

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549**

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): March 24, 2025

Larimar Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-36510
(Commission File Number)

20-3857670
(IRS Employer
Identification No.)

Three Bala Plaza East
Bala Cynwyd, Pennsylvania
(Address of Principal Executive Offices)

19004
(Zip Code)

Registrant's Telephone Number, Including Area Code: (844) 511-9056

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	LRMR	Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02 Results of Operations and Financial Condition.

On March 24, 2025, Larimar Therapeutics, Inc. (the “Company”) announced its financial results and operational highlights for the fourth quarter of 2024 and for the year ended December 31, 2024. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information furnished pursuant to this Item 2.02, including Exhibit 99.1 attached hereto, is intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On March 24, 2025, the Company posted on its website an updated slide presentation, which is attached as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. Representatives of the Company will use the presentation in various meetings with investors, analysts and other parties from time to time.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

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

<u>Exhibit No.</u>	<u>Document</u>
99.1	Press Release issued by Larimar Therapeutics, Inc. on March 24, 2025*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated March 24, 2025
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Larimar Therapeutics, Inc.

Date: March 24, 2025

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

Larimar Therapeutics Provides Nomlabofusp Development Update and Reports Fourth Quarter and Full Year 2024 Financial Results

- *FDA stated in written correspondence for a START pilot program meeting that it is open to considering skin FXN concentration as a reasonably likely surrogate endpoint in support of an accelerated approval*
- *FDA recommended measuring skin FXN concentrations to support evidence of effectiveness for accelerated approval pathway and acknowledged submitted data appear sufficient to support relationship between increased skin FXN concentrations and relevant tissues such as heart, dorsal root ganglia and skeletal muscle*
- *Larimar is continuing discussions with FDA on the adequacy of the safety data set to support BLA submission*
- *BLA seeking accelerated approval targeted for submission by year-end 2025*
- *FDA and EMA feedback obtained on global Phase 3 study protocol; study planned to initiate in mid-2025*
- *Topline 50 mg dose data from the OLE study and available data from the adolescent cohort of the PK run-in study planned for program update in September 2025*
- *Strong balance sheet of \$183.5 million cash, cash equivalents and marketable securities as of December 31, 2024, with projected cash runway into second quarter of 2026*

Bala Cynwyd, PA, March 24, 2025 – Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its fourth quarter and full year 2024 operating and financial results.

“The strong clinical and regulatory progress across our nomlabofusp program reinforces the timing of our planned Biologics License Application (BLA) submission expected by the end of 2025 to seek accelerated approval. Importantly, we are enthusiastic about the recent FDA interactions and their openness to consider skin frataxin (FXN) concentrations as a potential novel surrogate endpoint reasonably likely to predict clinical benefit in patients with Friedreich’s ataxia (FA). Acceptability of FXN as a novel surrogate endpoint will be based on review of the data by the Food and Drug Administration (FDA) in the future BLA. As we previously disclosed, we have been collecting skin FXN concentration data throughout the development program,” said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. “Overall, FDA’s recommendations are in line with our current approach, and we appreciate the frequent dialogue via our participation in the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program as we further refine our registrational plan.”

Dr. Ben-Maimon continued, “Our open label extension (OLE) study continues to advance, with some participants on daily treatment for up to one year. We continue to actively enroll study participants, and all participants are currently receiving the 50 mg dose. The 25 mg initial data we presented in December showed increased and sustained FXN levels over time and early trends in improved clinical outcomes in patients with FA. Our pediatric pharmacokinetic (PK) run-in study continues to progress, and we expect to complete the dosing of the adolescent cohort by the end of this month and report available data in September 2025. We also plan to enroll children 2-11 years of age in the clinical program. We have received feedback from both FDA and European Medicines Agency (EMA) on the protocol for our global Phase 3 trial and are on track to initiate this trial mid-2025. We believe our near-term data package strongly positions us to bring the first potential disease modifying therapy to patients with FA.”

Recent Highlights

- **Potential for Accelerated Approval Pathway Based on FXN Concentrations as a Reasonably Likely Surrogate Endpoint (RSLE):** FDA stated in written correspondence associated with a meeting through the START pilot program that they are open to considering the use of FXN concentration as a RLSE and the acceptability of FXN's use as an RLSE would ultimately be a matter of review of the data in a future marketing application.
 - **Increases in Skin FXN as Evidence of Effectiveness:** FDA recommended focusing on assessments of skin FXN concentrations rather than buccal FXN concentrations due to more consistent sampling and less variability. FDA acknowledged that recently submitted data appear to support a relationship between increased FXN concentrations in skin cells and relevant tissues such as the heart, dorsal root ganglion and skeletal muscle. FDA also acknowledged that the nonclinical studies were performed at relevant human doses.
 - **Additional Analyses Under Consideration:** FDA also suggested that Larimar consider exploring the relationship between increases in FXN in skin and changes in pharmacodynamic markers such as lipid profiles and/or clinical measures to provide additional support for the use of FXN as a RLSE.
 - **Planned Upcoming Regulatory Discussions:** Larimar is continuing discussions with FDA regarding the adequacy of the safety data set required to support BLA submission.
 - **50 mg OLE Data Expected in September 2025:** The ongoing OLE study continues to enroll, and all study participants are currently receiving the 50 mg dose of nomlabofusp. Larimar plans to provide an update on OLE data on at least 30 to 40 study participants, some of whom have been receiving nomlabofusp for over one year, in September 2025.
 - **OLE Protocol Amended to Include Premedication:** Larimar's Safety Monitoring Team has deemed anaphylaxis as an adverse drug reaction likely associated with nomlabofusp and therefore, Larimar expects to see additional reactions. To reduce the risk of allergic reactions, including anaphylaxis, Larimar has amended the OLE protocol to administer premedication for the first month of dosing.
 - **Continuing Dosing of Adolescents in Pharmacokinetic (PK) Run-In Study:** In January 2025, Larimar initiated dosing of adolescents (12-17 years of age) in a PK run-in study for pediatric patients with FA. In this study, adolescents are receiving a weight-based dose expected to match the PK of adults receiving the 50 mg dose. Following assessment of safety and PK data, participants will be eligible to screen for the OLE study. Larimar anticipates the dosing of this cohort will be completed by the end of this month. The data from this cohort is expected to be presented during the nomlabofusp program update in September 2025.
 - **FDA Acceptance to Transition to Lyophilized Form of Nomlabofusp:** In February 2025, FDA accepted the data supporting the comparability of the lyophilized drug product to the frozen solution and agreed with Larimar's plans to introduce the lyophilized product into our clinical development program in mid-2025. The frozen solution is the dosage form currently being used in the OLE. The lyophilized drug product is the formulation that Larimar intends to commercialize.
 - **BLA Submission and Initiation of Global Phase 3 Study on Track:** Larimar has obtained feedback from both FDA and EMA on the global Phase 3 study protocol and is on track to initiate the study by mid-2025 with potential sites in the U.S., Europe, U.K., Canada, and Australia. Larimar is targeting the BLA submission to seek accelerated approval by the end of 2025.
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Fourth Quarter and Full Year 2024 Financial Results

As of December 31, 2024, the Company had cash, cash equivalents and marketable securities totaling \$183.5 million.

The Company reported a net loss for the fourth quarter of 2024 of \$28.8 million, or \$0.45 per share, compared to a net loss of \$13.0 million, or \$0.30 per share, for the fourth quarter of 2023.

Research and development expenses for the fourth quarter of 2024 were \$26.7 million compared to \$10.6 million for the fourth quarter of 2023. The increase in research and development expenses was primarily driven by an increase of \$15.0 million in nonlabofusp manufacturing costs including lyophilization development, production scaling costs and manufacturing costs related to producing doses to be used in ongoing and planned clinical trials, an increase of \$1.3 million in personnel expense due to increased headcount, an increase of \$1.0 million of professional fees related to consulting costs, and an increase of \$0.5 million in stock compensation costs associated with 2024 grants, partially offset by a decrease of \$1.7 million in clinical trial costs primarily associated with decreased activity in the dose exploration study in 2024.

General and administrative expenses for the fourth quarter of 2024 were \$4.6 million compared to \$3.5 million for the fourth quarter of 2023. The increase in general and administrative expenses was primarily driven by an increase of \$0.6 million in professional fees primarily related to consulting costs related to commercial activity and other public company related expenses, and an increase of \$0.4 million in personnel expense due to increased headcount.

Other income (expense), net was \$2.5 million of income in the three months ended December 31, 2024 compared to \$1.2 million of income in the three months ended December 31, 2023. The increase primarily relates to interest income on a higher investment base and higher investment yields on that base during the current period.

For the full year 2024, the Company reported a net loss of \$80.6 million, or \$1.32 per share, compared to a net loss of \$36.9 million, or \$0.84 per share, for the same period in 2023.

Research and development expenses for the full year 2024 were \$73.3 million compared to \$27.7 million for the same period in 2023. The increase in research and development expenses was primarily driven by an increase of \$36.1 million in nonlabofusp manufacturing costs including lyophilization development, production scaling costs and manufacturing costs related to producing doses to be used in ongoing and planned clinical trials, an increase of \$4.5 million in personnel expense due to increased headcount, an increase of \$1.3 million of professional fees related to consulting costs, an increase of \$1.2 million in clinical costs primarily associated with the ongoing OLE study which began dosing participants in the first quarter of 2024, an increase of \$1.0 million in stock compensation costs associated with 2024 equity compensation grants, an increase of \$0.9 million related to the Friedreich's Ataxia Research Alliance (FARA) Track-FA program, an increase of \$0.3 million in internal lab costs and an increase of \$0.2 million in facility costs associated with the new lab space.

General and administrative expenses for the full year 2024 were \$17.6 million compared to \$14.1 million for 2023. The increase in general and administrative expenses was primarily driven by an increase of \$1.5 million in professional fees primarily related to consulting costs related to commercial activity and other public company related expenses, an increase of \$1.4 million in personnel expense due to increased headcount, an increase of \$0.4 million of other expense related to computer software, information technology services and recruiting and an increase of \$0.2 million in stock compensation costs associated with 2024 equity compensation grants.

Other income (expense), net was \$10.3 million of income in the twelve months ended December 31, 2024 compared to \$4.8 million of income in the twelve months ended December 31, 2023. The increase primarily relates to increased interest income on a higher investment base and higher investment yields on that base during 2024.

About Larimar Therapeutics

Larimar Therapeutics, Inc. (Nasdaq: LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. Larimar's lead compound, nomlabofusp, is being developed as a potential treatment for Friedreich's ataxia. Larimar also plans to use its intracellular delivery platform to design other fusion proteins to target additional rare diseases characterized by deficiencies in intracellular bioactive compounds. For more information, please visit: <https://larimartx.com>.

Forward-Looking Statements

This press release contains forward-looking statements that are based on Larimar's management's beliefs and assumptions and on information currently available to management. All statements contained in this release other than statements of historical fact are forward-looking statements, including but not limited to statements regarding Larimar's ability to develop and commercialize nomlabofusp and 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 potential for accelerated approval or accelerated access and time to market and overall development plans and other matters regarding Larimar's business strategies, ability to raise capital, use of capital, results of operations and financial position, and plans and objectives for future operations.

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

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Larimar Therapeutics, Inc.
Consolidated Balance Sheets

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	\$ 33,218	\$ 26,749
Marketable securities	150,236	60,041
Prepaid expenses and other current assets	11,850	3,385
Total current assets	195,304	90,175
Property and equipment, net	881	684
Operating lease right-of-use assets	2,838	3,078
Restricted cash	606	1,339
Other assets	596	659
Total assets	\$ 200,225	\$ 95,935
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 2,424	\$ 1,283
Accrued expenses	20,872	7,386
Operating lease liabilities, current	1,060	837
Total current liabilities	24,356	9,506
Operating lease liabilities	4,057	4,709
Total liabilities	28,413	14,215
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; \$0.001 par value per share; 5,000,000 shares authorized as of December 31, 2024 and December 31, 2023; no shares issued and outstanding as of December 31, 2024 and December 31, 2023	—	—
Common stock, \$0.001 par value per share; 115,000,000 shares authorized as of December 31, 2024 and December 31, 2023; 63,815,065 and 43,909,069 shares issued and outstanding as of December 31, 2024 and December 31, 2023, respectively	64	43
Additional paid-in capital	440,758	270,150
Accumulated deficit	(269,158)	(188,554)
Accumulated other comprehensive gain (loss)	148	81
Total stockholders' equity	171,812	81,720
Total liabilities and stockholders' equity	\$ 200,225	\$ 95,935

Larimar Therapeutics, Inc.
Consolidated Statements of Operations
(In thousands, except share and per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	\$ 26,738	\$ 10,648	\$ 73,278	\$ 27,670
General and administrative	4,555	3,514	17,612	14,088
Total operating expenses	31,293	14,162	90,890	41,758
Loss from operations	(31,293)	(14,162)	(90,890)	(41,758)
Other income, net	2,469	1,169	10,286	4,809
Net loss	\$ (28,824)	\$ (12,993)	\$ (80,604)	\$ (36,949)
Net loss per share, basic and diluted	\$ (0.45)	\$ (0.30)	\$ (1.32)	\$ (0.84)
Weighted average common shares outstanding, basic and diluted	63,810,823	43,906,281	61,256,084	43,901,241



Larimar Therapeutics
Corporate Deck

March 2025

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 any other planned product candidates, Larimar’s planned research and development efforts, including the timing of its nomlabofusp clinical trials and non-clinical investigations and overall development plan expectations with respect to the FDA START pilot program, interactions with FDA, expectations regarding potential for accelerated approval or accelerated access and time to market 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 non-clinical or clinical trials, and assessments; delays in patient recruitment, including as a result of changes in clinical protocols and adverse events; that the FDA may not ultimately agree with Larimar’s nomlabofusp development strategy; the potential impact of public health crises on Larimar’s future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and general economic conditions; Larimar’s ability and the ability of third-party manufacturers Larimar engages, to optimize and scale nomlabofusp’s manufacturing process; Larimar’s ability to obtain regulatory approvals for nomlabofusp and future product candidates; Larimar’s ability to develop sales and marketing capabilities, whether alone or with potential future collaborators, and to successfully commercialize any approved product candidates; Larimar’s ability to raise the necessary capital to conduct its product development activities; and other risks described in the filings made by Larimar with the Securities and Exchange Commission (SEC), including but not limited to Larimar’s periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. These forward-looking statements are based on a combination of facts and factors currently known by Larimar and its projections of the future, about which it cannot be certain. As a result, the forward-looking statements may not prove to be accurate. The forward-looking statements in this presentation represent Larimar’s management’s views only as of the date hereof. Larimar undertakes no obligation to update any forward-looking statements for any reason, except as required by law.

Clinical-Stage Novel Protein Replacement Therapy Platform

First potential disease modifying therapy for FA

Nomlabofusp is designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering a recombinant fusion protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), PRIME (EU) and ILAP (UK-MHRA) designations. Selected by FDA to participate in its START pilot program

Seeking FDA accelerated approval pathway

FDA stated in written correspondence for a START pilot program meeting that it is open to considering the use of skin FXN concentration as a reasonably likely surrogate endpoint (RLSE). The acceptability of using skin FXN concentrations as an RLSE would ultimately be a matter of review of the data in a future marketing application

Positive initial data from long-term OLE study

Daily nomlabofusp 25 mg was generally well-tolerated and increased and maintained tissue FXN concentrations over time with early trends in improvements across multiple clinical outcomes with some participants having received daily dosing for up to 1 year. Continuing to enroll with all patients currently receiving 50 mg dose

Adolescent PK run-in Study

Adolescent cohort of PK run-in study began in early 2025 and upon data analysis participants will be eligible to screen for entry into the OLE study

Near-term catalysts

Topline 50 mg data from OLE study planned for Sept 2025
Dosing in adolescent cohort in PK run-in study to be completed by end of March 2025; data planned for Sept 2025
On track to initiate global Phase 3 study in mid-2025
Biologics License Application (BLA) submission to seek accelerated approval targeted by year-end 2025



\$183.5 million in cash and investments as of December 31, 2024, with projected cash runway into Q2 2026

Potential for Accelerated Approval Pathway Based on Skin FXN Concentrations as a Surrogate Endpoint

FDA is open to considering the use of FXN concentration as a **reasonably likely surrogate endpoint** (RLSE)

Skin FXN concentrations recommended by FDA as a RLSE to **support evidence of effectiveness** for accelerated approval

FDA noted submitted data appear sufficient to support relationship between increased skin FXN concentrations and relevant tissues such as heart, dorsal root ganglia and skeletal muscle

FDA acknowledged nonclinical studies were **done at relevant doses**

BLA submission to seek accelerated approval targeted by year-end 2025

Potential Path to Bring Nomlabofusp to Patients Worldwide

OLE Study

Completed dosing of 25 mg dose with positive initial data

Continuing to enroll with all subjects on 50 mg dose

Plan to introduce the lyophilized dosage form (formulation intended for commercialization) into clinical studies in mid-2025

Long-term 50 mg data expected Sept 2025

Adolescent PK Run-In Study

Dosing of adolescents (12-17 yrs of age) at weight-based dose expected to match PK of adult 50 mg dose, expected to be completed by end of March

Participants eligible to transition into OLE after assessment of safety and PK data

Data expected Sept 2025

Global Phase 3 Study

Received feedback from FDA and EMA on study protocol for global double-blind placebo-controlled study

Potential sites in the U.S., E.U., U.K., Canada, and Australia

Plan to include children (2-17 yrs of age)

Plan to initiate mid-2025

Next Steps

Continuing discussions with FDA to determine adequacy of the safety data set to support the BLA submission

BLA submission to seek accelerated approval targeted by year-end 2025

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 Europe
~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**

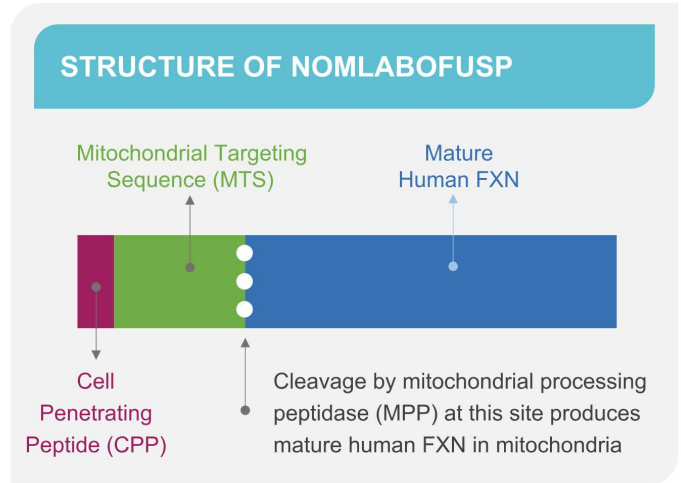
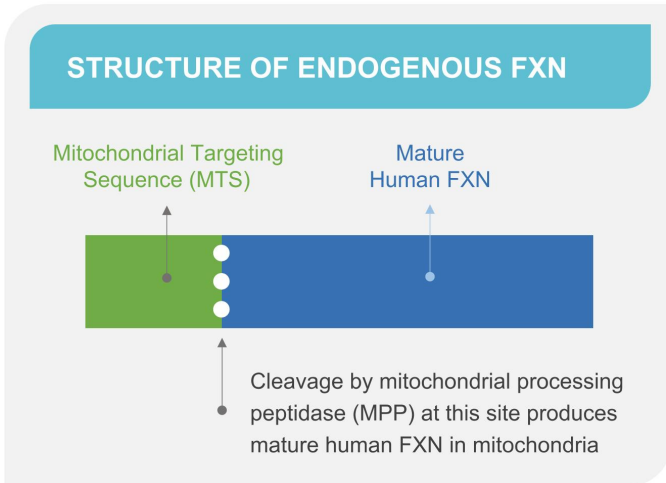
Only treatment approved for FA does not address frataxin deficiency



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

Nomlabofusp is Designed to Deliver Additional Frataxin

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



The presence of the cleavage site allows the CPP and MTS to be removed by mitochondrial processing peptidase to produce mature human FXN in the mitochondria

FXN Levels Clearly Predict Disease Progression in FA

Lower FXN levels are associated with earlier onset of disease, faster rate of disease progression, and shorter time to loss of ambulation

Median Age of Onset and Rate of Disease Progression in Relation to FXN Levels

FXN Level* (% of Normal Level)	Age of Onset (Years)	FARS** (Change/Year)
11.2	7	2.9
22.0	11	2.1
31.0	16	2.0
48.7	19	1.6

Adapted from H.L.Plasterer et al. PLoS ONE 2013 8(5):e63958

Median Age of Onset Predicts Time to Loss of Ambulation

Age of Onset (Years)	Median Time to Loss of Ambulation (Years)
< 15	11.5
15 to 24	18.3
> 24	23.5

Adapted from C. Rummey et al. EClinicalMedicine. 2020 18:100213



*FXN levels measured in peripheral blood mononuclear cells (PBMCs). FXN levels as measured by % of normal demonstrated to be equivalent in PBMCs, buccal cells, and whole blood.

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

Completed Ph 2 Dose Exploration Study (25 & 50 mg Cohorts)

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

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



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

 = No Administration

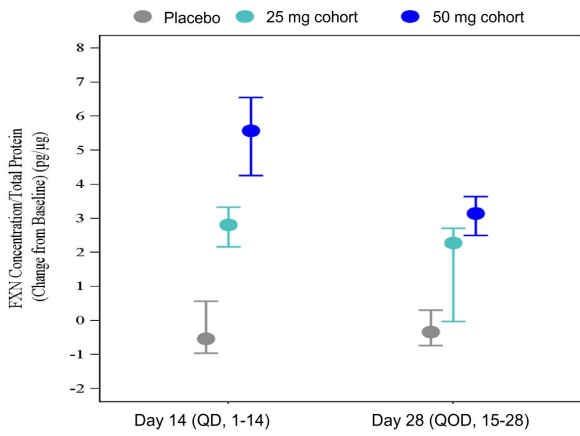
Study Details

Population	Ambulatory and non-ambulatory Friedreich's ataxia patients ≥18 years of age Nomlabofusp (CTI-1601) treatment naïve or participated (if eligible) in a previous Larimar study
Dose	Cohort 1: 25 mg Cohort 2: 50 mg
Key Endpoints	Frataxin levels in peripheral tissue, PK, safety and tolerability; other exploratory endpoints include lipids and gene expression levels
Number of Patients	Cohort 1: Enrolled 13 participants (9 on nomlabofusp; 4 on placebo) Cohort 2: Enrolled 15 participants (10 on nomlabofusp; 5 on placebo)
Key Results	Generally well tolerated; most common adverse events were mild and moderate injection site reactions Dose dependent increases of frataxin levels in tissues tested (skin and buccal cells) Baseline FXN levels in skin cells in the 50 mg cohort were < 17% of the average of healthy volunteers. After daily dosing for 14 days, FXN levels increased to 33% to 59% of the average of the healthy volunteers

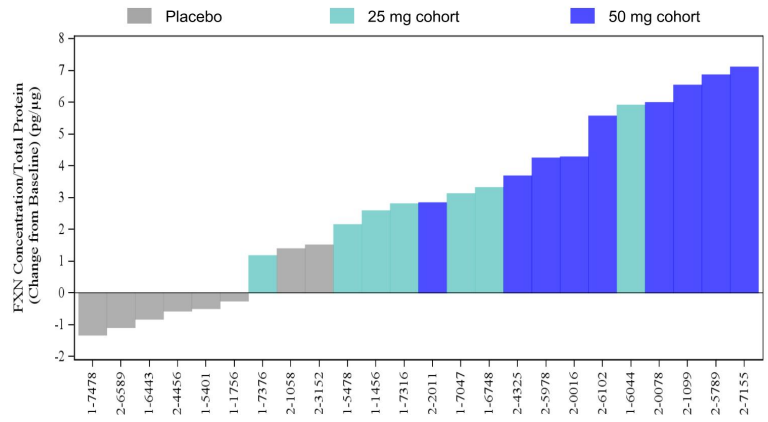
Dose-Dependent Increase in FXN Levels in Skin Cells

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

FXN Levels* in Skin Cells Change from Baseline**



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

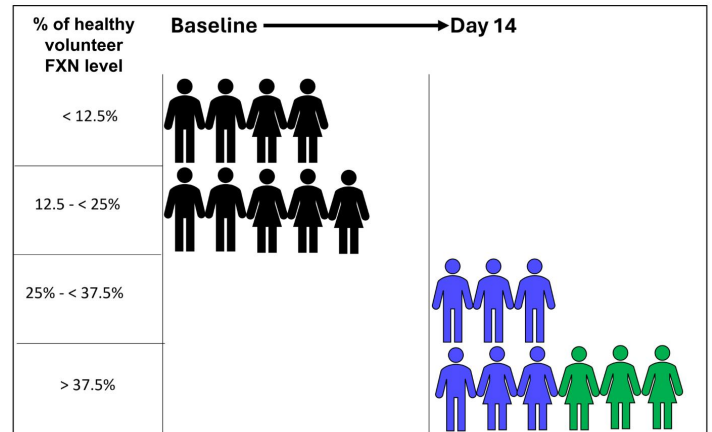
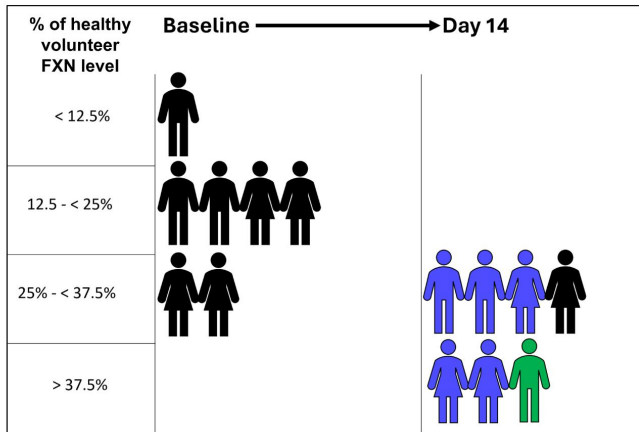


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.
**Median baseline FXN levels in patients were 3.5 pg/μg for the placebo, 3.7 pg/μg for the 25 mg cohort and 2.1 pg/μg for the 50 mg cohort.

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

25 mg of Nomlabofusp

50 mg of Nomlabofusp



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

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

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

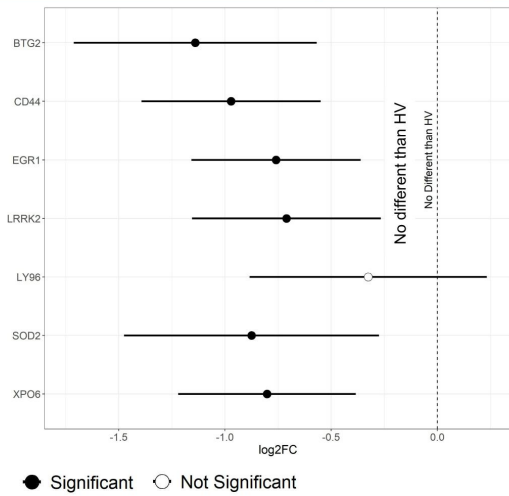


Only participants with quantifiable levels at baseline and day 14 are included in the figures.

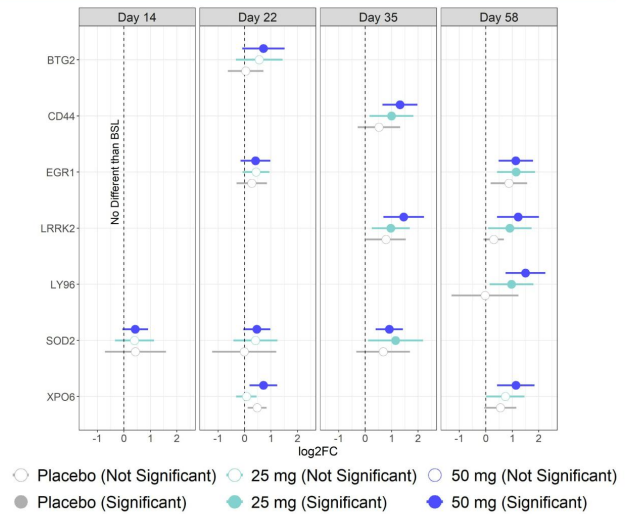
*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/μg) from the noninterventional healthy volunteer study (N=60).

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



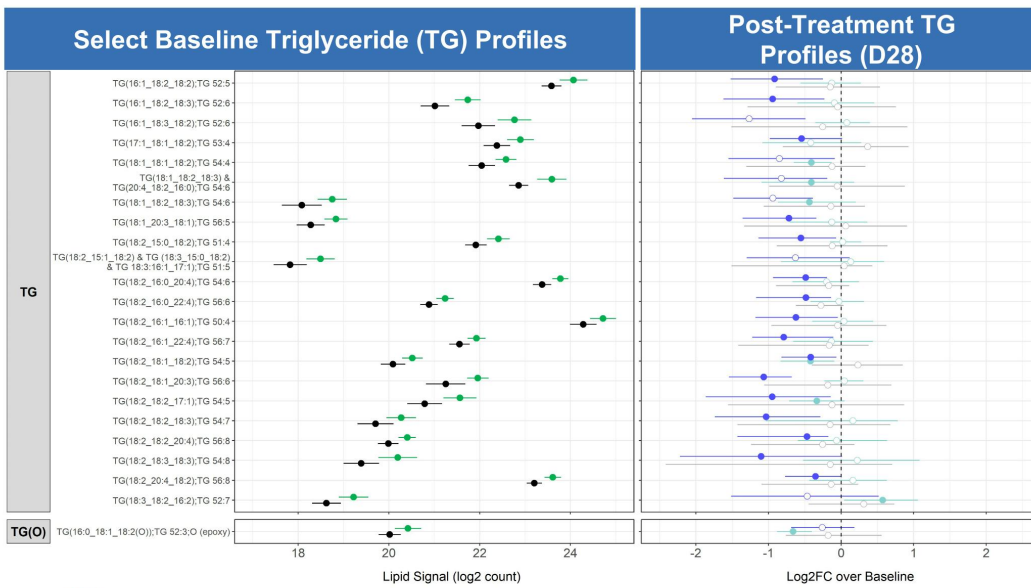
Data presented at the International Congress for Ataxia Research, November 2024

*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

Interest from FDA in Exploring the Correlation Between Lipids and FXN Concentrations

Decreases in elevated lipids in adults with FA* observed after nomlabofusp treatment



*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. Plasma samples were collected before, during, and after treatment for lipid profiling. Healthy volunteer (HV) data is from Larimar's non-interventional HV study

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



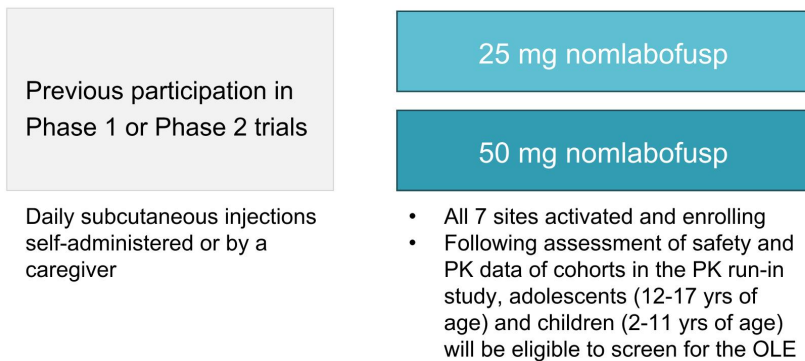


Nomlabofusp Long-term Open Label Extension Study

Open-label Extension: 25 mg Completed, Dosing at 50 mg Continues

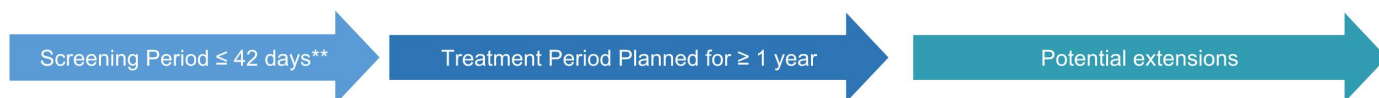
Long-term data from 50 mg dose expected September 2025

Key Eligibility Criteria



Key Study Objectives

- Safety and tolerability
- Long-term PK
- Tissue FXN concentrations
- Clinical efficacy measures compared to FACOMS* database once enrollment is complete



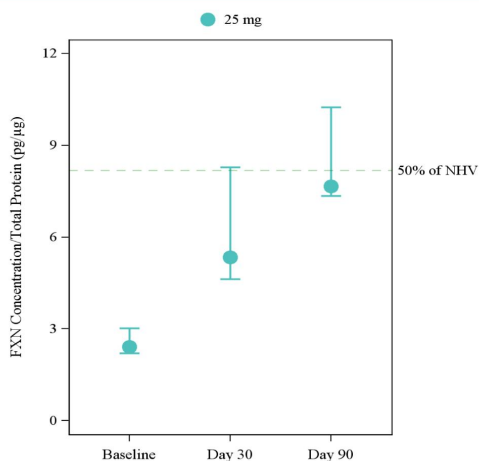
*FACOMS: Friedreich's Ataxia Clinical Outcome Measures Study.

**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.

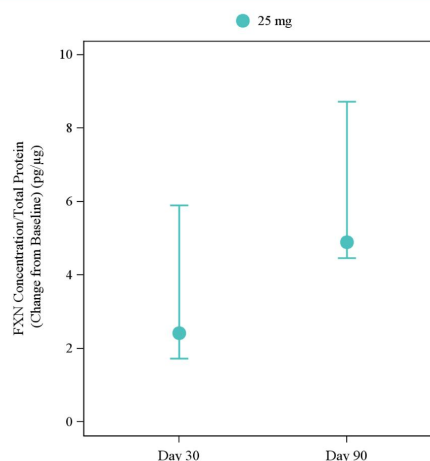
Increased FXN Levels in Skin Cells Sustained Over Time

Participants in the OLE dosed daily with 25 mg nomlabofusp for up to 90 days

Skin Cells - Absolute FXN Levels



Skin Cells - FXN Levels Change from Baseline

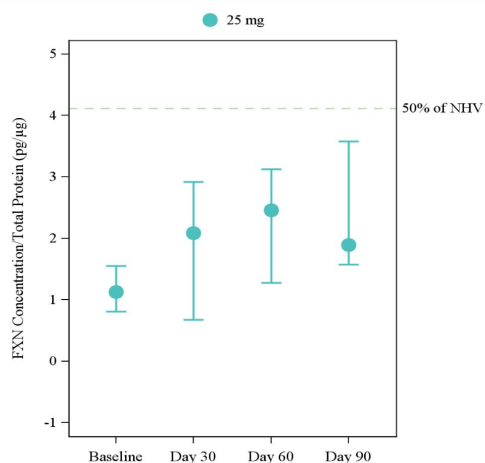


FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at all measurement points are included in the figures. 50% of normal healthy volunteer (NHV) FXN level is 8.17pg/µg from the noninterventional healthy volunteer study (N=60).

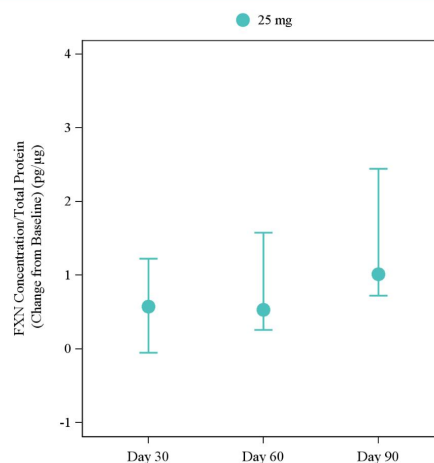
Increased FXN Levels in Buccal Cells Sustained Over Time

OLE participants dosed daily with 25 mg nomlabofusp for up to 90 days reached steady state by 30 days

Buccal Cells - Absolute FXN Levels

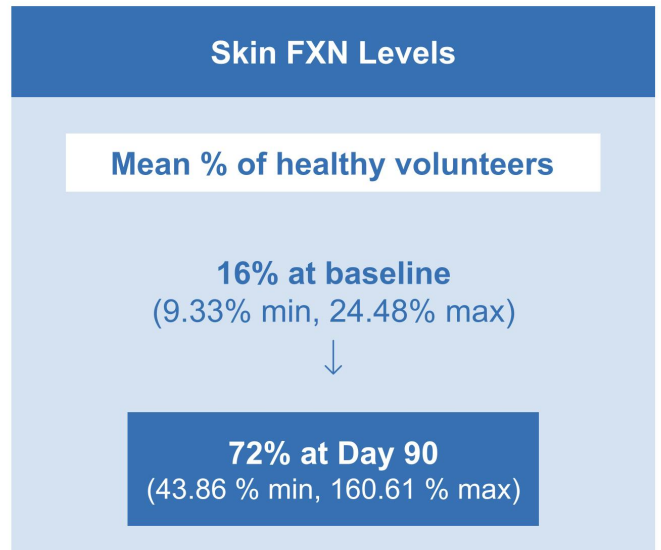
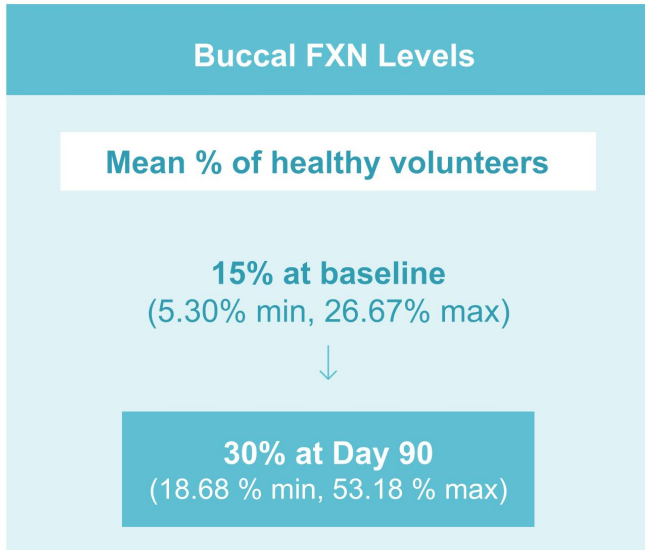


Buccal Cells - FXN Levels Change from Baseline



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 all measurement points are included in the figures. 50% of normal healthy volunteer (NHV) FXN level is 4.12 pg/µg from the noninterventional healthy volunteer study (N=60).

Tissue FXN Levels as a % of Healthy Volunteers are Higher at Day 90 Compared to Baseline in Subjects Treated with 25 mg Daily in the OLE



Only participants with quantifiable levels at each measurement point are included in the tables
Mean % of healthy volunteers is the mean of all the participants FXN levels relative to the mean FXN levels in skin cells (16.34 pg/ μ g) and in buccal cells (8.24 pg/ μ g) from the noninterventional healthy volunteer study (N=60).

Observed Increases in Tissue FXN Levels in OLE Are Comparable to the Phase 2 Dose Exploration Study

Absolute tissue FXN levels and increases from baseline after 25 mg nomlabofusp daily over time

	Open Label Extension					
	Buccal FXN levels (pg/μg)			Skin FXN levels (pg/μg)		
	n	Median	Mean	n	Median	Mean
Baseline	11	1.13	1.19	8	2.41	2.60
Day 30	11	2.08	3.62	8	5.34	7.45
Change from Baseline	11	0.58	2.43	8	2.42	4.85
Day 60	9	2.46	2.41			
Change from Baseline	9	0.53	1.13			
Day 90	6	1.89	2.48	5	7.65	11.73
Change from Baseline	6	1.01	1.32	5	4.89	9.28

	Phase 2 Dose Exploration					
	Buccal FXN levels (pg/μg)			Skin FXN levels (pg/μg)		
	n	Median	Mean	n	Median	Mean
Baseline	7	1.78	1.80	7	3.70	3.38
Day 14	7	2.24	2.22	7	5.53	6.4
Change from Baseline	7	0.56	0.42	7	2.81	3.02



Skin samples not collected at Day 60 per study protocol
Only participants with quantifiable levels at each measurement point are included in the tables

Observed Trends Towards Improvement in Clinical Outcomes at Day 90 in OLE After Daily 25 mg Nomlabofusp

Visit	Statistic	mFARS 93-Point Scale	FARS-ADL 36-Point Scale	Modified Fatigue Impact Scale 84-Point Scale	9 Hole Peg Test Dominant Hand Time (Seconds)
		N = 8	N = 8	N = 8	N = 8
Baseline	Mean (SD)	55.81 (13.296)	18.13 (6.064)	27.1 (14.23)	130.91 (99.366)
	Median (IQR)	53.5 (47.5, 68.3)	17.0 (12.8, 23.8)	29.5 (18, 38)	89.5 (48.7, 227.8)
	(Min, Max)	(35.0, 73.0)	(11.0, 27.0)	(2, 45)	(38.0, 277.3)
Day 90	Mean (SD)	55.13 (14.829)	15.88 (6.249)	18.5 (15.68)	113.11 (95.586)
	Median (IQR)	53.3 (43.8, 66.0)	14.8 (11.0, 21.3)	17.0 (5, 32)	67.15 (48.4, 176.7)
	(Min, Max)	(35.3, 79.5)	(8.0, 25.0)	(0, 42)	(33.50, 287.00)
Change from Baseline at Day 90	Mean (SD)	-0.69 (3.983)	-2.25 (3.082)	-8.6 (12.24)	-17.79 (27.450)
	Median (IQR)	-1.17 (-3.8, 1.2)	-2.25 (-3.8, 0.3)	-3.5 (-19, -3)	-9.00 (-32.0, 1.7)
	(Min, Max)	(-5.0, 7.0)	(-8.0, 1.5)	(-28, 9)	(-73.5, 9.8)



Timed 25-Foot Walk is not presented due to participants' ambulatory status

Nomlabofusp has Been Generally Well-Tolerated in OLE

Safety Data from OLE Update in Dec 2024

14 participants with FA were included in the safety data

Two participants withdrew for non-treatment related reasons

Two participants had serious adverse events that resolved within 24 hours and withdrew from the study

- Events were reviewed by Data Monitoring Committee and submitted to FDA and study is continuing as planned

Most common adverse event (AE) was injection site reactions (ISRs) which were mild, brief in duration and self limited

- No study discontinuations due to ISRs and all resolved

OLE Q1 2025 Updates

All patients in the OLE study are receiving 50 mg daily

Larimar's Safety Monitoring Team determined anaphylaxis to be an adverse drug reaction likely associated with nomlabofusp

To decrease the potential for allergic reactions, patients will be premedicated for the first month of dosing

Near-Term Milestones Support BLA Submission by End 2025

Larimar Selected to Participate in FDA START Pilot Program

Open-Label Extension Study



Introduction of lyophilized dosage form (formulation intended for commercialization) planned mid-2025

Topline 50 mg data from ongoing study expected September 2025

Adolescent PK Run-In Cohort



Dosing of study participants to be completed by end of March

Study participants are eligible to screen for the OLE after assessment of safety and PK data

Data expected September 2025

Global Phase 3 Study



Feedback on protocol received from FDA and EMA

On track to initiate mid- 2025

BLA Submission



Continuing discussions with FDA on adequacy of safety data set

BLA seeking accelerated approval expected by year-end 2025

Clinical-Stage Novel Protein Replacement Therapy Platform

First potential disease modifying therapy for FA

Nomlabofusp is designed to directly address frataxin deficiency in patients with Friedreich's ataxia (FA) by delivering a recombinant fusion protein to mitochondria. Granted Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), PRIME (EU) and ILAP (UK-MHRA) designations. Selected by FDA to participate in its START pilot program

Seeking FDA accelerated approval pathway

FDA stated in written correspondence for a START pilot program meeting that it is open to considering the use of skin FXN concentration as a reasonably likely surrogate endpoint (RLSE). The acceptability of using skin FXN concentrations as an RLSE would ultimately be a matter of review of the data in a future marketing application

Positive initial data from long-term OLE study

Daily nomlabofusp 25 mg was generally well-tolerated and increased and maintained tissue FXN concentrations over time with early trends in improvements across multiple clinical outcomes with some participants having received daily dosing for up to 1 year. Continuing to enroll with all patients currently receiving 50 mg dose

Adolescent PK run-in Study

Adolescent cohort of PK run-in study began in early 2025 and upon data analysis participants will be eligible to screen for entry into the OLE study

Near-term catalysts

Topline 50 mg data from OLE study planned for Sept 2025
Dosing in adolescent cohort in PK run-in study to be completed by end of March 2025; data planned for Sept 2025
On track to initiate global Phase 3 study in mid-2025
Biologics License Application (BLA) submission to seek accelerated approval targeted by year-end 2025



\$183.5 million in cash and investments as of December 31, 2024, with projected cash runway into Q2 2026

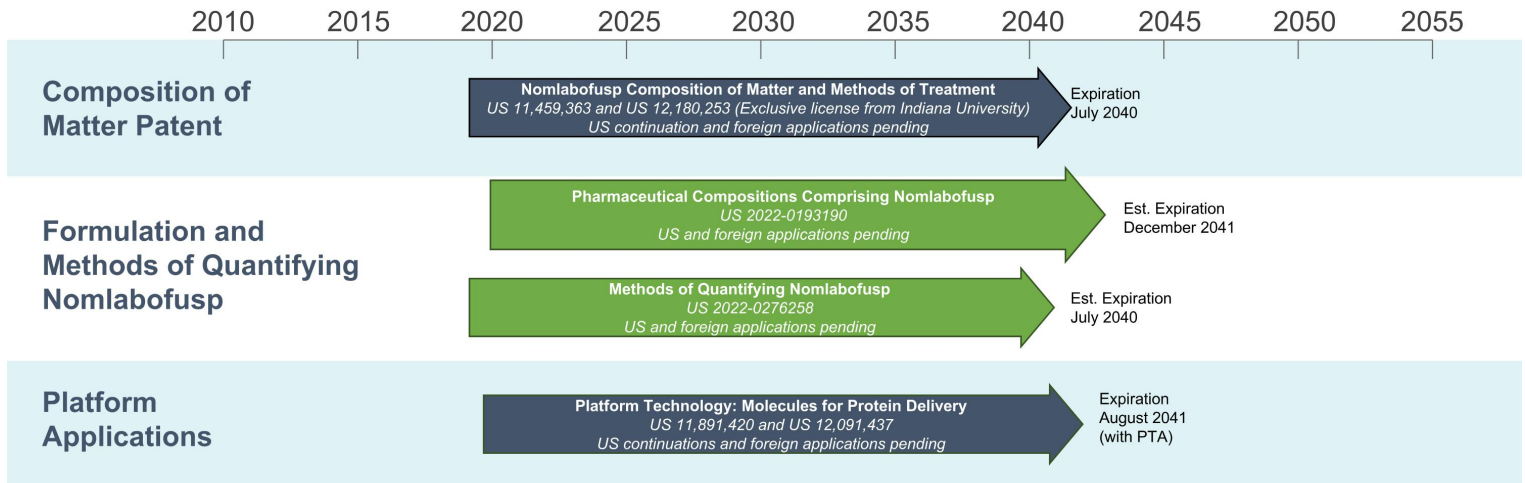


Larimar Therapeutics

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)



■ Granted ■ Pending

Nomlabofusp is a Competitively Differentiated Treatment Approach*

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



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	Frataxin Protein Replacement	Phase II
Mitochondrial Oxidative Stress Modifier	Omaveloxolone (SKYCLARYS™)	Biogen	Nrf2 Activator	Approved (US and EU)
	Vatiquinone	PTC Therapeutics	15-Lipoxygenase Inhibitor	Phase III
Gene Expression Regulator	DT-216P2 (new formulation)	Design Therapeutics	GeneTAC	Pre-clinical
Gene Therapy	LX2006	Lexeo Therapeutics	Frataxin Gene Replacement	Phase I/II

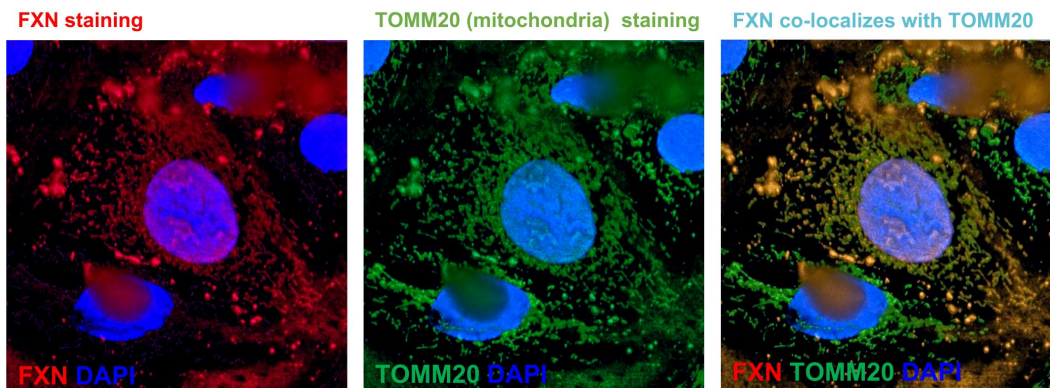


*Competitive landscape focuses on clinical-stage, industry-sponsored programs from public companies



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

Nomlabofusp Extends Survival in FXN-deficient KO Mice

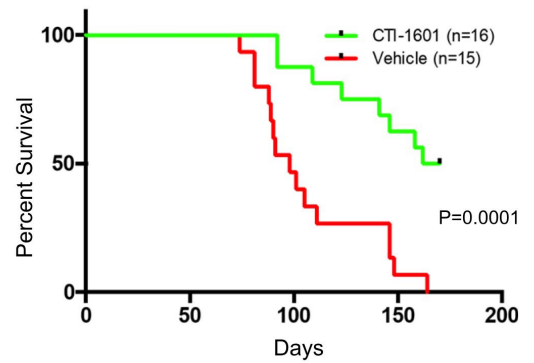
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

Median survival of MCK-Cre FXN-KO mice

- 166 days (nomlabofusp) vs. 98 days (Vehicle)
- Nomlabofusp administered 10 mg/kg SC every other day

Survival beyond vehicle mean (107.5 days)

- 87.5% (nomlabofusp) vs. 33% (Vehicle)
- Demonstrates that nomlabofusp is capable of delivering sufficient amounts of FXN to mitochondria



Nomlabofusp (CTI-1601) rescues a severe disease phenotype in a well-characterized cardiac mouse model of FA

Nomlabofusp Prevents Development of Ataxic Gait in Neurologic KO Mouse Model

In-Vivo Efficacy Data in Pvalb-Cre FXN-KO Mouse Model

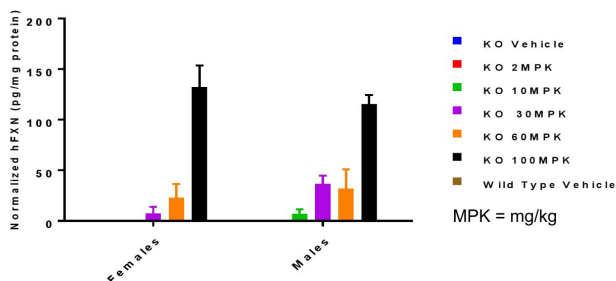
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

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

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

Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at varying SQ doses of nomlabofusp every other day for two weeks at Jackson Laboratories (Bar Harbor, ME). After dosing, animals were sacrificed, and heart and skeletal muscle were evaluated for hFXN concentration in mitochondrial extracts and SDH activity was assessed.

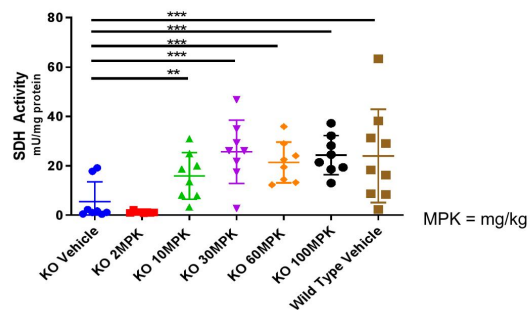
Mitochondrial FXN (Heart)



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



SDH Activity (Muscle)

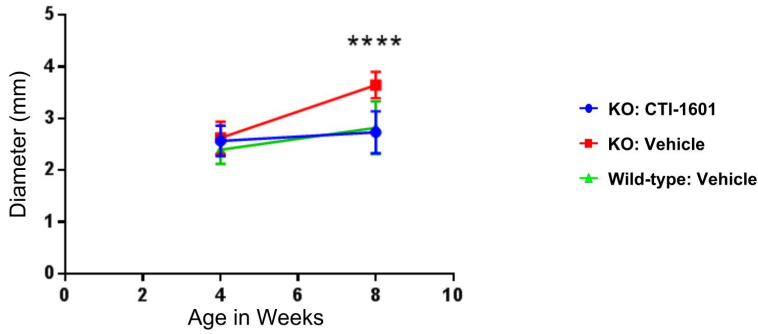


Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of nomlabofusp; activity plateaus at 30 mg/kg and is equivalent to activity in wild type

Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

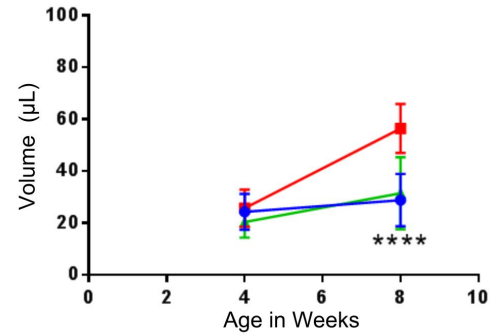
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

Left Ventricle Internal Diameter (Systole)



Left ventricular (LV) diameter 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)

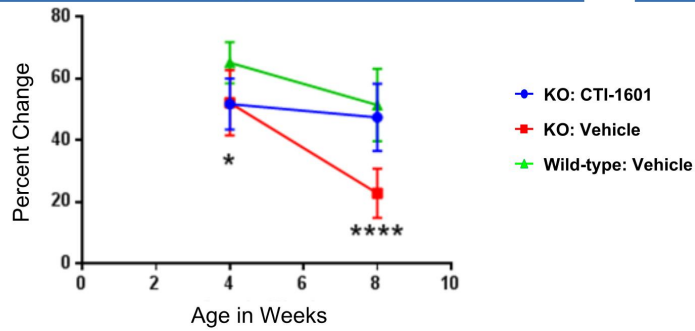


Nomlabofusp-treated mice have similar LV volume as wild type; echocardiogram shows significant differences between vehicle and nomlabofusp treated (10 mg/kg every other day) KO mice

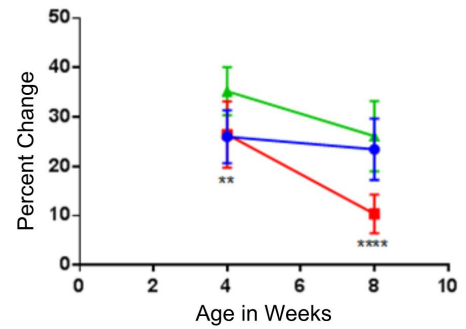
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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

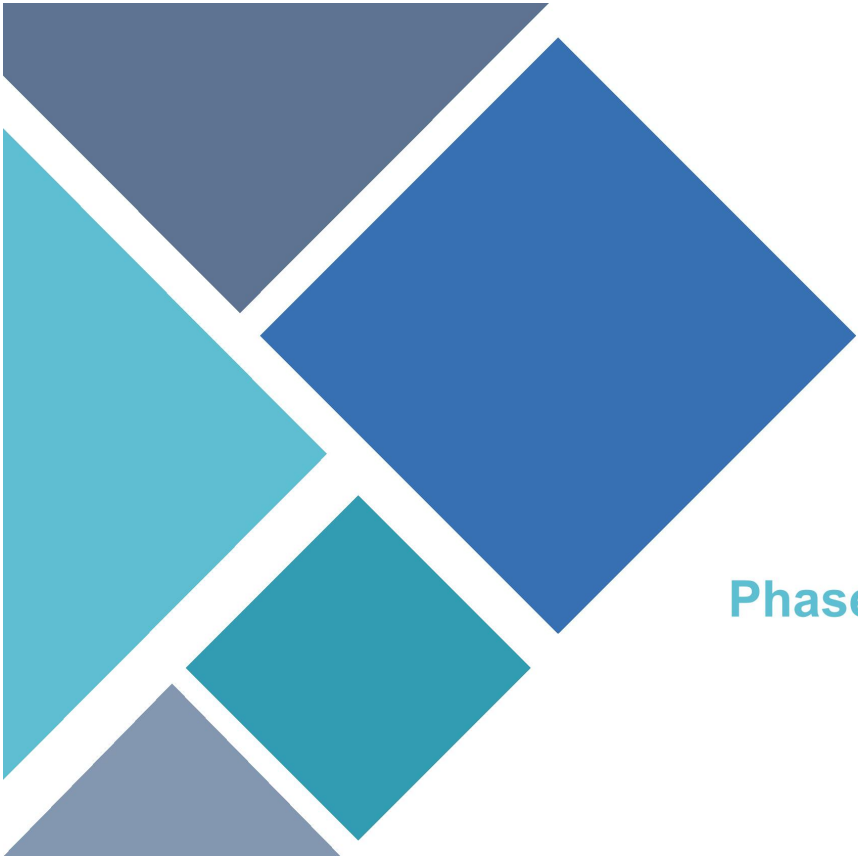


Left Ventricle Fractional Shortening



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



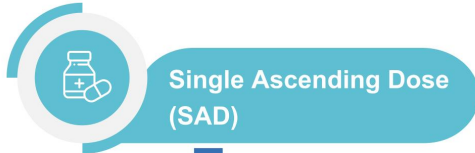
Phase 1 Clinical Data

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)

Eligible patients from SAD trial could enroll in MAD trial

Number of subjects: 28

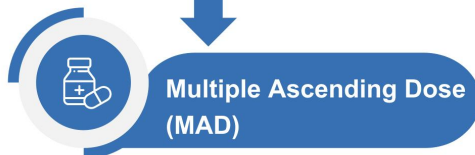
Dose levels: 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)

Treatment Duration: 1 day

1° Endpoint: Safety and tolerability

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

Status: Complete



Multiple Ascending Dose (MAD)

Number of Subjects: 27

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

Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days

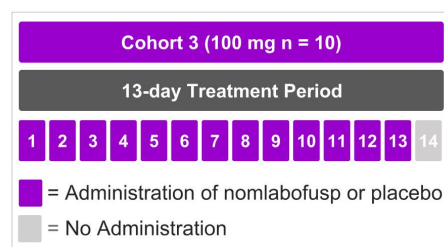
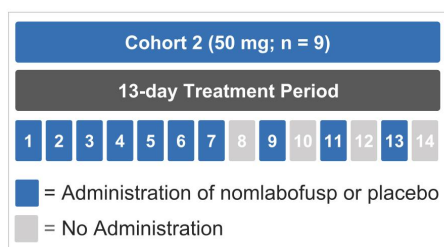
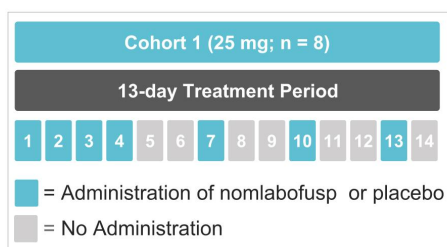
1° Endpoint: Safety and tolerability

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

Status: Complete

Completed Phase 1 Multiple Ascending Dose Study

Treatment Schedules for Each Cohort- nomlabofusp (CTI-1601) or placebo



FXN Level Sampling Days Presented for Each Cohort

Cohort 1 Sampling Days

Buccal Cells	Baseline, Day 4, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 4, Day 13

Cohort 2 Sampling Days

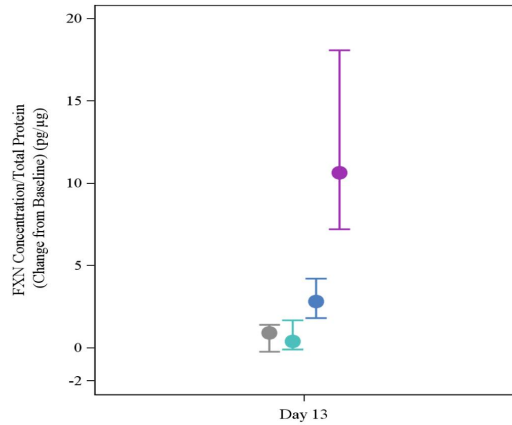
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Cohort 3 Sampling Days

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

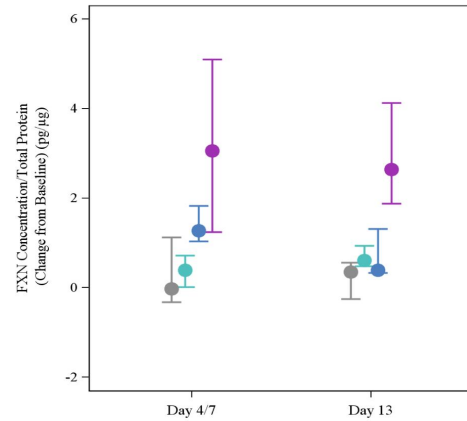
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

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



Placebo: Participants randomized to placebo in each cohort
 25 mg: Dosed daily for 4 days, every third day thereafter

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



50 mg: Dosed daily for 7 days, every other day thereafter
 100 mg: Dosed daily for 13 days



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

MAD Trial Patient Demographics

Parameter	Statistic	All placebo (n=7)	25 mg CTI-1601 (n=6)	50 mg CTI-1601 (n=7)	100 mg CTI-1601 (n=7)	All CTI-1601 (n=20)	Overall (n=27)
Sex							
Male	n (%)	5 (71.4)	3 (50.0)	4 (57.1)	3 (42.9)	10 (50.0)	15 (55.6)
Female	n (%)	2 (28.6)	3 (50.0)	3 (42.9)	4 (57.1)	10 (50.0)	12 (44.4)
Age (years)							
	Mean	25.7	39.7	34.7	28.0	33.9	31.7
	SD	6.37	16.59	9.03	8.96	12.13	11.40
	Median	23	37	36	24	34	28
	Min, Max	20,36	21,65	19,47	20,44	19,65	19,65
Race							
White	n (%)	6 (85.7)	6 (100.0)	6 (85.7)	6 (85.7)	18 (90.0)	24 (88.9)
Asian	n (%)	0	0	1 (14.3)	1 (14.3)	2 (10.0)	2 (7.4)
American Indian	n (%)	1 (14.3)	0	0	0	0	1 (3.7)
Ethnicity							
Hispanic/Latino	n (%)	2 (28.6)	0	0	0	0	2 (7.4)
Not Hispanic/Latino	n (%)	5 (71.4)	6 (100.0)	7 (100.0)	7 (100.0)	20 (100.0)	25 (92.6)

MAD Trial Patient Disease Characteristics

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

PK analyses support evaluating once-daily and every-other-day dosing regimens for CTI-1601

Summary of MAD Trial PK Analyses

- ✓ CTI-1601 was quickly absorbed after subcutaneous administration
- ✓ Dose-proportional increases in exposure observed with increasing doses of CTI-1601
- ✓ Mean half life of CTI-1601 in plasma was approximately 11 hours
- ✓ CTI-1601 appeared to be at or close to steady state exposure after 13 days of dosing 100 mg once daily



Additional Phase 2 Dose Exploration Data

Nomlabofusp: Predictable Long-Term Pharmacokinetics

1 Rapid absorption after subcutaneous administration

2 Nomlabofusp reached steady state in plasma by Day 30 with no further accumulation

3 Pharmacokinetic profile consistent with Phase 1 and Phase 2 studies

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

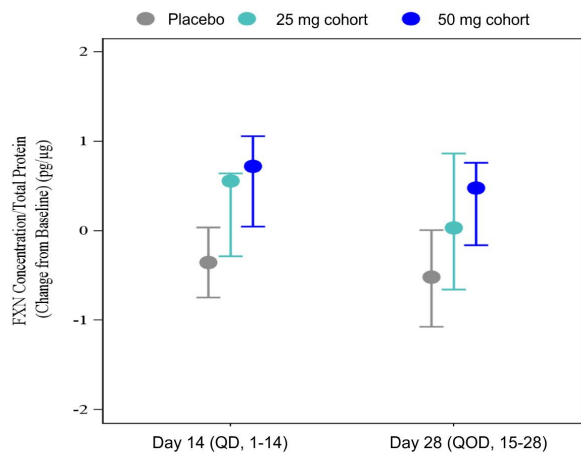


Only participants with quantifiable levels at baseline and day 14 and day 28 are included in the tables.

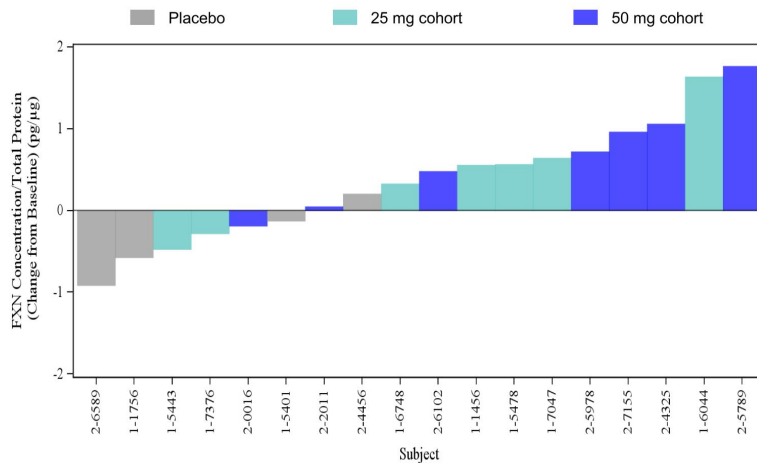
Dose-Dependent Increase in FXN Levels in Buccal Cells

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

Buccal Cells FXN Levels* Change from Baseline**



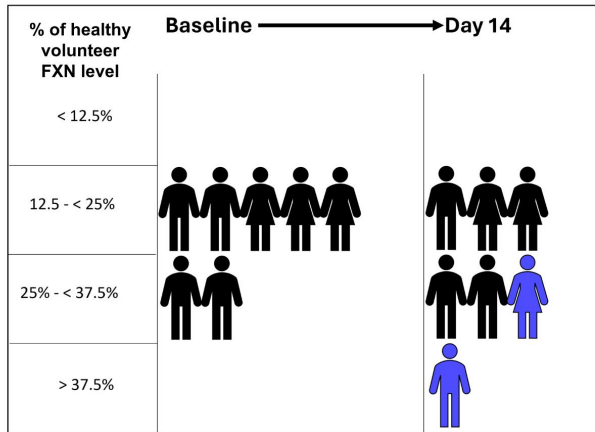
FXN Levels* in Buccal Cells Change from Baseline at Day 14



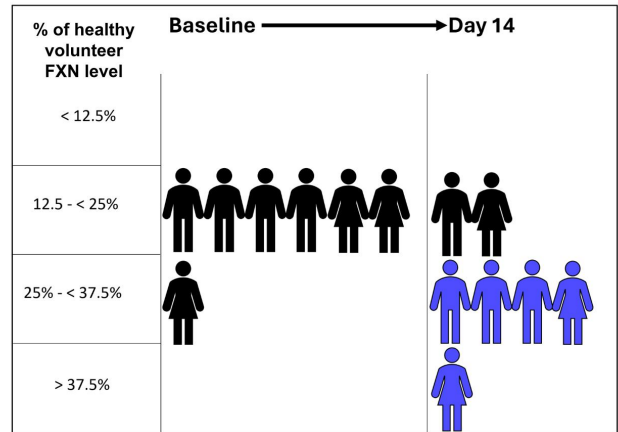
*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.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

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

25 mg of Nomlabofusp



50 mg of Nomlabofusp



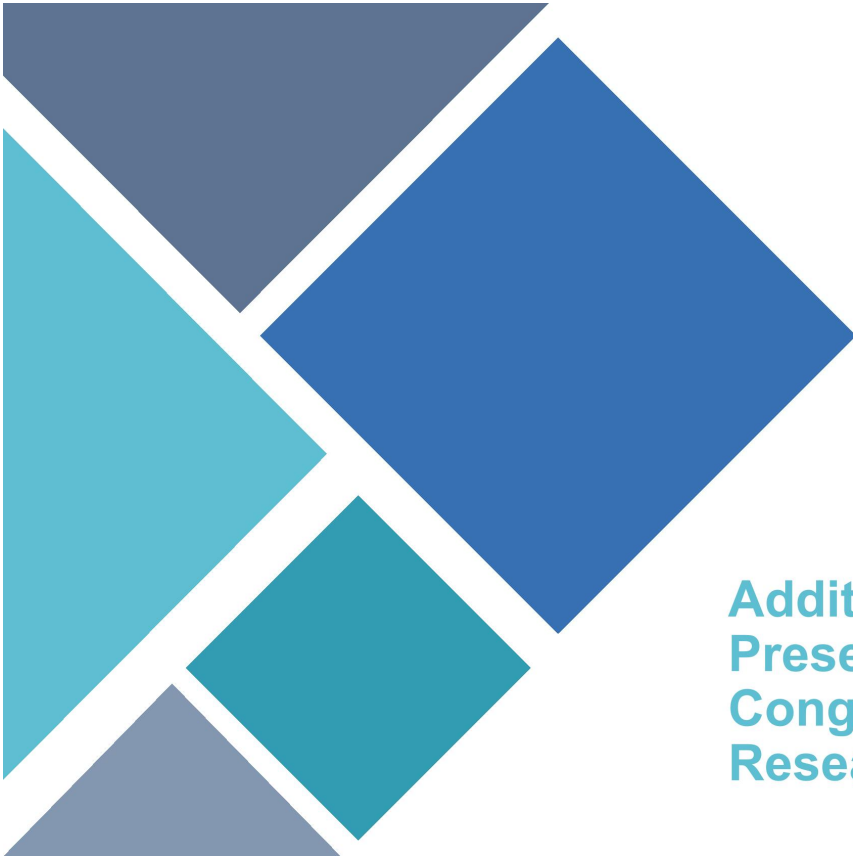
■ Baseline FXN levels as a % of average FXN level in healthy volunteers

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



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).



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

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

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

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

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

Over 50% of participants were non-ambulatory at baseline

*18 subjects participated in more than 1 study

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

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



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

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

	N*	Median	Mean	Min	Max
Age	61	28.0	31.9	19	69
Age of Onset	61	15.0	15.9	5	60
Age of Diagnosis	61	19.0	21.0	5	64
Shorter GAA (GAA₁)	60	550.0	555.8	99	1000
Longer GAA (GAA₂)	60	900.0	890.2	265	1300
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				

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

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

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

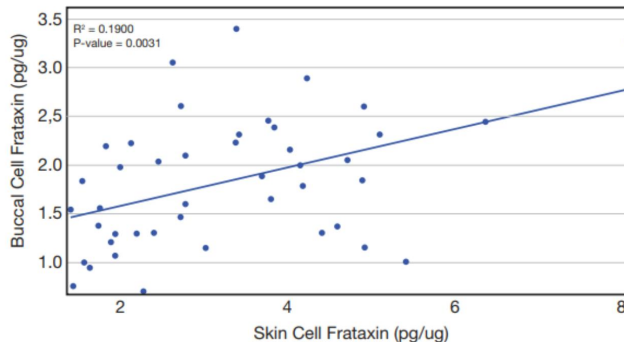
*Quantifiable buccal cell frataxin levels

**Median values

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

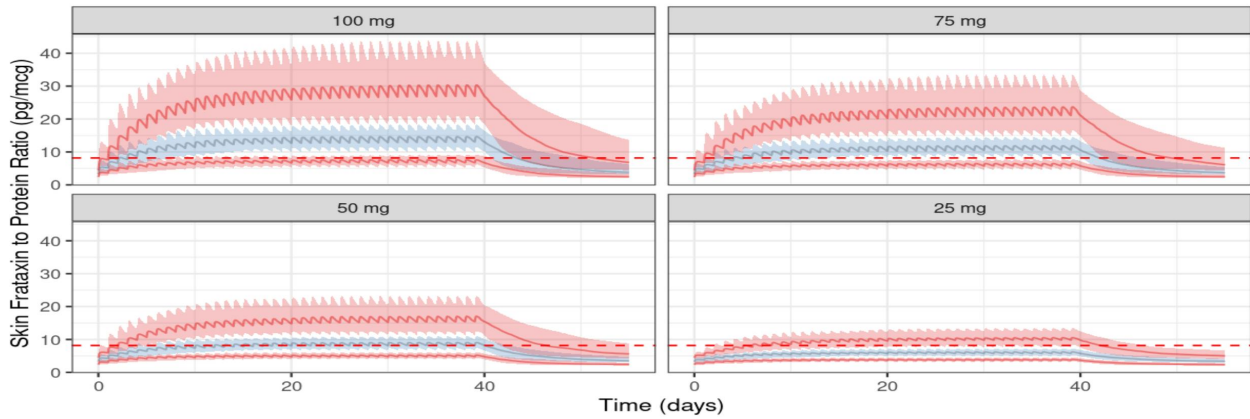
Buccal cell FXN levels correlated with age of onset and inversely correlated with the number of GAA repeats and rate of disease progression

Baseline Buccal and Skin Cell FXN Levels



Buccal cell FXN levels correlated with skin cell FXN levels

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



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

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

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

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



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



Additional OLE Study Information

Nomlabofusp: Predictable Pharmacokinetics

1

Quick absorption after subcutaneous administration

2

Dose-proportional increases in exposure observed

3

Pharmacokinetic profile consistent with Phase 1 studies

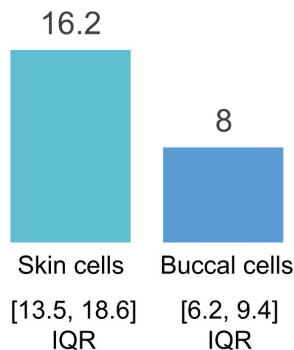


Non-Interventional Study Data

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

Non-interventional study measured FXN in homozygous healthy volunteers

**Median Frataxin Concentration (pg/ μ g)
in Homozygous Healthy Volunteers (n = 60)**



Most patients with FA only produce ~20-40%¹ of normal frataxin levels depending on the tissue, sampling technique, and assay considered

Lower FXN levels seen with typical onset² (5 to 15 years of age)

Higher FXN levels seen with late onset² (after 25 years of age)

Heterozygous carriers who show no signs of disease have buccal cell FXN levels of ~50% of unaffected healthy persons¹



FDA START Pilot Program

Nomlabofusp Selected by FDA for START Pilot Program

Highlights FDA commitment to augment formal meetings with more rapid, ad-hoc communications to accelerate program development of rare diseases

START Pilot Program

Support for Clinical Trials Advancing Rare Disease Therapeutics

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

Designed to **accelerate development of novel therapies** intended to address unmet medical needs in **rare diseases**

7 novel drugs selected

- 3 products by CDER (nomlabofusp) for rare neurodegenerative conditions
- 4 products by CBER for cell and gene therapy

CDER Selection Based On

Demonstrated development **program readiness** (e.g., sponsors who demonstrate the ability to move the program towards a marketing application)

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; CBER: Center for Biologics Evaluation and Research; CMC: Chemistry, Manufacturing, and Controls



FARA

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

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

FARA provides industry with several key items

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



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