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): May 12, 2022

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)**	**(I.R.S. Employer Identification No.)**

Three Bala Plaza East, Suite 506 **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 May 12, 2022, Larimar Therapeutics, Inc. (the "***Company***") announced its financial results and operational highlights for the first quarter ended March 31, 2022. 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 May 12, 2022, 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 May 12, 2022*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated May 12, 2022
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.

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

Date: May 12, 2022

Larimar Therapeutics Reports First Quarter 2022 Operating and Financial Results

-CTI-1601 Type C Meeting to discuss clinical hold has been granted by the U.S. Food and Drug Administration and is scheduled for early in the third quarter of 2022

- Cash at March 31, 2022 of $62.6 million provides projected cash runway into the third quarter of 2023

Bala Cynwyd, PA, May 12, 2022 – Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its first quarter 2022 operating and financial results.

"The FDA recently granted our request for a Type C meeting, which is expected to provide an important framework for interactions related to both the hold and our next proposed clinical trial," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar. "There remains an urgent need for therapies that can slow or prevent the progression of Friedreich's ataxia, which remains the key motivating factor behind our clinical development efforts. We believe our Phase 1 data demonstrate proof-of-concept for CTI-1601 as a frataxin replacement therapy and its differentiated mechanism of action (MOA). We believe this MOA leaves CTI-1601 uniquely positioned to address the urgent need for disease modifying therapies in Friedreich's ataxia, as it is designed to address the root cause of this devastating disease. We look forward to its continued development and our upcoming interactions with the FDA."

CTI-1601 Update

In February 2022, Larimar received feedback from the U.S. Food and Drug Administration (FDA) regarding the May 2021 clinical hold placed on the CTI-1601 program. The May 2021 hold followed the Company's notification to the agency of mortalities which occurred at the highest dose levels in a 26-week non-human primate (NHP) toxicology study that was designed to support extended dosing of patients with CTI-1601. At the time the hold was placed, Larimar had no interventional clinical trials with patients enrolled or enrolling. In the feedback provided in February 2022, the FDA stated it was maintaining the clinical hold and that additional data are needed to resolve the clinical hold. This feedback followed Larimar's submission of a complete response to the agency. Larimar subsequently submitted a request for an FDA Type C meeting, which has been granted and has been scheduled for early in the third quarter of 2022. Throughout the course of the interactions associated with the Type C meeting, Larimar intends to work with the FDA to resolve the CTI-1601 clinical hold and agree on the study design and timing of its next proposed clinical trial. The Company plans to provide a regulatory update on CTI-1601 following receipt of the minutes from the scheduled Type C meeting with the FDA.

First Quarter 2022 Financial Results

As of March 31, 2022, the Company had cash and cash equivalents totaling $62.6 million which provides projected cash runway into the third quarter of 2023.

The Company reported a net loss for the first quarter of 2022 of $8.9 million, or $0.49 per share, compared to a net loss of $12.1 million, or $0.76 per share, for the first quarter of 2021.

Research and development expenses for the first quarter of 2022 were $5.8 million compared to $9.0 million for the first quarter of 2021. The decrease in research and development expenses compared to the prior year period was primarily driven by lower clinical supply manufacturing costs of $2.2 million and a decrease of $1.9 million in clinical trial costs, partially offset by an increase of $0.3 million in personnel related costs due to headcount additions in our research and development functions, higher non-clinical costs of $0.2 million, an increase of $0.2 million in professional fees primarily related to regulatory and clinical consulting services and an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

General and administrative expenses for the first quarter of 2022 and the first quarter of 2021 were both $3.1 million. Increases in stock compensation expense associated with stock option grants that were made throughout 2021 and in the first quarter of 2022 and higher personnel costs were offset by lower recruiting and consulting fees.

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, CTI-1601, 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 expectations regarding its ability to resolve the clinical hold imposed by the FDA related to CTI-1601, Larimar's ability to develop and commercialize CTI-1601 and other planned product candidates, 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, 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, Larimar's ability to successfully engage with the FDA and satisfactorily respond to requests from the FDA for further information and data regarding CTI-1601, the timing and outcome of Larimar's planned interactions with the FDA concerning the clinical hold on CTI-1601, the success, cost and timing of Larimar's product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones; that preliminary 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; the ongoing impact of the COVID-19 pandemic on Larimar's future clinical trials, manufacturing, regulatory and nonclinical study timelines, the ongoing impact of the COVID-19 pandemic and the potential impact of the Russian invasion of Ukraine on Larimar's ability to raise additional capital and general economic conditions; 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 approval 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 press release represent Larimar's management's views only as of the date hereof. Larimar undertakes no obligation to update any forward-looking statements for any reason, except as required by law.

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Larimar Therapeutics, Inc.
Consolidated Balance Sheet
(Unaudited)

	March 31, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 62,564	$ 70,097
Prepaid expenses and other current assets	2,315	2,107
Total current assets	64,879	72,204
Property and equipment, net	1,067	1,049
Operating lease right-of-use assets	3,270	3,406
Restricted cash	1,339	1,339
Other assets	668	669
Total assets	$ 71,223	$ 78,667
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,417	$ 1,660
Accrued expenses	5,840	6,592
Operating lease liabilities, current	616	594
Total current liabilities	8,873	8,846
Operating lease liabilities	5,245	5,408
Total liabilities	14,118	14,254
Commitments and contingencies (See Note 9)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of March 31, 2022 and December 31, 2021; no shares issued and outstanding as of March 31, 2022 and December 31, 2021	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of March 31, 2022 and December 31, 2021; 17,710,450 shares issued and outstanding as of March 31, 2022 and December 31, 2021	18	18
Additional paid-in capital	182,280	180,645
Accumulated deficit	(125,193)	(116,250)
Accumulated other comprehensive loss	—	—
Total stockholders' equity	57,105	64,413
Total liabilities and stockholders' equity	$ 71,223	$ 78,667

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

	Three Months Ended March 31,	
	2022	2021
Operating expenses:		
Research and development	$ 5,806	$ 8,974
General and administrative	3,081	3,132
Total operating expenses	8,887	12,106
Loss from operations	(8,887)	(12,106)
Other income, net	(56)	18
Net loss	$ (8,943)	$ (12,088)
Net loss per share, basic and diluted	$ (0.49)	$ (0.76)
Weighted average common shares outstanding, basic and diluted	18,338,853	15,996,133

Exhibit 99

Larimar Therapeutics

Corporate Presentation

May 2022

Forward Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions of Larimar Therapeutics, Inc. (the "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 statements regarding the expectations and assumptions regarding the future of the Company's business, including its ability to resolve the clinical hold by the FDA related to CTI-1601and the timing of such resolution, the Company's ability to develop and commercialize CTI-1601 and other planned product candidates, the Company's planned research and development efforts, and other matters regarding the Company's business strategies, 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 the current request, and future requests, if any, from the FDA for further information and data regarding CTI-1601, the timing and outcomes of Larimar's interactions with the FDA, including with respect to the clinical hold on CTI-1601, the success, cost and timing of the Company's product development activities, non-clinical studies and clinical trials, including CTI-1601 clinical milestones; that 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 clinical trials, and that clinical trial data are subject to differing interpretations and assessments; the ongoing impact of the COVID-19 pandemic on the Company's clinical trials, manufacturing, regulatory and nonclinical study timelines, the ongoing impact of the COVID-19 pandemic and the potential impact of geopolitical instability, including as a result of the Russian invasion of Ukraine, on the Company's ability to raise additional capital and general economic conditions; the Company's ability and the ability of third-party manufactures the Company engages to optimize and scale CTI-1601's manufacturing process; the Company's ability to obtain regulatory approval for CTI-1601 and future product candidates; the Company's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators, and to successfully commercialize any approved product candidates; the Company's ability to raise the necessary capital to conduct its product development activities; and other risks described in the filings made by the Company with the Securities and Exchange Commission (SEC), including but not limited to the Company'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 the Company 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. These forward-looking statements are based on information currently available to us, and we assume no obligation to update any forward-looking statements, 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 other complex rare diseases based on a platform technology backed by a strong intellectual property portfolio

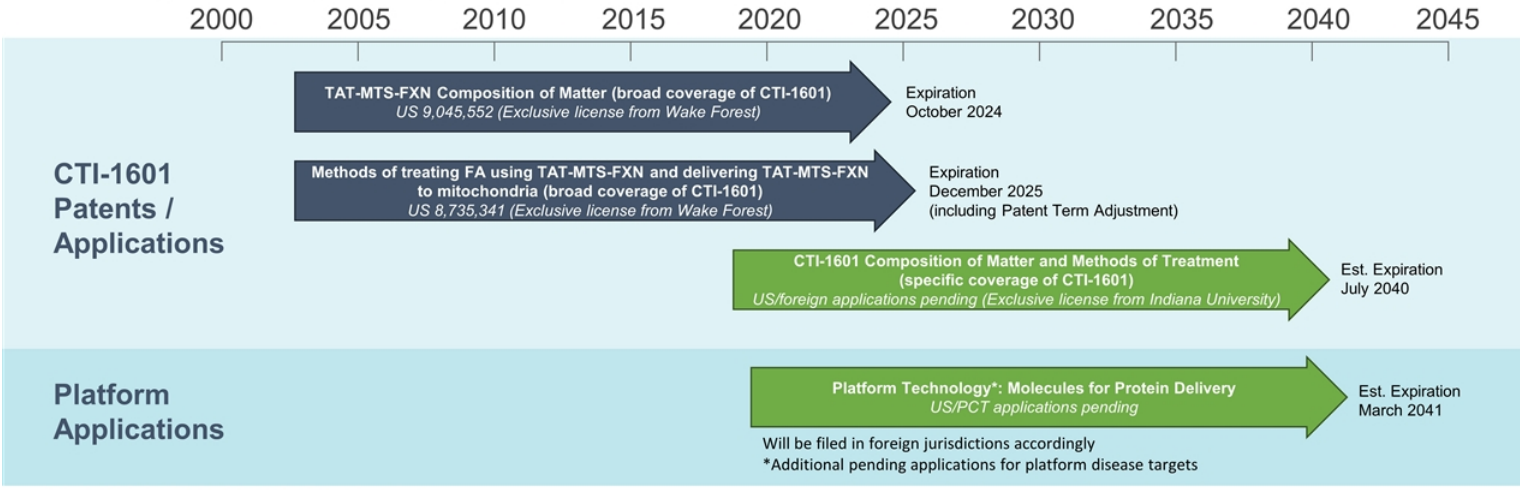
Lead candidate: CTI-1601, a recombinant fusion protein designed to deliver frataxin to mitochondria
Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations for FA

Double-blind, placebo-controlled Phase 1 proof-of-concept trials in FA patients complete
Data show dose dependent increases in frataxin (FXN) levels from baseline compared to placebo in al evaluated tissues with daily dosing & that CTI-1601 was generally well tolerated when dosed for up to 13 days -Currently on clinical hold; Type C meeting granted and scheduled with FDA early in the third quarter of 2022

Deerfield funded company; went public through a reverse merger/PIPE in May 2020
Shareholder base includes high-quality institutional investors; $62.6 million in cash at 3/31/22; Projected runway into the third quarter of 2023

Platform Technology is Supported by a Strong IP Portfolio

Pending CTI-1601 patent application, if issued, extends IP into 2040

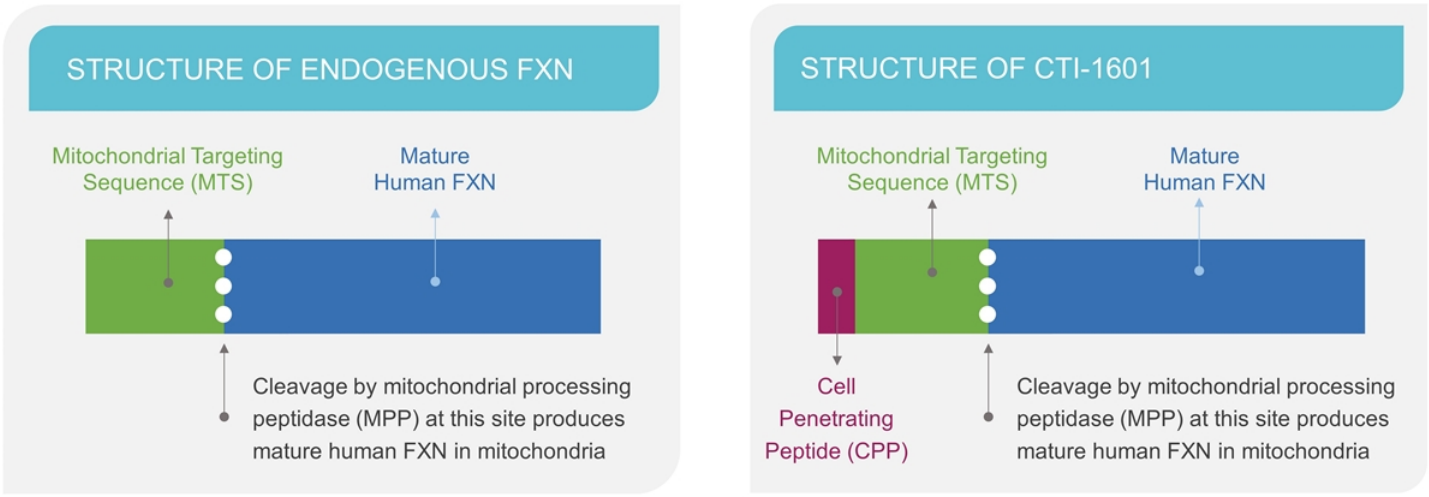


Additional CTI-1601 IP protection

- CTI-1601 pending applications cover key biomarkers, analytical tools and quantification methods
- CTI-1601 is 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)

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

CTI-1601 Maintains the Cleavage Site Between the MTS and Mature Human FXN



The maintenance 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

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

>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 approved therapies available

- Current treatment options are limited to symptom management

Larimar Therapeutics

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

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

Larimar Therapeutics

Executive Summary of Phase 1 POC Data

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 a once-daily dosing regimen for CTI-1601
Conclusion	Daily subcutaneous (SC) administration of 50mg and 100mg 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 phenotypically normal heterozygous carriers (who have FXN levels of ~50% of unaffected persons)

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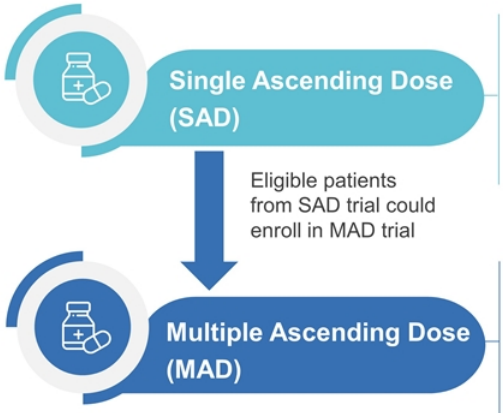
POC: Proof-of-concept

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

MAD Trial Patient Enrollment

16 out of 28 patients who participated in the SAD trial enrolled in the MAD trial

MAD Trial Patient Enrollment (n=27)		
Parameter	**Statistic**	**Overall**
Participated in SAD trial?		
Yes	n (%)	16 (59%)
No	n (%)	11 (41%)
Cohort 1 (25 mg) Active vs. Placebo		
Active	n (%)	6 (75%)
Placebo	n (%)	2 (25%)
Cohort 2 (50 mg) Active vs. Placebo		
Active	n (%)	7 (78%)
Placebo	n (%)	2 (22%)
Cohort 3 (100 mg) Active vs. Placebo		
Active	n (%)	7 (70%)
Placebo	n (%)	3 (30%)

Multiple Ascending Dose Study Design

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

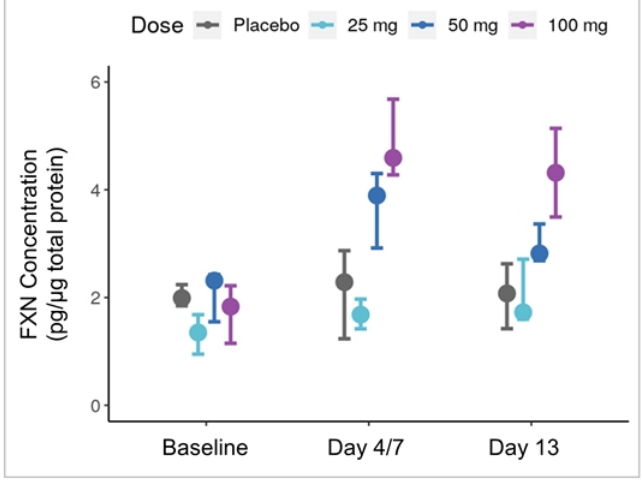
Cohort 3 Sampling Days	
Buccal Cells	Baseline, Day 7, Day 13
Skin	Baseline, Day 13
Platelets	Baseline, Day 7, Day 13

Larimar Therapeutics

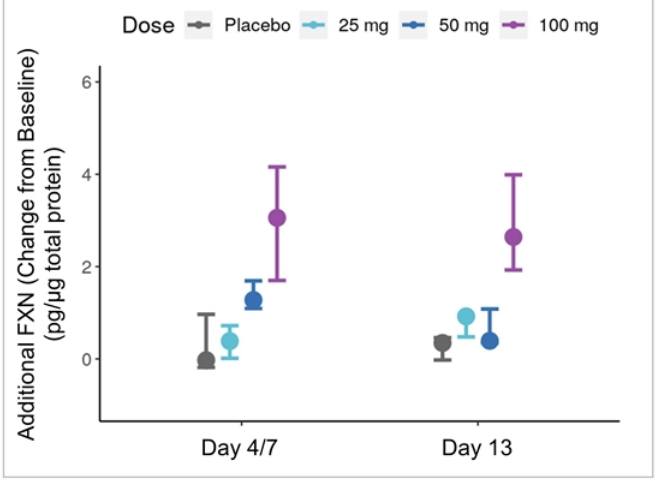
Dose Dependent Increases in FXN Levels Observed in Buccal Cells

Daily SC injections of 100 mg CTI-1601 resulted in an ~2.5 fold increase in FXN levels from baseline

FXN* Levels By Dose Group (Buccal Cells)



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

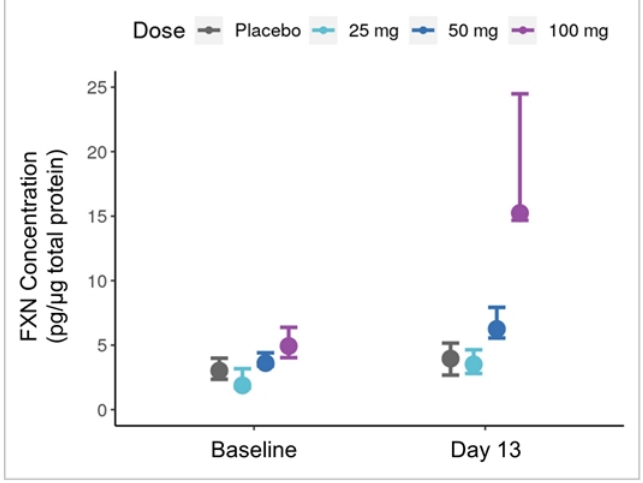


*FXN levels measured via detection of peptide derived from mature FXN; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from baseline, Day 7 & Day 13 measurements are shown for data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

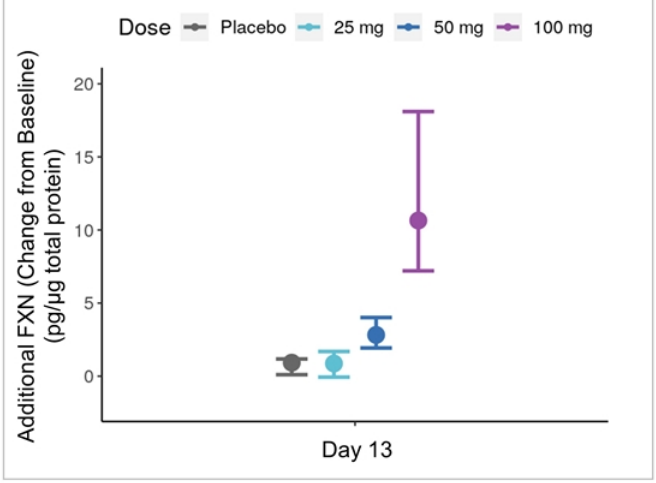
Dose Dependent Increases in FXN Levels Observed in Skin

Daily SC injections of 100 mg CTI-1601 resulted in an ~3 fold increase in FXN levels from baseline

FXN* Levels By Dose Group (Skin Biopsies)



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



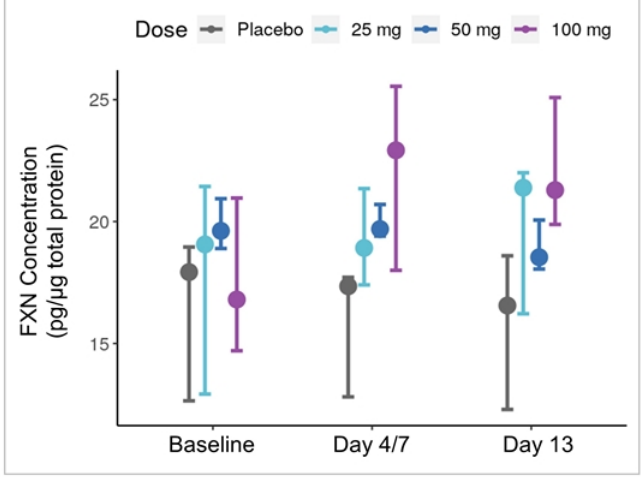
Larimar Therapeutics

*FXN levels measured via detection of peptide derived from mature FXN; Data represent median and 25th and 75th percentiles

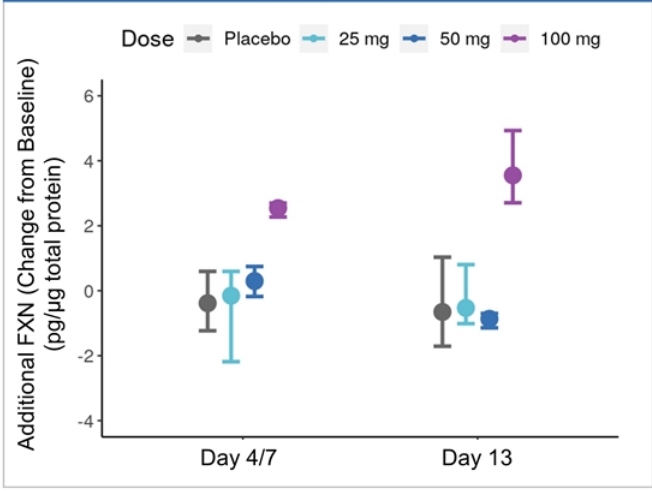
Dose Dependent Increases in FXN Levels Observed in Platelets with Daily Dosing

Daily SC injections of 100mg CTI-1601 resulted in increases in FXN levels from baseline compared to placebo

FXN* Levels By Dose Group (Platelets)



FXN* Change from Baseline By Dose Group (Platelets)

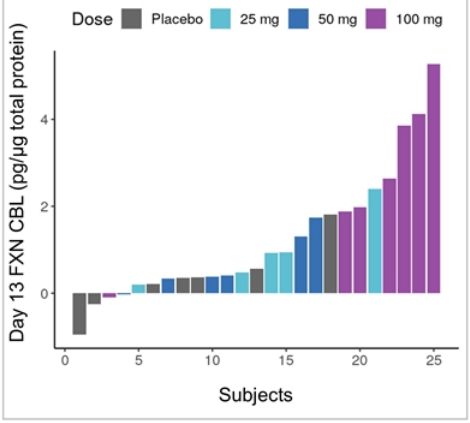


*FXN levels measured via detection of peptide derived from mature FXN; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from baseline, Day 7 & Day 13 measurements are shown for data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

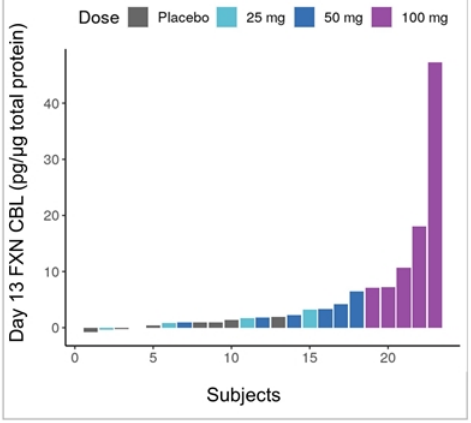
Increases in FXN Correlated with Increasing CTI-1601 Dose

Individual patient data further supports the dose-dependent effects of CTI-1601 in all tissues studied

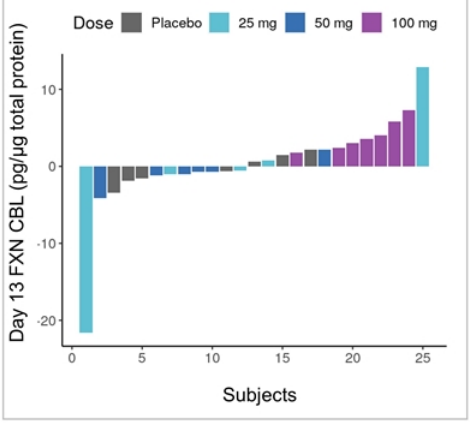
Buccal Cells Day 13 FXN* Change from Baseline



Skin Biopsies# Day 13 FXN* Change from Baseline



Platelets Day 13 FXN* Change from Baseline



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*FXN levels measured via detection of peptide derived from mature FXN; #Two patients in the 100 mg cohort declined skin biopsies
Day 13 observation excluded from one subject in 25 mg group that did not get a Day 13 dose.

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

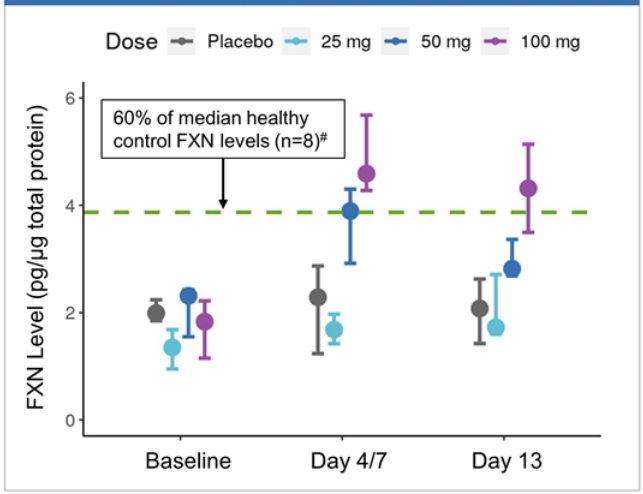
Benchmarking Clinical Relevance

- 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
- Data from additional healthy control buccal cells, skin, and platelets will be collected in a separate non-interventional study

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

Summary of MAD trial safety data:

Repeated doses (25 mg, 50 mg, and 100 mg) of CTI-1601 or placebo were administered subcutaneously. 27 patients were dosed in the trial. 26 patients completed the trial. 1 patient receiving CTI-1601 in Cohort 2 (50 mg) withdrew after experiencing mild/moderate symptoms (nausea and vomiting).

- No serious adverse events (SAEs), important medical events, or treatment-related severe adverse events
- The most common adverse events were mild and moderate injection site reactions (at least one injection site reaction was seen in 43% of placebo patients and in 100% of CTI-1601 patients)
- The number and severity of adverse events did not increase with increasing exposure to CTI-1601

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PK analyses support evaluating a once-daily dosing regimen for CTI-1601

Summary of 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 appears to be at or close to steady state exposure after 13 days of dosing 100 mg once daily

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Phase 1 Topline Data Demonstrated POC for CTI-1601 in FA

FXN levels in buccal cells & blood have been shown to correlate with disease severity in FA patients[1]

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

The most common AEs were mild and moderate injection site reactions

No SAEs have been reported

Frataxin Measurements

Daily SC injections of CTI-1601 resulted in dose-dependent increases in FXN levels from baseline compared to placebo controls in all evaluated tissues

With daily dosing (50mg and 100mg), achieved median FXN levels that were >60% of the median FXN levels observed in healthy controls

Pharmacokinetic Data

CTI-1601 was quickly absorbed after subcutaneous administration

Dose-proportional increases in exposure observed with increasing doses of CTI-1601

Data support evaluating a once-daily dosing regimen for CTI-1601

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1. Lazaropoulos et al. Ann Clin Transl Neurol. 2015 Aug; 2(8): 831–842; POC: Proof-of-concept; OLE: Open label extension; AE: Adverse events

Upcoming CTI-1601 Trials and Regulatory Interactions

Additional analyses from the Phase 1 program planned for presentation at a scientific meeting

Future Planned Trials and Regulatory Interactions Include:

Continued interactions with FDA including resolution of clinical hold and study design & timing of next proposed clinical trial. Type C meeting scheduled in early Q3 2022

Jive open label extension (OLE) trial for eligible patients who participated in SAD or MAD trials

MAD trial in patients 2 to 17 years of age. Participants eligible to screen for Jive OLE trial

Global double-blind placebo-controlled pivotal trial

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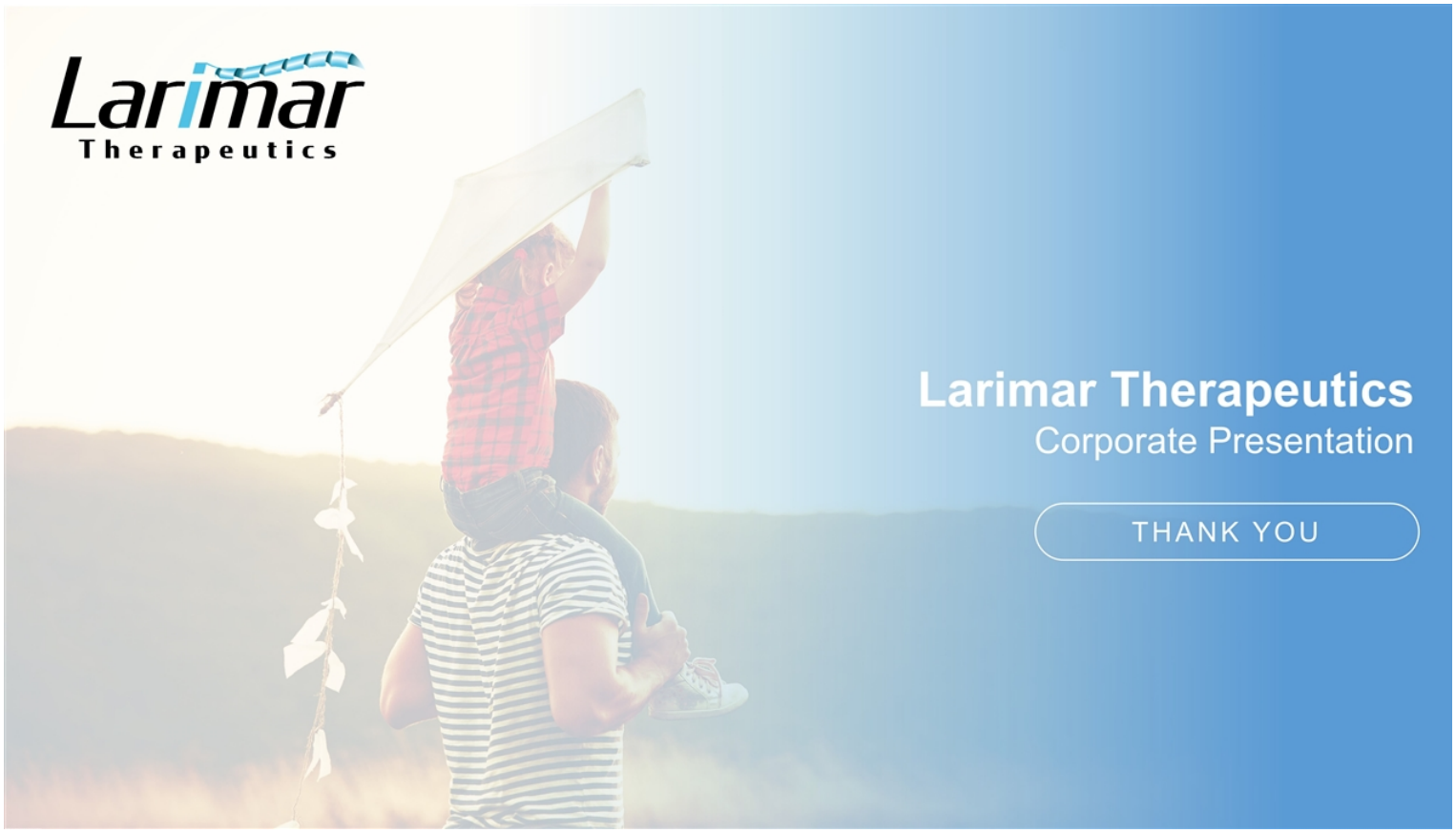
Investment Highlights

Clinical-stage biotechnology company with a novel protein replacement therapy platform
Focused on addressing unmet needs in Friedreich's ataxia (FA) and 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 FA patients complete
Data show dose dependent increases in frataxin (FXN) levels from baseline compared to placebo in al evaluated tissues with daily dosing & that CTI-1601 was generally well tolerated when dosed for up to 13 days -Currently on clinical hold; Type C meeting granted and scheduled with FDA early in the third quarter of 2022

Deerfield funded company; went public through a reverse merger/PIPE in May 2020
Shareholder base includes high-quality institutional investors; $62.6 million in cash at 3/31/22; Projected runway into the third quarter of 2023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MAD Trial Patient Demographics

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

SD: Standard deviation

MAD Trial Patient Disease Characteristics

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

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SD: Standard deviation

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

CTI-1601 Extends Survival in FXN-deficient KO Mice

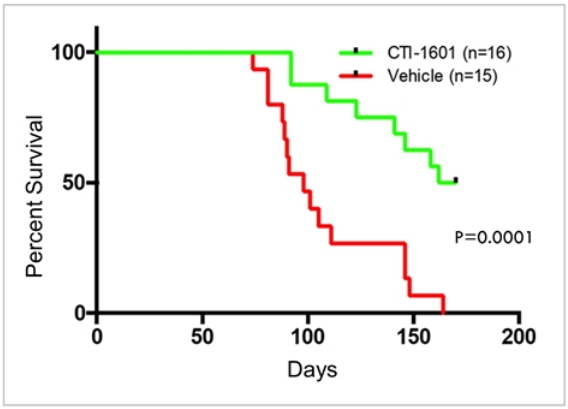
Initial Proof of Concept for FXN Replacement Therapy in Cardiac Mouse Model of FA

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

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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

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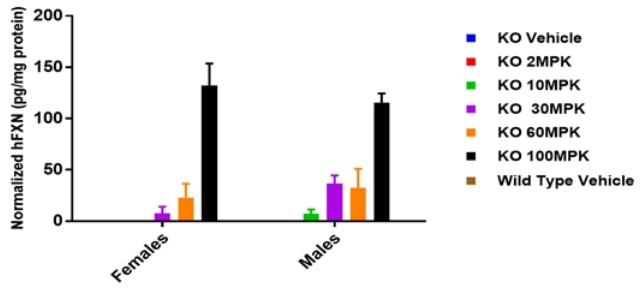
CTI-1601 Delivers hFXN to Mitochondria in KO Mice

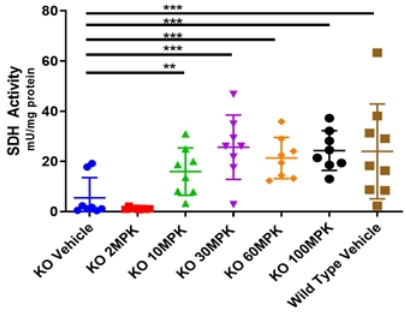
- hFXN concentration within mitochondria increases in a dose-dependent manner
- Given subcutaneously, CTI-1601 functionally replaces hFXN in mitochondria of KO mice
- Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration 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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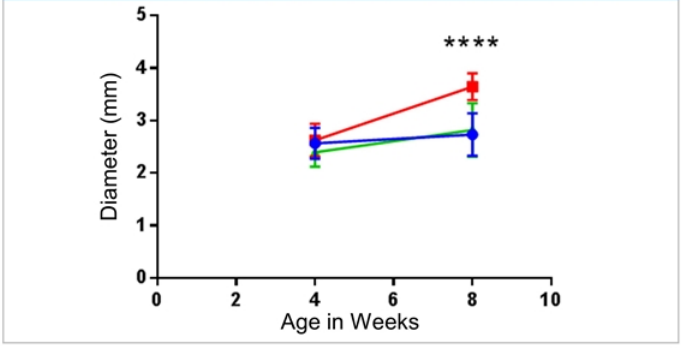
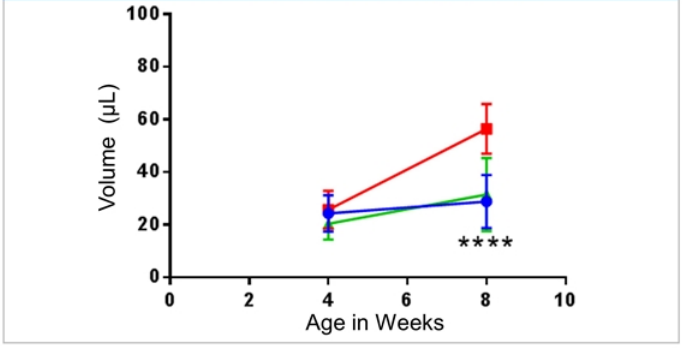
CTI-1601 Prevents Left Ventricle Dilation in KO Mice

- 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)

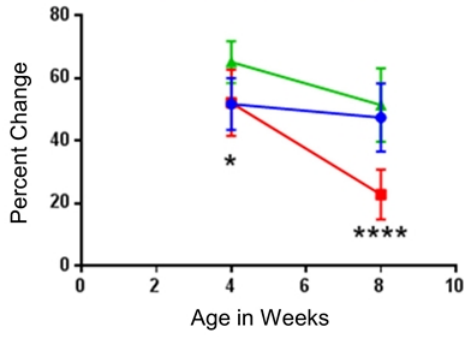


KO: CTI-1601 KO: Vehicle Wild-type: Vehicle

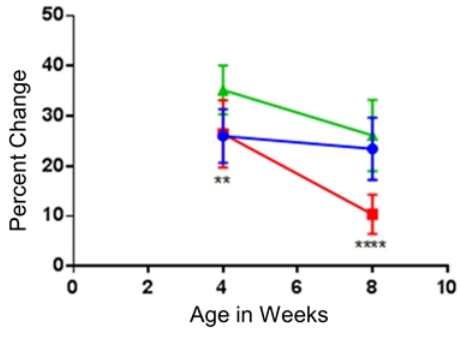
CTI-1601 Preserves Left Ventricle Function in KO Mice

- 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

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Favorable PK/PD Profile in Healthy Cynomolgus Monkeys

Study Design (14-Days of CTI-1601 dosing)

6 healthy cynomolgus monkeys (3M / 3F)

Pre-dosed for 2 days with Vehicle

Pre-dose collection of platelets, cerebrospinal fluid, buccal swab, skin punch
Dosing starts 15 mg/kg SC BID

Day 10 (7 days dosing)

Collection of platelets, buccal swab, skin punch

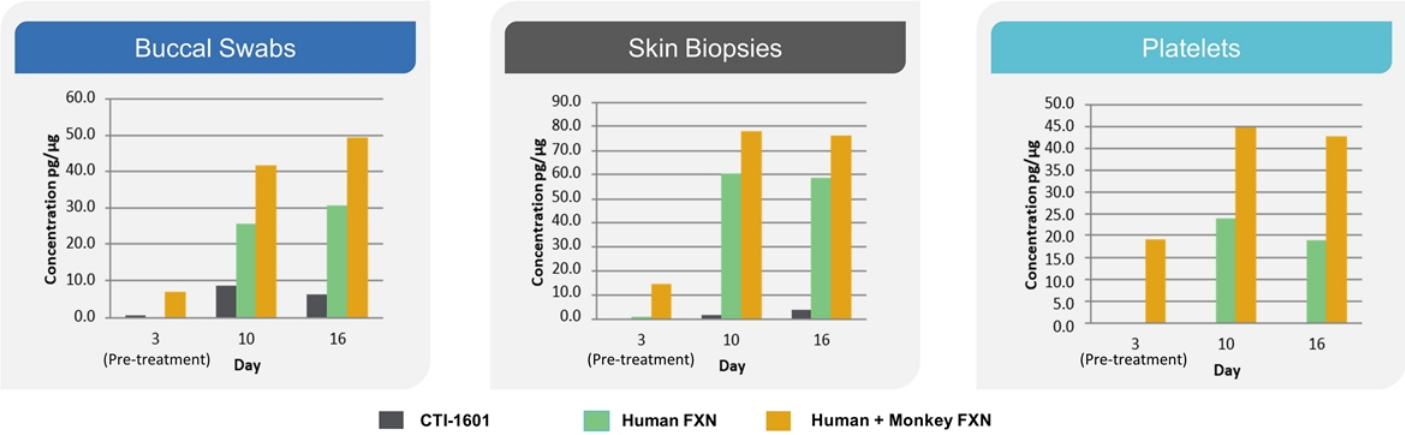
Day 16 (following 14th day of dosing)

Collection of cerebrospinal fluid, platelets, buccal swab, skin punch

- CTI-1601 is bioavailable when given subcutaneously
- Sustained levels of hFXN are found in blood cells (platelets) and peripheral tissues (buccal cells, skin) as early as the 7th day and still present after 14 days
- Sustained levels of hFXN are found after 14 days in the cerebrospinal fluid of monkeys, suggesting CNS penetration

Biodistribution in Healthy Cynomolgus Monkeys

Sustained levels of human FXN (hFXN) in peripheral tissues after 14 days of CTI-1601 dosing



- Treatment of monkeys with CTI-1601 results in sustained levels of hFXN in peripheral tissues that are accessible in the clinic
- FXN levels increase ~4X or more following CTI-1601 administration
  - For comparison, FA patients show FXN levels that range from ~20-40% of normal FXN levels depending on the tissue considered[1]
    - Heterozygous carriers show no phenotype and display levels of FXN representing ~2-3X higher than most FA patients[1]

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