Larimar Therapeutics

CTI-1601 Program Update

May 2023

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 expectations regarding its ability to resolve the partial clinical hold imposed by the FDA related to CTI-1601, Larimar’s ability to develop and commercialize CTI-1601, Larimar’s planned research and development efforts, including the timing of its CTI-1601 clinical development plan and other matters regarding Larimar’s business strategies, ability to raise capital, use of capital, results of operations and financial position, and plans and objectives for future operations.

In some cases, you can identify forward-looking statements by the words “may,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “ongoing” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, the Company’s ability to successfully engage with the FDA and satisfactorily respond to requests from the FDA for further information and data regarding the CTI-1601 clinical trial including the FDA review of data from cohort one from the Phase 2 dose exploration trial and FDA‘s agreement to escalate the dosing in cohort two, the timing and outcomes of Larimar’s interactions with the FDA concerning the partial clinical hold, the success, cost and timing of Larimar’s product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones; that preliminary and top-line clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of CTI-1601 may not be predictive of the results or success of later clinical trials, and assessments; Larimar’s ability and the ability of third-party manufacturers Larimar engages, to optimize and scale CTI-1601’s manufacturing process; Larimar’s ability to obtain regulatory approvals for CTI-1601 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.

Preliminary Top-line Data from 25 mg cohort of Ph 2 Trial

Participants dosed daily for 14 days and then every-other-day until end of treatment (day 28)

Safety and PK	Safety data indicate CTI-1601 was generally well tolerated in the cohort PK data suggest steady state was achieved by day 14
Frataxin (FXN) Levels	Median placebo-adjusted increase from baseline of 3.5 pg/µg in FXN levels in skin with 14 days daily dosing Median placebo-adjusted increase from baseline of 0.9 pg/µg in FXN levels in buccal cells with 14 days daily dosing Data build on proof-of-concept Phase 1 results
Next Steps	A meeting between Larimar and FDA is scheduled to discuss the information needed to gain clearance to initiate a 50 mg cohort in the Phase 2 trial Update on CTI-1601 program expected in Q3 2023

Larimar Therapeutics

Investment Highlights

Clinical-stage biotechnology company with a novel protein replacement therapy platform
Focused on addressing unmet needs in Friedreich's ataxia (FA) and potentially other complex rare diseases based on a platform technology backed by a strong intellectual property portfolio

Lead candidate: CTI-1601, a recombinant fusion protein designed to deliver frataxin to mitochondria
Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations for FA

Double-blind, placebo-controlled Phase 1 proof-of-concept trials in individuals with FA complete
Data show that CTI-1601 was generally well tolerated when dosed daily for up to 13 days & dose dependent increases in frataxin (FXN) levels from baseline compared to placebo in all evaluated tissues with daily dosing

Placebo-controlled, Phase 2, 4-week dose exploration study in individuals with FA
25 mg cohort data show that CTI-1601 was generally well tolerated & increases in FXN from baseline compared to placebo in skin & buccal cells. Per partial clinical hold, further clinical studies contingent on FDA review of data

Strong financial foundation with projected cash runway into 2H 2024
March 31, 2023 cash - $111.5M
High-quality institutional investor base includes founding investor Deerfield Management

Friedreich's Ataxia (FA)

Rare and Progressive Disease

Caused by genetic defect resulting in low levels of frataxin

- Patients with FA only produce ~20-40% of normal frataxin levels depending on the tissue, sampling technique, and assay considered[1]
- Affects ~20,000 patients globally, with ~5,000 patients in the U.S. and majority of the remaining patients in the EU

Approximately 70% of patients present before age 14

- Initial symptoms may include unsteady posture, frequent falling and progressive difficulty in walking. By the time symptoms occur, heart damage may have already occurred. Progressive disease: symptoms worsen and patients are eventually confined to a wheelchair with speech becoming hesitant and jerky (often referred to as "scanning of speech")

Life expectancy of 30-50 years

- Early death usually caused by heart disease

No available therapies increase frataxin levels

- Only treatment approved for FA does not address frataxin deficiency

LRMR continues to have a strong relationship with Friedreich's Ataxia Research Alliance

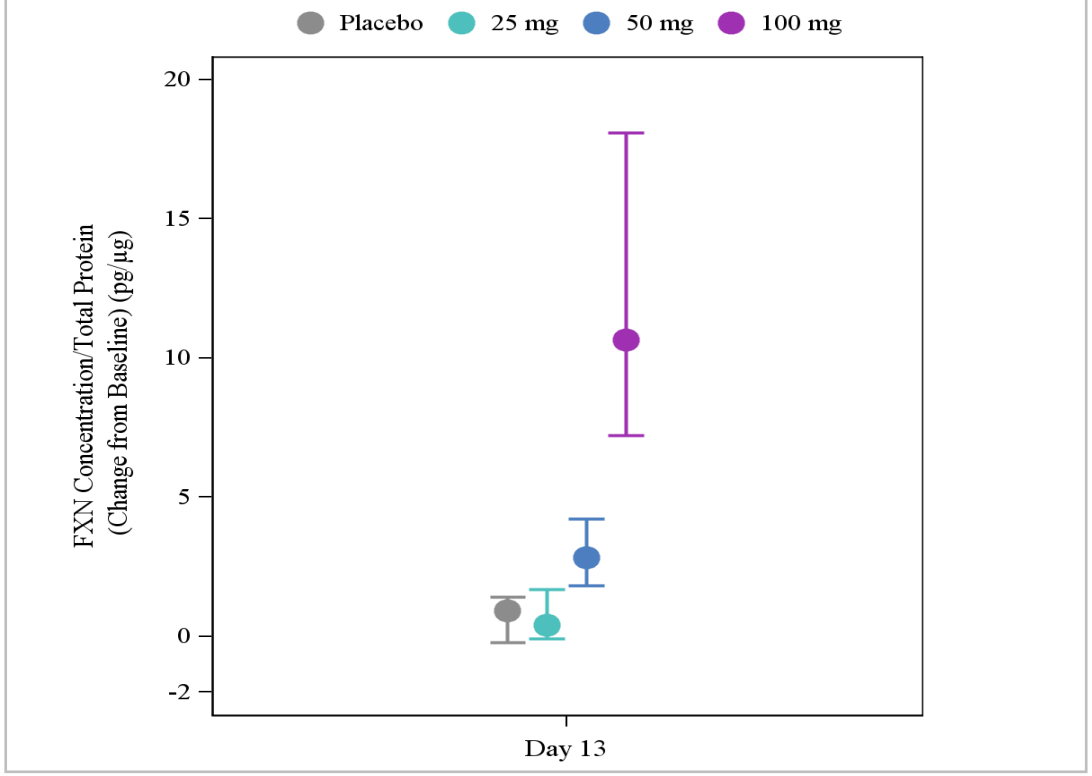
- Dedicated FA patient advocacy group focused on treatments for FA

Larimar Therapeutics

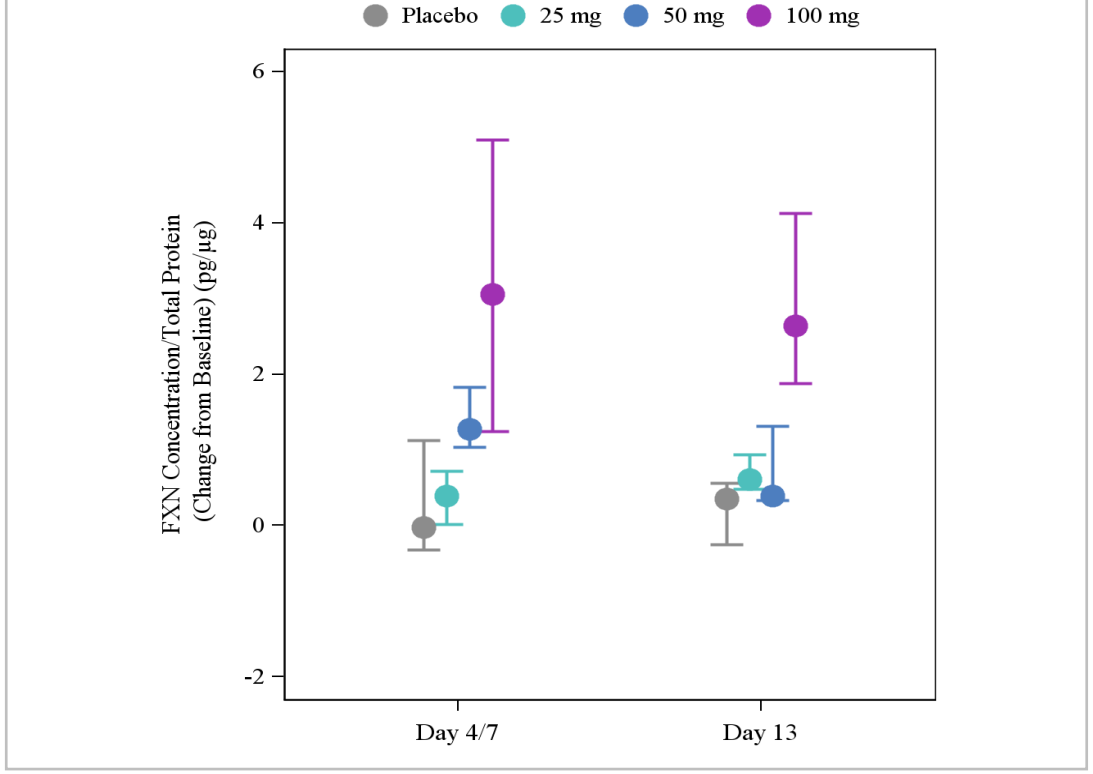
1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245

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

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



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

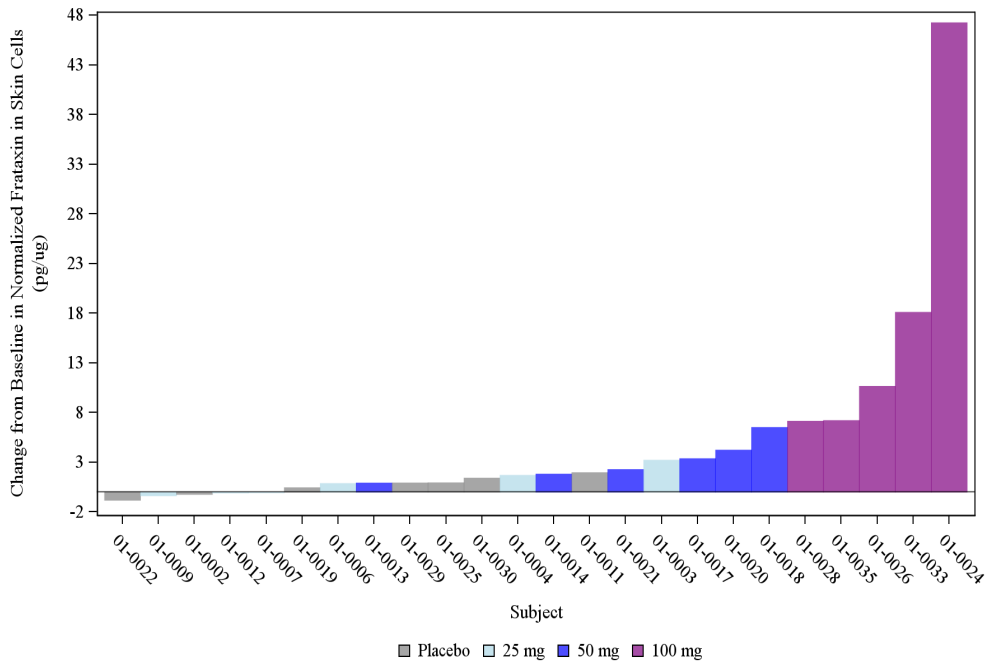
50 mg: Dosed daily for 7 days, every other day thereafter

100 mg: Dosed daily for 13 days

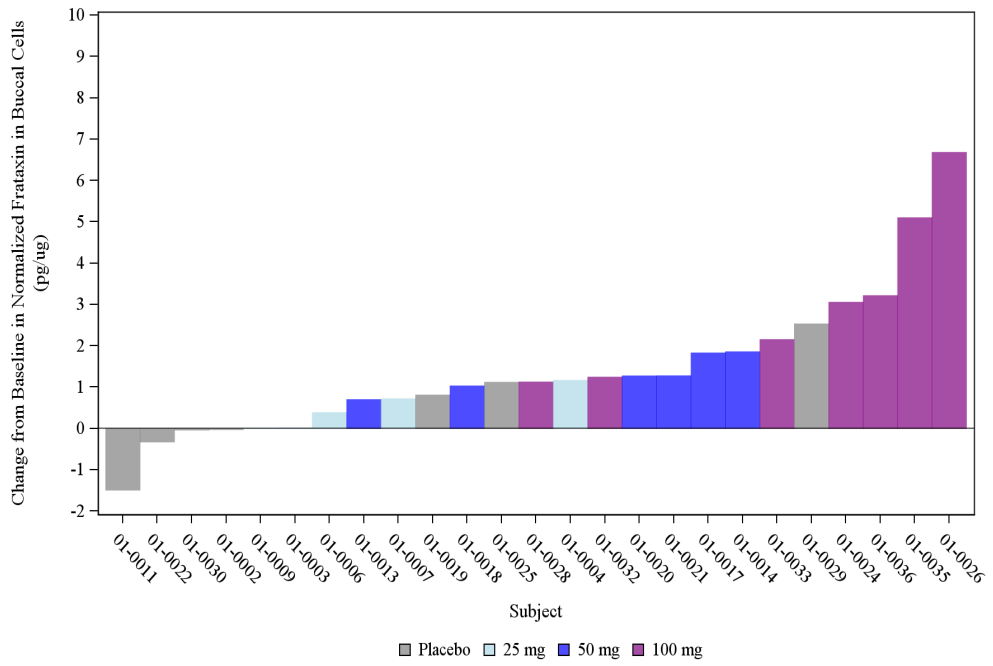
*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25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

Increases in FXN Correlated with Increasing CTI-1601 Dose in Phase 1 Trial

Skin Biopsies Day 13 FXN* Change from Baseline#



Buccal Cells Day 4/7 FXN* 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; #For 100 mg group, two participants did not have sample at Day 13. ◊For 25 mg group, one participant did not have sample at Day 4. ◊For 50 mg group, day 7 buccal cells were not collected from one participant who discontinued treatment and one participant did not have sample.

Ongoing Phase 2, Four-week Dose Exploration Study

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

Treatment Schedule

28-day Treatment Period

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

= Administration of CTI-1601 or placebo

= No Administration

Study Details

Population	Ambulatory and non-ambulatory Friedreich's ataxia patients ≥18 years of age. CTI-1601 treatment naïve or participated (if eligible) in a previous Larimar study.
Dose	**Cohort 1:** 25 mg (dosing complete) **Cohort 2:** Dose escalation contingent on a review of Cohort 1 data by FDA and IDMC.
Key Endpoints	Frataxin levels in peripheral tissue, PK, PD, safety and tolerability. PD endpoints include lipid profiles and gene expression data.
Number of Patients	**Cohort 1:** Enrolled 13 participants randomized 2:1 to receive CTI-1601 (n=9) or placebo (n=4). **Planned Cohort 2:** Designed to enroll ~12-15 participants randomized 2:1 to receive CTI-1601 or placebo
Timing	Expect to provide update on next steps in Q3 2023.

Larimar Therapeutics

IDMC: Independent data monitoring committee

Demographics

Demographics similar between Phase 1 and Phase 2 trials of CTI-1601

Parameter, n (%)	Placebo (N=4)	CTI-1601 25 mg (N=9)	Overall (N=13)
Mean Age (SD) (Years)	34.0 (9.20)	37.8 (14.93)	36.6 (13.16)
Male	2 (50.0%)	5 (55.6%)	7 (53.8%)
White	4 (100.0%)	8 (88.9%)	12 (92.3%)
Other	0	1 (11.1%)	1 (7.7%)
Not Hispanic or Latino	3 (75.0%)	8 (88.9%)	11 (84.6%)
Hispanic or Latino	1 (25.0%)	1 (11.1%)	2 (15.4%)
Mean BMI (SD) (kg/m^2)	23.66 (3.235)	25.26 (6.262)	24.77 (5.417%)
Previously participated in a CTI-1601 trial	1 (25.0%)	4 (44.4%)	5 (38.5%)

Larimar Therapeutics

Disease Characteristics

Parameter	Statistic	Placebo (N=4)	CTI-1601 (n=9)	Overall (n=13)
Age at Symptom Onset (years)				
	n	4	8	12
	Mean (SD)	14.5 (4.93)	13.0 (10.47)	13.5 (8.77)
	Median	14.5	10.0	11.0
	Q1, Q3	11, 19	8, 13	9, 15
	Min, Max	9, 20	5, 38	5, 38
Age at Diagnosis (years)				
	n	4	9	13
	Mean (SD)	17.5 (5.57)	18.6 (11.20)	18.2 (9.58)
	Median	16.5	16.0	16.0
	Q1, Q3	14, 22	14, 20	14, 20
	Min, Max	12, 25	5, 42	5, 42
Time Since Diagnosis (years)				
	n	4	9	13
	Mean (SD)	16.08 (5.965)	18.49 (11.523)	17.75 (9.938)
	Median	13.42	14.32	13.50
	Q1, Q3	12.9, 19.3	12.8, 21.6	12.8, 21.6
	Min, Max	12.5, 25.0	5.4, 45.0	5.4, 45.0

Larimar Therapeutics

CTI-1601 appeared to be generally well tolerated in Phase 2 trial's 25 mg cohort

Summary of Phase 2 trial safety data (25 mg cohort):

25 mg CTI-1601 or placebo were administered subcutaneously daily for 14 days and then every other day until day 28. 13 participants were dosed in the trial (9 active, 4 placebo). Of the 9 CTI-1601-treated participants, 8 completed the trial with 1 withdrawing due to an allergic reaction to study drug, which resolved with standard treatment

- Phase 2 and Phase 1 data consistent. 37 different adults with FA have been dosed with CTI-1601; data indicate CTI-1601 is generally well tolerated
- No serious adverse events. No important medical events. 1 severe adverse event (allergic reaction that resolved with standard treatment as referenced above).
- The most common adverse events were mild and moderate injection site reactions (at least one injection site reaction was seen in 50% of placebo participants and in 100% of CTI-1601 participants)

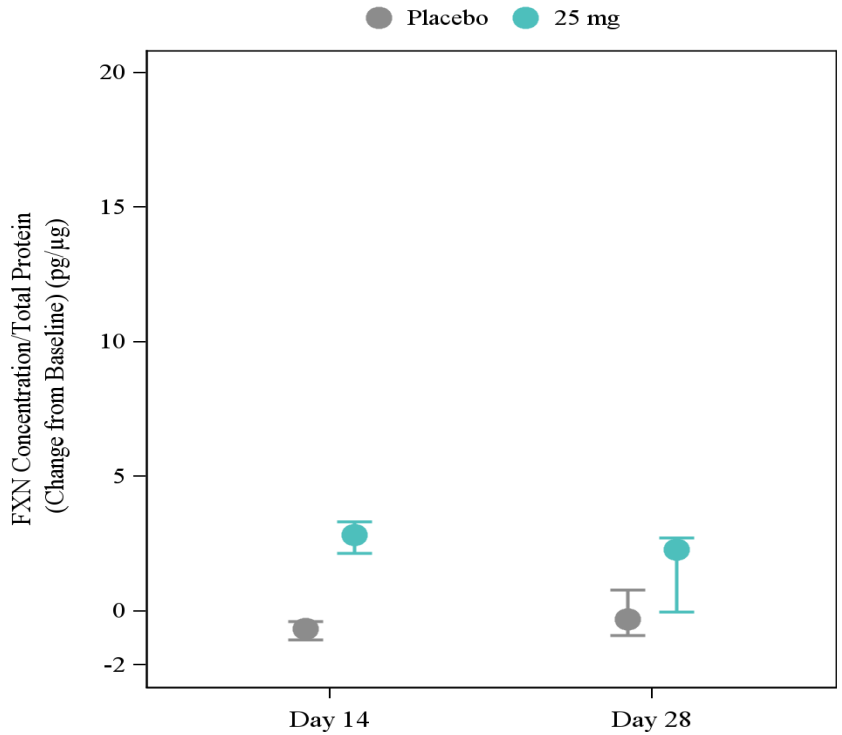
Pharmacokinetic Data

Suggest steady state achieved by day 14

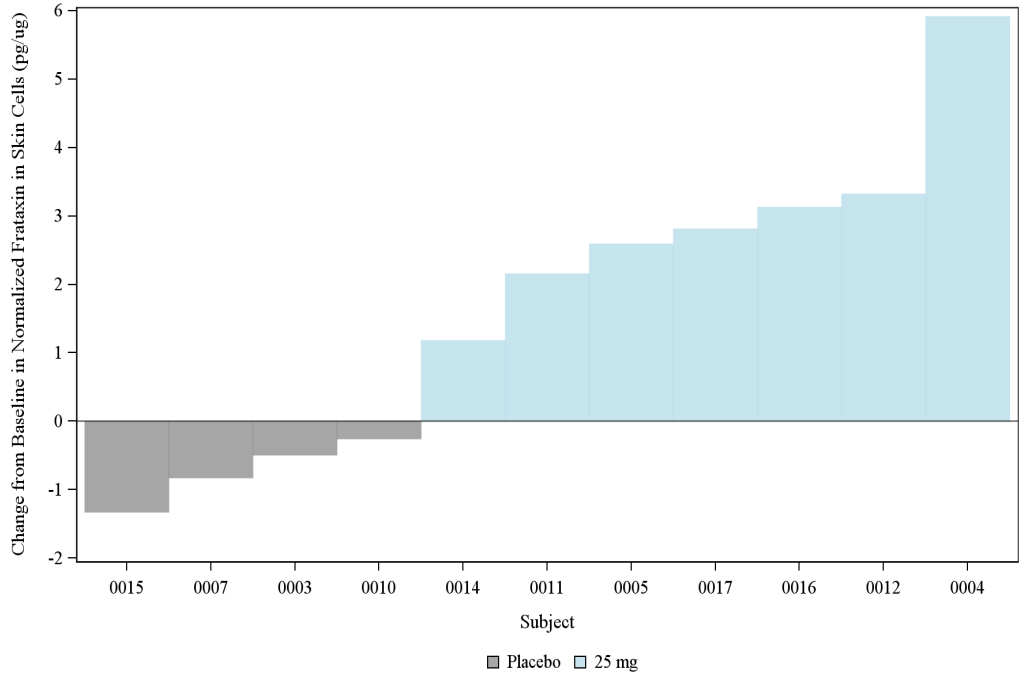
Increases in FXN Levels Observed in Skin Biopsies

Median placebo-adjusted increase from baseline of 3.5 pg/µg in FXN levels in skin with 14 days daily dosing

FXN* Change from Baseline (Skin Biopsies)



Day 14 FXN* Change from Baseline (Skin Biopsies)#

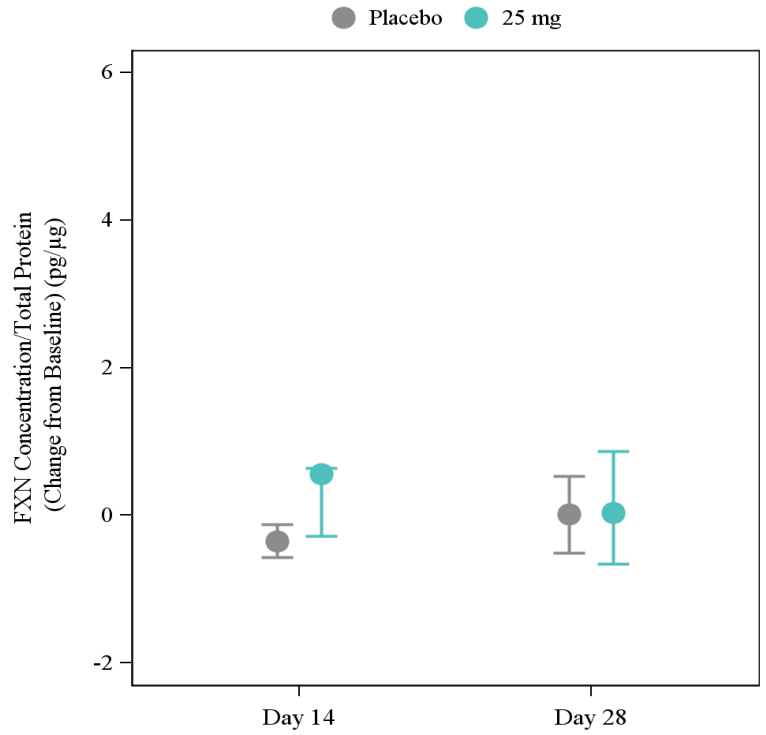


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data for left graph represent median and 25th and 75th percentiles. #: Subject 0006 (treated with CTI-1601) discontinued from study at Day 14 (Skin was not collected since post dose); Subject 0002 (treated with CTI-1601) had a FXN concentration value <lower limit of quantitation (LLOQ) at Day 14

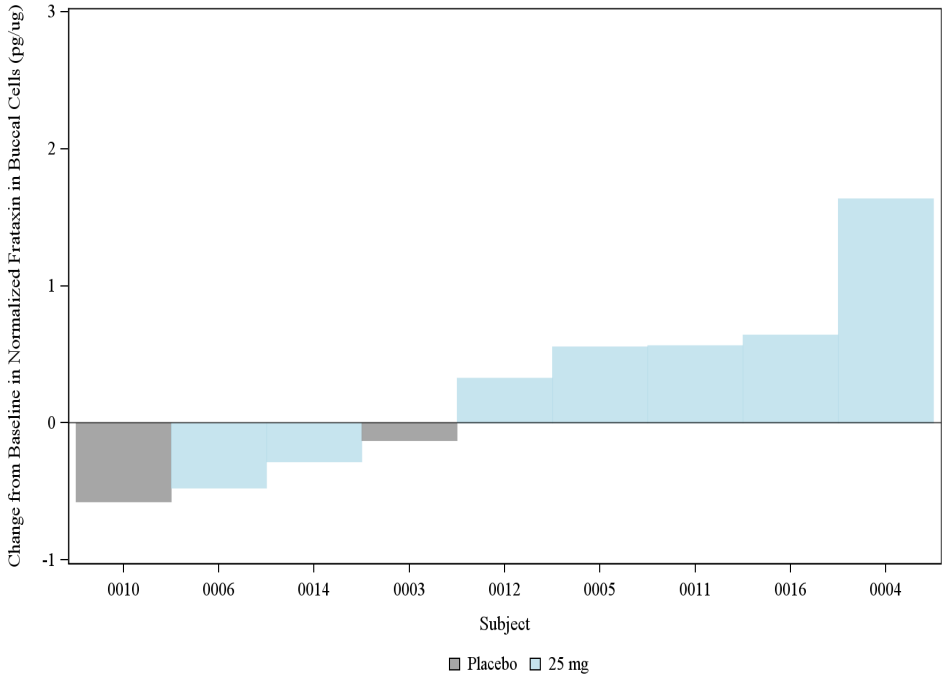
Increases in FXN Levels Observed in Buccal Cells

Median placebo-adjusted increase from baseline of 0.9 pg/µg in FXN levels in buccal cells with 14 days daily dosing

FXN* Change from Baseline (Buccal Cells)



Day 14 FXN* Change from Baseline (Buccal Cells)#



*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data for left graph represent median and 25th and 75th percentiles. Subject 0006 (treated with CTI-1601) discontinued from study at Day 14 (Skin was not collected since post dose); Subject 0002 (treated with CTI-1601) had a baseline and day 14 value < lower limit of quantitation (LLOQ); Subject 0007 (treated with placebo) had a FXN concentration value <LLOQ at baseline; Subject 0015 (treated with placebo) had a FXN concentration value <LLOQ at Day 14.

CTI-1601 Clinical Development Plan

Update on next steps expected in Q3 2023

Planned Trials Include:

Phase 2 four-week dose exploration study. Dosing in Cohort 1 is complete. Meeting with FDA scheduled for Q2 2023.

Jive open-label extension trial for eligible patients who participated in SAD, MAD, and/or four-week dose exploration studies.

MAD trial in patients 2 to 17 years of age. Participants eligible to screen for Jive open-label extension trial.

Global double-blind placebo-controlled pivotal trial.

Larimar Therapeutics

CTI-1601: In Development as the First Therapeutic Intended to Increase FXN

Phase 1

Generally well tolerated; Dose-dependent increases in FXN levels observed in all evaluated tissues with 7 days of daily dosing at 50 mg and 100 mg

Phase 2

Generally well tolerated

Median placebo-adjusted increase from baseline of 3.5 pg/µg in FXN levels in skin with 14 days daily dosing

Median placebo-adjusted increase from baseline of 0.9 pg/µg in FXN levels in buccal cells with 14 days daily dosing

Regulatory

Meeting with FDA scheduled to discuss the information needed to gain clearance to initiate a 50 mg cohort in Phase 2 trial; Expect to provide an update in Q3 2023

Larimar Therapeutics