Larimar Therapeutics

Corporate Deck

March 2024

Forward-Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions of Larimar Therapeutics, Inc. (“Company”) 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 Larimar’s ability to develop and commercialize nomlabofusp (CTI-1601) and other planned product candidates, Larimar’s planned research and development efforts, including the timing of its nomlabofusp clinical trials and overall development plan and other matters regarding Larimar’s business strategies, ability to raise capital, use of capital, results of operations and financial position, and plans and objectives for future operations.

In some cases, you can identify forward-looking statements by the words “may,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “ongoing” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, the success, cost and timing of Larimar’s product development activities, nonclinical studies and clinical trials, including nomlabofusp clinical milestones and continued interactions with the FDA; that preliminary clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of nomlabofusp may not be predictive of the results or success of later clinical trials, and assessments; that the FDA may not ultimately agree with Larimar’s nomlabofusp development strategy; the potential impact of public health crises on Larimar’s future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and general economic conditions; Larimar’s ability and the ability of third-party manufacturers Larimar engages, to optimize and scale nomlabofusp’s manufacturing process; Larimar’s ability to obtain regulatory approvals for nomlabofusp and future product candidates; Larimar’s ability to develop sales and marketing capabilities, whether alone or with potential future collaborators, and to successfully commercialize any approved product candidates; Larimar’s ability to raise the necessary capital to conduct its product development activities; and other risks described in the filings made by Larimar with the Securities and Exchange Commission (SEC), including but not limited to Larimar’s periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. These forward-looking statements are based on a combination of facts and factors currently known by Larimar and its projections of the future, about which it cannot be certain. As a result, the forward-looking statements may not prove to be accurate. The forward-looking statements in this presentation represent Larimar’s management’s views only as of the date hereof. Larimar undertakes no obligation to update any forward-looking statements for any reason, except as required by law.

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

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

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

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

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

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

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

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

OLE trial initiated for 25 mg daily dosing

High patient interest in study participation
First patient dosed in March 2024
Initial data expected Q4 2024

Intend to Pursue Accelerated Approval with FDA

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

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

Clinical-Stage Novel Protein Replacement Therapy Platform

Potential first therapy to increase frataxin levels	Lead candidate nomlabofusp (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering the protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations
Consistent Phase 1 and Phase 2 findings	Nomlabofusp was generally well tolerated and demonstrated dose-dependent increases in frataxin (FXN) levels from baseline in skin and buccal cells in a 4-week placebo-controlled Phase 2 study and a multiple ascending dose Phase 1 study
Intend to pursue accelerated approval with FDA	FDA acknowledgement that FXN deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. Discussions to support an accelerated approval are ongoing. BLA submission targeted for 2H 2025
OLE study with near-term catalysts	Dosed first patient in OLE study with 25 mg daily dosing in Q1 2024 with **interim data expected in Q4 2024** To potentially escalate dose in the OLE study, data from the 50 mg cohort of the Phase 2 study and available data from the OLE study will be submitted for FDA review due to continued partial clinical hold
Strong financial foundation	Cash $86.8 million estimated* at 12/31/23 plus $161 million net proceeds from February 2024 public offering provides projected cash runway into 2026

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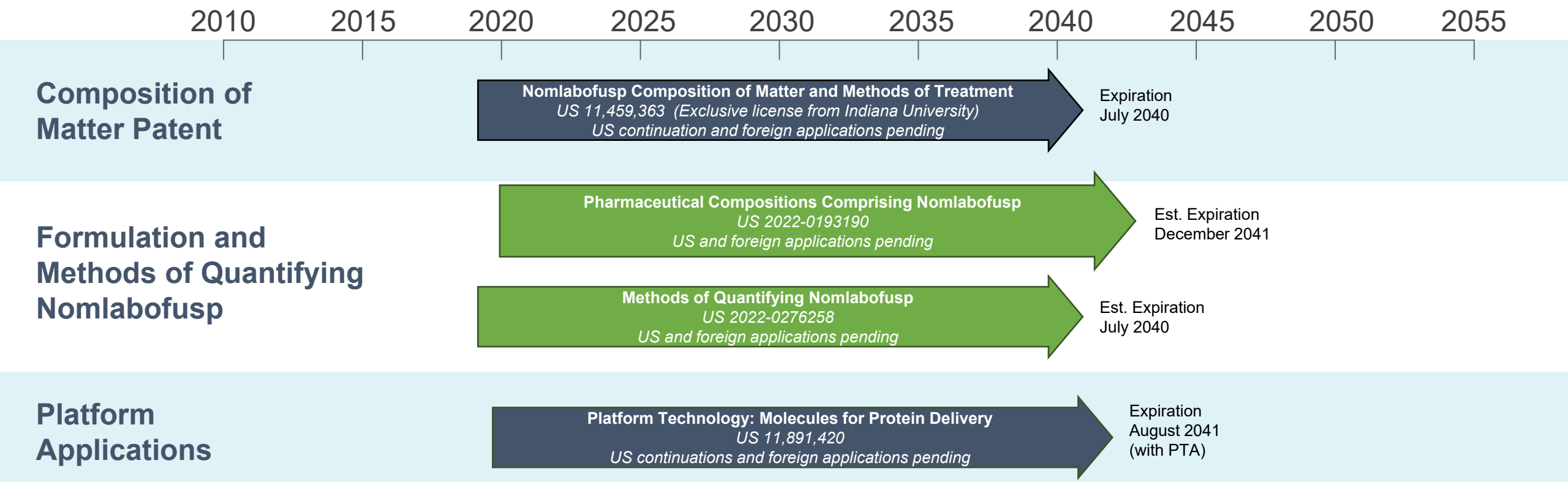
*This estimate is unaudited and preliminary and actual results may differ due to the completion of our fiscal 2023 closing procedures. As such, this estimate should not be viewed as a substitute for our full audited financial statements prepared in accordance with U.S. generally accepted accounting principles.

Larimar Technology is Supported by a Strong IP Portfolio

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



Additional nomlabofusp IP protection

- US and foreign pending applications cover key biomarkers, analytical tools and methods of treatment for additional disease indications for nomlabofusp
- Nomlabofusp should be eligible for **12 years of market exclusivity** upon approval in the US (independent of patents) and at least **10 years of market exclusivity** upon approval in EU (independent of patents)

Granted Pending

Friedreich's Ataxia (FA): A rare and progressive disease

Genetic defect on both alleles lowers frataxin levels

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

Affects ~20,000 patients globally

~5,000 patients in the U.S., with most remaining patients in the EU
~70% of patients present before age 14

Progressive disease

Initial symptoms include unsteady posture and frequent falling, and patients are eventually confined to a wheelchair
Life expectancy of 30-50 years with an early death usually caused by heart disease

No approved therapies increase frataxin levels

Only treatment approved for FA does not address frataxin deficiency

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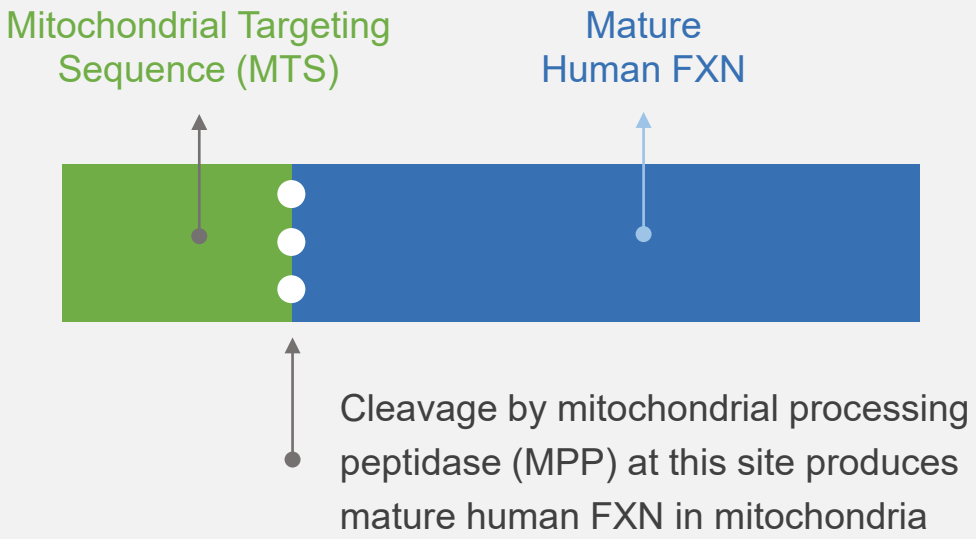
* E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245.

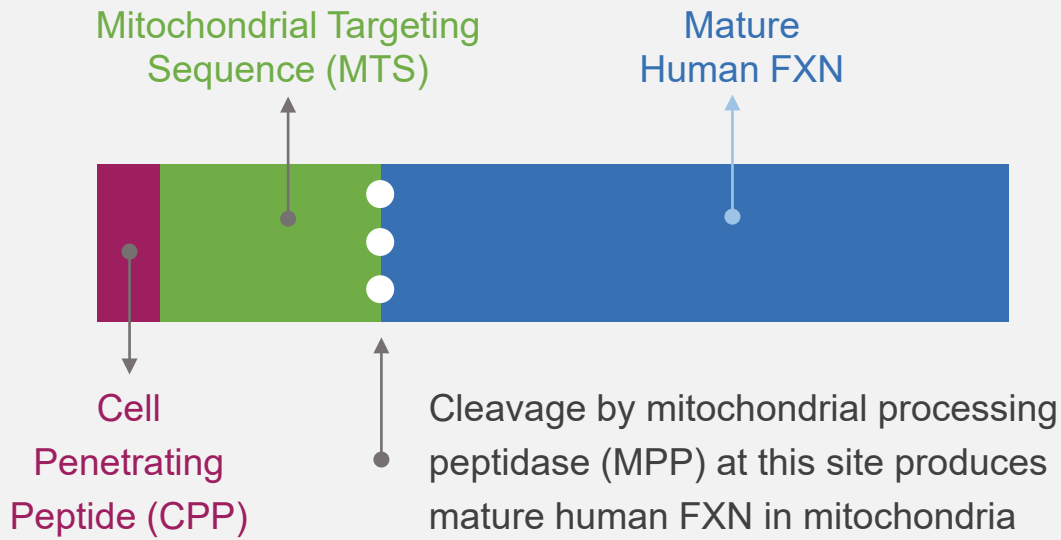
Nomlabofusp is Designed to Deliver Additional Frataxin

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

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 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.

Phase 2 Dose Exploration Study for 25 and 50 mg Cohorts

Goal: Further characterize PK/PD and assess safety to inform long-term dose and dose regimen

Treatment Schedule - nomlabofusp (CTI-1601) or placebo

28-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28

= 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)

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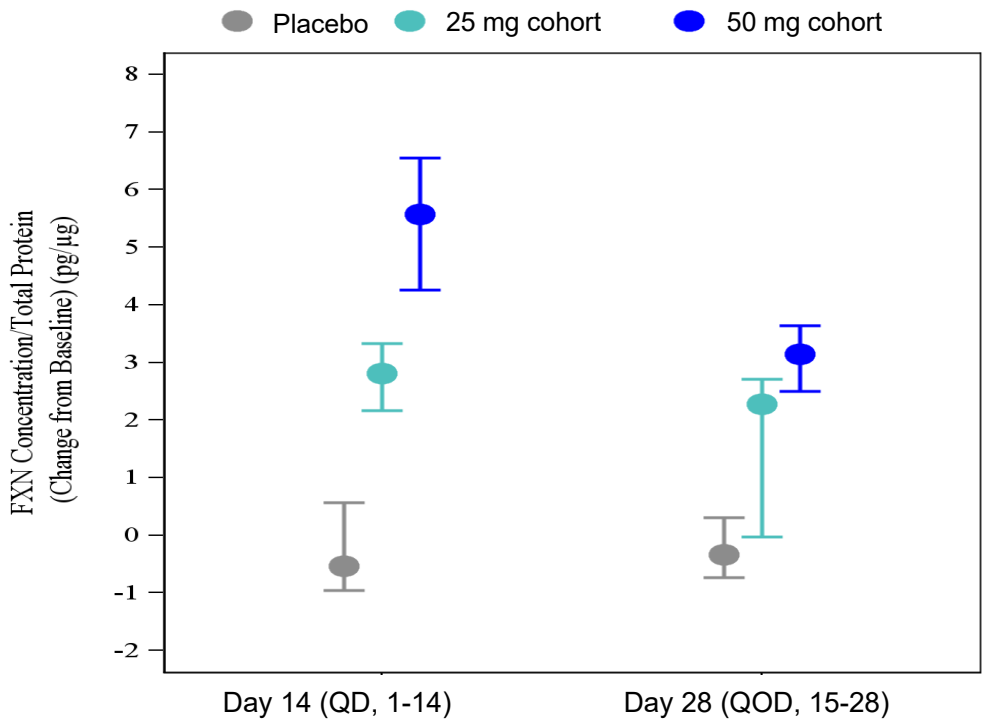
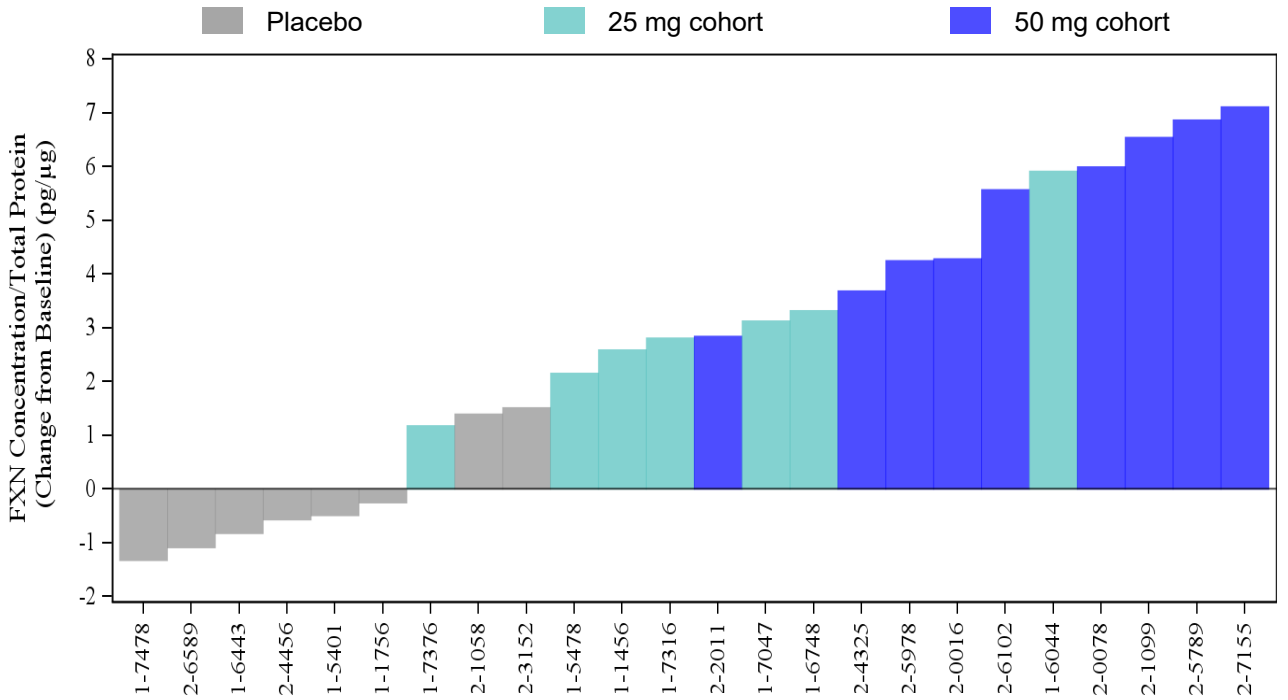
Dose-Dependent Increase in FXN Levels in Skin Cells

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

Skin Cells
FXN Levels* Change from Baseline**



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

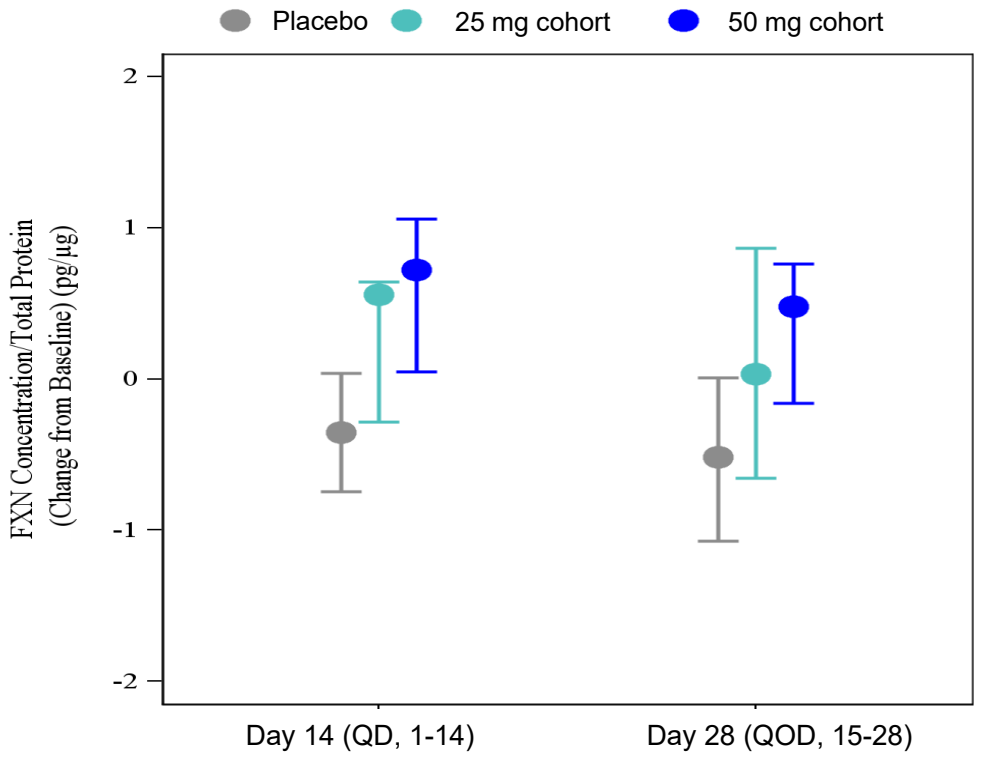


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.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

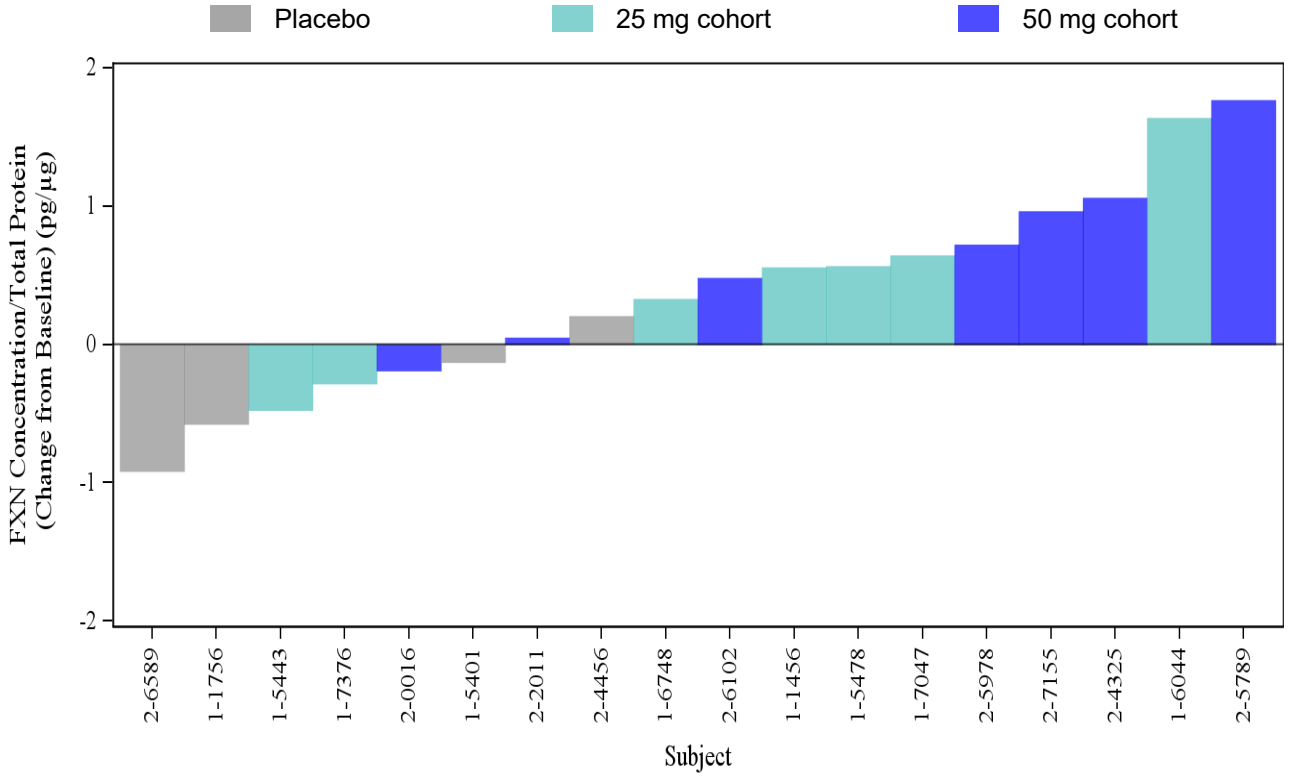
Dose-Dependent Increase in FXN Levels in Buccal Cells

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

Buccal Cells
FXN Levels* Change from Baseline**



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



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.
**Median baseline FXN level in patients were 2.1 pg/µg for the placebo, 1.8 pg/µg for the 25 mg cohort and 1.6 pg/µg for the 50 mg cohort.

Absolute Increases in Skin FXN Levels

Dose response in tissue FXN concentrations and increases from baseline after dosing

Day 14 Skin FXN Levels			
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 14	5.53	6.40
	Change from Baseline	**2.81**	**3.02**
50 mg	Baseline	2.12	2.08
	Day 14	7.40	7.32
	Change from Baseline	**5.57**	**5.24**

Day 28 Skin FXN Levels			
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	3.70	3.38
	Day 28	4.39	4.80
	Change from Baseline	**2.28**	**1.41**
50 mg	Baseline	2.12	2.08
	Day 28	5.23	5.24
	Change from Baseline	**3.14**	**3.17**

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

Absolute Increases in Buccal FXN Levels

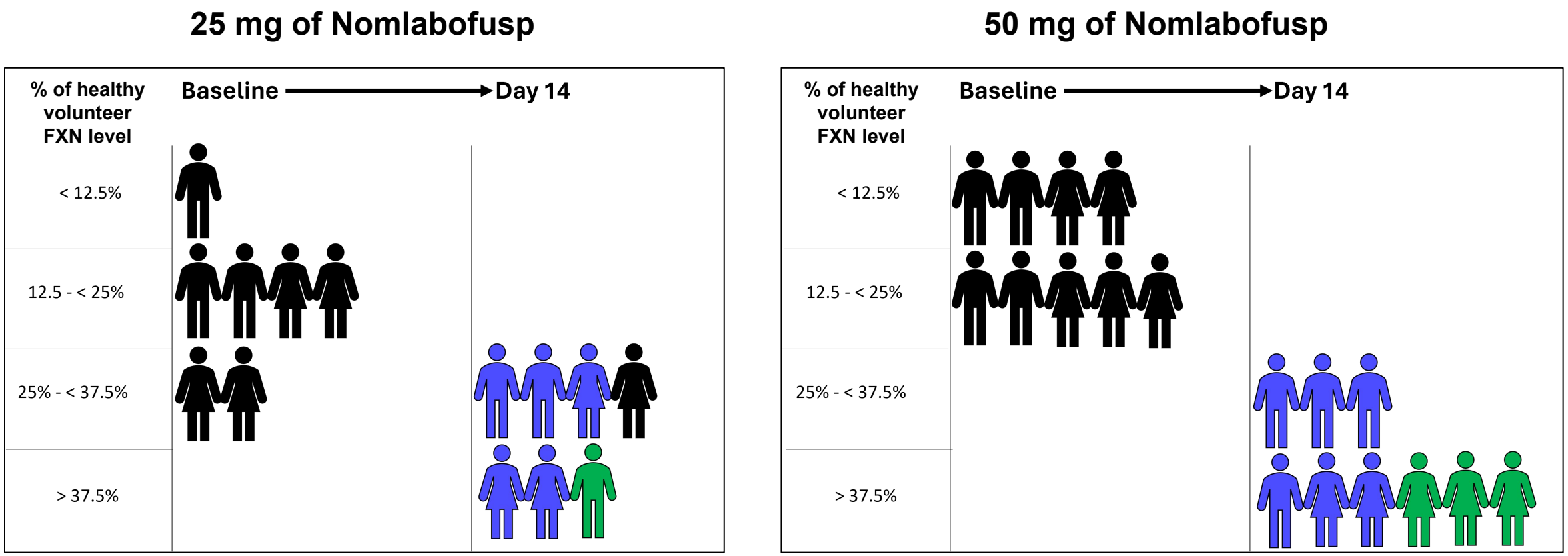
Dose response in tissue FXN concentrations and increases from baseline after dosing

Day 14 Buccal FXN Levels			
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	1.78	1.80
	Day 14	2.24	2.22
	Change from Baseline	**0.56**	**0.42**
50 mg	Baseline	1.61	1.69
	Day 14	2.44	2.38
	Change from Baseline	**0.72**	**0.69**

Day 28 Buccal FXN Levels			
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	1.70	1.65
	Day 28	1.73	1.76
	Change from Baseline	**0.03**	**0.11**
50 mg	Baseline	1.76	1.77
	Day 28	2.15	2.15
	Change from Baseline	**0.48**	**0.38**

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

Skin Cell FXN Levels Achieve Higher % of Healthy Volunteers* Following 14 days of Daily Nomlabofusp



Baseline FXN levels as a % of average FXN level in healthy volunteers

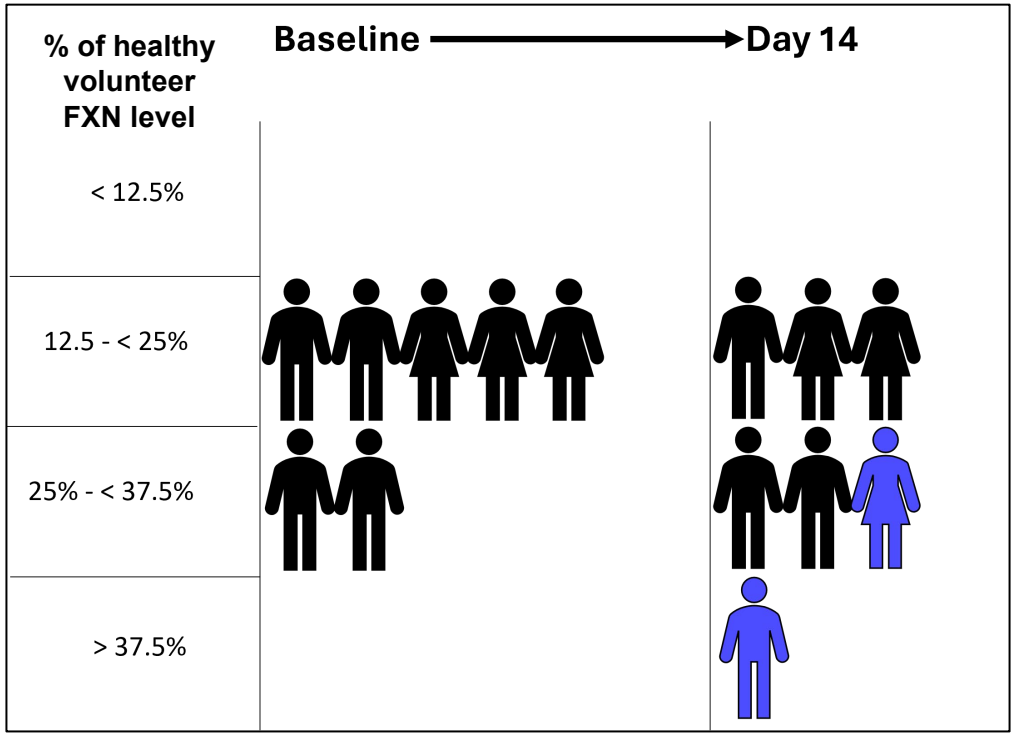
FXN levels increased from baseline and reached 25% to < 50% of average FXN level in healthy volunteers

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

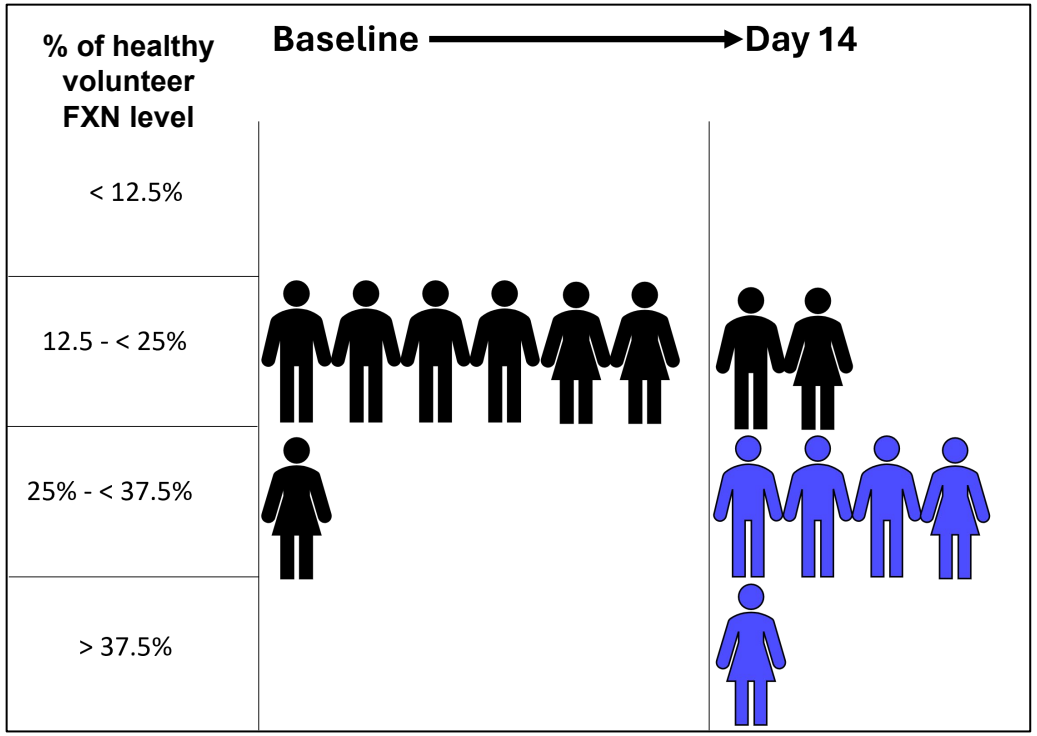
Only participants with quantifiable levels at baseline and day 14 are included in the figures.
*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/µg) from the noninterventional healthy volunteer study (N=60).

Buccal Cell FXN Levels Achieve Higher % of Healthy Volunteers* Following 14 days of Daily Nomlabofusp

25 mg of Nomlabofusp



50 mg of Nomlabofusp



Baseline FXN levels as a % of average FXN level in healthy volunteers

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

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Only participants with quantifiable levels at baseline and day 14 are included in the figures.
*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (8.24 pg/µg) from Larimar's noninterventional healthy volunteer study (N=60).

Increasing FXN Levels May Slow Disease Progression

Disease Characteristics* Based on Literature**

Patients with FXN levels 11% of average healthy volunteers

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

Patients with FXN levels > 30% of average healthy volunteers

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

Nomlabofusp Administration in Phase 2 Study

25 mg daily for 14 days shifted FXN levels in

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

50 mg daily for 14 days shifted FXN levels in

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

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

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*FARS: Friedreich's ataxia rating scale, measures disease progression with a higher score indicating a greater level of disability.
**FXN levels measured in peripheral blood mononuclear cells (PBMCs). FXN levels as measured by % of normal demonstrated to be equivalent in PBMCs, buccal cells, and whole blood.

Encouraging Therapeutic Potential for Nomlabofusp

Frataxin deficiency is the root cause of the disease

Lower levels of frataxin correlate with disease burden

Animal models show that increasing frataxin mitigates clinical outcomes

Dose-dependent increases in frataxin levels with nomlabofusp in several studies

Continue nomlabofusp clinical development

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Nomlabofusp: Predictable Pharmacokinetics

1. Quick absorption after subcutaneous administration
2. Dose-proportional increases in exposure observed
3. Pharmacokinetic profile consistent with Phase 1 studies

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Ph1 & Ph2 Data: Nomlabofusp is Generally Well Tolerated

61 patients have participated in our Phase 1 and Phase 2 studies with no serious adverse events in any nomlabofusp clinical study. One severe adverse event occurred, an allergic reaction that resolved with standard treatment referenced below.

44 of 46 clinical trial participants dosed with nomlabofusp completed their respective study

One Phase 2 participant in the 25 mg cohort withdrew due to allergic reaction that resolved with standard treatment

One Phase 1 participant in the 50 mg cohort withdrew due to mild-to-moderate nausea and vomiting

Most common adverse events (AEs) were mild and moderate injection site reactions (ISRs)

No study discontinuations due to ISRs and all resolved

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Open-label Extension Study: Dosed first patient in Q1 2024

Preliminary interim data expected in Q4 2024

Key Eligibility Criteria

Previous participation in Phase 1 or Phase 2 trials

Daily subcutaneous injection of 25 mg nomlabofusp;
self-administered or by a caregiver

1 site initiated and screening has begun
First patient dosed

Key Study Objectives

- Safety and tolerability
- Long-term PK
- Tissue FXN concentrations and potential use as surrogate endpoint to support accelerated approval
- Clinical efficacy measures compared to the matched set of untreated patients from FACOMS* database

Screening Period ≤ 42 days** → Treatment Period Planned for ≥ 1 year → Potential extensions

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*FACOMS: Friedreich's Ataxia Clinical Outcome Measures Study.
**Estimated screening period may be extended for those study participants who have not been on a stable regimen of omaveloxolone for at least six months.

Nomlabofusp Clinical Development Plan

Intend to pursue accelerated approval pathway with potential BLA submission targeted for 2H 2025

Ongoing open-label extension study with 25 mg daily dosing for eligible patients who participated in SAD, MAD, and/or four-week dose exploration studies

Initial data expected Q4 2024

Plan to include pediatric patients 2 to 17 years of age in clinical development*

Participants eligible to participate in long term studies

Planned global double-blind placebo-controlled registration/confirmatory study**

BLA submission targeted for 2H 2025

*Company is discussing with FDA as to what additional clinical trial data in adults would inform inclusion of pediatric patients ages 2 to 17 in our studies.
**Company initiated discussions with FDA on the potential use of FXN levels to support accelerated approval. Also, the Company is planning discussions with regulators and investigators outside the U.S. to expand clinical program to international geographies.
Initiation of additional U.S. clinical trials is contingent on FDA review of clinical data due to partial clinical hold.

Nomlabofusp is a Competitively Differentiated Treatment Approach*

$7.3B Acquisition supports the **robust market potential** for FA treatments

REATA PHARMACEUTICALS
Biogen

Nomlabofusp is a potential **first-and-only protein replacement therapy** designed to address the underlying cause of FA

Approach	Product	Company	Mechanism of Action	Clinical Status
Protein replacement	**Nomlabofusp (CTI-1601)**	**Larimar**	**Recombinant frataxin protein**	**Phase II**
Mitochondrial Oxidative Stress Modifier	Omaveloxolone (SKYCLARYS™)	Reata Pharma/Biogen	Nrf2 Activator	Approved (US and EU)
	Vatiquinone	PTC Therapeutics	15-Lipoxygenase Inhibitor	Phase III
Gene Expression Regulator	DT-216	Design Therapeutics	GeneTAC	Phase I
Gene Therapy	LX2006	Lexeo Therapeutics	Frataxin Gene Replacement	Phase I/II
Gene Therapy	TBD	Voyager/Neurocrine	Frataxin Gene Replacement	Phase 1 planned in 2025

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*Competitive landscape focuses on clinical-stage, industry-sponsored programs from public companies

Positive Topline 50 mg & 25 mg Ph 2 Data and Dosed First Patient in OLE

Consistent Ph 1 and Ph 2 Findings	Nomlabofusp is generally well tolerated at doses tested up to 4 weeks Dose-dependent increases in FXN levels from baseline in evaluated tissues (skin and buccal cells) Baseline FXN levels in skin cells in the 50 mg cohort were < 17% of the average of healthy volunteers. After daily dosing for 14 days, FXN levels increased to 33% to 59%
Regulatory Updates	Initiated discussions with FDA regarding use of FXN as a surrogate endpoint to support accelerated approval Intend to pursue accelerated approval with potential BLA submission for 2H 2025 Beginning preparations to expand nomlabofusp clinical program to ex-U.S. geographies
2024/2025 Milestones	**Q1 2024:** Dosed first patient in OLE study **Q4 2024:** Initial data from OLE study **2H 2024:** Final Phase 2 data planned to be presented at a conference **2H 2025:** BLA submission

Clinical-Stage Novel Protein Replacement Therapy Platform

Potential first therapy to increase frataxin levels	Lead candidate nomlabofusp (CTI-1601) is a recombinant fusion protein designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering the protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations
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Intend to pursue accelerated approval with FDA	FDA acknowledgement that FXN deficiency appears to be critical to the pathogenic mechanism of FA, and that there continues to be an unmet need for treatments that address the underlying disease pathophysiology. Discussions to support an accelerated approval are ongoing. BLA submission targeted for 2H 2025
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March 2024 Corporate Deck

THANK YOU

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Appendix

Scientific Advisory Board

Giovanni Manfredi, MD, PhD

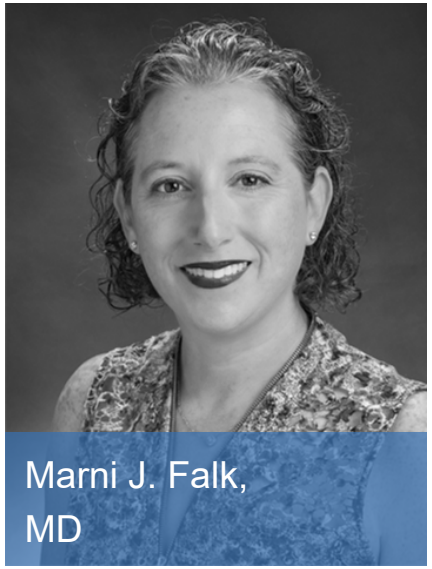
Finbar and Marianne Kenny Professor in Clinical and Research Neurology at Weill Cornell Medicine.

Professor of Neuroscience at Weill Cornell Medicine.

Mark Payne, MD

Co-founder of Chondrial Therapeutics, which became Larimar Therapeutics, Inc.

Professor of Pediatrics at Indiana University School of Medicine



Marni J. Falk, MD

Executive Director of the Mitochondrial Medicine Frontier Program at The Children's Hospital of Philadelphia (CHOP)

Professor in the Division of Human Genetics, Department of Pediatrics at University of Pennsylvania Perelman School of Medicine

Jill Ostrem, MD

Medical Director and Division Chief of the University of California San Francisco (UCSF) Movement Disorders and Neuromodulation Center.

Carlin and Ellen Wiegner Endowed Professor of Neurology

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Strong Relationship with FARA

Company has strong relationship with Friedreich's Ataxia Research Alliance (FARA)

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

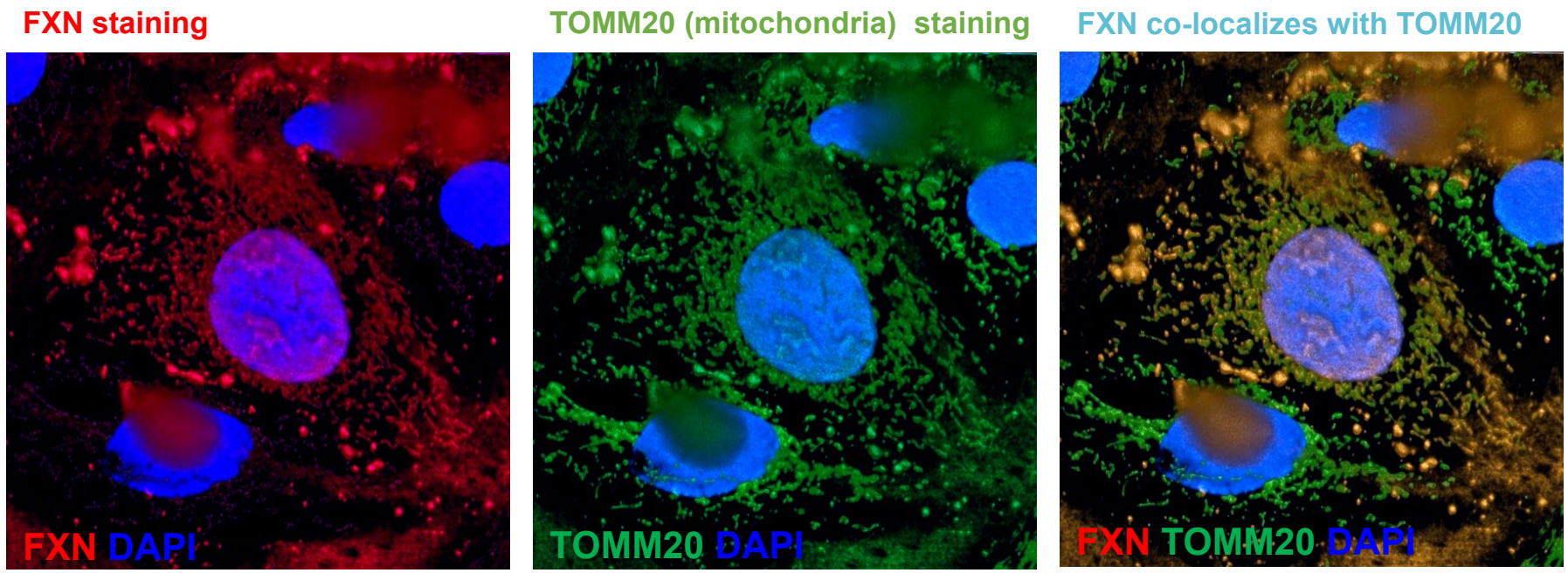
FARA provides industry with several key items

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

FARA Friedreich's Ataxia Research Alliance

Mitochondrial Localization and Preclinical Data

Nomlabofusp Transduction of Cells In Vitro Leads to hFXN Located in Mitochondria



- Rat cardiomyocytes (H9C2) were transduced with nomlabofusp
- Cells were fixed and analyzed by immunofluorescence microscopy to detect the presence of human frataxin (hFXN) and TOMM20 (a mitochondrial outer membrane protein)
- Nuclei were stained with DAPI

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Nomlabofusp Extends Survival in FXN-deficient KO Mice

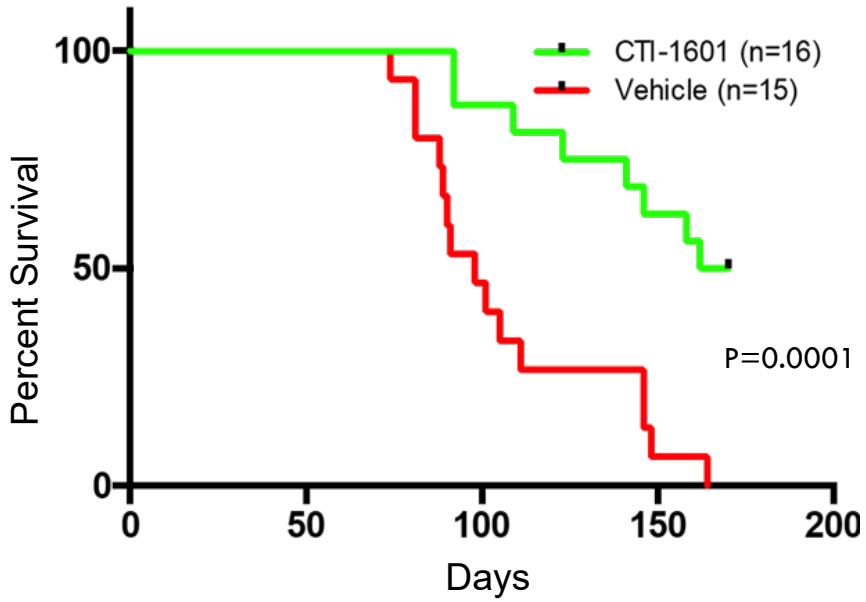
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

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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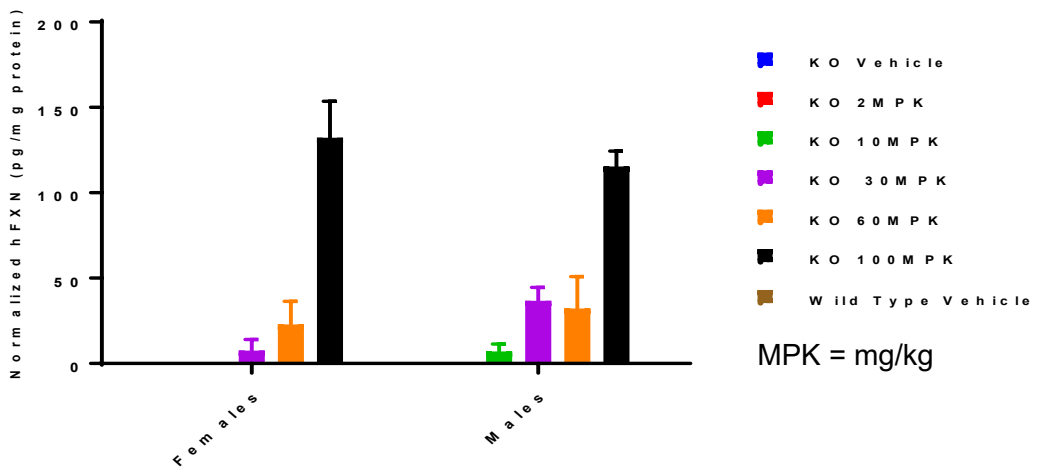
Nomlabofusp Delivers hFXN to Mitochondria and Restores SDH Activity in KO Mice

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)

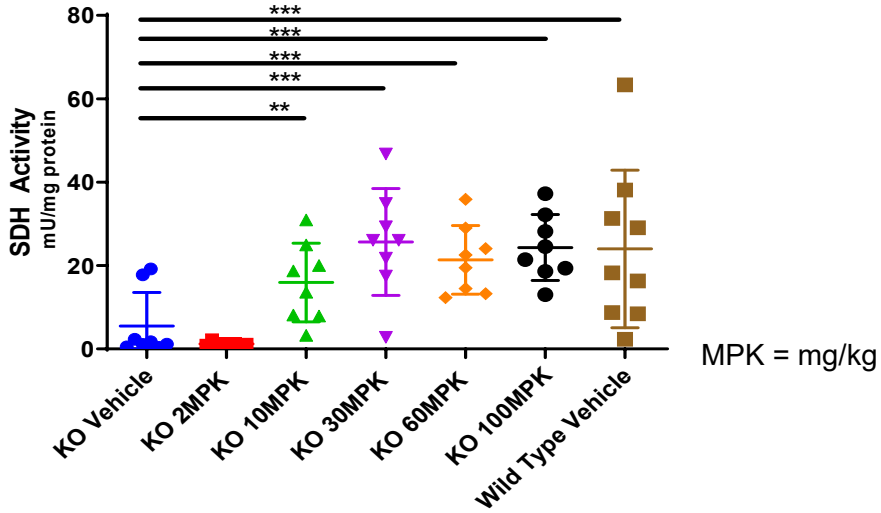


MPK = mg/kg

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

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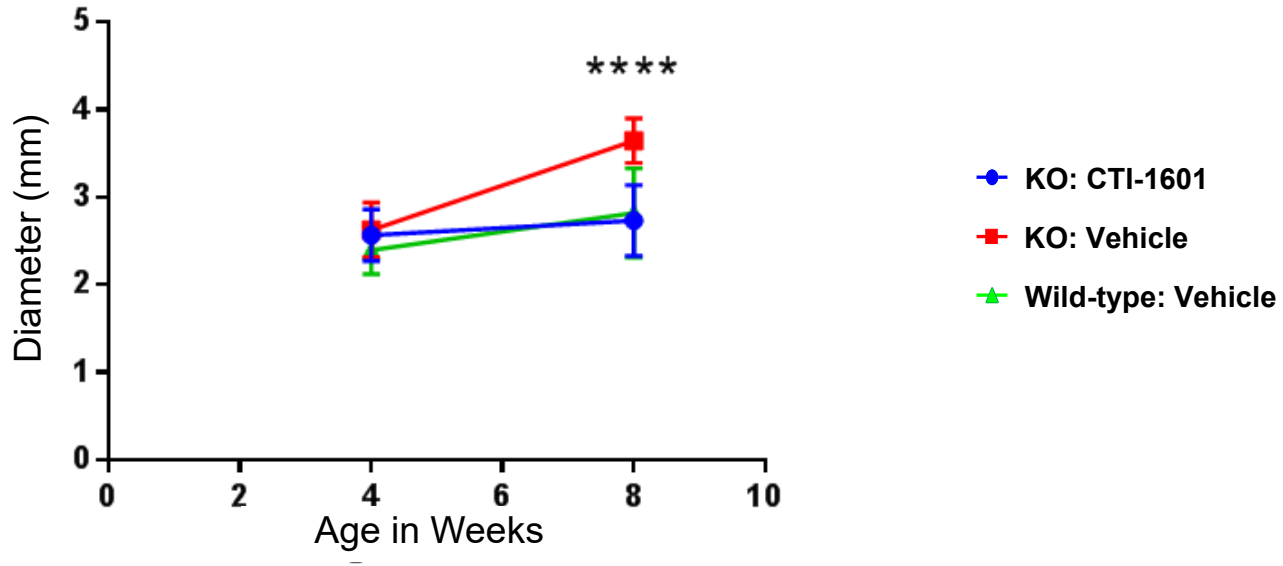
Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

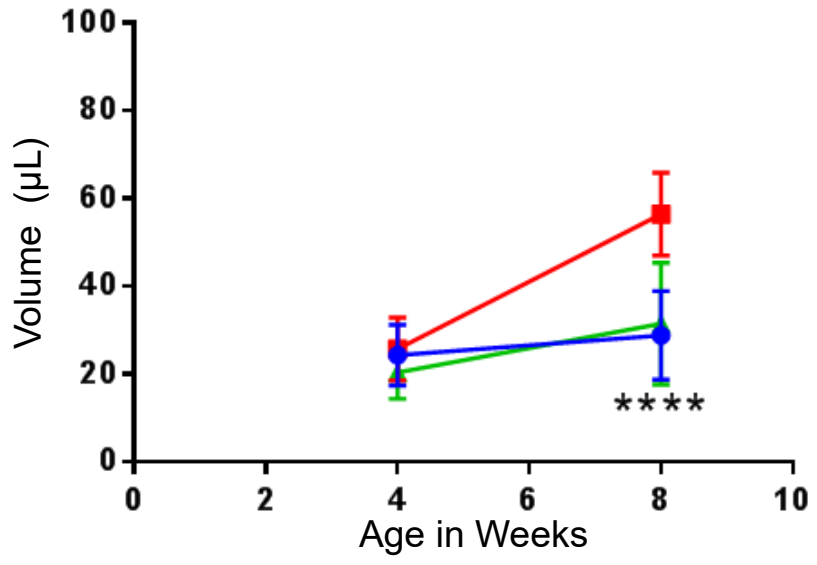
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

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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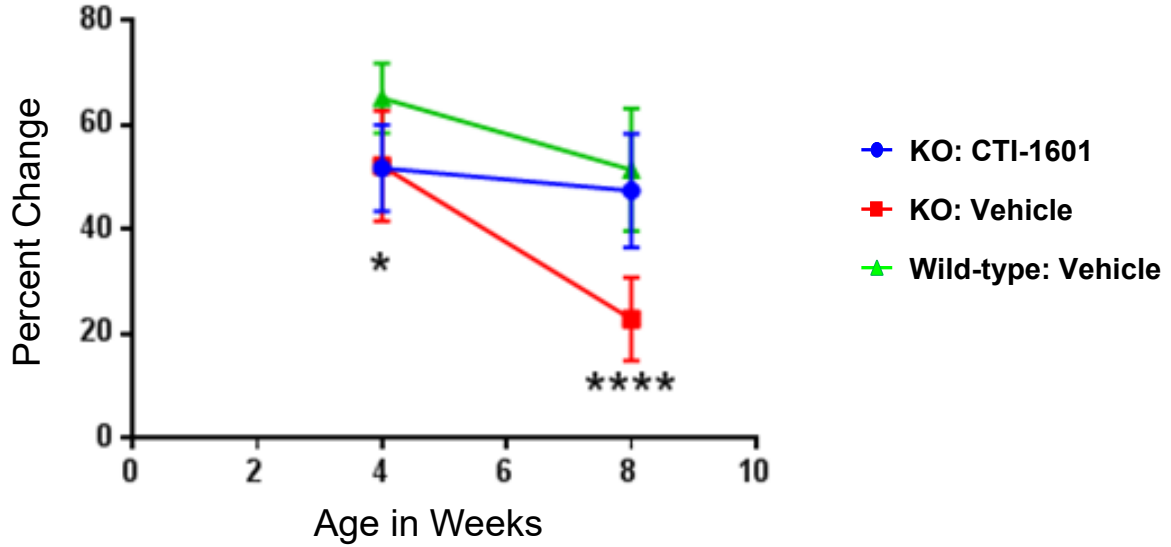
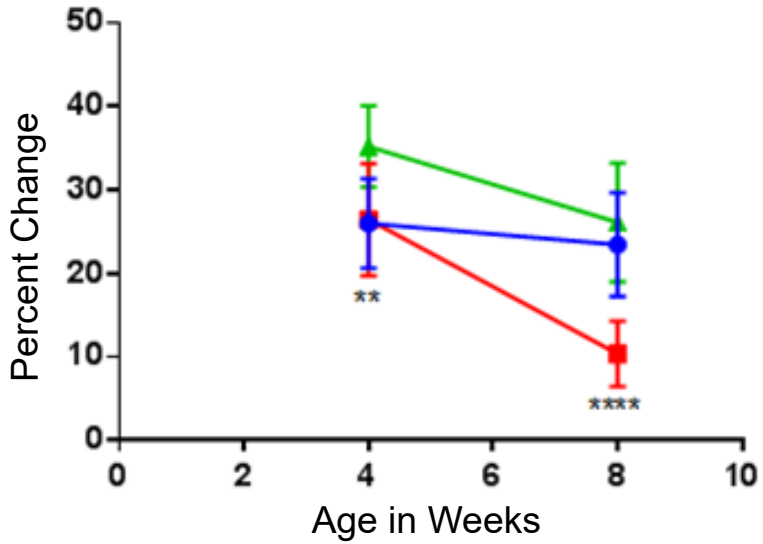
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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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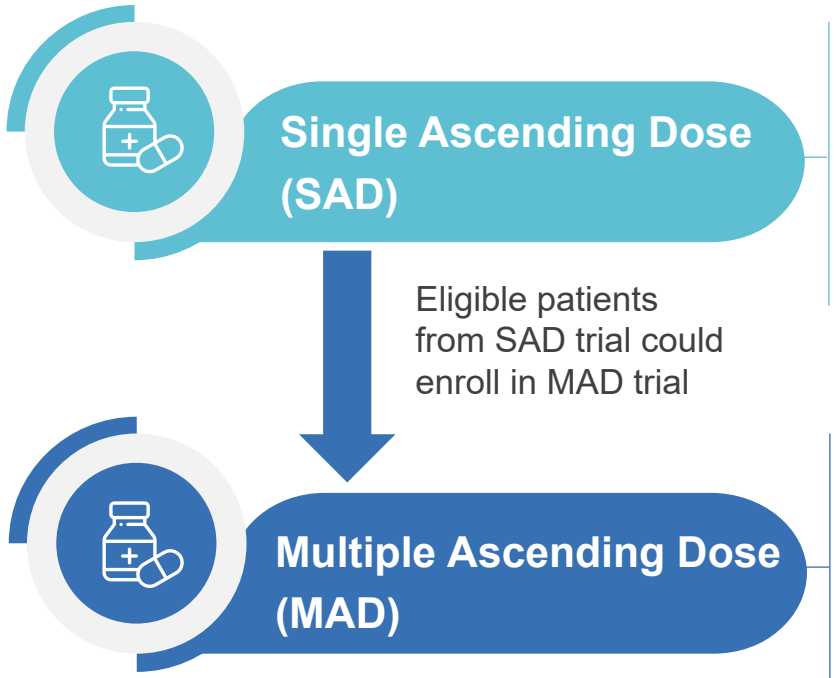
Phase 1 Clinical Data

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

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

Phase 1 Development Plan

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



Number of subjects: 28

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

Treatment Duration: 1 day

1º Endpoint: Safety and tolerability

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

Status: Complete

Number of Subjects: 27

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

Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days

1º Endpoint: Safety and tolerability

2º Endpoints: PK; PD; FXN levels (buccal cells, platelets, optional skin biopsies); multiple exploratory

Status: Complete

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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

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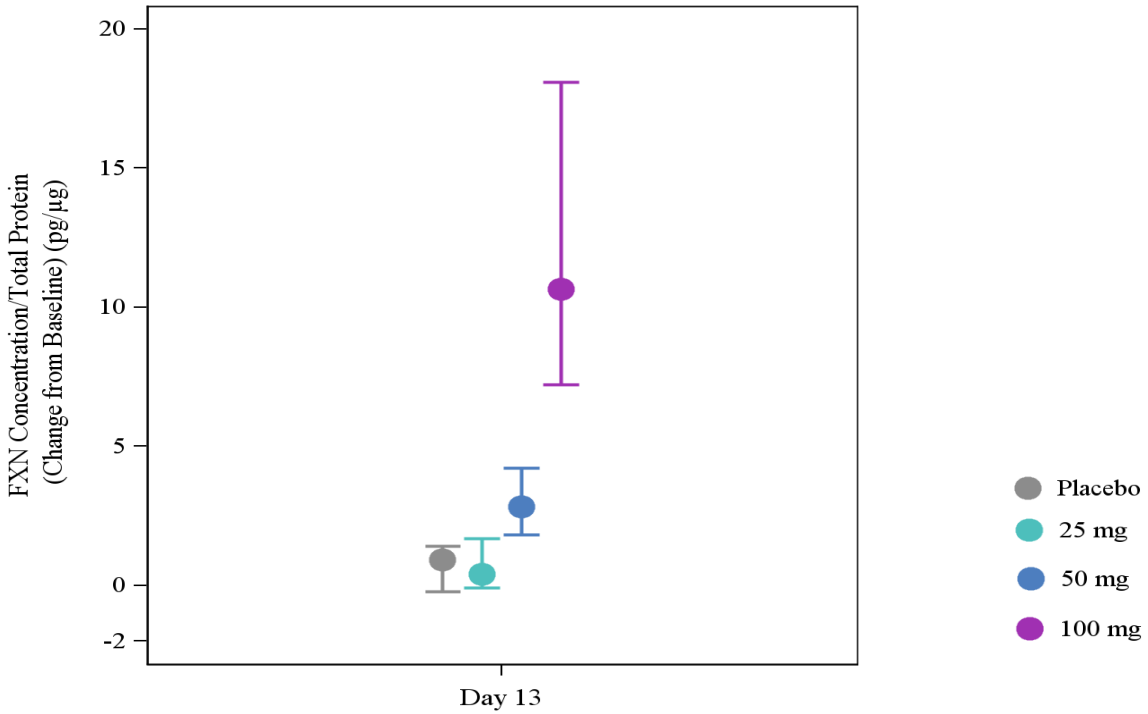
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

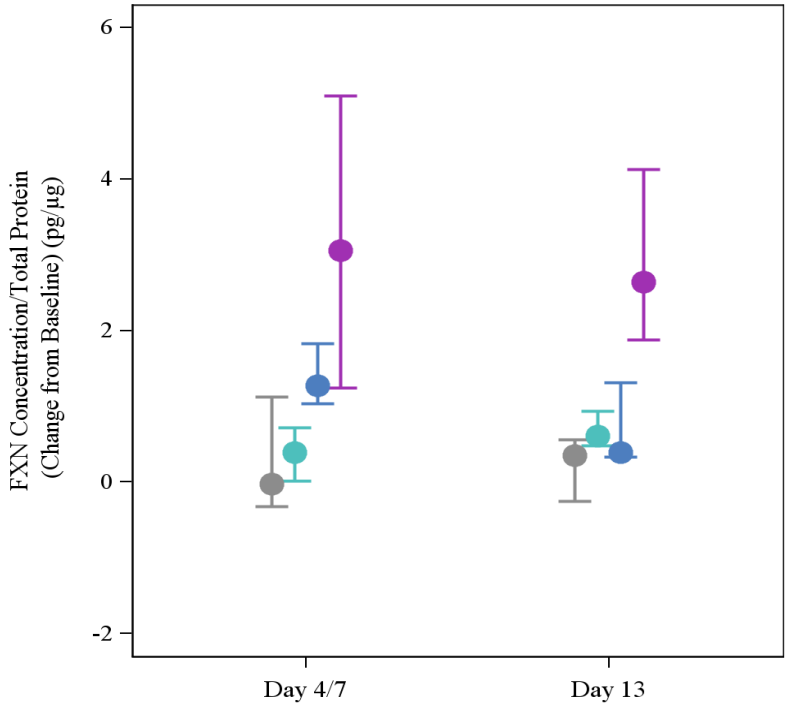
FXN* Change from Baseline By Dose Group (Skin Cells)



Placebo: Participants randomized to placebo in each cohort

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

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



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

100 mg: Dosed daily for 13 days

*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from Day 7 & Day 13 measurements are shown for data derived from the 50 & 100 mg cohorts;

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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 Demographic/ Disease Characteristics Data

Demographics – Phase 2 Trial

	25 mg Cohort			50 mg Cohort		
	Placebo *N = 4*	Nomlabofusp *N = 9*	Overall *N = 13*	Placebo *N = 5*	Nomlabofusp *N = 10*	Overall *N = 15*
Age at Screening (Years)						
Mean (SD)	34.0 (9.20)	37.8 (14.93)	36.6 (13.16)	28.6 (4.67)	28.1 (11.00)	28.3 (9.17)
Median	33	31	31	27	24	26
Q1, Q3	27, 42	27, 42	27, 42	26, 30	21, 32	21, 32
Min, Max	25, 45	25, 69	25, 69	24, 36	19, 54	19, 54
Sex n (%)						
Male	2 (50.0)	5 (55.6)	7 (53.8)	1 (20.0)	4 (40.0)	5 (33.3)
Female	2 (50.0)	4 (44.4)	6 (46.2)	4 (80.0)	6 (60.0)	10 (66.7)
Previously Treated with Nomlabofusp n (%)						
Yes	1 (25.0)	3 (33.3)	4 (30.8)	0	1 (10.0)	1 (6.7)
No	3 (75.0)	6 (66.7)	9 (69.2)	5 (100.0)	9 (90.0)	14 (93.3)

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Disease Characteristics – Phase 2 Study

	25 mg Cohort			50 mg Cohort		
	Placebo *N = 4*	Nomlabofusp *N = 9*	Overall *N = 13*	Placebo *N = 5*	Nomlabofusp *N = 10*	Overall *N = 15*
Age at Symptom Onset (Years)						
Mean (SD)	14.5 (4.93)	13.0 (10.47)	13.5 (8.77)	15.2 (7.26)	13.7 (8.37)	14.2 (7.78)
Median	14.5	10	11	14	12.5	14
Q1, Q3	11, 19	8, 13	9, 15	11, 16	7, 18	7, 18
Min, Max	9, 20	5, 38	5, 38	8, 27	5, 30	5, 30
Age at Diagnosis (Years)						
Mean (SD)	17.5 (5.57)	18.6 (11.20)	18.2 (9.58)	18.6 (6.80)	16.6 (8.03)	17.3 (7.46)
Median	16.5	16	16	19	13.5	14
Q1, Q3	14, 22	14, 20	14, 20	13, 20	10, 21	12, 21
Min, Max	12, 25	5, 42	5, 42	12, 29	9, 30	9, 30
Time Since Diagnosis (Years)						
Mean (SD)	16.1 (5.97)	18.5 (11.52)	17.8 (9.94)	9.5 (3.72)	11.9 (7.05)	11.1 (6.10)
Median	13.42	14.32	13.5	11	11.26	11
Q1, Q3	12.9, 19.3	12.8, 21.6	12.8, 21.6	5.8, 11.3	7.4, 15.3	5.8, 15.2
Min, Max	12.5, 25.0	5.4, 45.0	5.4, 45.0	5.6, 14.0	2.3, 25.1	2.3, 25.1

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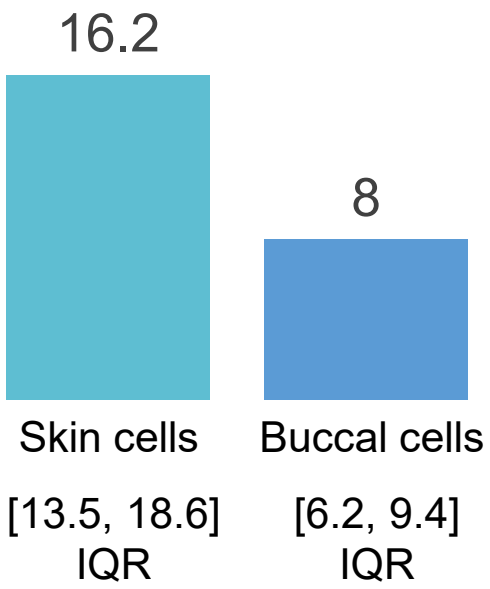
Non-Interventional Study Data

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