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): August 14, 2025

Larimar Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-36510**	**20-3857670**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(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 August 14, 2025, Larimar Therapeutics, Inc. (the "***Company***") announced its financial results and operational highlights for the second quarter ended June 30, 2025. 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 August 14, 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.

Exhibit No.	Document
99.1	Press Release issued by Larimar Therapeutics, Inc. on August 14, 2025*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated August 14, 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: August 14, 2025

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

Exhibit 99.1

Larimar Therapeutics Reports Second Quarter 2025 Financial Results

- *Initial data from the 50 mg dose in the open label study and the adolescent PK run-in study planned for program update in September 2025*
- *Adolescent participants from the PK run-in study and patients with FA who have not participated in prior nomlabofusp clinical studies are currently screening and enrolling in the open label study; planning to enroll children (2 to 11 years of age) directly into the open label study*
- *FDA recommended that the safety database include at least 30 participants with continuous study drug exposure for 6 months, and a subset of at least 10 participants for 1-year; large majority of safety data should be from participants receiving 50 mg nomlabofusp*
- *Published two peer-reviewed articles; the nonclinical data included in the publications were part of the data submitted to FDA to support the mechanism of action of nomlabofusp and the potential use of skin FXN concentrations as a reasonably likely surrogate endpoint*
- *BLA seeking accelerated approval on track to be submitted in the second quarter of 2026*
- *Global Phase 3 study activities ongoing including qualification of identified sites with patient recruitment expected to initiate later this year*
- *$203.6 million in pro forma* cash, cash equivalents and marketable securities as of June 30, 2025, with projected cash runway into the fourth quarter of 2026*

**Pro forma cash, cash equivalents, and marketable securities of $203.6 million reflects $138.5 million of cash, cash equivalents and marketable securities as of June 30, 2025 combined with the $65.1 million in net proceeds from the recently completed July 2025 public offering.*

Bala Cynwyd, PA, August 14, 2025 – Larimar Therapeutics, Inc. (Larimar) (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its second quarter 2025 operating and financial results.

“We are pleased with our continued strong execution as we further advance our nomlabofusp program towards potential registration. Importantly, we have written communications in hand from the Food and Drug Administration (FDA) for key elements of our Biologics License Application (BLA) submission including safety database recommendations and a potential accelerated approval pathway based on the use of skin frataxin levels as a novel surrogate endpoint. We also recently published two peer-reviewed papers, including nonclinical data contributing to the FDA’s openness to using skin frataxin (FXN) concentrations as a reasonably likely surrogate endpoint (RLSE),” said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. “Enrollment in our open label study is ongoing, and new study participants are

now receiving the lyophilized formulation of nomlabofusp. We have several important near-term catalysts ahead including initial data from the 50 mg dose of our open label study and data from the adolescent pharmacokinetic (PK) run-in study expected in September 2025. Global sites have been identified for our Phase 3 trial, and we expect to initiate patient recruitment later this year. With our balance sheet strengthened through our recent capital raise, key clinical data approaching, and a clear regulatory path in place, we are well-positioned to submit our BLA for nomlabofusp in the second quarter of 2026 as the first potential disease modifying therapy for Friedreich's ataxia (FA)."

Highlights

- **Initial 50 mg Open Label Study Data Expected in September 2025:** The ongoing open label study continues to enroll, and active study participants are currently receiving the 50 mg dose of nomlabofusp. Larimar plans to provide an update on open label data on at least 30 to 40 study participants who received at least one dose of nomlabofusp in September 2025.

- **Adolescent PK Run-In Data in September 2025:** Larimar completed dosing of 14 adolescents (12-17 years of age) in a PK run-in study for pediatric patients with FA in March 2025. Adolescents received a weight-based dose expected to match the PK of adults receiving the 50 mg dose or placebo. The safety and PK data from this study are expected during the nomlabofusp program update in September 2025.

- **Recent Expansion of Open Label Study Participants**: Adolescent participants from the PK run-in study are currently being screened and enrolled in the open label study. Also, patients with FA who have not participated in prior nomlabofusp clinical studies are being screened and enrolled into the open label study. In addition, Larimar plans to enroll children (2 to 11 years of age) directly into the open label study.

- **Announced FDA Safety Database Recommendations:** In June 2025, Larimar announced that the FDA recommended evaluating safety in at least 30 participants with continuous study drug exposure for 6-months, with a subset of at least 10 of those participants on continuous study drug exposure for 1-year. The FDA also recommended that the large majority of safety data should be from participants receiving the 50 mg dose.

- **Published Nonclinical Data on Nomlabofusp in Two Peer-Reviewed Articles:** In July 2025, Larimar announced the publication of nonclinical data evaluating the mechanism of action, pharmacodynamics, and pharmacology of nomlabofusp as a novel FXN protein replacement therapy designed to address the underlying cause of FA in two peer-reviewed articles. These data were included in the briefing package reviewed by the FDA in support of using skin FXN concentrations as a RLSE for Larimar's registrational program seeking accelerated approval for nomlabofusp.

- **BLA Submission on Track:** In the second quarter of 2026, Larimar plans to submit a BLA seeking accelerated approval.

- **Identified Global Phase 3 Sites:** Following feedback from FDA and European Medicines Agency (EMA) on the study protocol, global Phase 3 study sites in the U.S., E.U., U.K., Canada, and Australia were identified and are currently being qualified and initiated. Larimar expects to begin patient recruitment later this year.

- **Initiated Transition to Lyophilized Form of Nomlabofusp:** In May 2025, Larimar began to introduce the lyophilized product formulation intended for commercialization into the open label

study.

- **Strengthened Balance Sheet With $65.1 Million Public Offering:** In July 2025, Larimar announced a public offering of common stock with net proceeds of $65.1 million supported by existing and new leading healthcare investors that extends its projected cash runway into the fourth quarter of 2026.

Second Quarter 2025 Financial Results

As of June 30, 2025, the Company had cash, cash equivalents and marketable securities totaling $138.5 million. Together with net proceeds of approximately $65.1 million from the July 2025 public offering, the company has projected cash runway into the fourth quarter of 2026.

Second quarter of 2025 compared to the second quarter of 2024

The Company reported a net loss for the second quarter of 2025 of $26.2 million, or $0.41 per share, compared to a net loss of $21.6 million, or $0.34 per share, for the second quarter of 2024.

Research and development expenses for the second quarter of 2025 were $23.4 million compared to $19.7 million for the second quarter of 2024. The increase in research and development expenses was primarily attributable to an increase of $2.4 million in professional consulting fees related to ongoing clinical trial activity, an increase of $1.3 million in personnel costs associated with increased headcount as BLA activities expand, and an increase of $0.6 million in clinical costs primarily associated with initial activities related to the design and initial activities associated with the Company's planned confirmatory study required as part of the planned BLA filing.

General and administrative expenses were $4.4 million in the second quarter of 2025 compared to $4.9 million in the second quarter of 2024. The decrease in general and administrative expenses was primarily due to a decrease of $0.4 million in noncash stock compensation costs and a decrease of $0.3 million in professional services primarily related to legal services performed.

Six months ended June 30, 2025, compared to the six months ended June 30, 2024

The Company reported a net loss for the first six months of 2025 of $55.5 million, or $0.87 per share, compared to a net loss of $36.3 million, or $0.62 per share, for the first six months of 2024.

Research and development expenses for the six months ended June 30, 2025, were $49.9 million compared to $32.6 million for the six months ended June 30, 2024. The increase in research and development expenses was primarily attributable to an increase of $7.1 million in nomlabofusp clinical trial material manufacturing costs, an increase of $3.5 million in professional consulting fees related to ongoing clinical trials, an increase of $3.4 million in clinical costs primarily associated with initial activities related to the design and initial activities associated with the Company's planned confirmatory study, and an increase of $2.9 million in personnel costs associated with increased headcount.

General and administrative expenses were $9.1 million for the first six months of 2025 compared to $8.7 million for the six months ended June 30, 2024. The increase in general and administrative expenses was primarily due to an increase of $0.8 million in personnel costs associated with an increased headcount and an increase of $0.3 million in professional services primarily related to pre-marketing-related consulting fees, partially offset by a decrease of $0.7 million in stock compensation costs.

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; 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.

Investor Contact:	**Company Contact:**
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LifeSci Advisors	Chief Financial Officer
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(212) 915-2569	(484) 414-2715

Larimar Therapeutics, Inc.
Consolidated Balance Sheet
(In thousands except share data)
(unaudited)

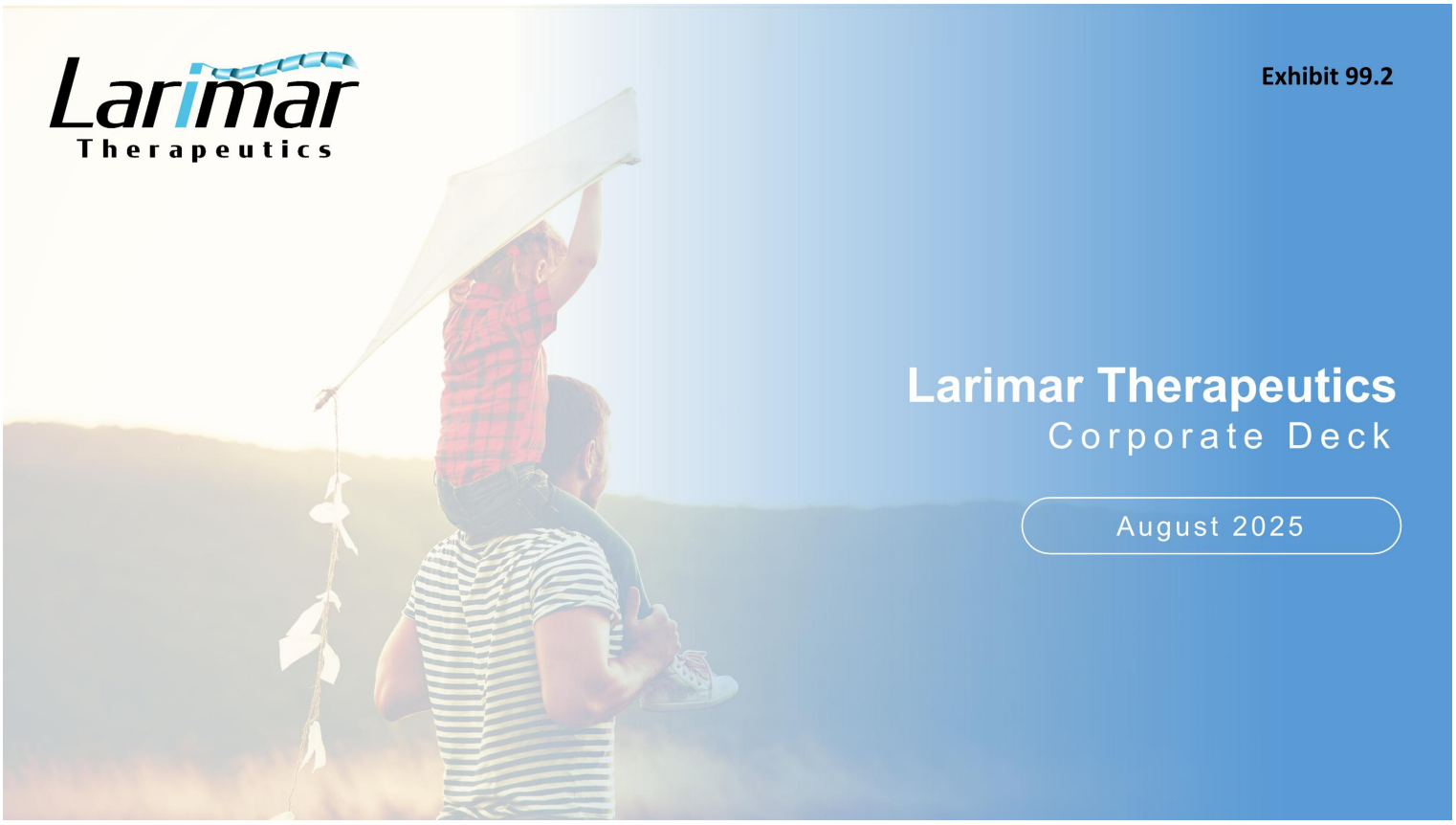
	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 20,587	$ 33,218
Short-term marketable securities	117,937	150,236
Prepaid expenses and other current assets	7,032	11,850
Total current assets	145,556	195,304
Property and equipment, net	797	881
Operating lease right-of-use assets	2,468	2,838
Restricted cash	606	606
Other assets	561	596
Total assets	$ 149,988	$ 200,225
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,169	$ 2,424
Accrued expenses	21,351	20,872
Operating lease liabilities, current	1,129	1,060
Total current liabilities	26,649	24,356
Operating lease liabilities	3,485	4,057
Total liabilities	30,134	28,413
Commitments and contingencies		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of June 30, 2025 and December 31, 2024; no shares issued and outstanding as of June 30, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2025 and December 31, 2024; 64,027,892 and 63,815,065 shares issued and outstanding as of June 30, 2025 and December 31, 2024, respectively	64	64
Additional paid-in capital	444,420	440,758

Accumulated deficit	(324,621)	(269,158)
Accumulated other comprehensive gain (loss)	(9)	148
Total stockholders' equity	119,854	171,812
Total liabilities and stockholders' equity	$ 149,988	$ 200,225

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 23,368	$ 19,682	$ 49,919	$ 32,621
General and administrative	4,424	4,917	9,060	8,712
Total operating expenses	27,792	24,599	58,979	41,333
Loss from operations	(27,792)	(24,599)	(58,979)	(41,333)
Other income, net	1,610	2,972	3,516	5,052
Net loss	$ (26,182)	$ (21,627)	$ (55,463)	$ (36,281)
Net loss per share, basic and diluted	$ (0.41)	$ (0.34)	$ (0.87)	$ (0.62)
Weighted average common shares outstanding, basic and diluted	64,027,892	63,801,792	63,996,126	58,677,749
Comprehensive loss:				
Net loss	$ (26,182)	$ (21,627)	$ (55,463)	$ (36,281)
Other comprehensive loss:				
Unrealized loss on marketable securities	(63)	(125)	(157)	(231)
Total other comprehensive loss	(63)	(125)	(157)	(231)
Total comprehensive loss	$ (26,245)	$ (21,752)	$ (55,620)	$ (36,512)

Exhibit 99.2



Larimar Therapeutics

Corporate Deck

August 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.
Clear FDA expectations for accelerated approval path	Written FDA recommendations on key elements for Biologics License Application (BLA) seeking accelerated approval Safety database of at least 30 participants with continuous exposure for 6 months and a subset of at least 10 with 1-year; large majority of safety data should be from participants receiving the 50 mg dose. Open to use of skin FXN concentrations as a reasonably likely surrogate endpoint (RLSE), supported by published peer-reviewed nonclinical data
Positive initial data from long-term open label 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 active patients currently receiving 50 mg dose.
Recent updates to open label study design	Began introducing to new study participants lyophilized drug product formulation intended for commercialization. Included antihistamine premedication for patients who participated in a prior nomlabofusp study. Enrolling eligible adolescents from the PK run-in study, as well as patients with FA who have not participated in a prior nomlabofusp study.
Near-term catalysts	Open label data from 30-40 participants who received at least one dose of nomlabofusp, including subjects on 50 mg dose, expected Sept 2025. Adolescent PK run-in data from 14 participants (some on placebo) expected Sept 2025. BLA seeking accelerated approval on track to be submitted in the second quarter 2026 to include adults and children.

Larimar Therapeutics

$203.6 M in pro forma cash and investments as of June 30, 2025, with projected cash runway into Q4 2026.
Pro forma cash and investments reflect our $138.5 M of cash and investments as of June 30, 2025, combined with the $65.1 M in net proceeds from our recently completed July 2025 public offering.

Clear FDA Expectations for Path to BLA Submission Seeking Accelerated Approval Planned in Q2 2026

Elements of BLA Submission	FDA Recommendations
Use of Skin FXN Concentrations as a Surrogate Endpoint	• Open to use of increases in skin FXN concentrations as a RLSE • Acknowledged submitted data appears to support a relationship between increased skin FXN and relevant tissues such as the heart, dorsal root ganglion, and skeletal muscle • Acceptability of increases in skin FXN for accelerated approval will be decided during future BLA review
Safety Database	• At least 30 participants with continuous exposure for 6-months • A subset of at least 10 participants with continuous exposure for 1-year • A large majority of the exposure should be on the 50 mg dose
Clinical Data Package and Global Phase 3 Study	• SAD and MAD Phase 1 studies established safety and tolerability • Phase 2 dose exploration study data and PK/PD data from ongoing open label study supported 50 mg as the recommended dose • Long-term data from ongoing open label study including increases in skin FXN concentrations and safety data • Global Phase 3 study, intended as the confirmatory study, to evaluate clinical outcomes including upright stability and mFARS expected to be underway at the time of BLA submission
Pharmacology and Toxicology	• Nonclinical data supporting the use of FXN as a novel surrogate endpoint • Complete toxicology package including juvenile toxicology study • Clinical data includes trends towards normalization of patient lipid profiles and gene expression
Chemistry Manufacturing and Controls	• Data supporting the lyophilized drug product with stability at room temperature • Data on batches manufactured at a commercial scale • Analytical methods and proposed specifications

Larimar Therapeutics

FXN: Frataxin; BLA: Biologics License Application; FDA: Food and Drug Administration; RLSE: reasonably likely surrogate endpoint; SAD: Single ascending-dose ; MAD: Multiple ascending dose

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 (FXN) 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 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
Unmet Medical Need	The only treatment currently approved for FA does not address frataxin deficiency

Larimar is developing nomlabofusp, the first potential disease modifying therapy designed to systemically address the underlying FXN deficiency in FA

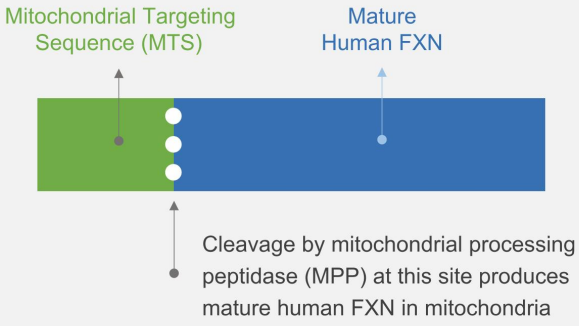
Larimar Therapeutics

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

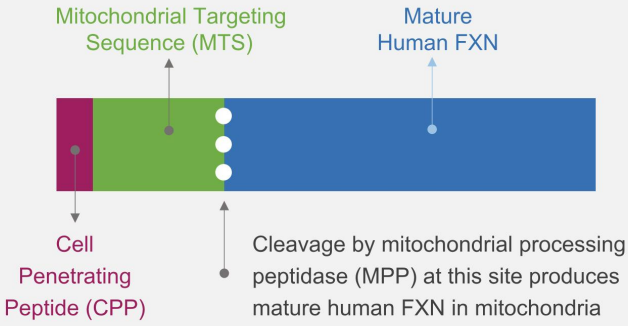
Nomlabofusp is Designed to Deliver Additional Frataxin

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

Larimar Therapeutics

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

Larimar Therapeutics

*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

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

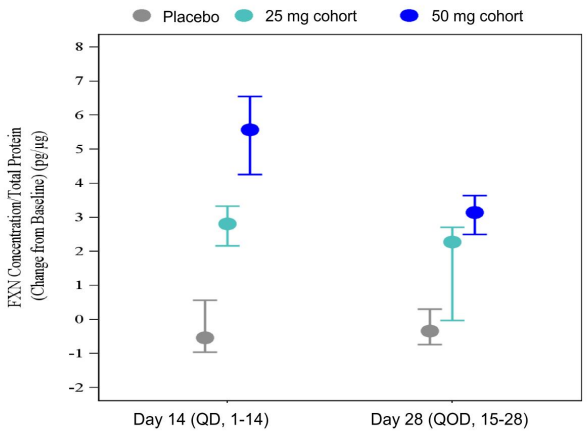
= No Administration

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)
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

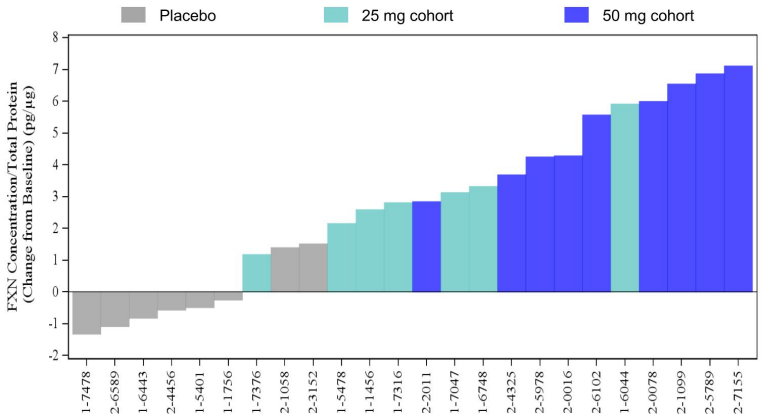
Dose-Dependent Increase in FXN Levels in Skin Cells

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



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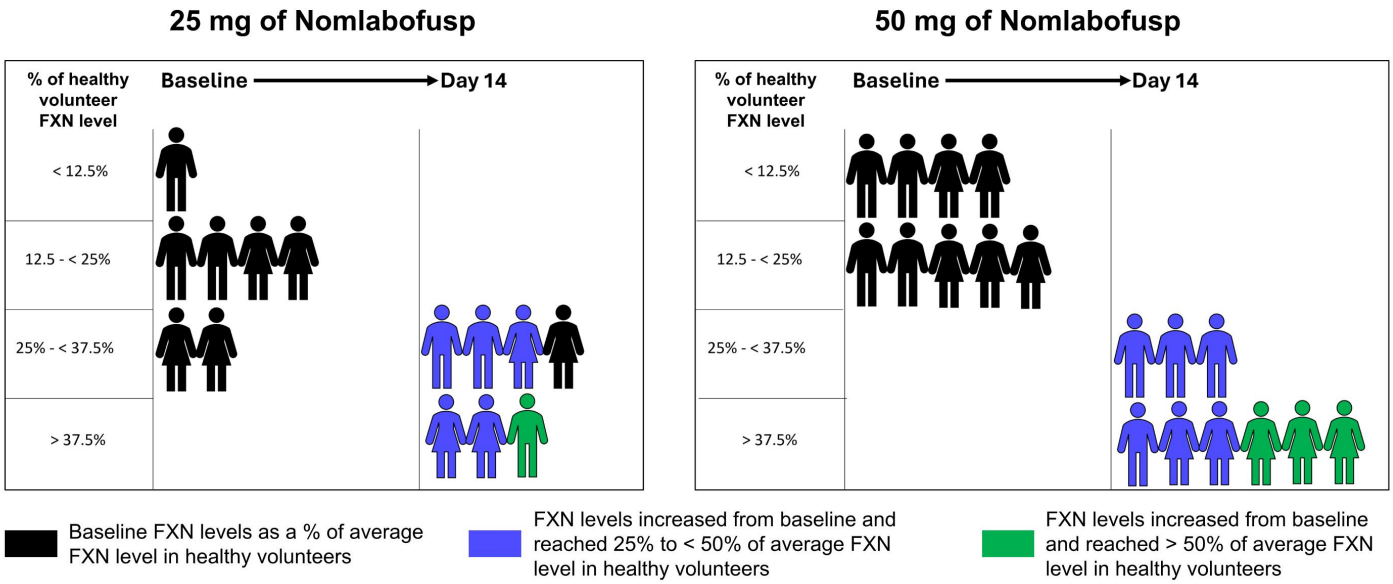
*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at both baseline and Day 14 are included in the figures.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

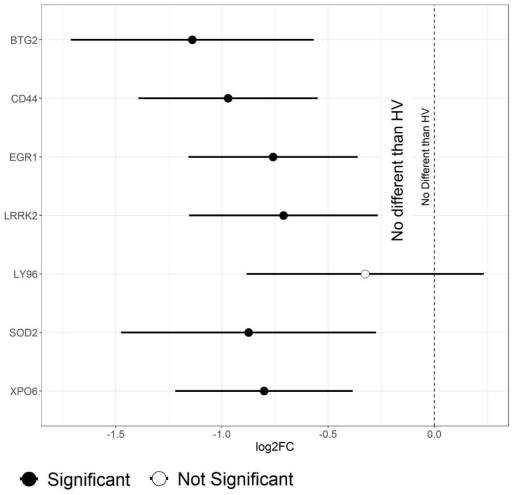
Skin Cell FXN Levels Achieve Higher % of Healthy Volunteers* Following 14 days of Daily Nomlabofusp



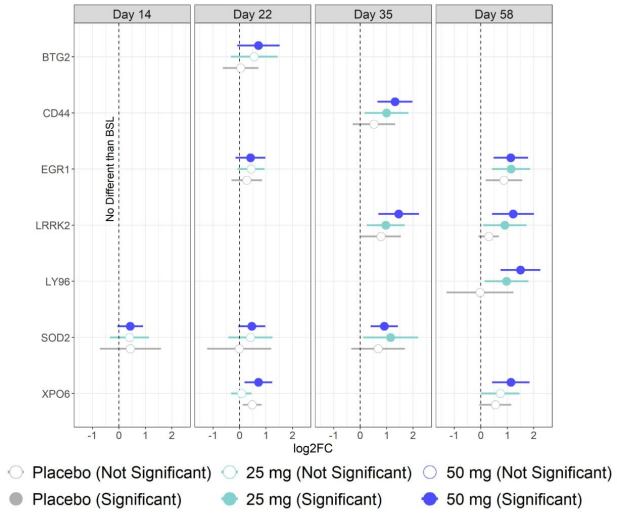
Only participants with quantifiable levels at baseline and day 14 are included in the figures.
*% of healthy volunteer FXN level is calculated by dividing each participant's FXN level by the average FXN level (16.34 pg/µg) from the noninterventional healthy volunteer study (N=60).

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

Select Baseline Gene Expression
Patients with FA* vs. Healthy Volunteers (HV)**



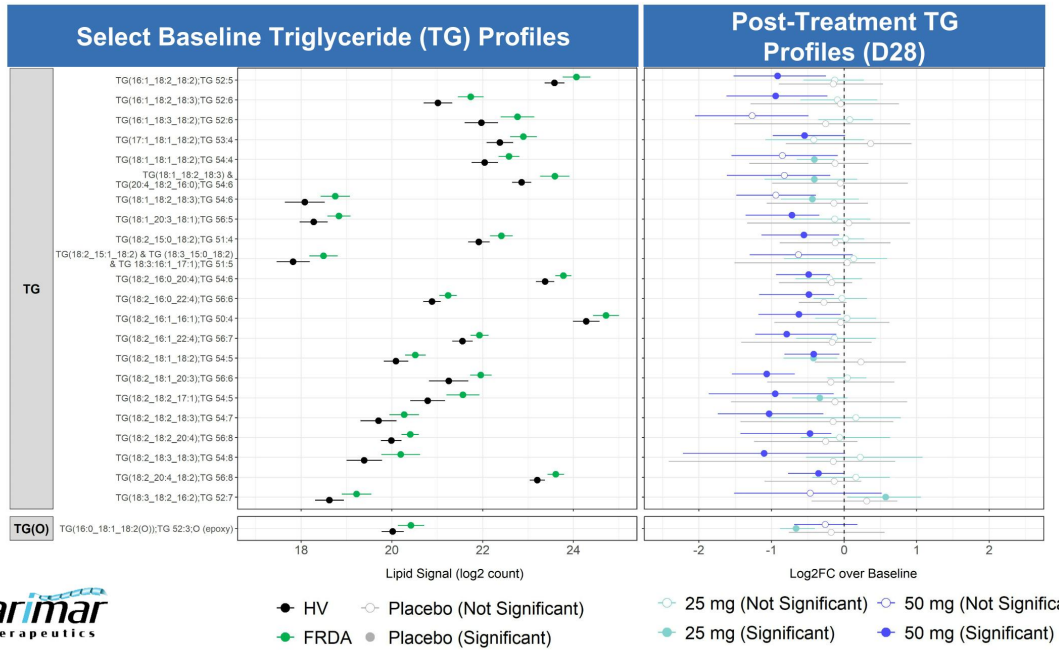
Post-treatment Changes in Gene Expression From Baseline



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

Larimar Therapeutics

Nomlabofusp Long-term Open Label Study

Open Label Study*: 25 mg Dose Completed, 50 mg Dose Ongoing

Initial data from 50 mg dose expected September 2025

Patient Population

- Initially, participation in a prior Phase 1 or Phase 2 trial required
- Currently screening and enrolling
  - adolescents (12-17 yrs) from the PK run-in study
  - patients who did not participate in prior nomlabofusp clinical studies
- Plan to enroll children (2 to 11 yrs) directly in study

25 mg nomlabofusp

Patients switched from 25 mg to 50 mg dose from Nov 2024 to Q1 2025

50 mg nomlabofusp

Daily subcutaneous injections self-administered or by a caregiver

Key Study Objectives

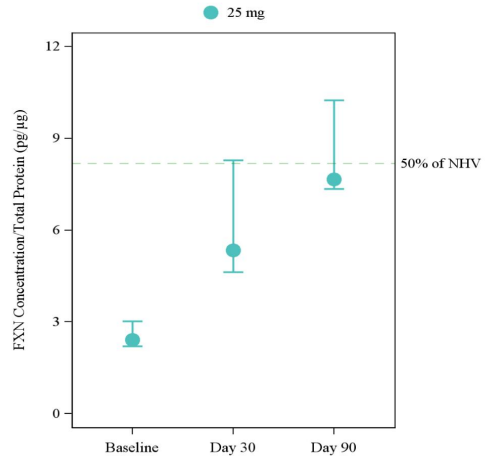
- Safety and tolerability
- Long-term PK
- Tissue FXN concentrations
- Clinical efficacy measures compared to FACOMS** database using propensity matching at the time of BLA

*Due to inclusion of participants who have not participated in prior nomlabofusp clinical studies, this study is now referred to as Open Label Study (previously called the Open Label Extension study)
**FACOMS: Friedreich's Ataxia Clinical Outcome Measures Study.

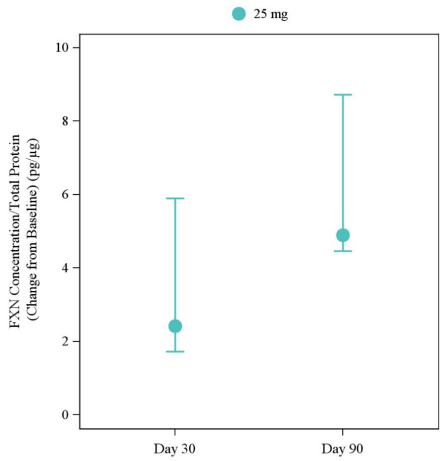
Increased FXN Levels in Skin Cells Sustained Over Time

Participants in the open label study dosed daily with 25 mg nomlabofusp for up to 90 days

Skin Cells - Absolute FXN Levels



Skin Cells - FXN Levels Change from Baseline

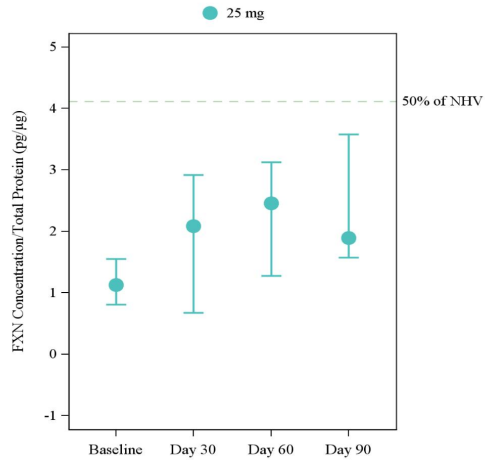


FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample. Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at all measurement points are included in the figures.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

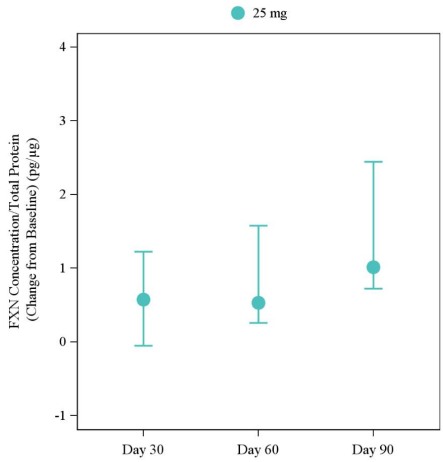
Increased FXN Levels in Buccal Cells Sustained Over Time

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

Buccal Cells - Absolute FXN Levels



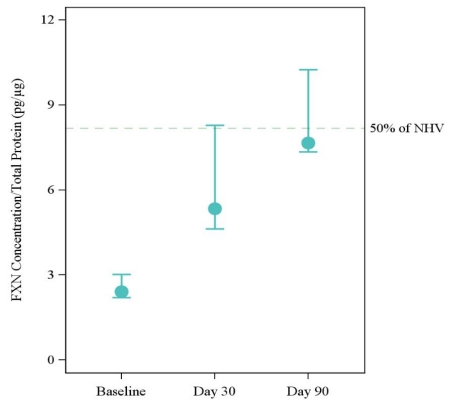
Buccal Cells - FXN Levels Change from Baseline



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).

Nomlabofusp 25 mg Daily Increased Skin FXN Levels in Open Label Study

Absolute FXN Levels



Time dependent increase in skin FXN levels

Mean % of healthy volunteers

16% at baseline
(9.33% min, 24.48% max)

↓

72% at Day 90
(43.86 % min, 160.61 % max)

Skin FXN levels as a % of healthy volunteers are higher at Day 90 vs. baseline in subjects

Larimar Therapeutics

FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample.
Data represent median and 25th and 75th percentiles. Only participants with quantifiable levels at all measurement points are included. 50% of normal healthy volunteer (NHV) FXN level is 8.17pg/µg from the noninterventional healthy volunteer study (N=60).
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/µg) and in buccal cells (8.24 pg/µg) from the noninterventional healthy volunteer study (N=60).

Observed Trends Towards Improvement in Clinical Outcomes at Day 90 in Open Label Study 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) Median (IQR) (Min, Max)	55.81 (13.296) 53.5 (47.5, 68.3) (35.0, 73.0)	18.13 (6.064) 17.0 (12.8, 23.8) (11.0, 27.0)	27.1 (14.23) 29.5 (18, 38) (2, 45)	130.91 (99.366) 89.5 (48.7, 227.8) (38.0, 277.3)
Day 90	Mean (SD) Median (IQR) (Min, Max)	55.13 (14.829) 53.3 (43.8, 66.0) (35.3, 79.5)	15.88 (6.249) 14.8 (11.0, 21.3) (8.0, 25.0)	18.5 (15.68) 17.0 (5, 32) (0, 42)	113.11 (95.586) 67.15 (48.4, 176.7) (33.50, 287.00)
Change from Baseline at Day 90	**Mean (SD)** **Median (IQR)** **(Min, Max)**	**-0.69 (3.983)** **-1.17 (-3.8, 1.2)** **(-5.0, 7.0)**	**-2.25 (3.082)** **-2.25 (-3.8, 0.3)** **(-8.0, 1.5)**	**-8.6 (12.24)** **-3.5 (-19, -3)** **(-28, 9)**	**-17.79 (27.450)** **-9.00 (-32.0, 1.7)** **(-73.5, 9.8)**

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

Nomlabofusp is Generally Well-Tolerated with Long-Term Treatment

First potential disease modifying therapy to treat FA, a rare and progressive neurodegenerative disease

Safety Data

Nomlabofusp has been generally well tolerated

Includes some participants on treatment for up to 15 months

Most common adverse events are local injection site reactions, with most being mild to moderate, brief in duration, and self-limited. These injection site reactions occur in the majority of the patients.

No participant has withdrawn from the study due to injection site reactions

Anaphylaxis has been deemed an adverse drug reaction likely related to nomlabofusp by the Larimar Safety Team

Participants with prior exposure who have been off treatment for some time seem to be more likely to develop an allergic reaction

- Premedication with antihistamines starting 5 days prior to the first dose and continuing for the first month in this population

Recent Updates to the Open Label* Study Design

Commercial Formulation	Introduction of lyophilized drug product formulation (stable at room temperature) intended for commercialization
Antihistamine Premedication	Premedication with antihistamines starting 5 days prior to the first dose and continuing for the first month in participants with prior exposure who have been off treatment for some time
Adolescent and Children Expansion	Inclusion of adolescents (12-17 yrs of age) from the PK run-in study is ongoing. Plan to enroll children (2-11 yrs of age) directly into study
Program Expansion	Inclusion of participants who have not participated in prior nomlabofusp clinical trials

Larimar Therapeutics

*Due to inclusion of participants who have not participated in prior nomlabofusp clinical studies, this study is now referred to as Open Label Study (previously called the Open Label Extension study)

Potential Path to Bring Nomlabofusp to Patients Worldwide

Open Label Study

Continuing to enroll participants on 50 mg dose

Introduced lyophilized dosage form in Q2 2025

Enrolling patients who have not participated in a prior nomlabofusp trial

Planning to enroll children (2 - 11 yrs of age) directly into the study

Data expected Sept 2025
30-40 participants who received at least one dose of nomlabofusp

Adolescent PK Run-In Study

Dosing of adolescents (12-17 yrs of age) with weight-based dose expected to match PK of adult 50 mg dose completed

Participants eligible to transition into open label study

Ongoing screening and enrollment of patients into the open label study

Adolescent data expected Sept 2025
14 participants, some on placebo

Global Phase 3 Study

Received feedback from FDA and EMA on study protocol

Sites in the U.S., Europe, U.K., Canada, and Australia currently being qualified and initiated. Patient recruitment expected later this year

Study Design*

- Double-blind placebo-controlled study
- Ambulatory patients (n = 100 – 150) weighted to younger patients
- Includes patients 2 - 40 yrs
- 18 months dosing
- Upright stability and mFARS as primary outcome measures

Next Steps

BLA submission to seek accelerated approval planned for Q2 2026

U.S. launch planned for early 2027

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*Study will initiate with subjects 12-40 yrs of age and will change to 2-40 yrs when dose is confirmed in children 2-11 yrs of age

Nomlabofusp Advancing Towards BLA Submission for FA

First potential disease modifying therapy
Designed to systemically address FXN deficiency in FA

FDA clarity on key BLA elements
Skin FXN concentrations as a surrogate endpoint

Safety database of at least 30 for 6-mos, and a subset of at least 10 for 1-year; large majority of exposure should be on the 50 mg dose

Long-term data from the Open Label Study & PK and safety data from adolescent PK-run in study
Expected in September 2025

BLA submission seeking accelerated approval expected Q2 2026
To include data from adults & children

U.S. launch planned for early 2027

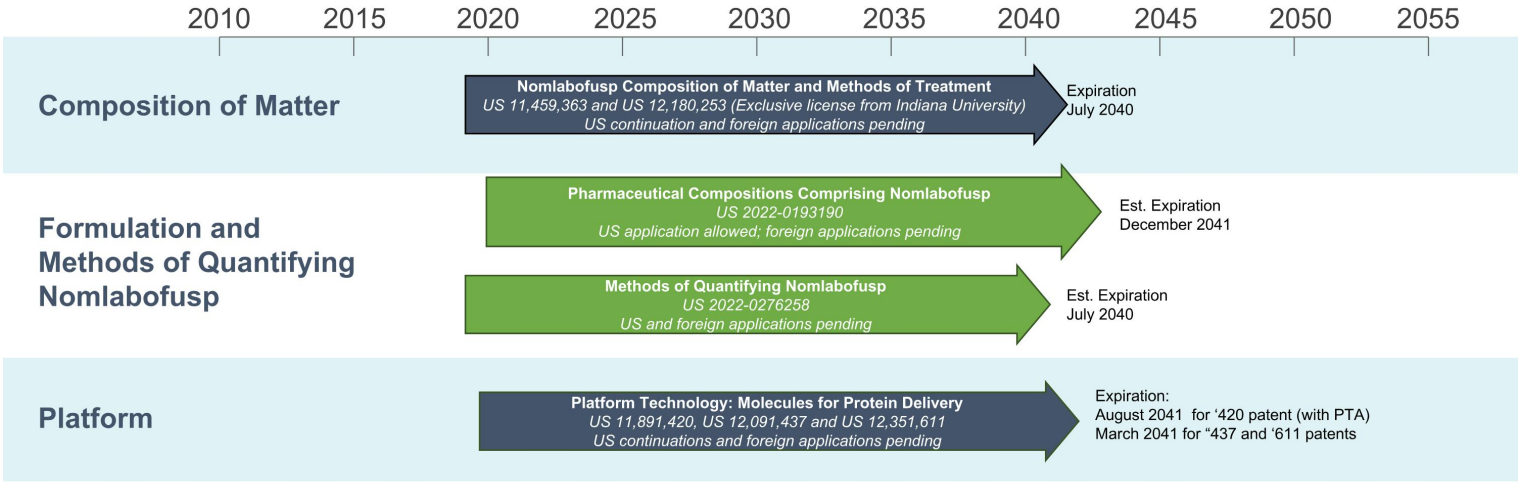
$203.6 M in pro forma cash and investments as of June 30, 2025, with projected cash runway into Q4 2026.
Pro forma cash and investments reflects our $138.5 M of cash and investments as of June 30, 2025 combined with the $65.1 M in net proceeds from our recently completed July 2025 public offering.

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

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**	**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

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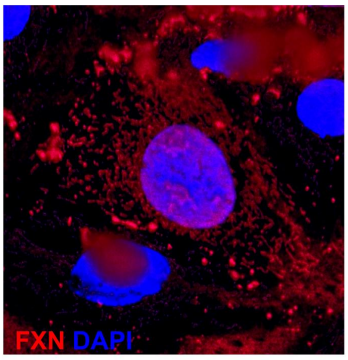
*Competitive landscape focuses on clinical-stage, industry-sponsored programs from public companies

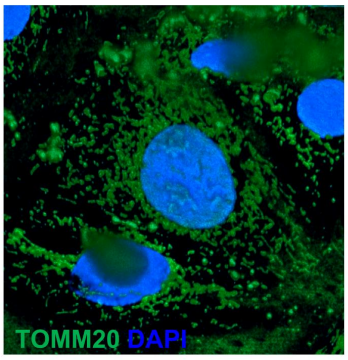
Mitochondrial Localization and Preclinical Data

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

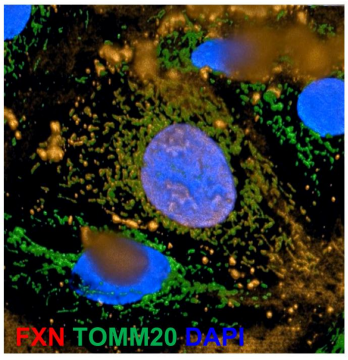
FXN staining



TOMM20 (mitochondria) staining



FXN co-localizes with TOMM20



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

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Nomlabofusp Extends Survival in FXN-deficient KO Mice

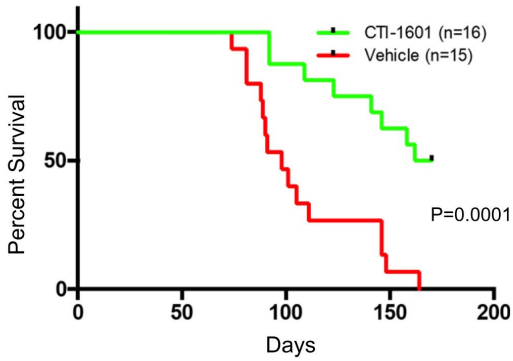
Initial proof-of-concept for FXN replacement therapy in cardiac mouse model of FA

Median survival of MCK-Cre FXN-KO mice

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

Survival beyond vehicle mean (107.5 days)

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



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

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Nomlabofusp Prevents Development of Ataxic Gait in Neurologic KO Mouse Model

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

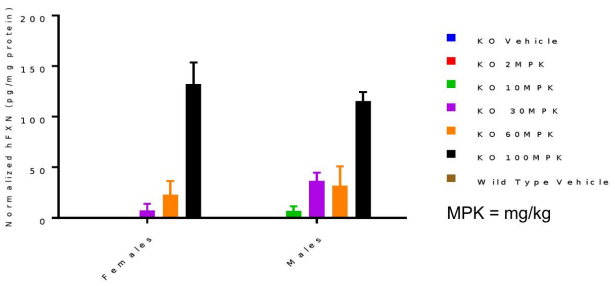
Single dose level: 10 mg/kg nomlabofusp or vehicle given intraperitoneally three times per week

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

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

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

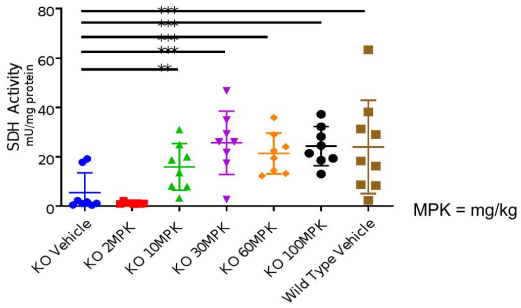
Mitochondrial FXN (Heart)



Mitochondria hFXN concentration increases dose-dependently

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

SDH Activity (Muscle)



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

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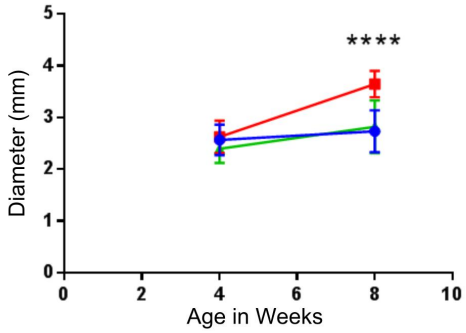
Nomlabofusp Prevents Left Ventricle Dilation in KO Mice

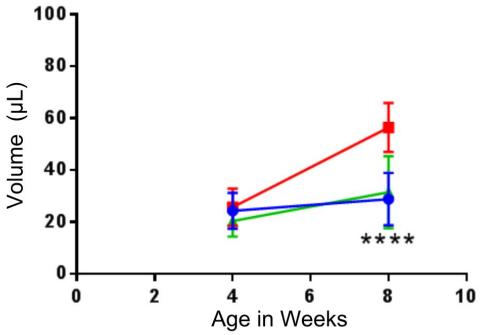
Study Design – Cardiac and skeletal muscle FXN knockout mice (MCK-CRE) were treated at 10 mg/kg every other day at Jackson Laboratories (Bar Harbor, ME). Echocardiograms were performed pre-dose and post dose.

Left Ventricle Internal Diameter (Systole)



Left ventricular (LV) volume increases in systole in untreated mice by 8 weeks (after 4 weeks of dosing with vehicle), but remains similar to wildtype when treated with nomlabofusp (10 mg/kg every other day)

Left Ventricle Volume (Systole)



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

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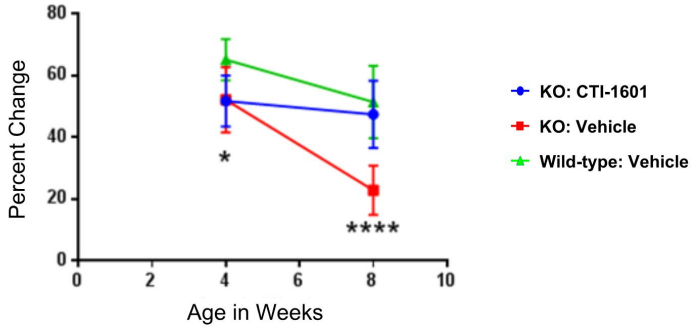
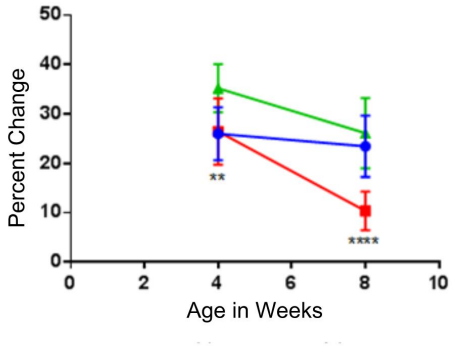
Nomlabofusp Preserves Left Ventricle Function in KO Mice

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

Left Ventricle Ejection Function



Left Ventricle Fractional Shortening



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

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

Larimar Therapeutics

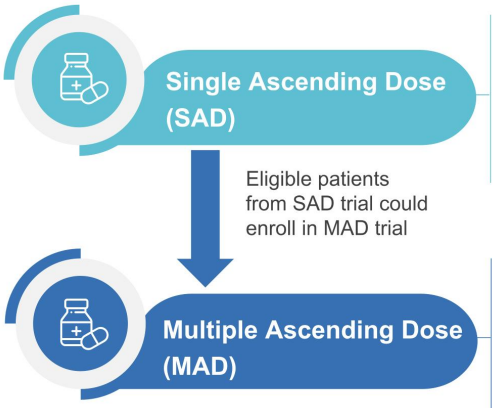
Phase 1 Clinical Data

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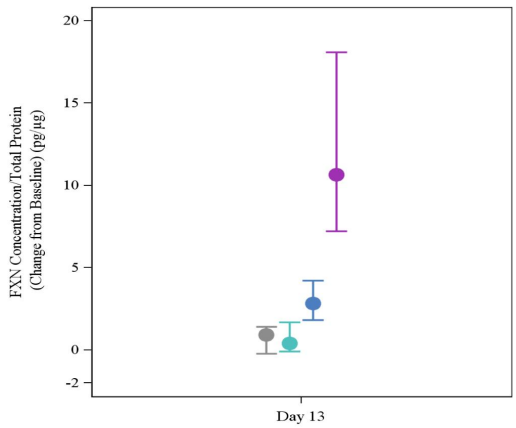
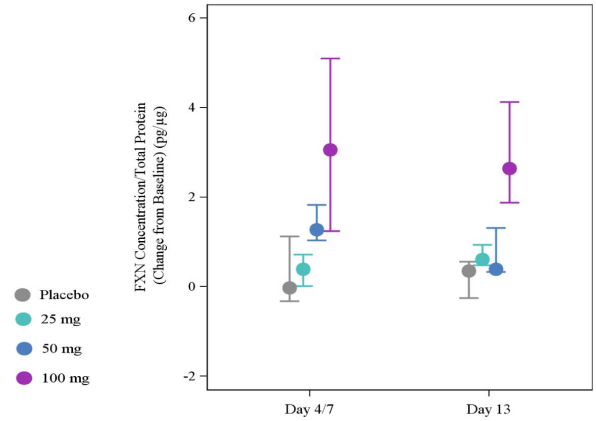
Dose Dependent Increases in FXN Levels Observed in Skin and Buccal Cells in Phase 1

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



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



Placebo: Participants randomized to placebo in each cohort
25 mg: Dosed daily for 4 days, every third day thereafter
50 mg: Dosed daily for 7 days, every other day thereafter
100 mg: Dosed daily for 13 days

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

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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)

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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

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**

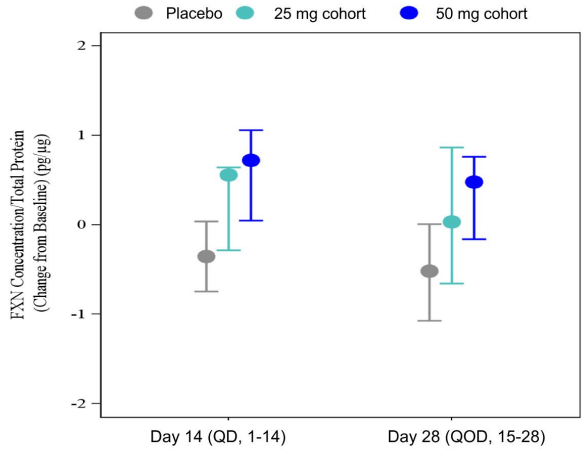
Day 28 Buccal FXN Levels			
Dose	Visit	Absolute Values (pg/µg)	
		Median	Mean
25 mg	Baseline	1.70	1.65
	Day 28	1.73	1.76
	Change from Baseline	**0.03**	**0.11**
50 mg	Baseline	1.76	1.77
	Day 28	2.15	2.15
	Change from Baseline	**0.48**	**0.38**

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

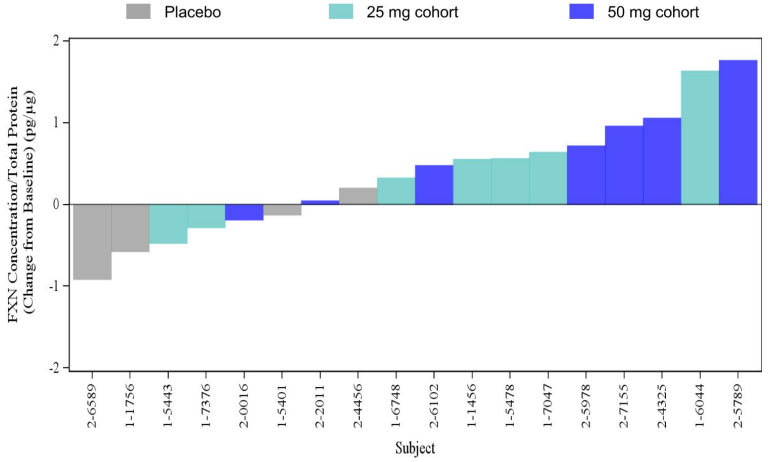
Dose-Dependent Increase in FXN Levels in Buccal Cells

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

Buccal Cells
FXN Levels* Change from Baseline**

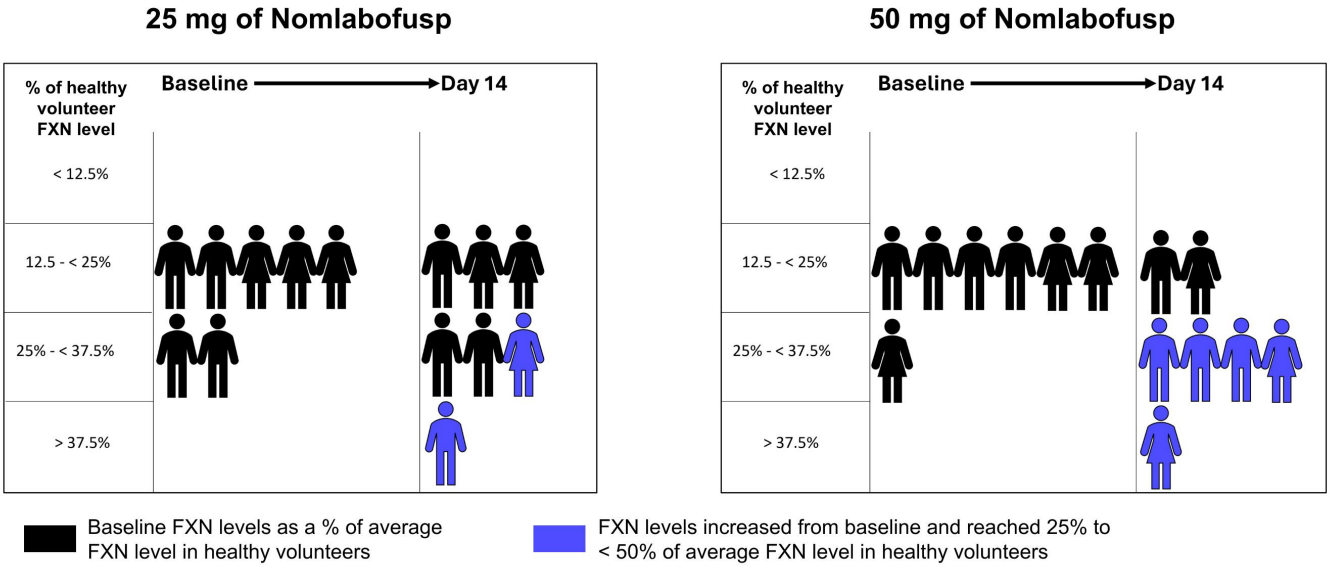


FXN Levels* in Buccal Cells
Change from Baseline at Day 14



*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



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 (GAA_1)	60	550.0	555.8	99	1000
Longer GAA (GAA_2)	60	900.0	890.2	265	1300
Frataxin, % of Control**	57	24.4	23.9	8.7	61.9
mFARS Score	61	52.0	49.5	13.2	74.5
Upright Stability Score	61	32.0	26.9	7.0	35.0
Dominant hand 9-hole peg test	61	71.0	84.8	26.0	229.2
T25-FW Test Score	51	9.9	13.4	4.3	48.5
Left Ventricular Mass (g)	61	163.4	168.0	73.7	398.8
LVEF %	61	63.0	63.5	52	76
Ambulatory Status***					
No	36				
Yes	25				

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

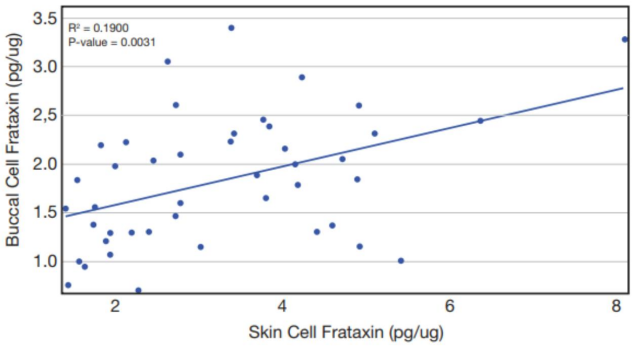
Disease Characteristics by Quartiles Based on Buccal Cell FXN Levels at Baseline

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

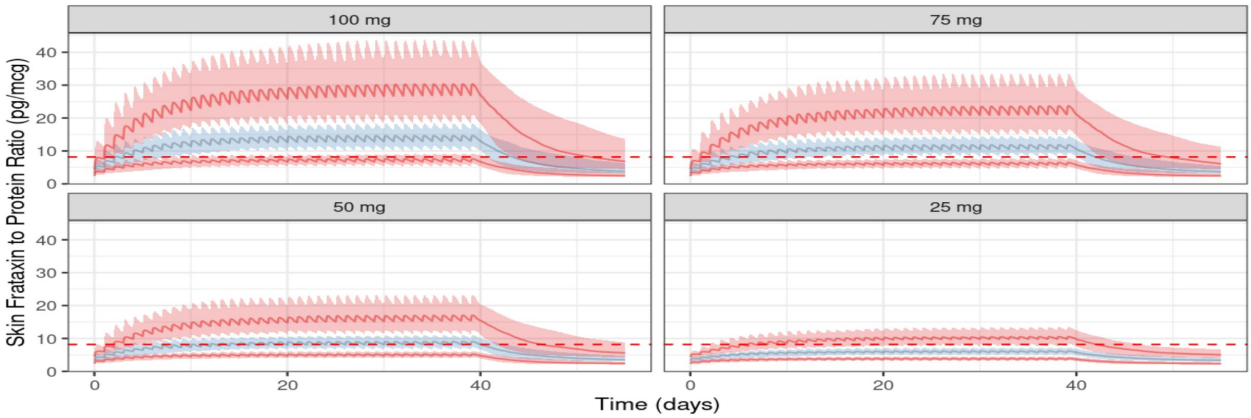
Buccal cell FXN levels correlated with age of onset and inversely correlated with the number of GAA repeats and rate of disease progression

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 ≥50% of Healthy Controls in Most Patients



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

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

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

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

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**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
Open Label Study Information

Observed Increases in Tissue FXN Levels in Open Label Study 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**

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

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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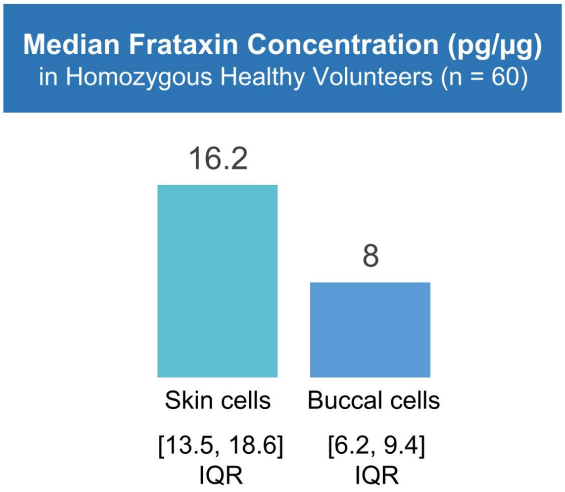
Non-Interventional Study Data

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

Non-interventional study measured FXN in homozygous healthy volunteers



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]

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

FDA START Pilot Program

START Pilot Program Continues to Expedite the Clinical and Regulatory Development of Nomlabofusp

START Pilot Program
Support for Clinical Trials Advancing Rare Disease Therapeutics

1 of 7 novel drugs development programs selected by FDA

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

Designed to accelerate the development of novel therapies for rare diseases

Sponsors selected can benefit from:
- more frequent and rapid ad-hoc FDA interactions
- help facilitating the development of programs to pre-BLA meeting stage
- guidance on generating high-quality and reliable data intended to support a BLA

CDER Selection Based On

Demonstrated development **program readiness**

Potential to address serious and unmet medical need in a **rare neurodegenerative condition**

Alignment of CMC development timelines with clinical development plans

Proposed plan where **enhanced communication can improve efficiency of product development**

FDA: Food and Drug Administration; CDER: Center for Drug Evaluation and Research; CMC: Chemistry, Manufacturing, and Controls

FARA

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

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

FARA provides industry with several key items

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

FARA Friedreich's Ataxia Research Alliance

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