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): June 01, 2023

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 8.01 Other Events.

On June 1, 2023, Larimar Therapeutics, Inc. (the "Company") posted on its website an updated slide presentation, which is attached as Exhibit 99.1 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	Larimar Therapeutics, Inc. Corporate Presentation, dated June 1, 2023*
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith

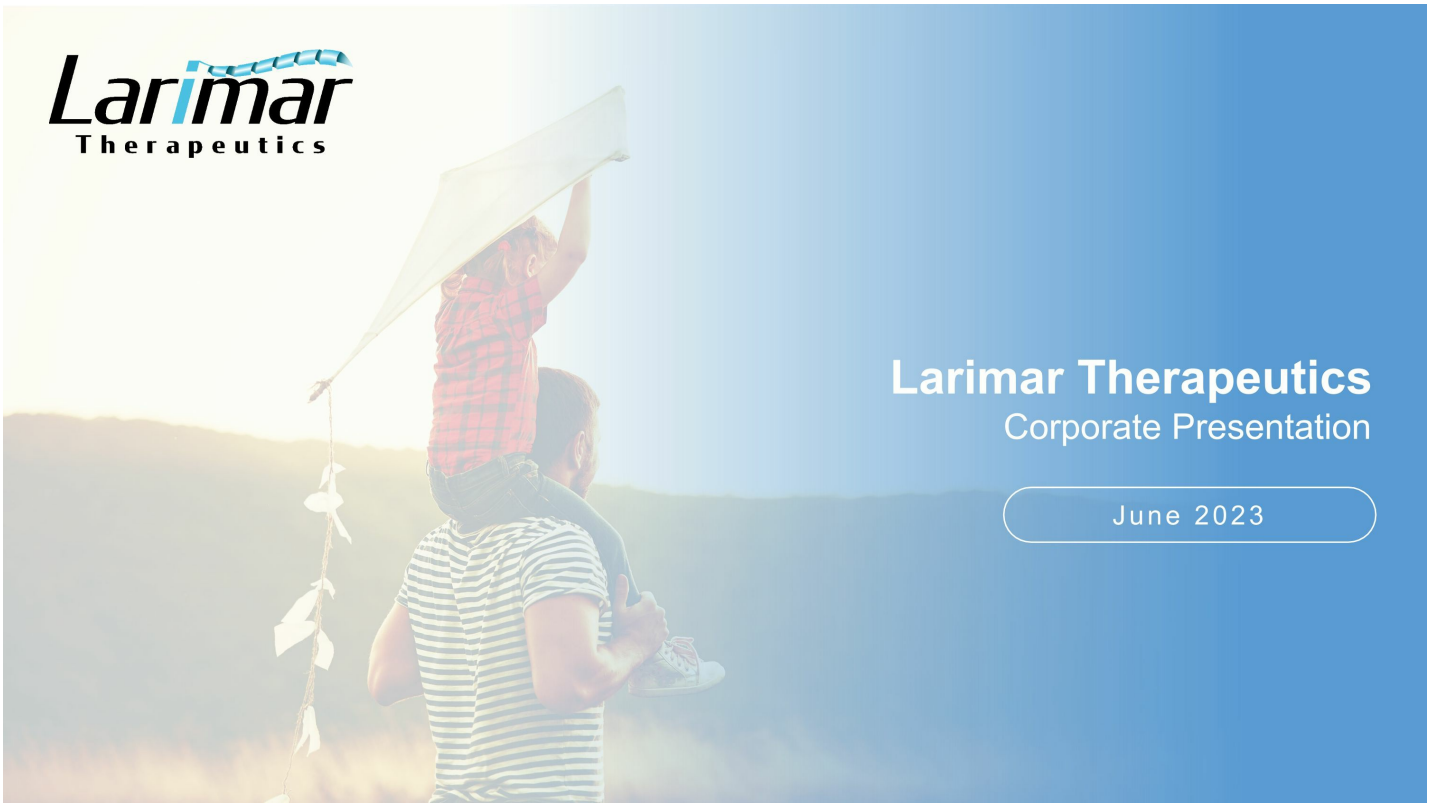
SIGNATURES

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: June 1, 2023

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



Larimar Therapeutics

Corporate Presentation

June 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.

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

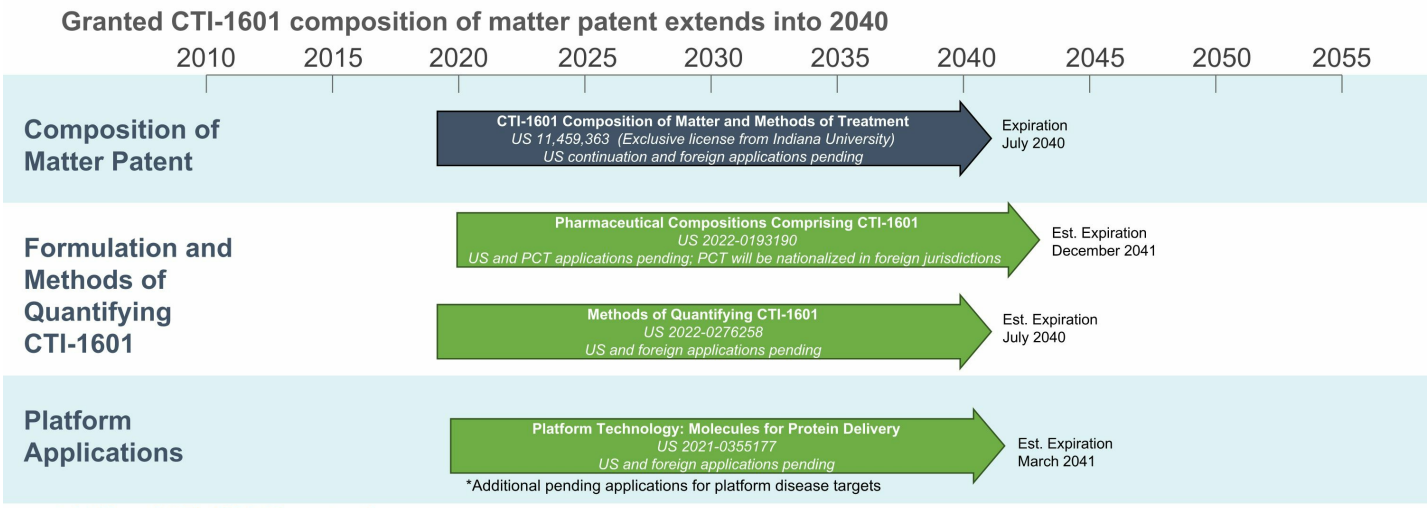
Lead candidate: CTI-1601, a recombinant fusion protein designed to deliver frataxin to mitochondria
Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations for FA

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

Placebo-controlled, Phase 2, 4-week dose exploration study in individuals with FA
25 mg cohort data show CTI-1601 was generally well tolerated; Increases in FXN from baseline compared to placebo in skin & buccal cells were observed; Given partial clinical hold, further clinical studies are 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

Platform Technology is Supported by a Strong IP Portfolio



Additional CTI-1601 IP protection

- CTI-1601 pending applications cover key biomarkers and analytical tools
- CTI-1601 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)

Larimar Therapeutics

Granted Pending

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

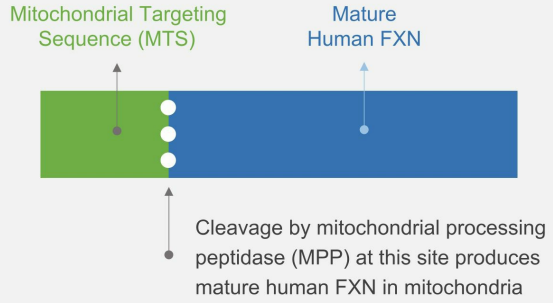
Larimar Therapeutics

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

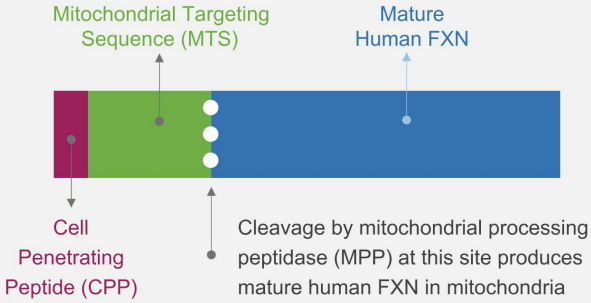
CTI-1601 is Designed to Deliver Additional Frataxin (FXN)

CTI-1601 maintains the cleavage site between the MTS and mature human FXN

STRUCTURE OF ENDOGENOUS FXN



STRUCTURE OF CTI-1601



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

Completed Phase 1 Multiple Ascending Dose Study

Treatment Schedules for Each Cohort

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 CTI-1601 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 CTI-1601 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 CTI-1601 or placebo

= No Administration

FXN Level Sampling Days Presented for Each Cohort

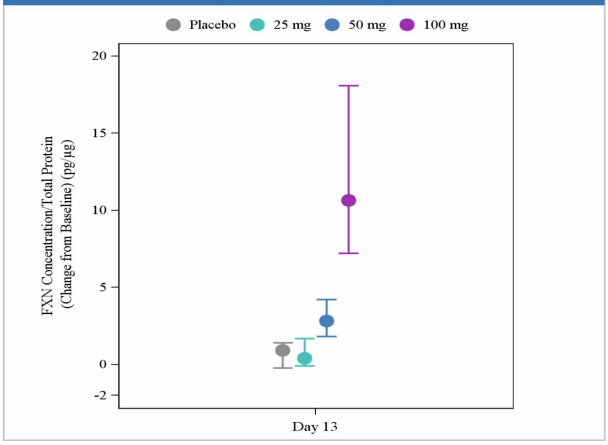
Cohort 1 Sampling Days	
Buccal Cells	Baseline, Day 4, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 4, Day 13

Cohort 2 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

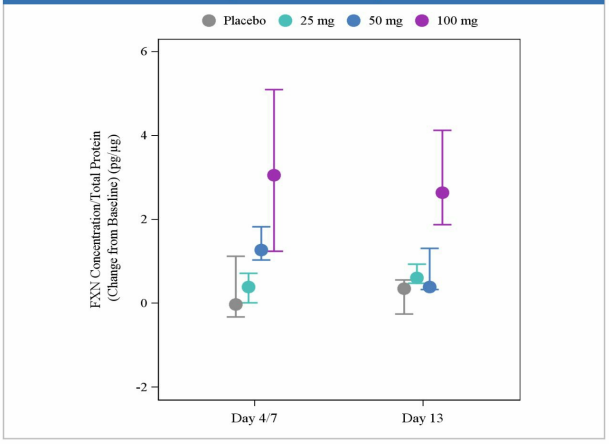
Cohort 3 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

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)

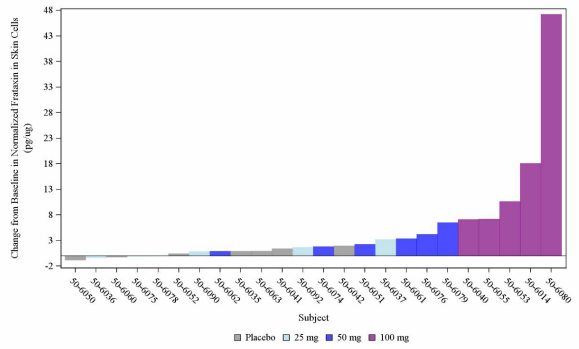


Placebo: Participants randomized to placebo in each cohort
25 mg: Dosed daily for 4 days, every third day thereafter
50 mg: Dosed daily for 7 days, every other day thereafter
100 mg: Dosed daily for 13 days

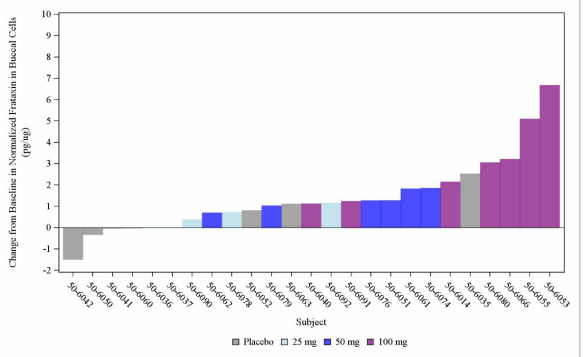
*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25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

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.

IDMC: Independent data monitoring committee

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

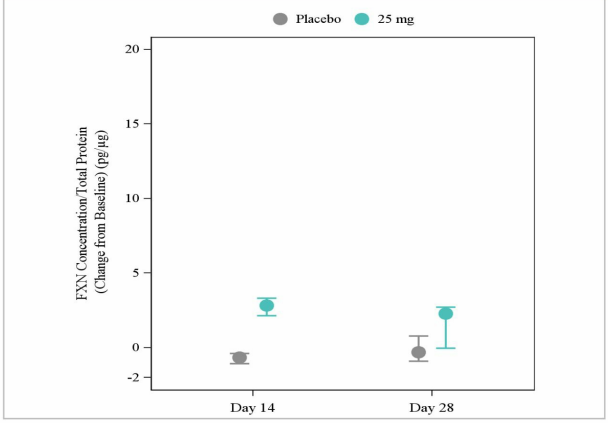
Participants dosed daily for 14 days and then every-other-day until end of treatment (day 28)

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

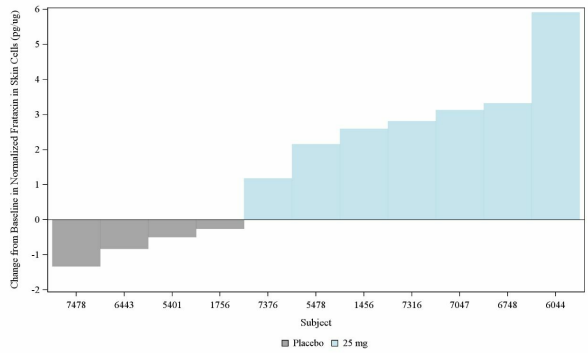
Increases in FXN Levels Observed in Skin Biopsies

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

FXN* Change from Baseline (Skin Biopsies)



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

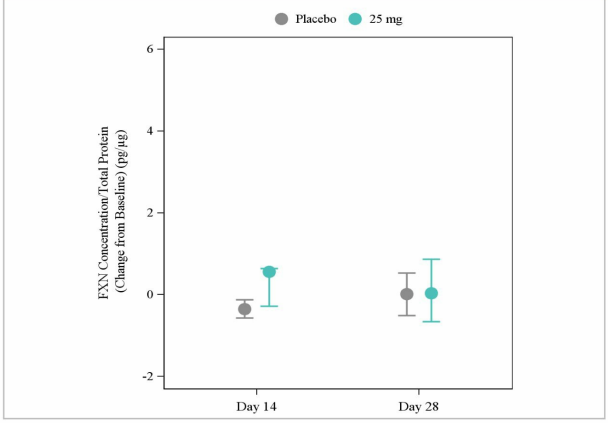


*FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations are normalized to total cellular protein content in each sample; Data for left graph represent median and 25th and 75th percentiles.
#: One participant treated with CTI-1601 discontinued from study at Day 14 (Day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

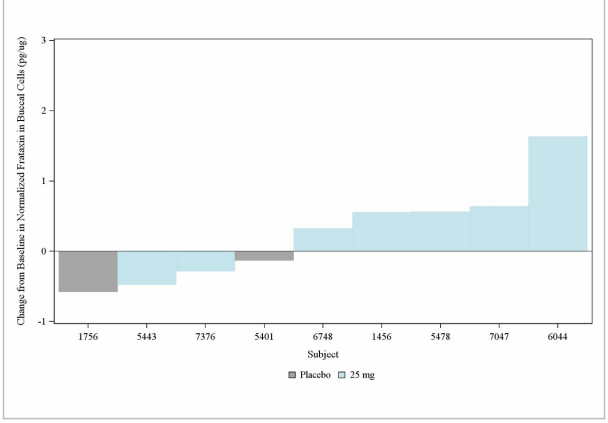
Increases in FXN Levels Observed in Buccal Cells

Median placebo-adjusted increase from baseline of 0.9 pg/µg in 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.

#:One participant treated with CTI-1601 had a baseline value < lower limit of quantitation (LLOQ) and another participant treated with CTI-1601 had a baseline and Day 14 value < lower limit of quantitation (LLOQ); One participant treated with placebo had a FXN concentration value <LLOQ at baseline and another participant treated with placebo had a FXN concentration value <LLOQ at Day 14.

Larimar Therapeutics

Clinical Data Indicate CTI-1601 is Generally Well Tolerated

CTI-1601 administered to 37 different adults with FA in multiple studies

35 of 37 clinical trial participants dosed with CTI-1601 completed their respective study
One Phase 2 participant withdrew due to an allergic reaction that resolved with standard treatment
One Phase 1 participant in the 50 mg cohort withdrew due to mild-to-moderate nausea and vomiting

No serious adverse events or important medical events in any CTI-1601 clinical trial
One severe adverse event (allergic reaction that resolved with standard treatment referenced above)

Most common adverse events (AEs) were mild and moderate injection site reactions (ISRs)
No study discontinuations due to ISRs and all resolved without intervention
ISRs in 100% of CTI-1601-treated participants and 40% of placebo-treated participants across all trials

In MAD study, except for ISRs, number & severity of AEs did not increase with increasing dose

Larimar Therapeutics

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 to discuss information needed to initiate a 50 mg cohort.

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.

Due to the FDA partial clinical hold, the conduct of additional cohorts in the Phase 2 study, as well as other studies, will be subject to FDA review of Phase 2 cohort 1 data and possibly other data.

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

Phase 1

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

Phase 2 (25 mg cohort)

- 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

- 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

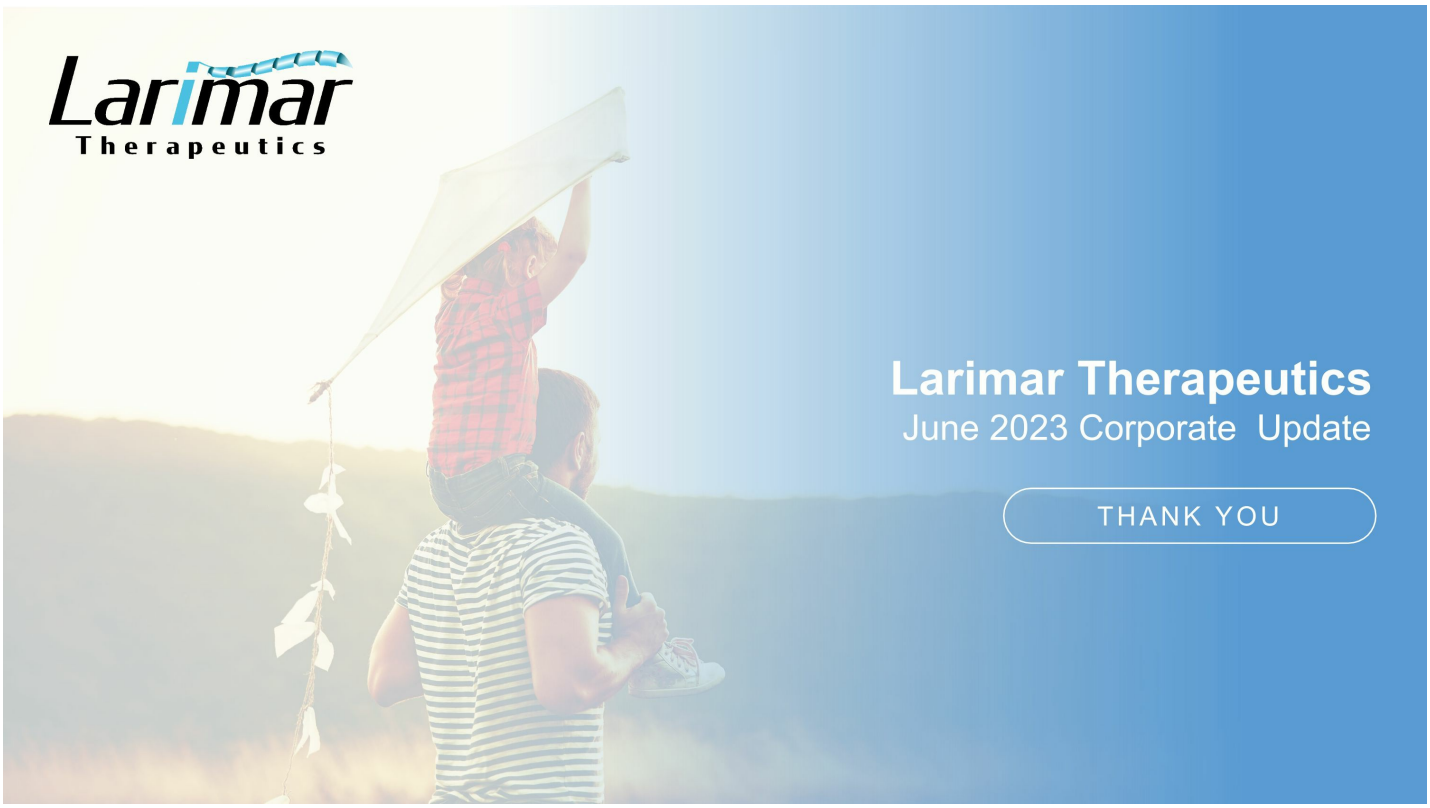
Clinical-stage biotechnology company with a novel protein replacement therapy platform
Focused on addressing unmet needs in Friedreich's ataxia (FA) and potentially other complex rare diseases based on a platform technology backed by a strong intellectual property portfolio

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 CTI-1601 was generally well tolerated when dosed daily for up to 13 days; Dose dependent increases were observed in frataxin (FXN) levels from baseline compared to placebo in all evaluated tissues

Placebo-controlled, Phase 2, 4-week dose exploration study in individuals with FA
25 mg cohort data show CTI-1601 was generally well tolerated; Increases in FXN from baseline compared to placebo in skin & buccal cells were observed; Given partial clinical hold, further clinical studies are 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


Larimar
Therapeutics
Larimar Therapeutics
June 2023 Corporate Update
THANK YOU

Larimar Therapeutics

Appendix

Leadership Team

Carole Ben-Maimon, MD
Chief Executive Officer

Impax teva

Michael Celano
Chief Financial Officer

OraSure Technologies Kensey Nash

Gopi Shankar, PhD, MBA, FAAPS
Chief Development Officer

janssen

Nancy Ruiz, MD, FACP, FIDSA
Chief Medical Officer

Schering-Plough MERCK

Jennifer Johansson, JD
VP, Regulatory Affairs & Counsel

Andrx SQUIRE PATTON BOGGS

Mohamed Hamdani
VP, Biometrics

Apellis Shire

Keith E. Lynch, Jr.
VP, Technical Operations

MERCK Oncobiologics

John Berman, CPA
VP, Finance & Operations

teva Cell Pathways, Inc.

Noreen Scherer
VP, Clinical Operations

Shire Wyeth Pharmaceuticals

Francis Michael Conway
VP, Controller

TELA BIO stryker

Larimar Therapeutics

Scientific Advisory Board

Russell Clayton, DO (Chairman)

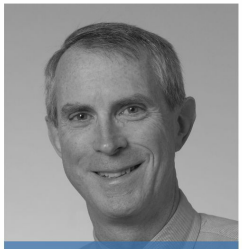
Former Chief Medical Officer at Alcresta Therapeutics, a medical device company

Former Senior Vice President of Research and Development at Discovery Labs, a pharmaceutical and medical device company

Giovanni Manfredi, MD, PhD

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

Professor of Neuroscience at Weill Cornell Medicine.



Mark Payne, MD

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

Professor of Pediatrics at Indiana University School of Medicine

Marni J. Falk, MD

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

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

Jill Ostrem, MD

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

Carlin and Ellen Wiegner Endowed Professor of Neurology

Strong Relationship with FARA

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

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

FARA provides industry with several key items

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

FARA Friedreich's Ataxia Research Alliance

Additional Phase 1 Data

Phase 1 Top-line Data Demonstrated POC for CTI-1601 in FA

Safety	CTI-1601 appears to be generally well tolerated at doses up to 100 mg administered daily for 13 days
Pharmacodynamics	Daily dosing of CTI-1601 resulted in dose-dependent increases in FXN levels from baseline compared to placebo controls in all evaluated tissues
Pharmacokinetics	Pharmacokinetic analyses support evaluating once-daily and every-other-day dosing regimens for CTI-1601
Conclusion	Daily subcutaneous (SC) administration of 50 mg and 100 mg doses of CTI-1601 resulted in FXN levels in buccal cells that are at, or in excess of, those we would expect to see in asymptomatic heterozygous carriers (who have FXN levels of ~50% of homozygous healthy people)[1]

Larimar Therapeutics

POC: Proof-of-concept

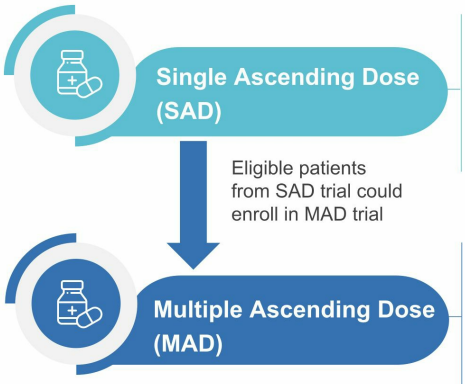
1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245

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

Larimar Therapeutics

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

Data Compare Favorably to FXN Levels Expected in Heterozygous Carriers

Achieved median FXN levels that were >60% of the median FXN levels observed in healthy controls

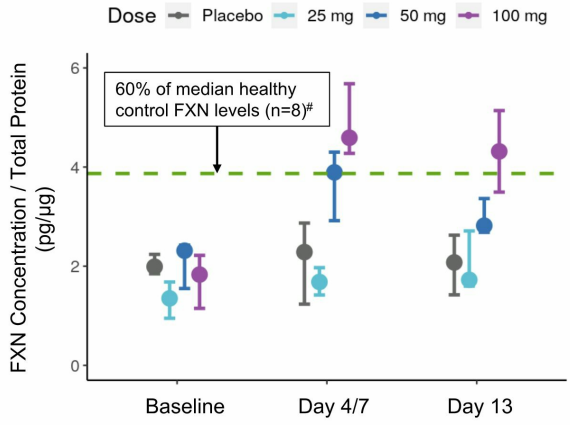
Benchmarking Clinical Relevance

- FXN levels in buccal cells and blood have been shown to correlate with neurological function in FA patients[1]
- Patients with FA only produce ~20-40% of normal frataxin levels depending on the tissue considered[2]
- Heterozygous carriers who show no signs of disease have FXN levels of ~50% of unaffected healthy persons[2]

Comparison to Healthy Controls

- FXN levels were measured in buccal cells from 8 healthy controls using the same assay and sampling technique employed in the Phase 1 MAD trial
- With daily administration, patients in Cohorts 2 & 3 of the Phase 1 MAD trial achieved median buccal cell FXN levels that were >60% of the median FXN levels observed in healthy controls

FXN* Levels By Dose Group (Buccal Cells)



*FXN levels measured via detection of peptide derived from mature FXN; #Data on file; 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. 1. Lazaropoulos et al. Ann Clin Transl Neurol. 2015 Aug; 2(8): 831–842; 2. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245.

Repeated subcutaneous injections of CTI-1601 were generally well tolerated in Phase 1 MAD trial

Summary of MAD trial safety data:

Repeated doses (25 mg, 50 mg, and 100 mg) of CTI-1601 or placebo were administered subcutaneously.

- No serious adverse events (SAEs), important medical events, or treatment-related severe adverse events were observed.
- Most common adverse events (AEs) were mild and moderate injection site reactions (ISR). At least one ISR was seen in 43% of patients receiving placebo, and all patients receiving CTI-1601 experienced ISRs.
- Most ISRs resolved within an hour after injection, and all ISRs resolved without intervention. There were no study discontinuations due to ISRs.
- Except for ISRs, the number & severity of AEs did not increase with increasing dose.

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

Clinical & Non-clinical Safety Data Supported Initiation of 4-Week, Phase 2 Dose Exploration Study at 25 mg

FDA cleared Phase 2 study's initiation following review of clinical and non-clinical data

SUMMARY OF MULTIPLE-ASCENDING DOSE (MAD) TRIAL SAFETY DATA

Repeated SC injections of CTI-1601 appear to be generally well tolerated at doses up to 100 mg administered daily for 13 days.

- No serious adverse events (SAEs), important medical events, or treatment-related severe adverse events were observed.
- Most common AEs were mild and moderate injection site reactions (ISR). At least one ISR was seen in 43% of patients receiving placebo, and all patients receiving CTI-1601 experienced ISRs. Most ISRs resolved within an hour after injection, and all ISRs resolved without intervention. There were no study discontinuations due to ISRs.
- Except for ISRs, the number and severity of AEs did not increase with increasing exposure to CTI-1601.

SUMMARY OF NON-HUMAN PRIMATE (NHP) DATA

- The clinical hold was put in place following deaths that occurred during the 26-week toxicology study in 3 out of a total of 34 NHPs. All 3 of these NHPs were in the two highest dose groups. All NHPs in the two lower dose groups survived to the end of the 26-week toxicology study.
- Based on AUC, C_{max}, and C_{trough} from the Phase 1 studies at the 25 mg and 50 mg levels, and the no observed adverse effect levels from the 4-, 13-, and 26-week toxicology studies, the safety margins calculated for CTI-1601 are generally greater than 10.
- Though the precise mechanism of toxicity in NHPs was not determined, we believe the toxicity was associated with accumulation and high levels of exposure as demonstrated by the safety margins. We believe the presence of persistent edema at the injection sites in some NHPs may explain the accumulation associated with adverse events, as well as higher plasma levels of CTI-1601. In the clinic, injection sites will be closely monitored and we intend to avoid the use of injection sites where persistent edema is present.

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SC: Subcutaneous

Additional Phase 2 Cohort 1 Data

Demographics of Phase 2 (Cohort 1)

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%)

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Disease Characteristics (Phase 2 Cohort 1)

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

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

- 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

Top-line Results from Non-interventional Study

Recently completed non-interventional study (CLIN-1601-002) measured FXN in homozygous HVs

FXN Levels in Homozygous Healthy Volunteers (HVs)

FXN concentrations were measured[1] in skin and buccal cells from 60 homozygous healthy volunteers:

- Median FXN in Skin = 16 pg/µg
- Median FXN in Buccal cells = 8 pg/µg

Study utilized the same sampling technique and assay as clinical trials of CTI-1601

Based on published literature, asymptomatic heterozygous carriers have frataxin levels that are approximately 50% of those of homozygous healthy people[2]

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1. FXN levels measured via detection of peptide derived from mature FXN; FXN concentrations normalized to total cellular protein content in each sample.
2. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245.

Preclinical Data

CTI-1601: Positive Mouse Model Data Support Development

Proof-of-Concept Demonstrated In Mouse Models of FA

Cardiac Knock Out Mouse Model Studies
(MCK-Cre FXN KO Mouse)

- Extended survival
- Demonstrated ability to deliver hFXN to mitochondria
- Increased in a dose dependent manner, succinate dehydrogenase (SDH) activity. SDH is an FXN dependent enzyme, whose activity is indicative of mitochondrial function
- Prevented left ventricle dilation and maintained function

Neurologic Knock Out Mouse Model Study
(Pvalb-CRE FXN KO Mouse)

- Prevented development of ataxic gait
- Showed that treated mice survive longer than untreated mice
- Demonstrated CNS penetration, as hFXN was present in brain, dorsal root ganglia & spinal cord

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CTI-1601 Extends Survival in FXN-deficient KO Mice

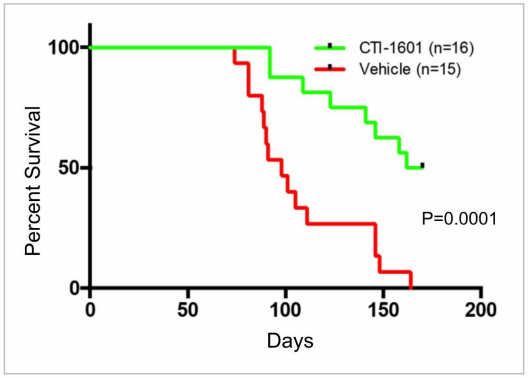
Initial Proof-of-Concept for FXN Replacement Therapy in Cardiac Mouse Model of FA

Median Survival of MCK-Cre FXN-KO Mice

- 166 days (CTI-1601) vs. 98 days (Vehicle)
- CTI-1601 was administered 10 mg/kg SC every other day

Survival beyond vehicle mean (107.5 days)

- 87.5% (CTI-1601) vs. 33% (Vehicle)
- Demonstrates that CTI-1601 is capable of delivering sufficient amounts of FXN to mitochondria



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

CTI-1601 Prevents The Development of Ataxic Gait in KO mice

In-Vivo Efficacy Data in Neurologic KO Mouse Model

Pvalb-Cre FXN-KO mouse

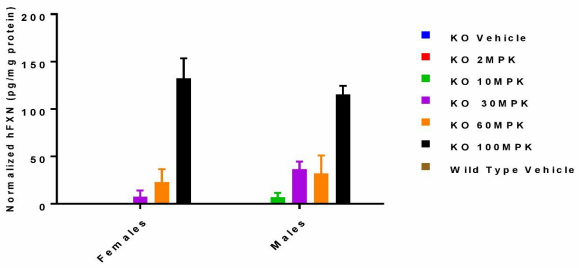
Single dose level: 10 mg/kg CTI-1601 or vehicle given intraperitoneally three times per week

- hFXN replacement with CTI-1601 **prevents the development of ataxic gait**
- CTI-1601-treated mice **survive longer** than untreated mice
- Human frataxin **present in brain, dorsal root ganglia and spinal cord** demonstrating central nervous system penetration

CTI-1601 Delivers hFXN to Mitochondria in KO Mice

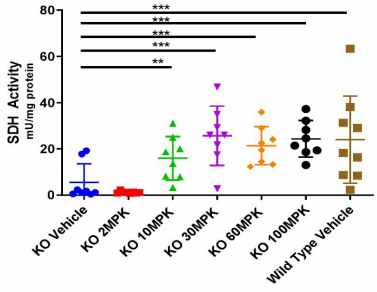
- hFXN concentration within mitochondria increases in a dose-dependent manner
- Given subcutaneously, CTI-1601 functionally replaces hFXN in mitochondria of KO mice
- Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration of CTI-1601; activity plateaus at 30 mg/kg and is equivalent to activity in wild type animals
- Demonstrated normalization of gene expression in cardiac tissue

Normalized Mitochondrial FXN (Heart)



MPK = mg/kg

Normalized SDH Activity (Muscle)



MPK = mg/kg

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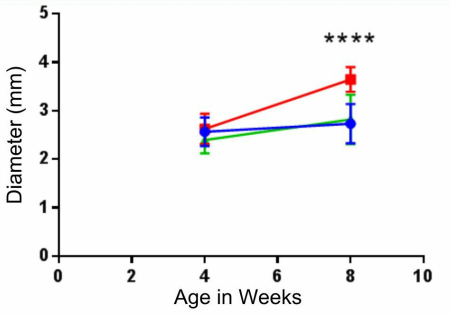
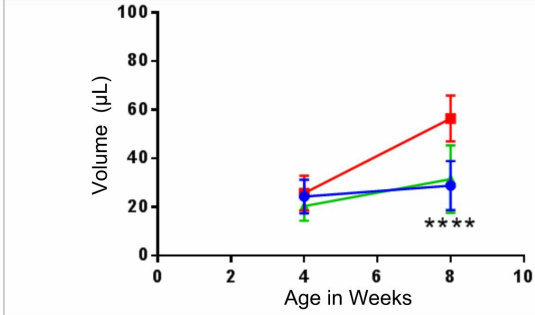
CTI-1601 Prevents Left Ventricle Dilation in KO Mice

- 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 CTI-1601 (10 mg/kg every other day)
- CTI-1601-treated mice have similar LV volume as healthy controls; echocardiogram shows significant differences between vehicle and CTI-1601 treated (10 mg/kg every other day) KO mice

Left Ventricle Internal Diameter (Systole)



Left Ventricle Volume (Systole)



KO: CTI-1601 KO: Vehicle Wild-type: Vehicle

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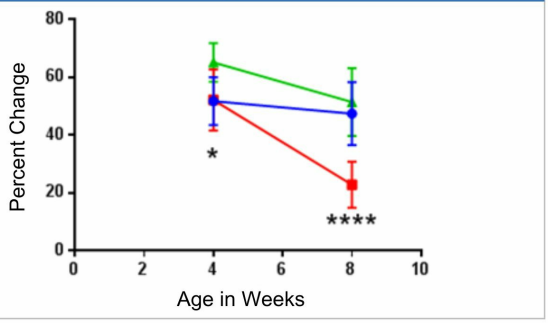
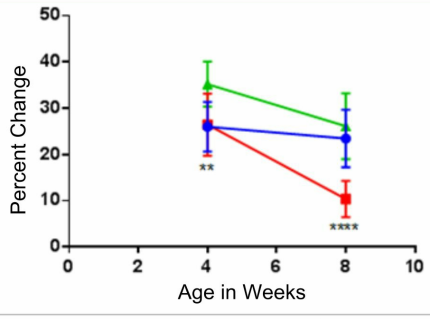
CTI-1601 Preserves Left Ventricle Function in KO Mice

- Left ventricular (LV) function drops significantly in vehicle treated mice by week 8
- CTI-1601-treated (10 mg/kg every other day) mice have similar LV as healthy controls; echocardiogram shows significant differences between vehicle and CTI-1601 treated KO mice

Left Ventricle Ejection Function



Left Ventricle Fractional Shortening



KO: CTI-1601 KO: Vehicle Wild-type: Vehicle