

Larimar Therapeutics

Corporate Deck

November 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; the potential of nomlabofusp to address the underlying cause of FA and provide clinical benefit; the interpretation of clinical, biomarker and safety data and their ability to support the BLA; expectations regarding the FDA's acceptance for filing, review and potential approval of the BLA, including consideration of skin FXN as a potential novel surrogate endpoint; Larimar's planned research and development efforts, including the design, timing and conduct of FORWARD-FA; interactions with FDA; expectations regarding the potential for, accelerated approval or accelerated access and the impact of such accelerated access on the FDA's review timeline Larimar's preparations for and timing of a potential launch, if approved; the potential award and future sale of a Rare Pediatric Priority Review Voucher; and Larimar's overall development plans, business strategies, ability to raise capital, use of capital, results of operations and financial position, and plans and objectives for future operations.

In some cases, you can identify forward-looking statements by the words "may," "will," "could," "would," "should," "expect," "intend," "plan," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue," "ongoing" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, the success, cost and timing of Larimar's product development activities, nonclinical studies and clinical trials, including nomlabofusp clinical milestones and continued interactions with the FDA; that preliminary clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of nomlabofusp may not be predictive of the results or success of later 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 or determine that skin FXN is not an acceptable surrogate endpoint for accelerated approval; that completion of the BLA submission does not assure acceptance for filing or accelerated approval and that FDA may require additional information or studies; 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 successfully 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.

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

Targeting the Root Cause of Disease	Nomlabofusp is a first-in-class mitochondrial protein replacement therapy designed to directly address systemic frataxin deficiency in patients with FA, a rare neurodegenerative disease
Strong & Consistent Data Package	Data from 4 successfully completed studies (Phase 1 SAD and MAD, Phase 2 dose exploration, and adolescent PK); ongoing long-term OL study supports sustained increases in tissue FXN levels and continued directional improvements in clinical outcomes (Jun 2026 data release).
Completed BLA Submission	Completed rolling BLA submission seeking accelerated approval of nomlabofusp using skin FXN levels as a novel surrogate endpoint following FDA alignment on key elements of BLA package for submission. Patients currently enrolling in FORWARD-FA, the global confirmatory Phase 3 study.
Regulatory Designations	Breakthrough Designation, START Pilot Program, Rare Pediatric Disease Designation (US), Orphan Drug (US & EU), Fast Track (US), PRIME (EU) and ILAP (UK) designations
Potential Catalysts (estimated timing)	BLA submission acceptance – Early 2027 Open label study safety update – Q1 2027 U.S. approval-Mid-2027* and launch – Q3 2027

$128.7** million in cash and investments as of September 30, 2026, with projected cash runway into late Q2 2027;

*Expect to be eligible for rare pediatric disease priority review voucher.

**Estimate is unaudited and preliminary and actual results may differ due to the completion of our September 30, 2026 closing procedures.

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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*
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.
Intended to help patients avoid profound suffering and maintain or improve their quality of life.

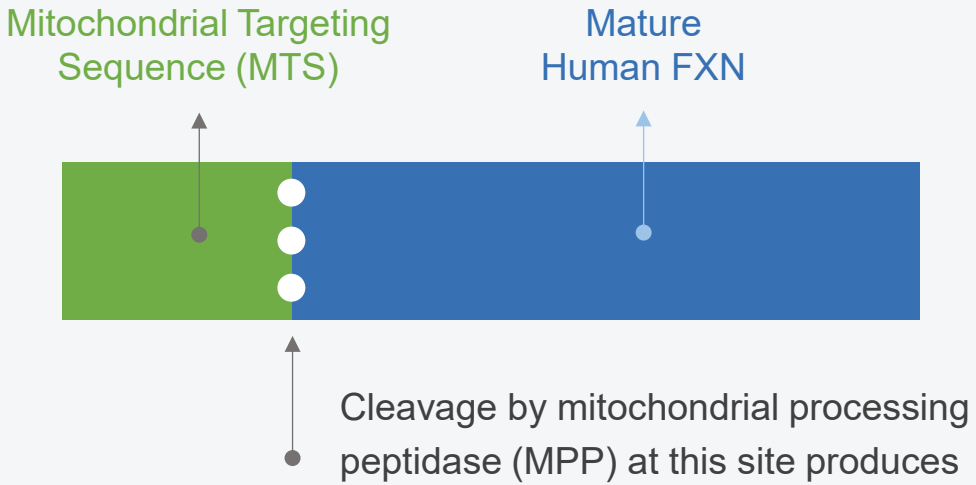
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* E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245.

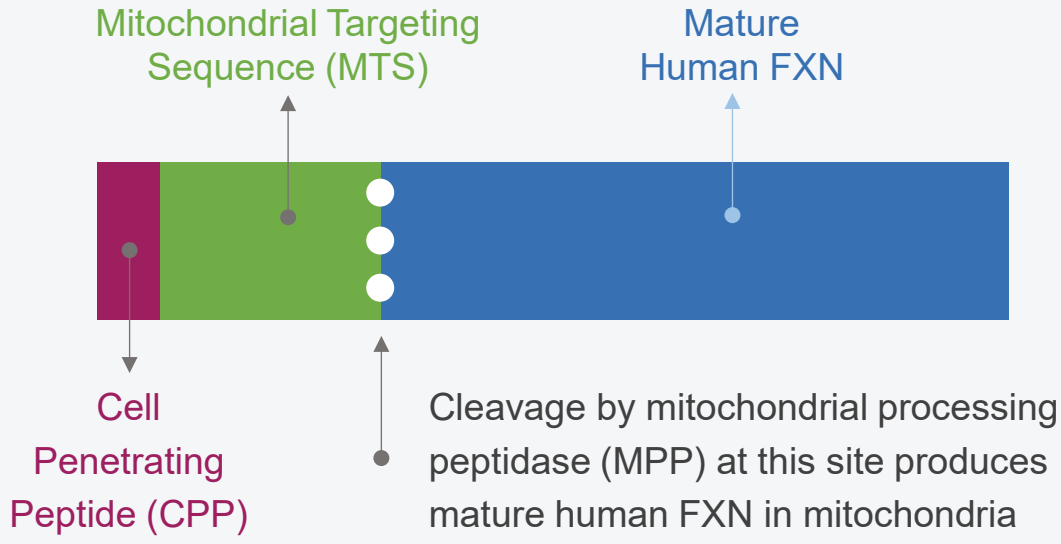
Nomlabofusp is Designed to Target the Root Cause of FA, FXN Deficiency

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

Structure of Endogenous FXN



Structure of nomlabofusp



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

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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.

Completed BLA Submission Seeking Accelerated Approval Following FDA Alignment of Key Elements of the BLA Package for Submission

Existing Clinical Data Package Appears Sufficient for Submission

FDA reviewed OL data submitted in a briefing package and confirmed that the **existing data package appears capable of supporting submission and review of a BLA seeking accelerated approval;** Approval will be a matter of review

FXN as Novel Surrogate Endpoint

FDA **reaffirmed willingness to consider FXN as a novel surrogate endpoint** and that Larimar's exposure-response analysis linking nomlabofusp exposures to clinical outcomes is the type that could support a BLA submission

BLA Submission Completed

November 2026

Potential Submission Acceptance

Early 2027

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Comprehensive and Long-Term Data Package*

Across All Studies

76
Participants

66
Received at least one dose of nomlabofusp

>10,000
Doses administered to date in the OL study

Open Label Study

32 had nomlabofusp exposure in prior studies

11 had no nomlabofusp exposure in prior studies

43 Participants dosed

22 Active
Maximum duration >800 days

Sustained increases in tissue FXN levels

Continued improvements in clinical outcomes

21 Discontinued

10 due to anaphylaxis (9 with prior nomlabofusp exposure; all recovered without sequelae)

3 due to generalized urticaria (none following initiation of antihistamine therapy)

3 due to other adverse events

5 for reasons unrelated to treatment (primarily logistical)

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*Data as of June 2026 data release.

Study Participants with Longer Exposure in OL Study* Reinforces the Disease-Modifying Potential of Nomlabofusp

13 participants completed 1 year of dosing, 7 of the 13 completed 18 months, and 3 of the 13 completed 2 years

Increased Tissue FXN Levels

Sustained increases in skin FXN levels

Achieved and maintained skin FXN levels within the range expected in asymptomatic carriers (~50% or higher)

- 82% (9/11) at 6 months
- **100% (9/9) at 1 year**
- **100% (3/3) at 18 months**

Observed Directional Improvement of Clinical Outcomes

At 1 year: a 2.6-point mFARS advantage when nomlabofusp treatment is compared to a FACOMS reference group

At 18 months: a 4.6-point mFARS advantage when nomlabofusp treatment is compared to a calculated value in a FACOMS reference group

Well-Characterized Safety Profile

Long term dosing continues to be generally well tolerated for up to a maximum of 800 days

Most common AEs are mild to moderate injection site reactions that decrease over time

10 cases of anaphylaxis,
9 with exposure to nomlabofusp in a prior study

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*Data as of June 2026 data release.

Nomlabofusp Long-term Open Label Study (Ongoing)

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

Patient Population

Initially, adults who had participated 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**

50 mg***
Nomlabofusp

Daily subcutaneous injections self-administered or by a caregiver

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

FACOMS is a longitudinal natural history study, includes patients with confirmed FA diagnosis

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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.
**Current dosing regimen includes a 5 mg Day 1 test dose; One hour after test dose, then 25 mg daily for the first 30 days
***Participants under 18 years of age receive a weight-based dose equivalent.

Participant Characteristics Represent a Broad Range of Disease Severity

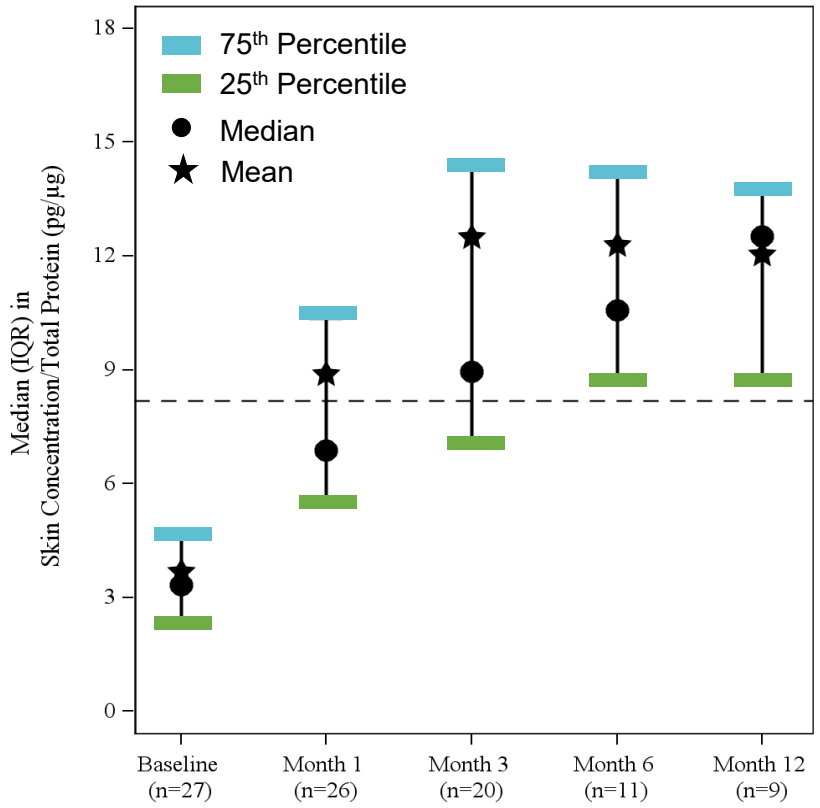
OL Study	Nomlabofusp* N = 41
Age of Screening (Years)	
Mean (SD)	29.0 (11.30)
Min, Max	12, 55
Age Group	
≥12 to <18	8 (19.5)
≥18	33 (80.5)
Age of Symptom Onset (Years)	
Mean (SD)	12.4 (5.98)
Min, Max	5, 30
Sex	
Male, n (%)	17 (41.5)
Ambulatory Status	
Ambulatory, n (%)	21 (51.2)
Previous Exposure to Nomlabofusp	
Yes, n (%)	31 (75.6)

OL Study	Nomlabofusp* N = 41
Previous Exposure to Omaveloxolone	
Yes, n (%)	20 (48.8)
Gait Score, n (%)	
1 - Mild ataxia	4 (9.7)
2 - Walks with definite ataxia	4 (9.7)
3 - Moderate ataxia	5 (12.2)
4 - Severe ataxia	8 (19.5)
5 - Cannot walk with assistance (wheelchair bound)	20 (48.8)
mFARS Total Score	
Mean (SD)	54.99 (16.96)
Min, Max	16.8, 85.5
9-HPT Average Time of the Dominant Hand(s)	
Mean (SD)	93.79 (65.5)
Min, Max	36.0, 277.3
FARS-ADL Score	
Mean (SD)	17.1 (7.1)
Min, Max	0.0, 27.0

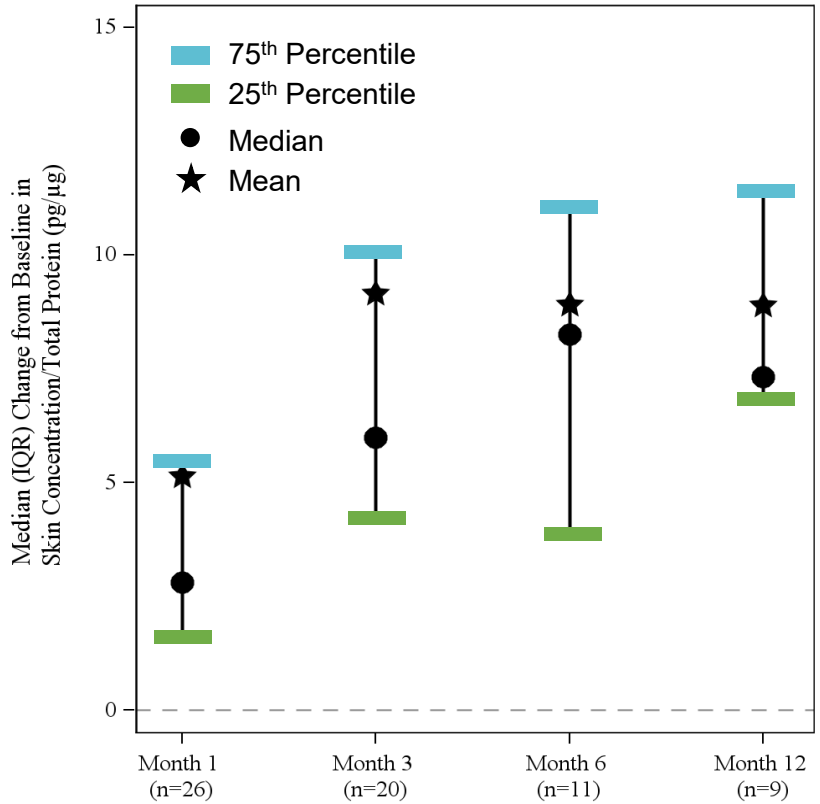
*As of June 2026, there were 43 participants including 2 dosed after the March 2026 cutoff.

Increases in Skin FXN Levels* Reached Steady State by 3 Months and were Sustained Over Time

Skin FXN Levels*



Change from Baseline in Skin FXN Levels*



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample.
Dotted Line indicates 50% of the average FXN concentrations of healthy volunteers.
Data include all participants with quantifiable FXN levels at baseline and at least 1 post-baseline FXN level.
Data are presented as of the March 2026 cutoff date.

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Nomlabofusp Treatment Increased and Sustained FXN Levels in Open Label Study to Range Expected in Asymptomatic Carriers

% of Participants with Skin FXN Levels in the Range of Asymptomatic Heterozygous Carriers (> 8.2 pg/µg; ~50% of Mean Healthy Volunteer FXN Concentration)					
Baseline	**1 month**	**3 months**	**6 months**	**1 year**	**18 months**
4% 1/27	**38%** 10/26	**50%** 10/20	**82%** 9/11	**100%** 9/9	**100%** 3/3

Absolute Skin FXN Levels* Increased Over Time with Nomlabofusp Treatment						
Statistic	**Baseline**	**1 month**	**3 months**	**6 months**	**1 year**	**18 months**
N	*27*	*26*	*20*	*11*	*9*	*3*
Mean	**3.7**	**8.9**	**12.5**	**12.3**	**12.1**	**10.7**
(Min, Max)	(1.5, 8.8)	(2.9, 22.9)	(5.6, 37.1)	(5.6, 26.7)	(8.1, 16.1)	(9.9, 11.8)

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*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. Data are presented as of the March 2026 cutoff date.

Safety Profile of Nomlabofusp Administration* is Well-Characterized

Nomlabofusp is generally well-tolerated long-term; >10,000 doses have been administered in the OL study

- Most common adverse events were local injection site reactions that were mild to moderate and decreased in frequency over time
- 21 participants discontinued
  - 10 experienced anaphylaxis
    - 9 with prior nomlabofusp exposure; one with no prior exposure
    - These participants returned to their usual state of health after standard treatment with no further sequelae
  - 3 experienced generalized urticaria (none since initiating antihistamine therapy)
  - 8 withdrew (3 associated with other adverse events; 5 for non-treatment related reasons (primarily logistical))
- 11 participants with no prior exposure; one had anaphylaxis
- Long-term daily dosing was generally well tolerated with 13 adults on treatment for 1 year, 7 of the 13 for 18 months, and 3 of the 13 for 2 years

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*Data as of June 2026 data release.

Baseline Characteristics Used to Generate FACOMS Reference Group

Methodology for generating reference group was reviewed by FDA and recommendations were incorporated

	Nomlabofusp* N = 41	FACOMS N = 362
Age of screening (years)		
Mean (SD)	29.0 (11.30)	25.6 (9.55)
Min, Max	12, 55	12, 55
Age of symptom onset (years)		
Mean (SD)	12.4 (5.98)	12.6 (5.25)
Min, Max	5, 30	5,30
Baseline mFARS Total Score		
Mean (SD)	55.0 (16.96)	50.6 (13.19)
Min, Max	16.8, 85.5	24.0, 83.0

	Nomlabofusp* N = 41	FACOMS N = 362
Baseline FARS-ADL Overall Score		
Mean (SD)	17.1 (7.1)	14.7 (5.30)
Min, Max	0, 27	2, 27
Baseline 9-HPT Average Time of Dominant Hand(s)		
Mean (SD)	93.8 (65.5)	75.4 (42.45)
Min, Max	36.0, 277.3	36.0, 262.1

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 2 years for each participant

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*Participants in open label study included 49% female, 49% with exposure to omaveloxolone and 51% non-ambulatory.

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

Comparison to external reference group intended to support the use of FXN for an accelerated approval

Clinical Outcome Measure Change from Baseline, Mean (Range)								
	mFARS [0- 93]		FARS-ADL [0- 36]		9-HPT Dominant Hand [Seconds]		MFIS [0- 84]	
	Nomlabofusp	FACOMS[1]	Nomlabofusp	FACOMS[1]	Nomlabofusp	FACOMS[1]	Nomlabofusp	FACOMS[1]
Baseline	**55.0** (16.8, 85.5) *n=41*	**50.6** (24.0, 83.0) *n=362*	**17.1** (0.0, 27.0) *n=41*	**14.7** (1.5, 27.0) *n=362*	**93.8** (36.0, 277.3) *n=37*	**75.4** (36.0, 262.1) *n=362*	**34.5** (2.0, 79.0) *n=41*	*NA*
Change at 1 year	**-1.0** (-6.5, 3.0) *n=13*	**1.6** (-15.7, 18.0) *n=189*	**-1.1** (-9.0, 2.5) *n=13*	**1.5** (-5.5, 9.0) *n=211*	**-15.6** (-46.7, 15.4) *n=12*	**6.1** (-40.1, 203.7) *n=194*	**-5.2** (-25.0, 10.0) *n=13*	*NA*
Change at 18 months	**-2.3** (-10.0, 4.5) *n=7*	*2.3*[2]	**-0.3** (-1.5, 1.0) *n=7*	*NA*	**-11.8** (-13.6, 6.5) *n=7*	*NA*	**0.6** (-16.0, 15.0) *n=7*	*NA*

Range = min, max.
Data are presented as of the March 2026 cutoff date.
[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] Data collected annually in FACOMS; 18-month value was interpolated using a linear equation constructed with annual data up to 3 years.

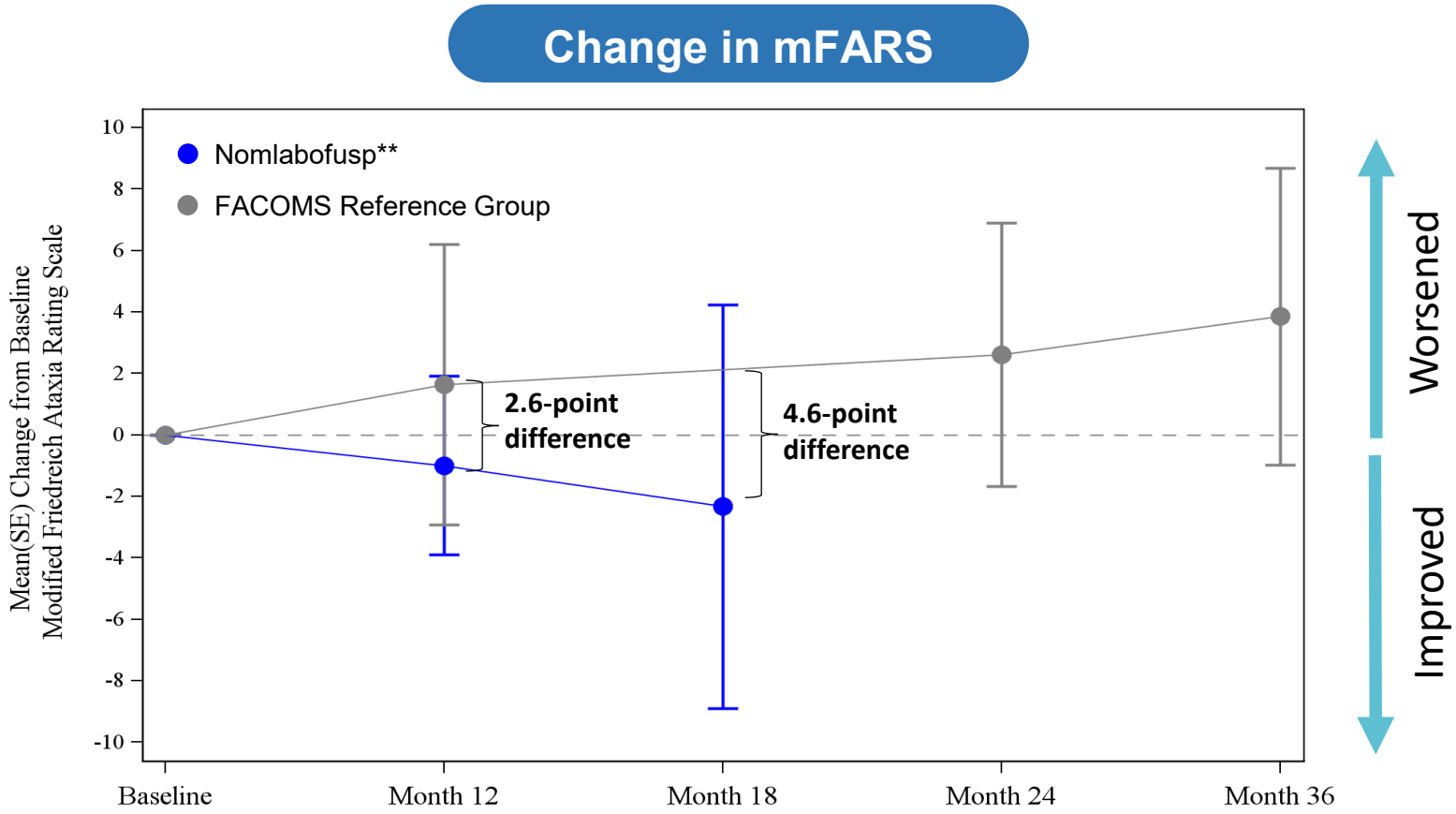
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2.6-point Difference in Mean mFARS Score for Nomlabofusp Treated Participants and FACOMS Reference Group at 1 year

A 4.6-point calculated* difference in mean mFARS score for treated participants and FACOMS Reference Group at 18 months

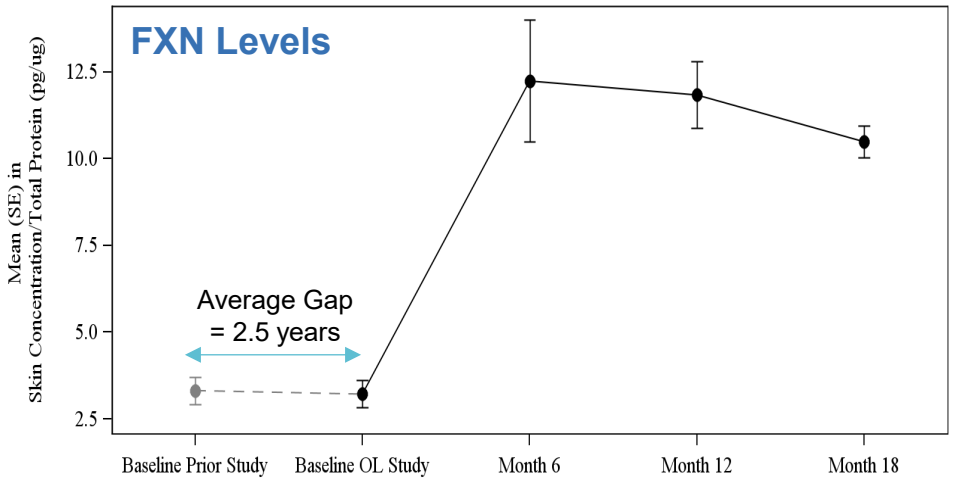
Change from baseline after treatment with nomlabofusp in OL study:

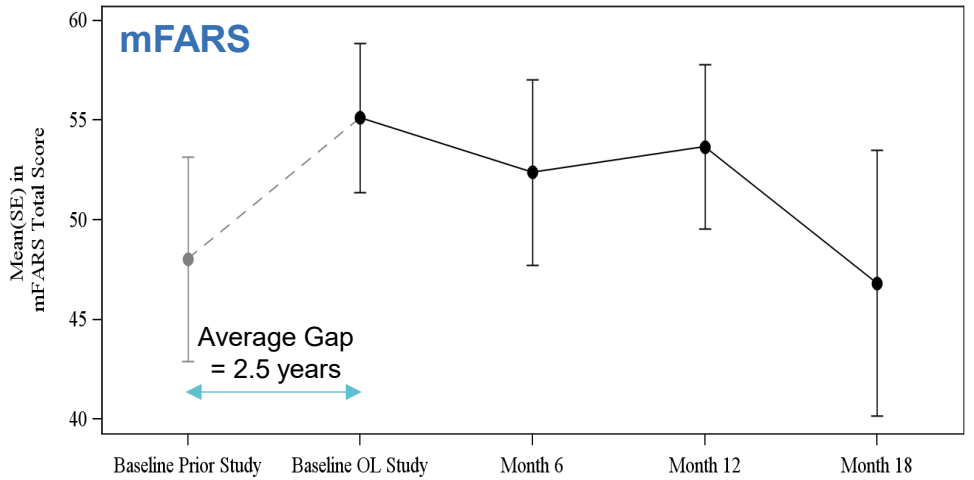
- **Mean 1.0-point improvement** at 1 year in 13 participants
- **Mean 2.3-point improvement** at 18 months in 7 participants

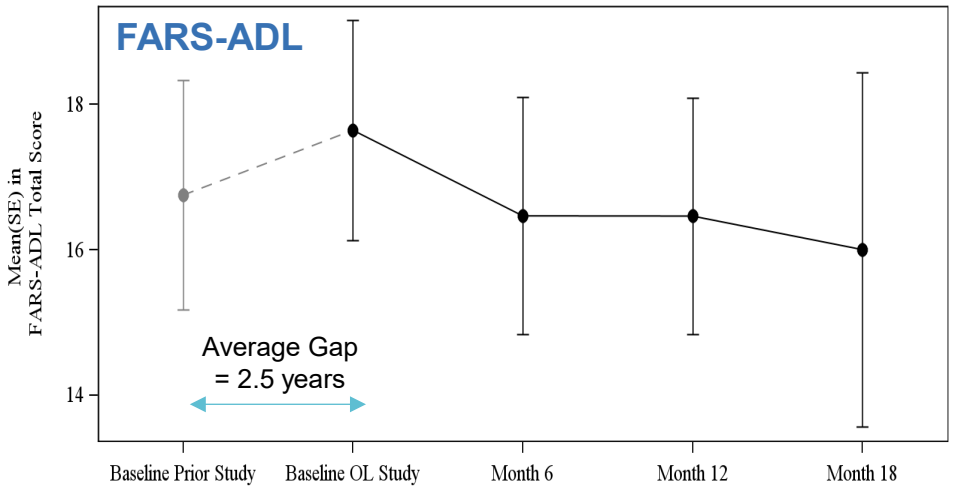


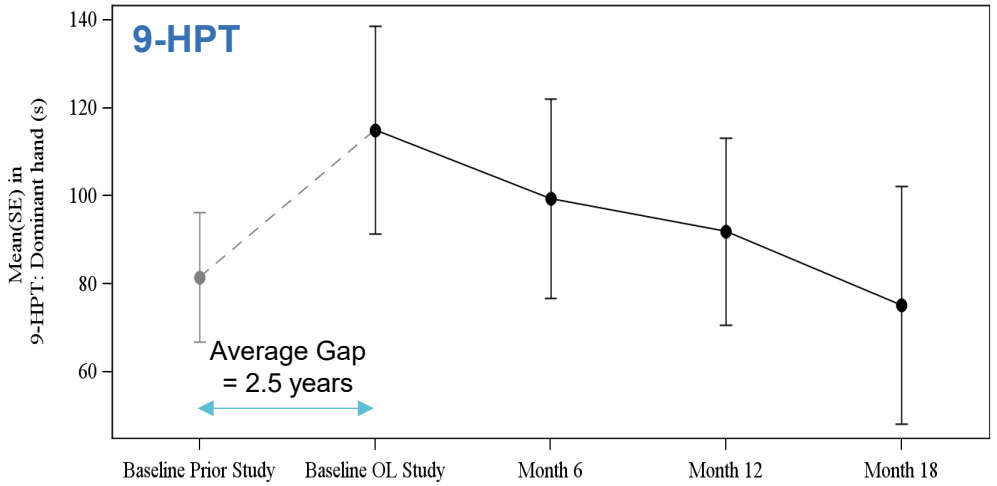
* Data collected annually in FACOMS; 18-month value was interpolated using a linear equation constructed with annual data up to 3 years.
** N at 1 year = 13; N at 18 months = 7.

Patients Who Worsened Between Prior Nomlabofusp Study and OL Study* Improved On Key Clinical Outcome Measures During Treatment









OL study: N at 1 year = 13; N at 18 months = 7.
*Data are presented as of the March 2026 cutoff date.

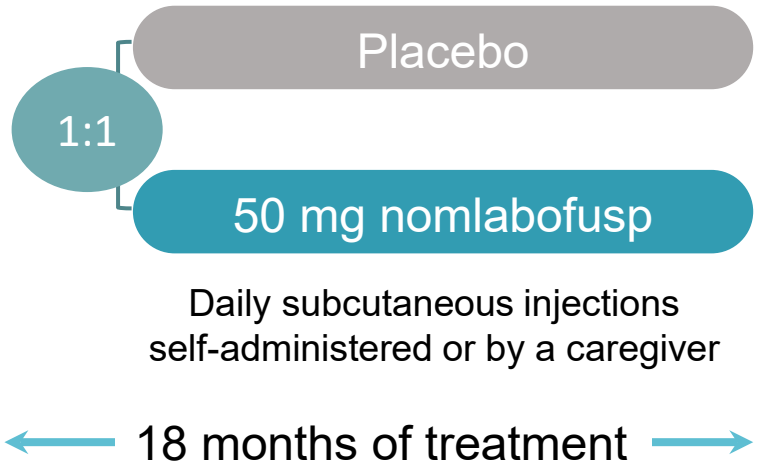
FORWARD-FA, a Global Confirmatory Phase 3 Double-Blind Placebo-Controlled Study

Currently enrolling patients

Patient Population

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

Sites in U.S., E.U., U.K., Canada, and Australia planned



Key Study Objectives

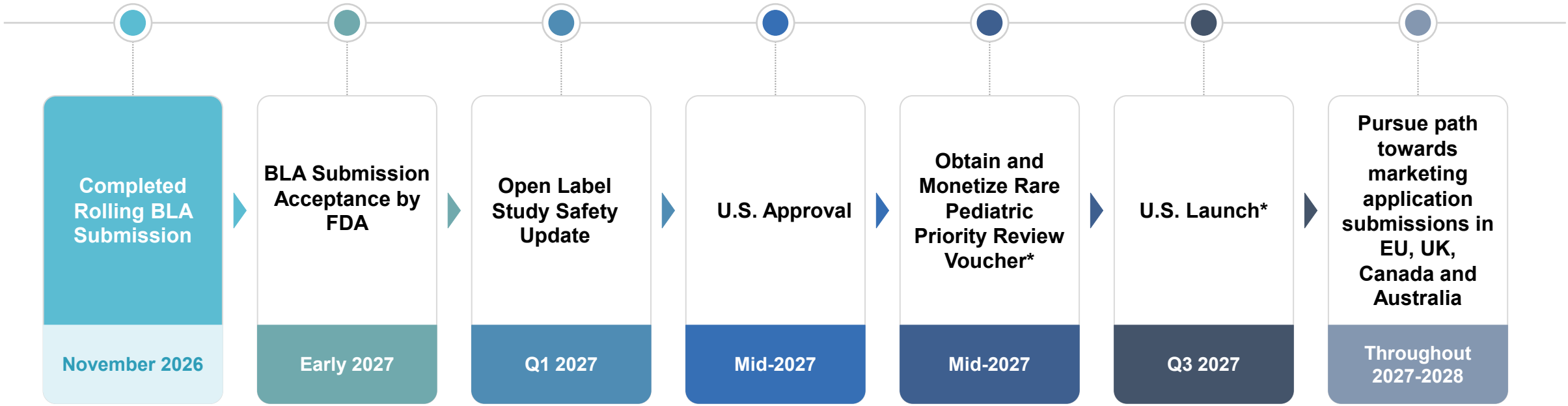
- Upright stability (U.S.) and mFARS (E.U.) as primary outcome measures
- Safety and tolerability

Phase 3 participants differ from OL study, with all expected to be ambulatory and ~2/3 under age 21

*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

Approaching Potential Approval Following Submission of BLA

Potential Upcoming Milestones



*Contingent on FDA approval

Nomlabofusp Advancing as the First Potential Disease-Modifying Therapy for Broad FA Population

BLA submitted seeking accelerated approval for nomlabofusp; potential submission acceptance in early 2027

Patients currently enrolling in FORWARD-FA, a global confirmatory Phase 3 study

Well-characterized and generally well-tolerated long term safety profile

Sustained FXN levels up to 18 months; Directional improvements in clinical outcomes at 1 year and at 18 months; Pursuit of broad label potentially supported by OL study data

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Appendix

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)

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Nomlabofusp Awarded Multiple U.S., EU, and U.K. Regulatory Designations Intended to Expedite the Development Program

US DESIGNATIONS

Breakthrough Therapy Designation

START Pilot Program

Orphan Drug Designation

Fast Track Designation

Rare Pediatric Disease Designation

Rare pediatric disease priority review voucher
program extended to 2029;
Expected to be available at time of approval

GLOBAL DESIGNATIONS

Orphan Drug Designation (EU)

PRIME Designation (EU)

Innovative Licensing and

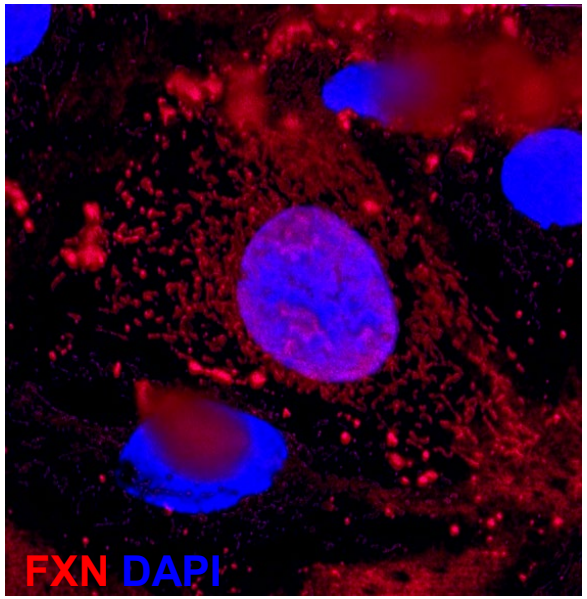
Access Pathway (ILAP) (UK)

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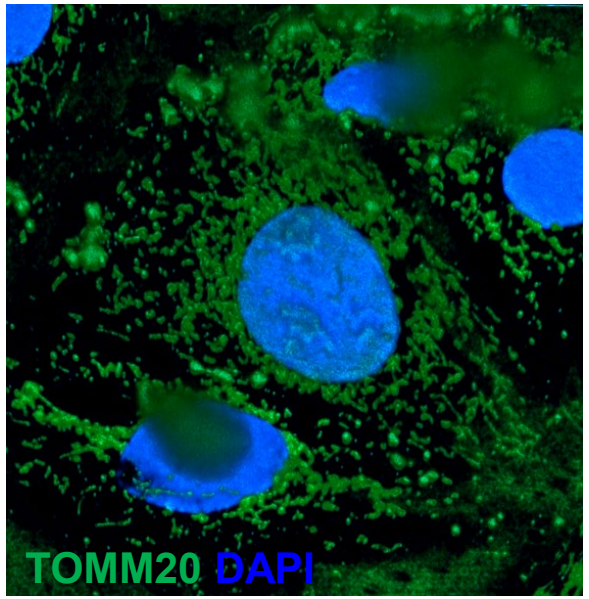
Mitochondrial Localization and Preclinical Data

Nomlabofusp Cell Transduction In Vitro Leads to hFXN in Mitochondria

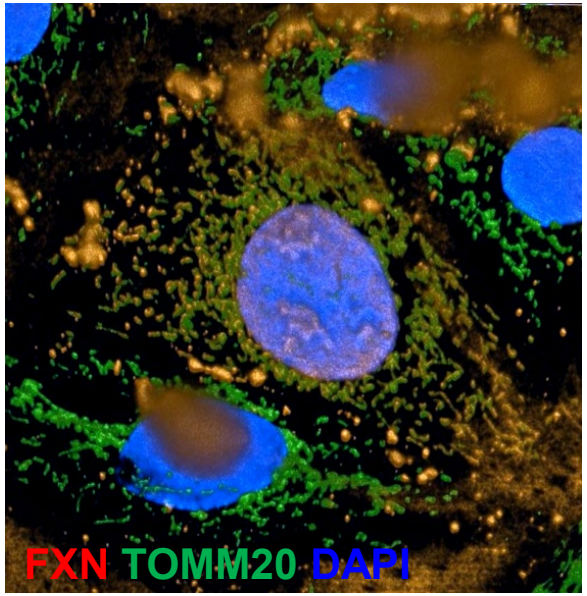
FXN staining



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

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Nomlabofusp Extends Survival in FXN-deficient KO Mice

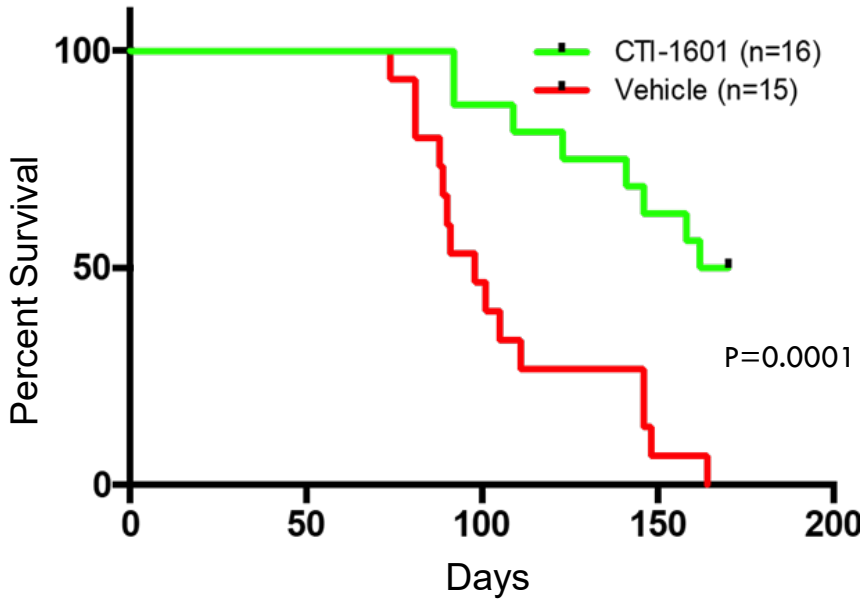
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

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

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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

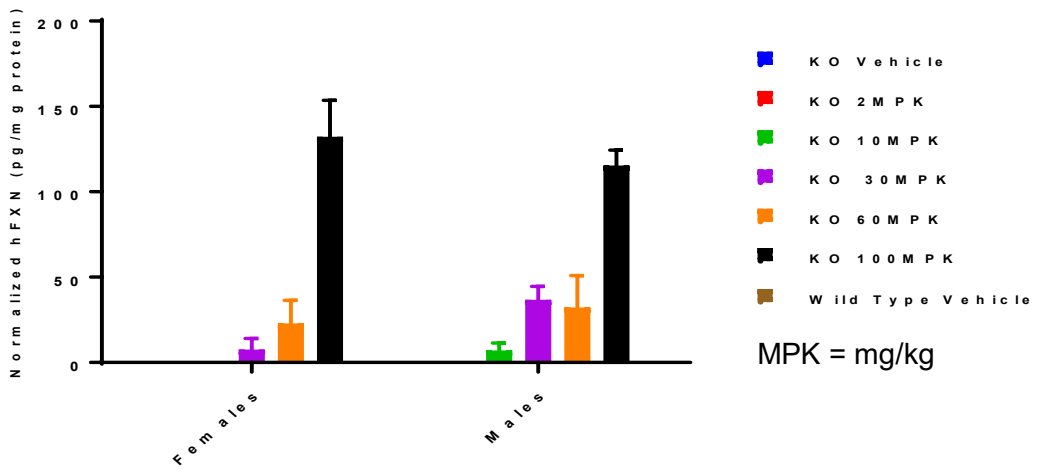
- hFXN replacement with nomlabofusp **prevents development of ataxic gait**
- Nomlabofusp-treated mice **survive longer** than untreated mice
- Human frataxin **present in brain, dorsal root ganglia and spinal cord** demonstrating central nervous system penetration

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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.

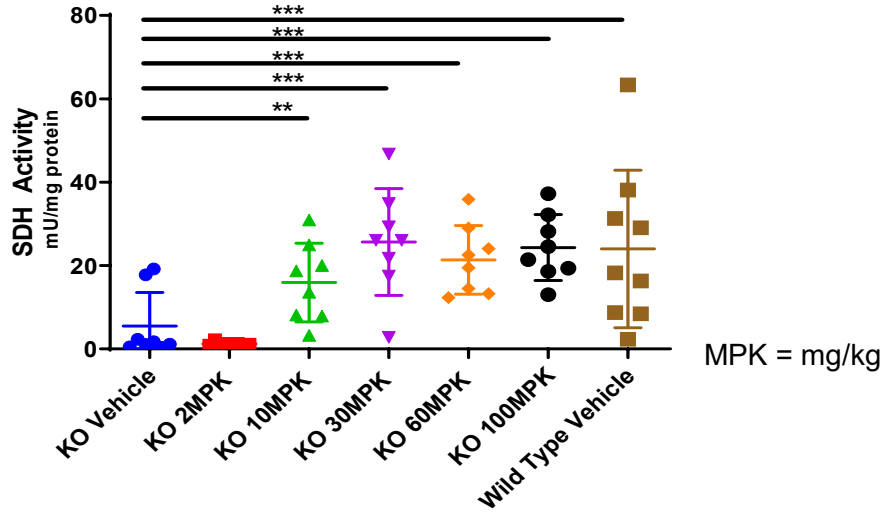
Mitochondrial FXN (Heart)



Mitochondria hFXN concentration increases dose-dependently

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

SDH Activity (Muscle)



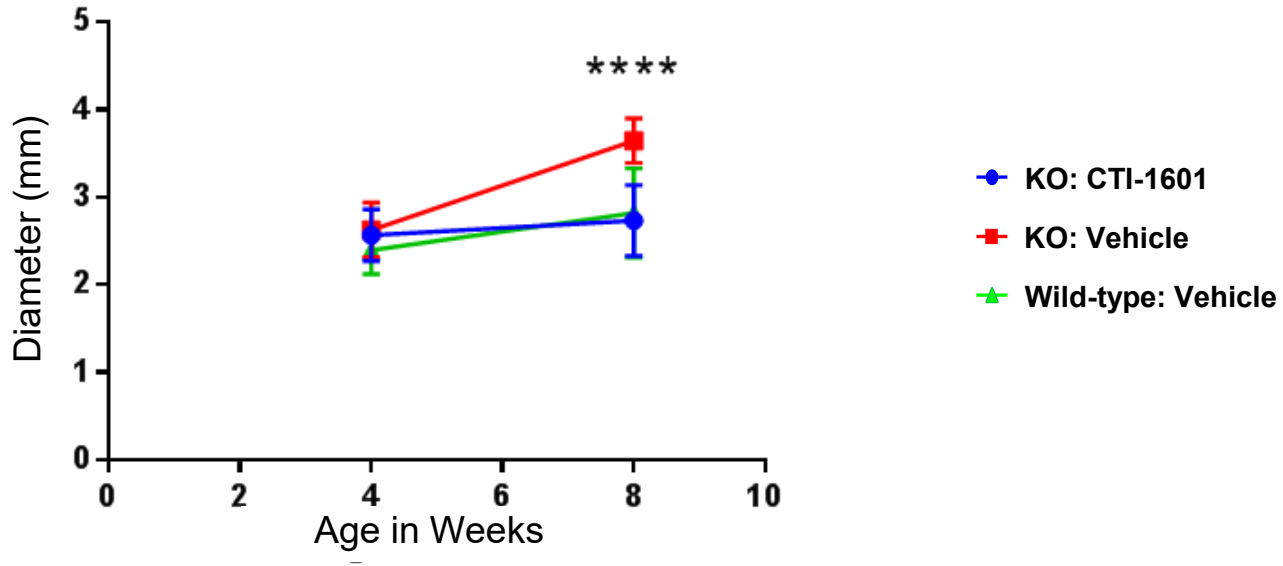
Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type

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Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

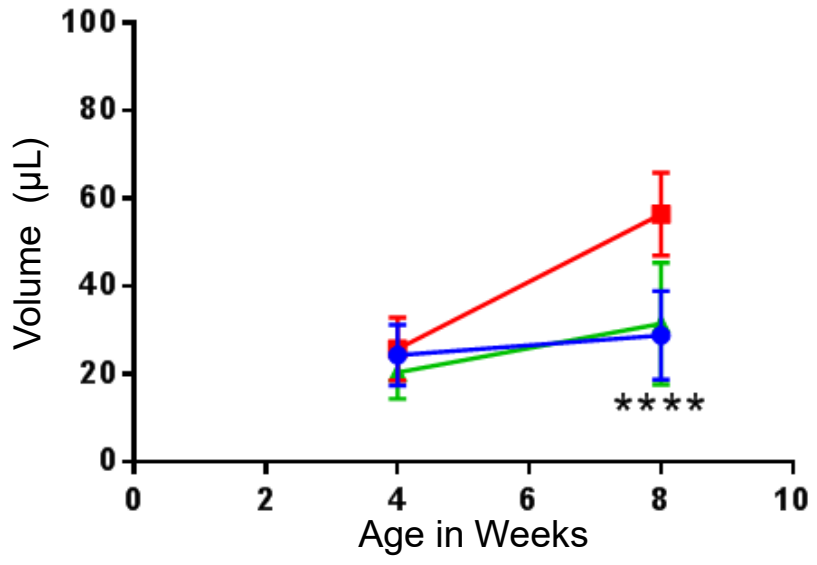
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

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)



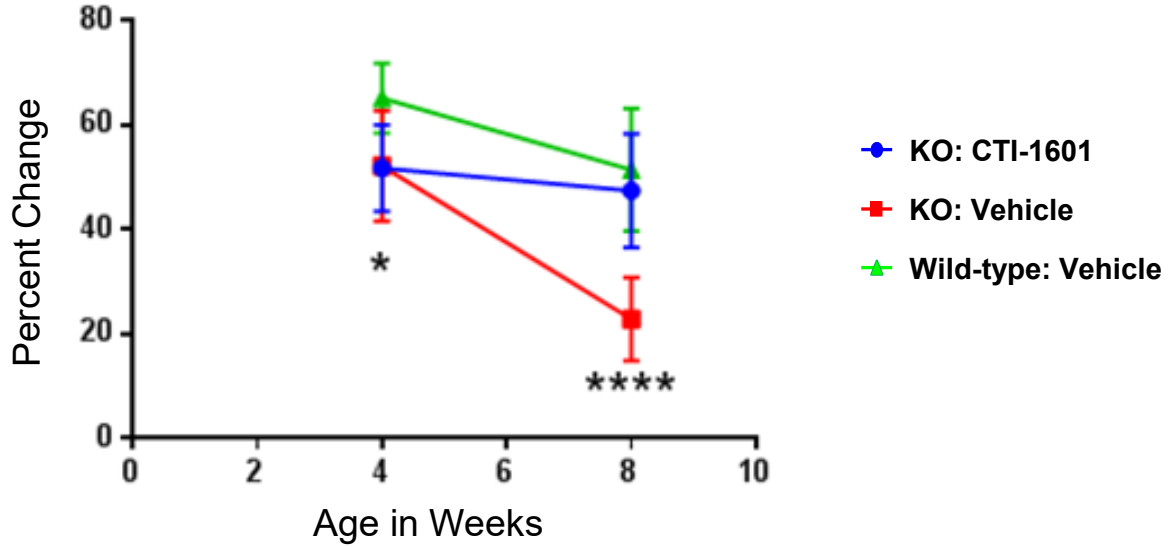
Nomlabofusp-treated mice have similar LV volume as wild type; echocardiogram shows significant differences between vehicle and nomlabofusp treated (10 mg/kg every other day) KO mice

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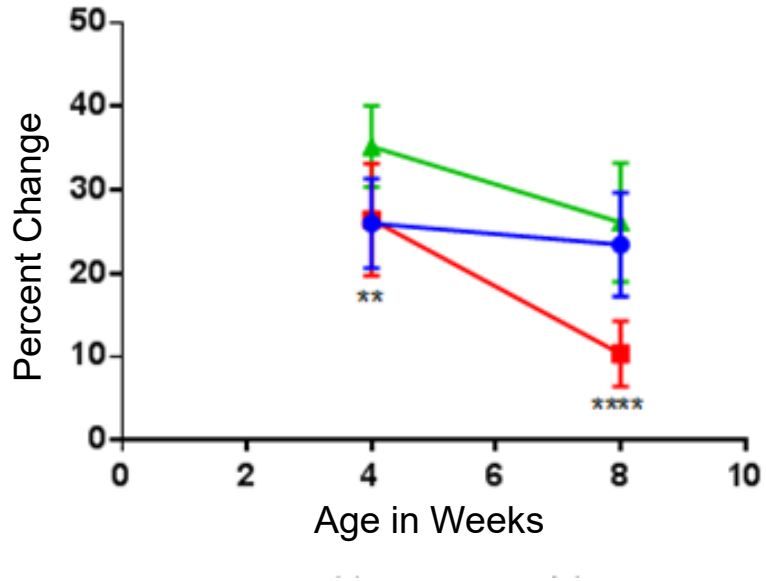
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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

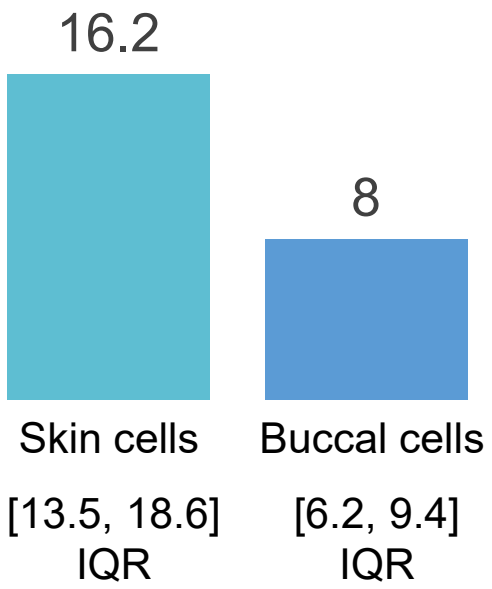
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Non-Interventional Study Data

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 Expedited 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**

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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