

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): July 25, 2023

Larimar Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware  
(State or Other Jurisdiction  
of Incorporation)

001-36510  
(Commission File Number)

20-3857670  
(IRS Employer  
Identification No.)

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

19004  
(Zip Code)

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

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

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

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

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

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

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

Emerging growth company

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

**Item 8.01 Other Events.****Press Release**

On July 25, 2023, Larimar Therapeutics, Inc. (the “**Company**”) issued a press release announcing that the U.S. Food and Drug Administration has (i) cleared the Company’s four-week, placebo-controlled, Phase 2 dose exploration trial of CTI-1601 in patients with Friedreich’s ataxia to proceed to a 50 mg cohort in which participants will be dosed daily for the first 14 days, and then every other day until day 28 and (ii) cleared for initiation the Company’s open label extension trial in which participants will receive 25 mg of CTI-1601 daily. A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

**Investor Presentation**

On July 25, 2023, the Company posted on its website an updated slide presentation, which is attached as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. Representatives of the Company will use the presentation in various meetings with investors, analysts and other parties from time to time.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

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

<u>Exhibit No.</u>	<u>Document</u>
99.1	<a href="#">Press Release issued by Larimar Therapeutics, Inc. on July 25, 2023*</a>
99.2	<a href="#">Larimar Therapeutics, Inc. Corporate Presentation, dated July 25, 2023*</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

\* Filed herewith

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**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: July 25, 2023

By: /s/ Carole S. Ben-Maimon, M.D.

*Name: Carole S. Ben-Maimon, M.D.*

*Title: President and Chief Executive Officer*

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## Larimar Therapeutics Receives FDA Clearance to Proceed to 50 mg Cohort in CTI-1601's Phase 2 Friedreich's Ataxia Trial and to Initiate Open Label Extension Trial

- *Top-line safety, pharmacokinetic, and pharmacodynamic (frataxin level) data from Phase 2 trial's 50 mg cohort expected in 1H 2024*
- *Initiation of open label extension trial with 25 mg daily dosing expected in Q1 2024; interim data expected in Q4 2024*
- *Company management hosting a webcast and conference call today at 8:00 a.m. ET*

**Bala Cynwyd, PA**, July 25, 2023 – Larimar Therapeutics, Inc. ("Larimar") (Nasdaq: LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Company's four-week, placebo-controlled, Phase 2 dose exploration trial of CTI-1601 in patients with Friedreich's ataxia (FA) to proceed to a 50 mg cohort in which participants will be dosed daily for the first 14 days, and then every other day until day 28. In addition, Larimar's open label extension (OLE) trial was also cleared for initiation by the FDA. Participants in the OLE will receive 25 mg of CTI-1601 daily. CTI-1601 is a novel protein replacement therapy designed to deliver frataxin to the mitochondria of patients with FA who have low levels of frataxin.

Larimar received clearance to advance its Phase 2 trial to a 50 mg cohort and initiate its OLE trial following a review by the FDA of Larimar's complete response to its partial clinical hold that included unblinded safety, pharmacokinetic (PK), and pharmacodynamic (PD) data from the Phase 2 trial's completed 25 mg cohort. Data from the completed 25 mg cohort (n = 13) indicated that CTI-1601 was generally well tolerated and showed increases in frataxin (FXN) levels from baseline compared to placebo in all evaluated tissues (skin and buccal cells) at day 14 (the final day of daily dosing in the trial). Further dose escalation in the Phase 2 and OLE trials and the initiation of additional U.S. clinical trials evaluating CTI-1601 are contingent on FDA review of results from the Phase 2 trial's 50 mg cohort in accordance with a partial clinical hold.

"Gaining clearance to advance to a 50 mg cohort in our Phase 2 trial and initiate the OLE trial are crucial steps in CTI-1601's development as potentially the first therapy to increase frataxin levels in patients with FA," said Carole Ben-Maimon, MD, President, and Chief Executive Officer of Larimar. "Given the inability of current treatments to address the frataxin deficiency underlying Friedreich's ataxia, we believe CTI-1601 has the potential to improve the treatment paradigm for this devastating disease. We now look forward to data from our Phase 2 trial's 50 mg cohort in the first half of 2024, which will help us further characterize the safety and PK profiles of CTI-1601 and its ability to increase frataxin levels in a dose-dependent fashion as seen in our earlier Phase 1 studies."

Participants who complete treatment in the Phase 2 dose exploration trial, or who previously completed a prior clinical trial of CTI-1601, are eligible to screen for Larimar's OLE trial. Participants in the OLE trial will receive subcutaneous injections of 25 mg of CTI-1601 administered daily. The trial is expected to begin in Q1 2024 with interim data expected in Q4 2024.

Dr. Ben-Maimon continued, "We are pleased to have clearance to begin our OLE trial and look forward to what we expect will be important interim data from the study in Q4 2024. Alongside our efforts to advance both our OLE and Phase 2 trials in the United States, we have also begun to engage with regulators and investigators outside the U.S. as we prepare to expand our clinical

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program to additional geographies. With approximately 75% of individuals with FA living outside the U.S., establishing global clinical trial capabilities is important for addressing the pressing unmet needs of the FA community.”

CTI-1601 has been granted Orphan Drug (U.S. and Europe), Rare Pediatric Disease (U.S.), Fast Track (U.S.), and PRIME (Europe) designations for FA.

#### **Webcast and Conference Call**

Larimar will host a conference call and webcast today, July 25, 2023 at 8:00 a.m. ET. To access the webcast please visit this link to the event. To participate by phone, please dial 1-877-407-9716 (domestic) or 1-201-493-6779 (international) and refer to conference ID 13740205 or click on this link and request a return call. Following the live event, the archived webcast will be available on the “Events & Presentations” page of the Larimar website. Following the live event, the archived webcast will be available for 90 days.

#### **About the Phase 2 Trial**

The Phase 2 trial is a placebo-controlled, four-week, dose exploration trial designed to further characterize CTI-1601’s safety, pharmacokinetic, and pharmacodynamic profiles. Eligible participants include ambulatory and non-ambulatory individuals with FA who are at least 18 years old. Participants enrolled in each cohort of the trial are randomized 2:1 to receive CTI-1601 or placebo daily via subcutaneous injections for the first 14 days, and then every other day until day 28. The trial currently includes two cohorts. Cohort 1 included 13 participants (9 on active treatment and 4 on placebo) and evaluated a 25 mg dose of CTI-1601. Cohort 2, which is initiating, will include 12-15 participants randomized 2:1 to CTI-1601 or placebo and will evaluate a 50 mg dose of CTI-1601. Key endpoints include safety assessments, pharmacokinetic assessments, as well as measures of frataxin levels and other pharmacodynamic markers (e.g., lipid profiles and gene expression data) in peripheral tissues. For more information on the trial, please visit [clinicaltrials.gov](https://clinicaltrials.gov) under the identifier: NCT05579691.

#### **About the Open Label Extension Trial**

The open label extension trial is a multi-center study designed to enroll patients with FA who have previously completed a clinical trial of CTI-1601. Participants in the trial will receive daily subcutaneous injections of 25 mg of CTI-1601 with injections being self-administered or administered by a caregiver. The safety and pharmacodynamic objectives of the open-label extension trial are to evaluate the safety, tolerability, and pharmacokinetics of long-term subcutaneous administration of CTI-1601 as well as measures of frataxin levels and other pharmacodynamic markers (e.g., lipid profiles and gene expression data) in peripheral tissues. Other objectives include evaluation of the effects of long-term subcutaneous administration of CTI-1601 on measures of clinical function. Data collected during the trial will be compared to a matched set of untreated patients derived from participants in the Friedreich’s Ataxia Clinical Outcome Measures Study (FACOMS) database.

#### **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**

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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 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 trials and overall 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 success, cost and timing of Larimar's product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones and continued interactions with the FDA regarding the partial clinical hold; 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 potential impact of public health crises on Larimar's future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and general economic conditions; Larimar's ability and the ability of third-party manufacturers Larimar engages, to optimize and scale 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](http://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

## CTI-1601 Program Update

July 2023

# Forward-Looking Statements

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In some cases, you can identify forward-looking statements by the words “may,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “ongoing” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, , the success, cost and timing of Larimar’s product development activities, nonclinical studies and clinical trials, including CTI-1601 clinical milestones and continued interactions with the FDA regarding the partial clinical hold; 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 potential impact of public health crises on Larimar’s future clinical trials, manufacturing, regulatory, nonclinical study timelines and operations, and general economic conditions; Larimar’s ability and the ability of third-party manufacturers Larimar engages, to optimize and scale 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](http://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.

# CTI-1601 Cleared by FDA to Advance Clinical Development

FDA review of Larimar Complete Response to its partial clinical hold clears initiation of both 50 mg cohort in Phase 2 dose exploration trial and OLE trial

## Phase 2 Trial Proceeding to 50 mg Cohort

FDA cleared initiation of 50 mg cohort after review of unblinded data from 25 mg cohort  
Participants in 50 mg cohort to be dosed daily for 14 days, then every other day until day 28

## OLE Trial Cleared for Initiation

Participants in either of the Phase 1 trials or Phase 2 dose exploration trial eligible to screen  
Dosing: 25 mg daily with subcutaneous injections self-administered or administered by a caregiver (data collected periodically in 1<sup>st</sup> year and every 3 months thereafter)

## Next Steps

Continue preparations to expand CTI-1601 clinical program to geographies outside the U.S.  
Submit Phase 2 data from the 2<sup>nd</sup> cohort to FDA to obtain clearance to potentially escalate the dose in the Phase 2 study or the OLE study

# Investment Highlights

## Novel protein replacement therapy platform

Clinical-stage biotechnology company 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

## Potential first-ever therapy to increase frataxin levels

Lead candidate CTI-1601 is a recombinant fusion protein designed to directly address frataxin deficiency by delivering the protein to mitochondria. CTI-1601 has received Orphan Drug (US & EU), Rare Pediatric Disease (US), Fast Track (US), & PRIME (EU) designations

## Completed Phase 1 proof-of-concept

Two double-blind, placebo-controlled Phase 1 trials in FA demonstrating CTI-1601 was **generally well tolerated** when dosed daily for up to 13 days; **dose-dependent increases in frataxin (FXN) levels** from baseline vs. placebo were observed in all evaluated tissues

## Phase 2 and OLE studies with near-term catalysts

Ongoing Phase 2, placebo-controlled, 4-week dose exploration study in FA; 25 mg cohort data show CTI-1601 is generally well tolerated, increasing FXN levels from baseline vs. placebo in skin and buccal cells; **trial advancing to 50 mg cohort with data expected in 1H 2024; OLE trial with 25 mg daily dosing cleared for initiation in Q1 2024**. To potentially escalate dose in Phase 2 study or the OLE study, submit Phase 2 data from 50 mg cohort to FDA due to continued partial clinical hold.

## Strong financial foundation

\$111.5 M cash balance (March 31, 2023) with projected cash runway into Q4 2024

# 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<sup>1</sup>
- 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 speech")

### Life expectancy of 30-50 years

- Early death usually caused by heart disease

### No available therapies increase frataxin levels

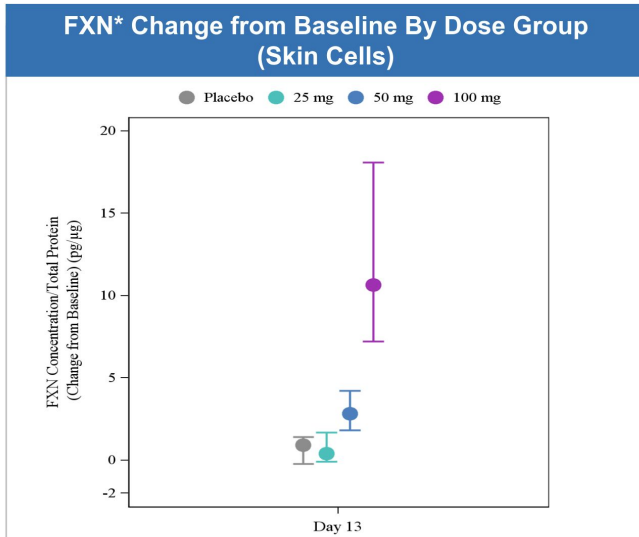
- Only treatment approved for FA does not address frataxin deficiency

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

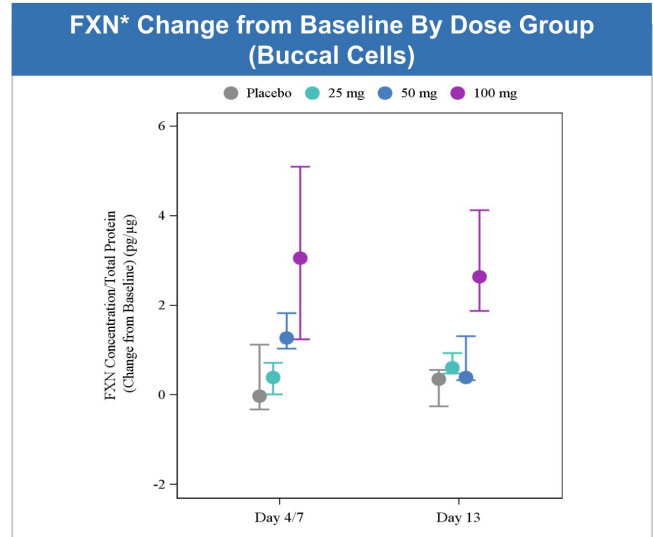
- Dedicated FA patient advocacy group focused on treatments for FA



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



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



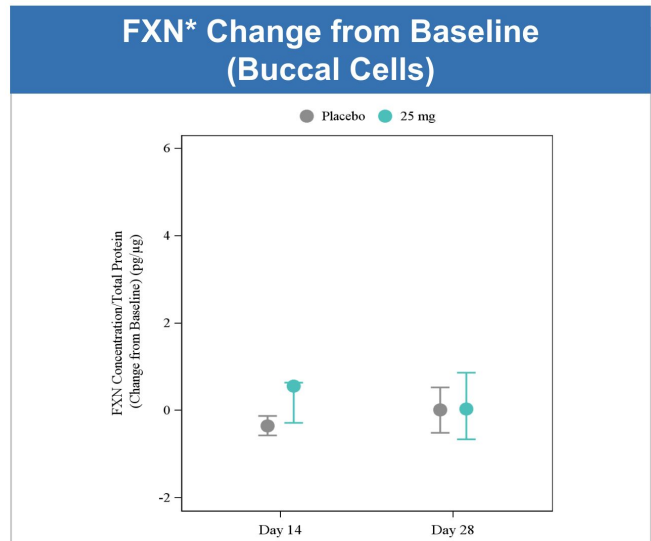
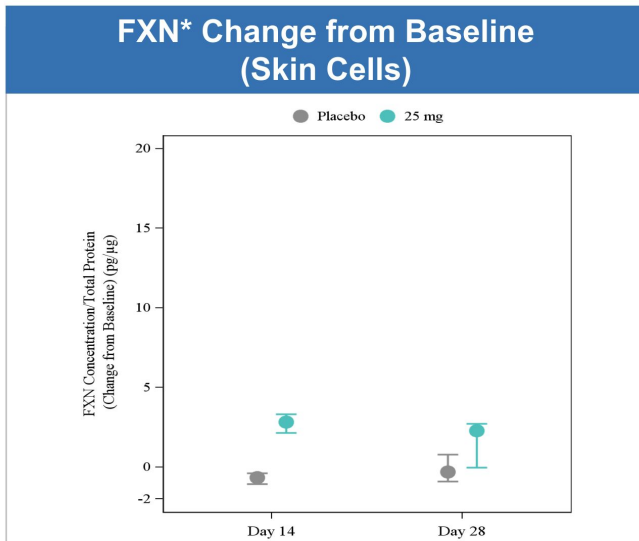
50 mg: Dosed daily for 7 days, every other day thereafter  
 100 mg: Dosed daily for 13 days



\*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 25<sup>th</sup> and 75<sup>th</sup> percentiles; FXN levels from Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from Day 7 & Day 13 measurements are shown for data derived from the 50 & 100 mg cohorts; Sample collection days varied in each cohort per the trial protocol

# Increases in FXN Levels in Phase 2 Trial's 25 mg Cohort

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



\*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 25<sup>th</sup> and 75<sup>th</sup> percentiles.

# 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**



# Phase 2 Dose Exploration Study Advancing to 50 mg Cohort

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

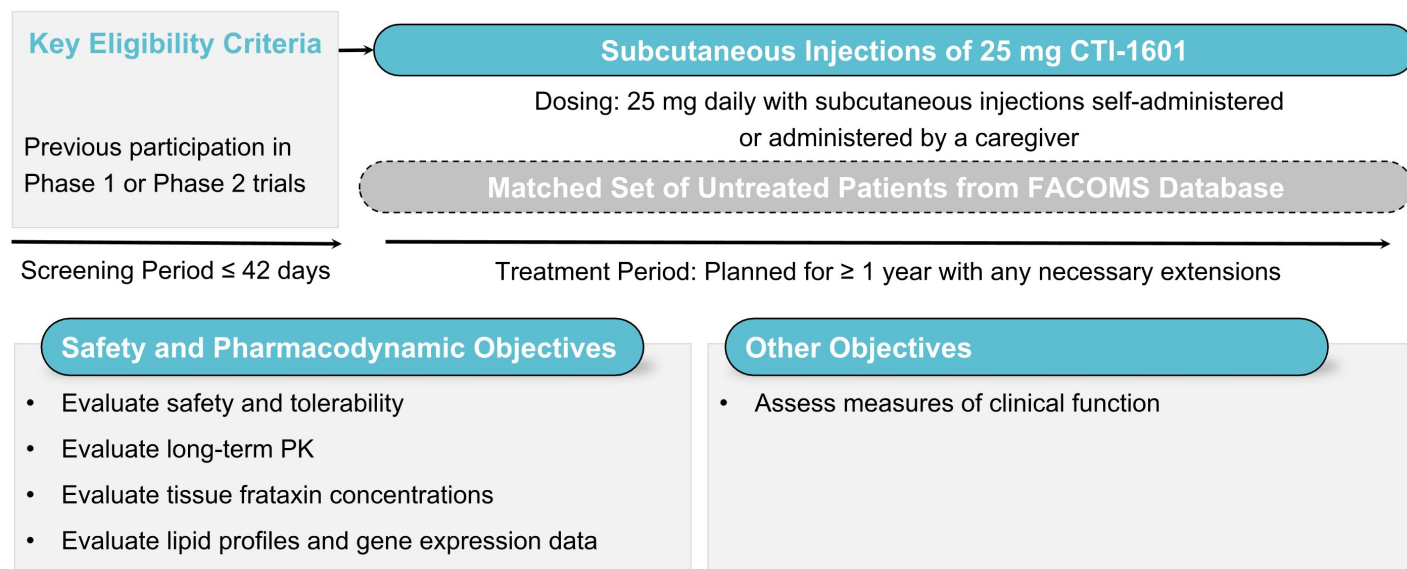


**Study Details**

<b>Population</b>	Ambulatory and non-ambulatory Friedreich's ataxia patients $\geq 18$ years of age. CTI-1601 treatment naïve or participated (if eligible) in a previous Larimar study.
<b>Dose</b>	<b>Cohort 1:</b> 25 mg <b>Cohort 2:</b> 50 mg
<b>Key Endpoints</b>	Frataxin levels in peripheral tissue, PK, PD, safety and tolerability. PD endpoints include lipid profiles and gene expression data.
<b>Number of Patients</b>	<b>Cohort 1:</b> Enrolled 13 participants (9 on CTI-1601; 4 on placebo). <b>Cohort 2:</b> Designed to enroll ~12-15 participants randomized 2:1 to receive CTI-1601 or placebo.
<b>Timing</b>	Dosing in Cohort 1 is complete. Cohort 2 has been cleared for initiation. Top-line data from Cohort 2 expected in 1H 2024.

# Open-label Extension Trial: Initiation Expected in Q1 2024

Preliminary interim data expected in Q4 2024



# CTI-1601 Clinical Development Plan

Top-line data from 50 mg cohort of Phase 2 dose exploration trial expected in 1H 2024

Ongoing and Planned Trials\* Include:



Phase 2, four-week dose exploration study intended to identify dose and dose regimen for long-term studies. Advancing to 50 mg cohort.



Open-label extension trial with 25 mg daily dosing for eligible patients who participated in SAD, MAD, and/or four-week dose exploration studies. Initiation expected Q1 2024.



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



Global double-blind placebo-controlled pivotal trial\*\*\*

\*Initiation of additional U.S. clinical trials are contingent on submission to FDA of additional data from adult patients due to partial clinical hold.

\*\*Company is planning discussions with FDA on how to best include patients 2 to 17 years of age in clinical development

\*\*\*Company plans to initiate discussions with FDA on appropriate pivotal trial endpoints, including value of FXN levels. Also, the Company is planning discussions with regulators & investigators outside the U.S. to expand clinical program to international geographies.

## Summary: CTI-1601 Cleared by FDA to Advance Clinical Development

### CTI-1601

Generally well tolerated  
Dose-dependent increases in FXN levels in all evaluated tissues in Phase 1  
Increases in FXN from baseline compared to placebo in all evaluated tissues (skin and buccal cells) in Phase 2

### Regulatory Updates

Phase 2 dose exploration trial of CTI-1601 in FA cleared to proceed to 50 mg cohort  
Open label extension trial with 25 mg daily dosing cleared for initiation  
Beginning preparations to expand CTI-1601 clinical program to ex-U.S. geographies

### Expected Milestones

**Q3 2023:** Initiation of 50 mg cohort in Phase 2 dose exploration trial  
**Q1 2024:** Initiation of open-label extension trial  
**1H 2024:** Top-line data from Phase 2 trial's 50 mg cohort  
**Q4 2024:** Interim data from open-label extension trial