependent Increase in FXN Levels in Skin Cells

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

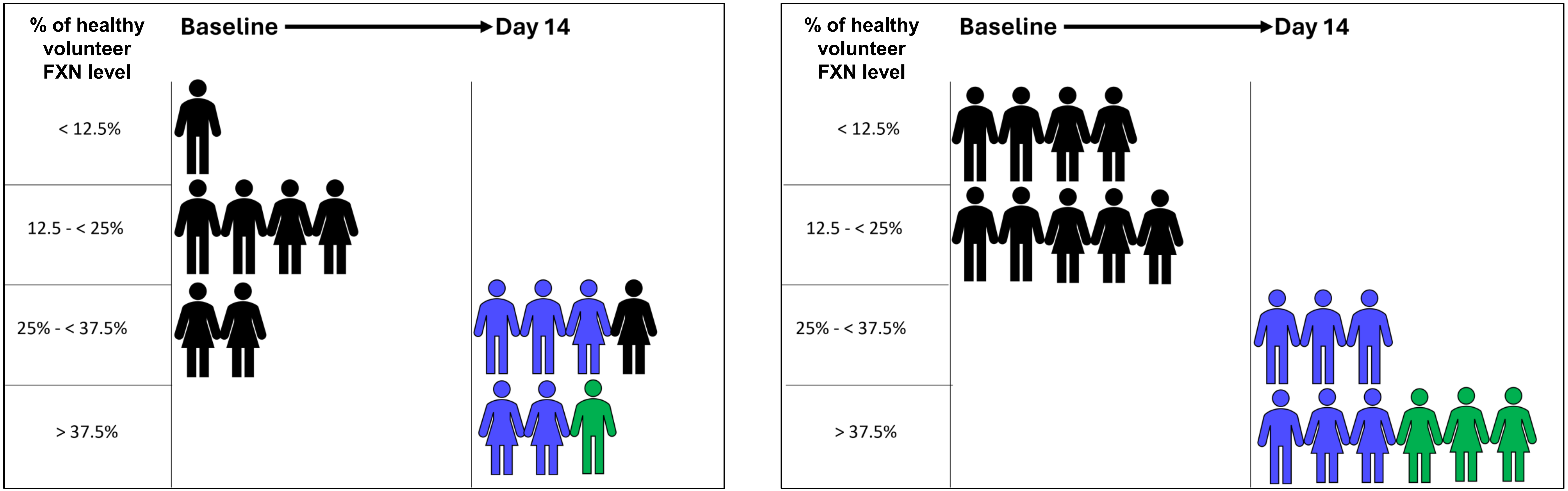


Larimar Therapeutics

*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

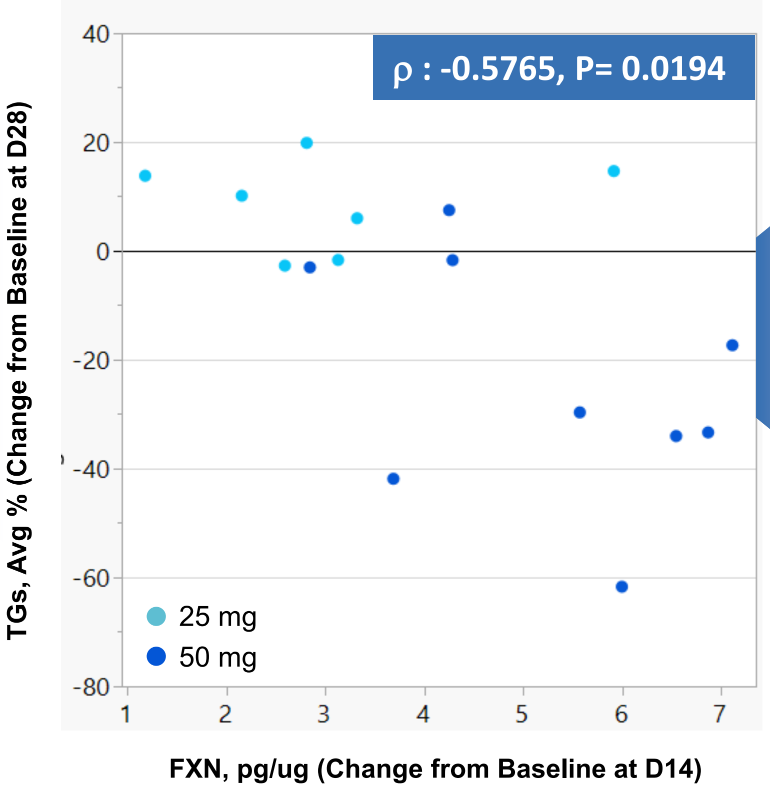
Plasma lipids at baseline were compared to Day 28 after nomlabofusp treatment

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

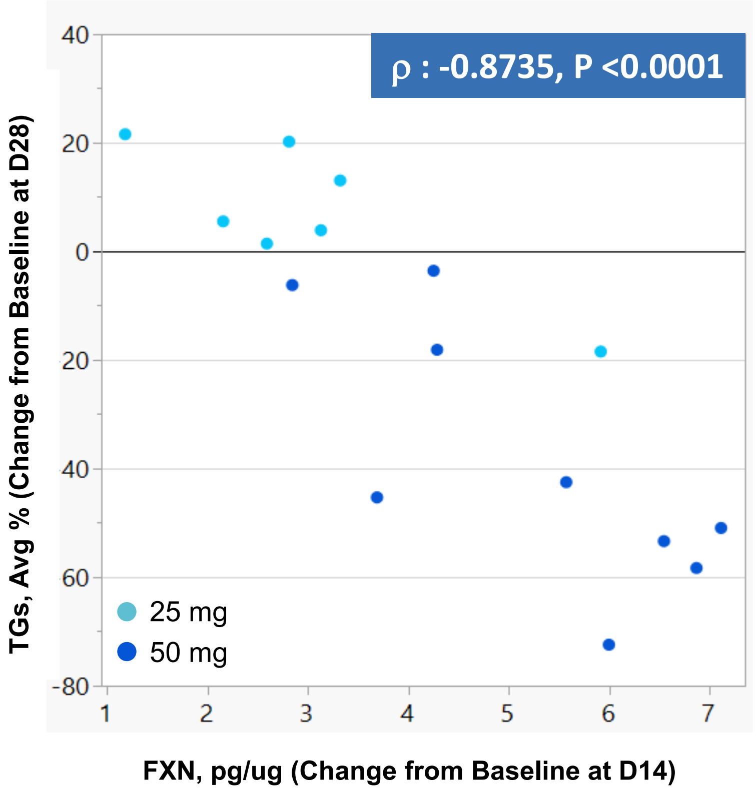
27 elevated TGs correlated with skin FXN levels

Representing ~28% of total TGs



Subset of 4 TGs were highly correlated with skin FXN levels

Representing ~22% of total TGs



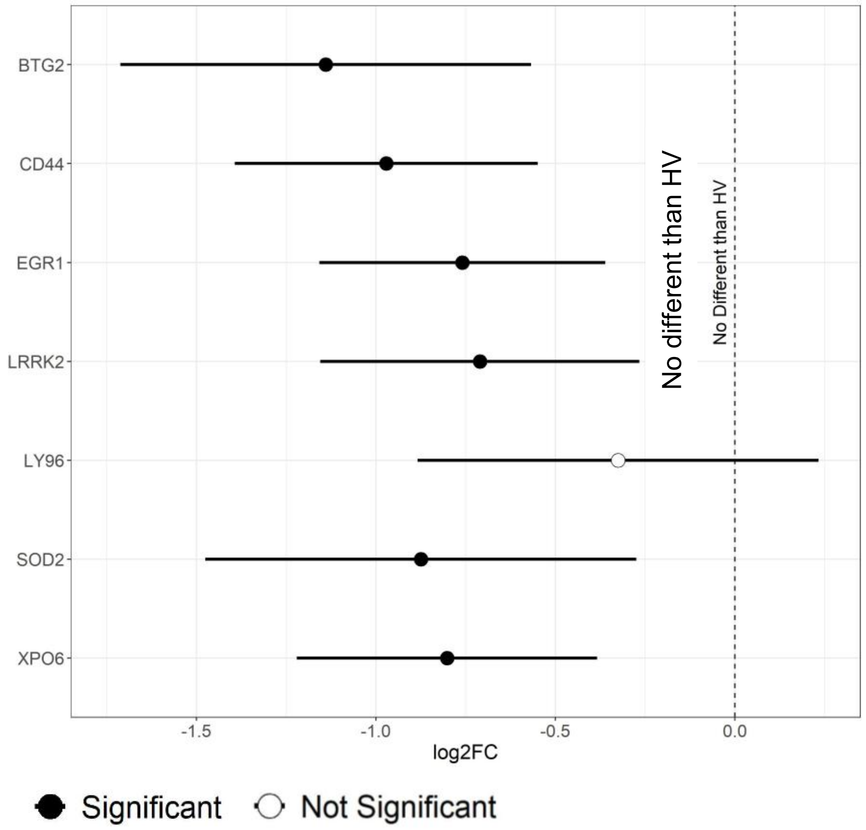
Plasma samples were collected before, during, and after treatment for lipid profiling from the Phase 2 dose exploration study evaluating nomlabofusp 25 mg and 50 mg or placebo daily for 14 days followed by alternate day administration for 14 days

**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*

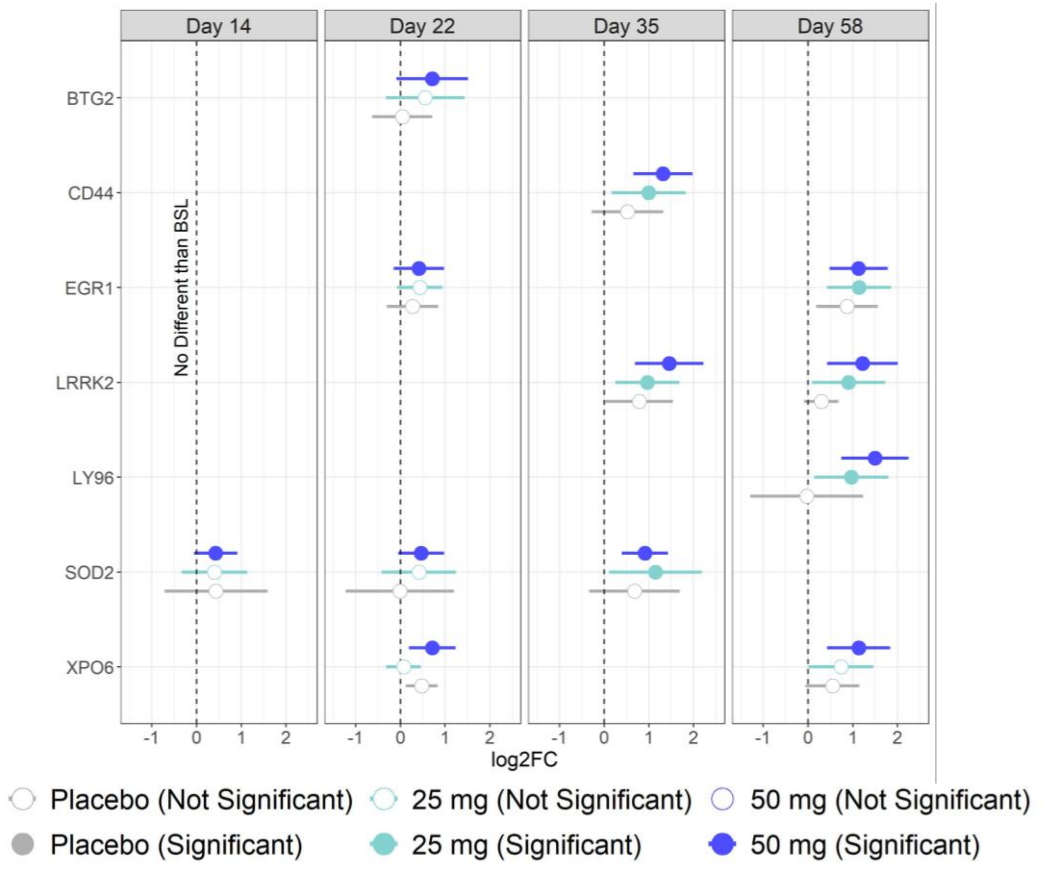
Increase Towards Normal Gene Expression in Adults with FA* Observed After Nomlabofusp Treatment

Select Baseline Gene Expression

Patients with FA* vs. Healthy Volunteers (HV)**



Post-treatment Changes in Gene Expression From Baseline



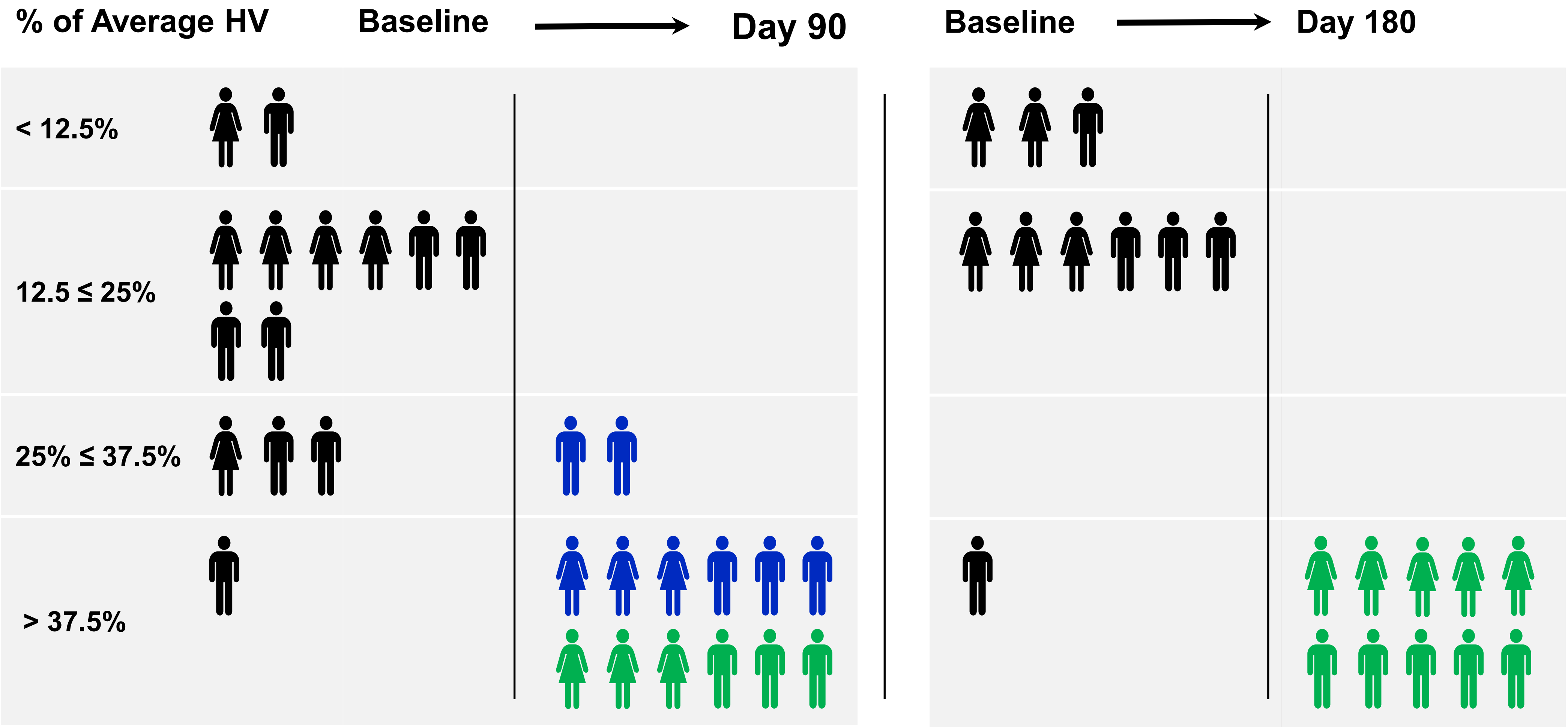
Data presented at the International Congress for Ataxia Research, November 2024

**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 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

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 (GAA_1)	60	550.0	555.8	99	1000
Longer GAA (GAA_2)	60	900.0	890.2	265	1300
Frataxin, % 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				

**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*

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

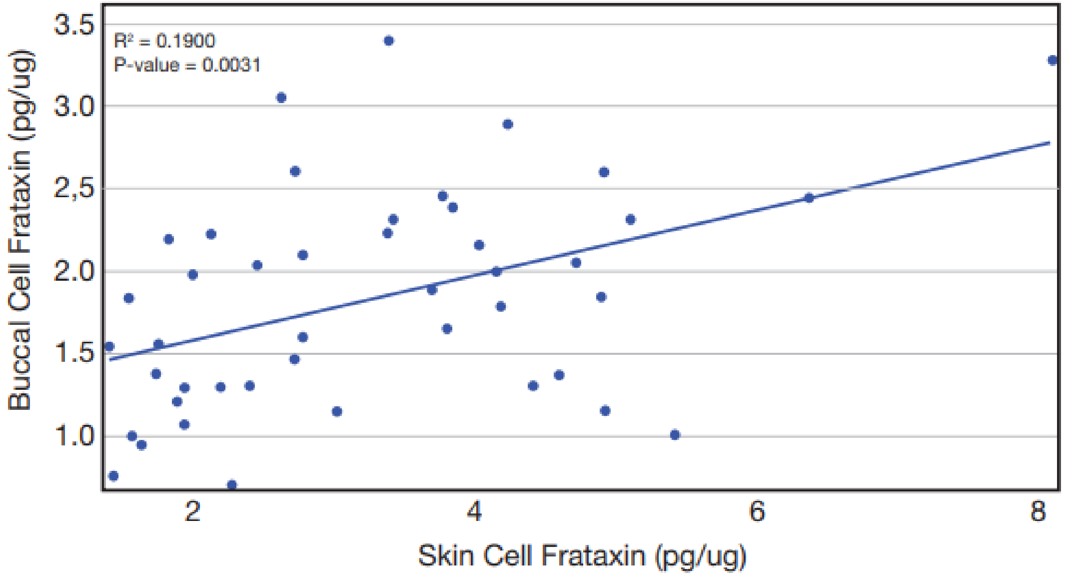
Disease Characteristics by Quartiles Based on Buccal Cell FXN Levels at Baseline

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

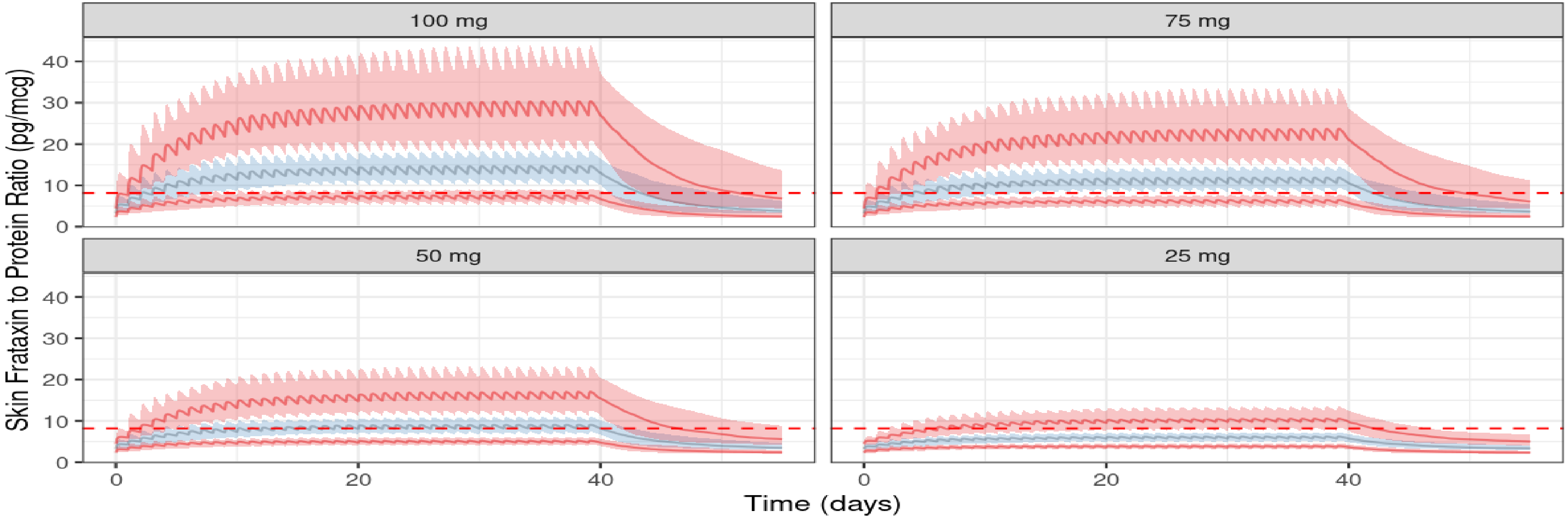
Baseline Buccal and Skin Cell FXN Levels



Buccal cell FXN levels correlated with skin cell FXN levels

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

Modeling/Simulation Predicts* 50mg Daily Can Achieve Skin FXN Levels ≥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/µg in FXN levels from baseline

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

Larimar Therapeutics

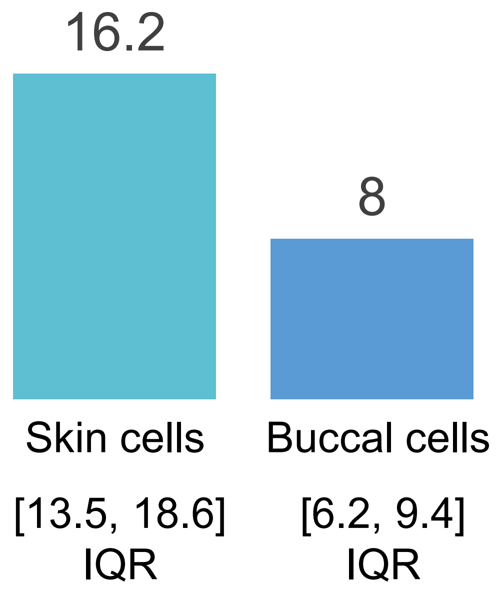
**PK/PD model was developed with data collected from 3 completed studies in adults with FA. A population of virtual FA patients (n = 100, 100 trials) receiving subcutaneous daily doses of 25, 50, 75, or 100 mg nomlabofusp for 40 days was simulated*

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/µg)
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 onset[2] (5 to 15 years of age)

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

Heterozygous carriers who show no signs of disease have buccal cell FXN levels of ~50% of unaffected healthy persons[1]

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

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"

FARA | Friedreich's Ataxia Research Alliance

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