UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): August 11, 2020

Larimar Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**001-36510**	**20-3857670**
(State or other jurisdiction of incorporation or organization)	**(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 (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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

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

Emerging growth company ☐

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

Item 2.02 Results of Operations and Financial Condition

On August 11, 2020, Larimar Therapeutics, Inc. (the "***Company***") announced its financial results and operational highlights for the second quarter ended June 30, 2020. 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 in this Current Report on Form 8-K and 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 (the "***Securities Act***"), or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01 Regulation FD Disclosure

On August 11, 2020, the Company posted on its website an updated slide presentation, which is being furnished 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.

The information in this Item 7.01 (including Exhibit 99.2) is being furnished solely to satisfy the requirements of Regulation FD and shall not be deemed to be "filed" for purposes of Section 18 of the Exchange Act or otherwise subject to the liabilities of that section, nor shall it be deemed to be incorporated by reference in any filing under the Securities Act or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits

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

Exhibit No.	Document
99.1	Press Release issued by Larimar Therapeutics, Inc. on August 11, 2020**
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated August 11, 2020**

** 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, thereunto 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: August 11, 2020

Larimar Therapeutics Reports Second Quarter 2020 Operating and Financial Results

- *Merger between Chondrial Therapeutics and Zafgen completed and company began operating as Larimar Therapeutics*
- *Phase 1 clinical trial of CTI-1601 for treatment of Friedreich's ataxia restarts after delay due to COVID-19 related restrictions*
- *$80 million private placement financing completed with biotechnology-focused institutional investors*
- *Positive opinion on Orphan Drug Designation for CTI-1601 from the European Medicines Agency Committee for Orphan Medicinal Products*
- *New Board Chair, Chief Medical Officer and Chief Financial Officer appointed*

Bala Cynwyd, PA – August 11, 2020 – Larimar Therapeutics, Inc. (Nasdaq:LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its second quarter 2020 operating and financial results.

"The second quarter was a transformative period for Larimar as we completed the merger between Chondrial and Zafgen, strengthened our leadership team and raised significant capital. We believe these accomplishments have well positioned us to execute our strategy of developing treatments for complex rare diseases using our novel cell penetrating peptide technology platform," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar Therapeutics. "We have sustained the momentum created by the merger with two recent important milestones, resumption of our Phase 1 clinical trial for CTI-1601 for the treatment of Friedreich's ataxia (FA) which allows our Phase 1 program to continue moving forward, and receipt of a positive opinion on orphan drug designation for CTI-1601 from the European Medicines Agency (EMA) Agency Committee for Orphan Medicinal Products (COMP)."

Second Quarter and Subsequent Highlights

- In May 2020, Larimar announced the completion of the reverse merger between Chondrial Therapeutics, Inc. and Zafgen, Inc. The combined, publicly traded clinical-stage biotechnology company began operating under the name Larimar Therapeutics, Inc. and its shares commenced trading on the Nasdaq Global Market on May 29, 2020, under the ticker symbol "LRMR."
- In May 2020, Larimar completed a private placement of common stock and pre-funded warrants to purchase common stock for $80 million of gross proceeds before placement agent fees and expenses. The financing was led by Cowen Healthcare Investments, and includes participation from biotechnology specialist funds Acuta Capital, funds managed by Janus Henderson Investors, Logos Capital, OrbiMed, RA Capital Management, and Vivo Capital, along with other healthcare-focused institutional investors. These new investors in the financing, along with Deerfield Management, the company's largest pre-financing investor, and Atlas Ventures created a strong institutional shareholder base for the company. Together with approximately $40 million in cash on Zafgen's balance sheet at the time of the merger, the combined company had approximately $116 million in cash immediately following the completion of the merger and the private placement.

- In May 2020, Larimar announced the appointment of Joseph Truitt as Chair of its Board of Directors. Larimar's board of directors also includes Peter Barrett, PhD, Carole S. Ben-Maimon, MD, Thomas O. Daniel, MD, Tom Hamilton, Jonathan Leff and Frank E. Thomas. In addition, in May 2020, the company appointed Nancy Ruiz, MD, FACP, FIDSA, as Chief Medical Officer and Michael Celano as Chief Financial Officer.
- In July 2020, Larimar resumed dosing of patients in its Phase 1 clinical trial to evaluate the safety and tolerability of single ascending doses of CTI-1601 for the treatment of FA, allowing the program to continue moving forward. The trial was previously delayed due to the impact of the COVID-19 pandemic. Topline results are expected in the first half of 2021.
- In July 2020, the EMA COMP issued a positive opinion on the company's application for orphan drug designation for CTI-1601. Larimar expects that the European Commission, based on this positive opinion of the COMP, will formally grant the orphan drug designation for the European Union this year.

Second Quarter 2020 Financial Results

As of June 30, 2020, the Company had cash, cash equivalents, and marketable debt securities totaling $113.7 million.

The Company reported a net loss for the second quarter of 2020 of $11.3 million, or $1.21 per share, compared to a net loss of $3.7 million, or $0.61 per share, for the second quarter of 2019.

Research and development expenses for the second quarter of 2020 were $8.9 million compared to $3.1 million for the second quarter of 2019. The increase in research and development expenses compared to the prior year period was primarily due to an increase in external development costs for CTI-1601, an increase in personnel related costs due to headcount additions in our research and development functions and an increase in stock-based compensation.

General and administrative expenses for the second quarter of 2020 were $2.5 million, compared to $0.6 million for the second quarter of 2019. The increase in general and administrative expenses as compared to the prior year period was primarily due to an increase in professional fees resulting from the reverse merger and the costs of operating as a public company, an increase in legal fees associated with intellectual property filings and an increase in stock-based compensation.

About CTI-1601

CTI-1601 is a recombinant fusion protein intended to deliver human frataxin into the mitochondria of patients with Friedreich's ataxia (FA) who are unable to produce enough of this essential protein. Currently in a Phase 1 clinical trial in the U.S., CTI-1601 has been granted Rare Pediatric Disease designation, Fast Track designation and Orphan Drug designation by the U.S. Food and Drug Administration (FDA). Topline results from the Phase 1 clinical program are planned for the first half of 2021.

About Friedreich's ataxia

Friedreich's ataxia (FA) is a rare, progressive, multi-symptom genetic disease that typically presents in mid-childhood and affects the functioning of multiple organs and systems. The most common inherited ataxia, FA is a debilitating neurodegenerative disease resulting in multiple symptoms including

progressive neurologic and cardiac dysfunction – poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing and speech. FA affects an estimated 4,000-5,000 individuals living in the United States and approximately 20,000 in the European Economic Area and United Kingdom. FA results from a deficiency of the mitochondrial protein, frataxin (FXN), which is found in cells throughout the body. To date, there are no medical treatment options approved for patients with FA.

About Larimar Therapeutics

Larimar Therapeutics, Inc. (Nasdaq:LRMR), is a clinical-stage biotechnology company focused on developing treatments for complex rare diseases. The company's lead compound, CTI-1601, is currently being evaluated in a Phase 1 clinical program in the U.S. as a potential treatment for Friedreich's ataxia, a rare and progressive genetic disease. 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 the receipt of Orphan Drug Designation for the EU for CTI-1601 in FA from the European Commission, Larimar's ability to develop and commercialize CTI-1601 and other planned product candidates, Larimar's planned research and development efforts, 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, the success, cost and timing of Larimar's product development activities, studies and clinical trials; the ongoing impact of the COVID-19 pandemic on Larimar's clinical trial timelines, ability to raise additional capital and general economic conditions; Larimar's ability to optimize and scale CTI-1601's manufacturing process; Larimar's ability to obtain regulatory approval for CTI-1601 and future product candidates; the fact that the European Commission may not grant Orphan Drug Designation for the EU for CTI-1601 in FA or may do so in a longer than anticipated timeframe; Larimar's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators, and 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 the Company 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 views 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.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$112,673	$ 1,009
Marketable debt securities	1,011	—
Prepaid expenses and other current assets	5,427	3,741
Total current assets	119,111	4,750
Property and equipment, net	675	274
Operating lease right-of-use assets	4,252	87
Restricted cash	1,339	—
Other assets	80	90
Total assets	$125,457	$ 5,201
Liabilities and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,258	$ 3,539
Accrued expenses	3,796	2,259
Operating lease liabilities, current	591	97
Total current liabilities	6,645	5,895
Operating lease liabilities	6,268	—
Total liabilities	12,913	5,895
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of June 30, 2020 and December 31, 2019; no shares issued and outstanding as of June 30, 2020 and December 31, 2019	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2020 and December 31, 2019; 15,356,206 and 6,091,250 shares issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	15	6
Additional paid-in capital	153,668	22,432
Accumulated deficit	(41,136)	(23,132)
Accumulated other comprehensive loss	(3)	—
Total stockholders' equity (deficit)	112,544	(694)
Total liabilities and stockholders' equity (deficit)	$125,457	$ 5,201

LARIMAR THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	8,907	3,128	13,914	7,350
General and administrative	2,492	576	4,159	1,078
Total operating expenses	11,399	3,704	18,073	8,428
Loss from operations	(11,399)	(3,704)	(18,073)	(8,428)
Other income, net	69	—	69	—
Net loss	$ (11,330)	$ (3,704)	$ (18,004)	$ (8,428)
Net loss per share, basic and diluted	$ (1.21)	$ (0.61)	$ (2.33)	$ (1.38)
Weighted average common shares outstanding, basic and diluted	9,381,412	6,091,250	7,736,331	6,091,250

Exhibit 99.2

Larimar Therapeutics

Corporate Presentation

August 2020 (8.11.20 update)

Forward Looking Statements

This presentation contains forward-looking statements that are based on the Company's beliefs and assumptions 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 potential benefits of the merger, the use of proceeds of the private placement, Company's ability to develop and commercialize CTI-1601 and other planned product candidates, Company's planned research and development efforts, and other matters regarding 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 success, cost and timing of the Company's product development activities, studies and clinical trials; the ongoing impact of the COVID-19 pandemic on the Company's clinical trial timelines, ability to raise additional capital and general economic conditions; the Company's ability 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 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 Form 8-K/A filed on June 26, 2020, and the Company's subsequent 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 Securities and Exchange Commission 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.

Larimar Therapeutics Introduction

- **Created by reverse merger of Zafgen and Chondrial Therapeutics**
- **Traded under ticker "LRMR"**
- **Merger accompanied by $80 million private placement – World-class life science investor shareholder base**
  - Financing led by Cowen Healthcare Investments with Acuta, Janus, Logos, OrbiMed, RA Capital and Vivo
  - Continued shareholder support of Deerfield Management and Atlas Ventures
  - Company has ~ $116 million in cash from merger and financing
- **Leadership team bolstered**
  - Joseph Truitt – Board chair
  - Nancy Ruiz, MD, FACP, FIDSA – CMO
  - Michael Celano - CFO

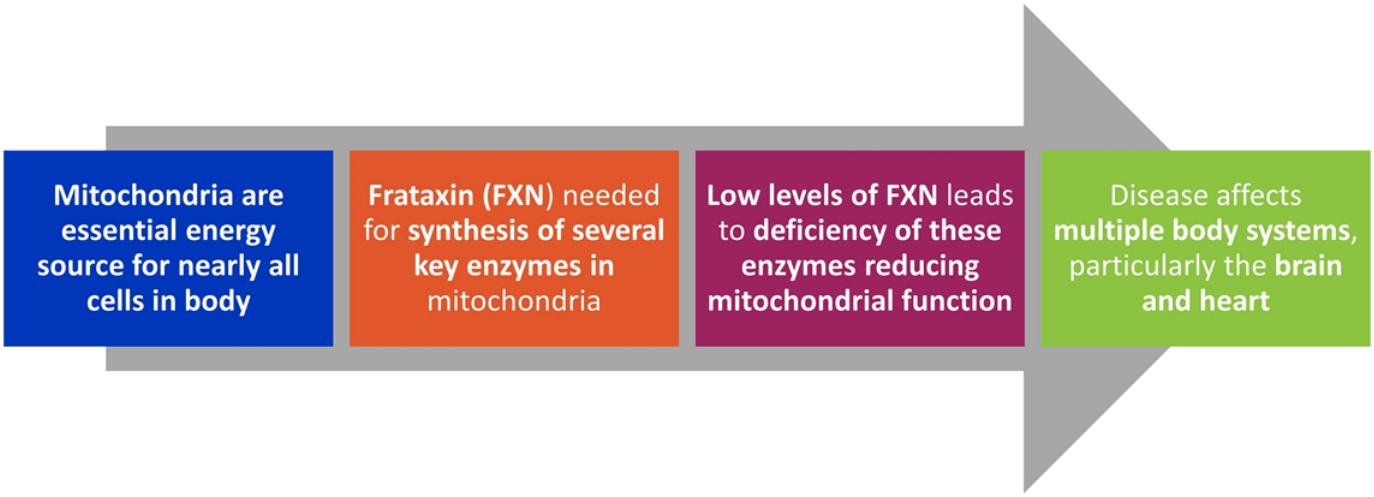
Larimar Therapeutics

Investment Highlights

- Clinical-stage biotech with **novel protein replacement therapy platform** to address untreated, serious and complex rare diseases
- Lead candidate, **CTI-1601**, in **ongoing Phase 1 clinical development** for treatment of **Friedreich's ataxia (FA)**
  - To our knowledge, **frataxin (FXN) protein replacement therapy** is only protein replacement therapy in clinical development
  - Nonclinical studies demonstrated **promising results in several models of FA**, including heart, brain and muscle function, and overall survival
  - Multiple FDA designations: **Orphan Drug, Rare Pediatric Disease, Fast Track**; Recent **EMA Committee for Orphan Medicinal Products positive opinion for Orphan Drug designation**
  - **Topline Phase 1 data expected in 1H 2021**
- **Experienced** leadership team
- **Extensive IP** with 12 years market exclusivity expected if approved; patents pending around efficacy biomarkers
- **Strong balance sheet** with ~ $114M in cash and investments at 6/30/20; based on current estimates of funding needs, cash expected to last for ~ 2 years into first half of 2022

Larimar Therapeutics

Frataxin (FXN): Crucial for Mitochondrial Function



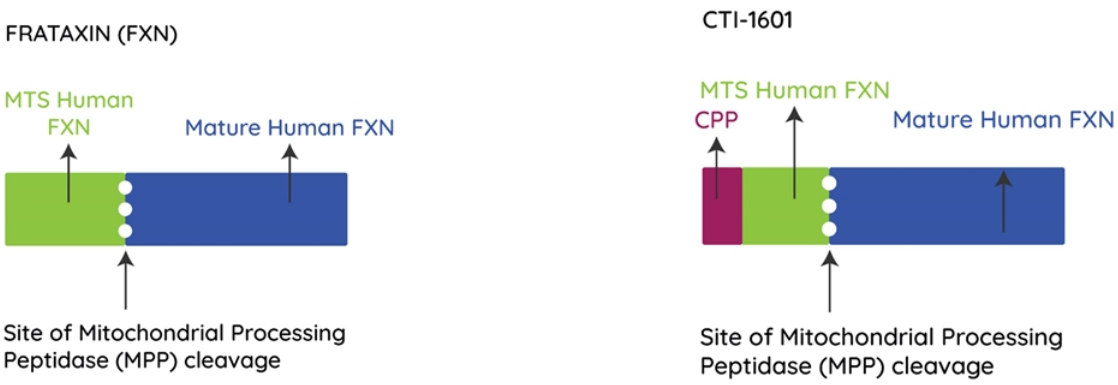
Larimar Therapeutics

Friedreich's ataxia (FA): Rare and Progressive Disease

- **Rare disease** caused by genetic defect resulting in **abnormally low levels of FXN**
  - Affects ~5,000 patients in U.S. and ~20,000 patients in EU
- **Onset:** Age of **onset correlated with severity and speed of progression**
- **>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 has already occurred**
- **Progression of disease:** Symptoms worsen and patients are eventually confined to wheelchair
- **Life expectancy of 30-50 years**, early death usually caused by **heart disease**
- **Treatment limited to symptom management; currently no approved therapies**

Larimar Therapeutics

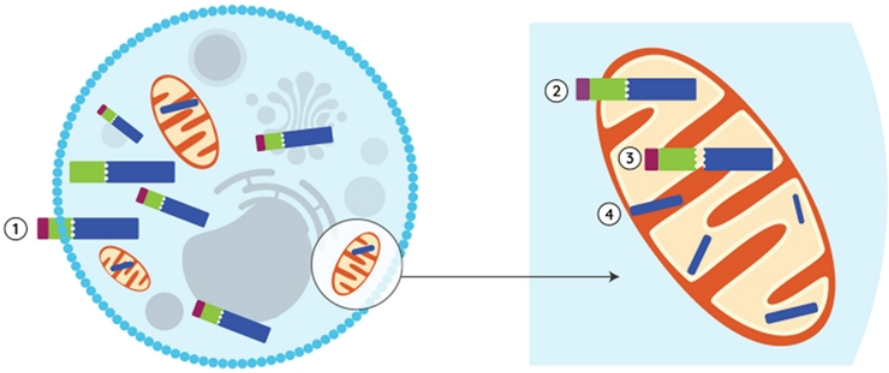
CTI-1601 – Designed to Deliver Frataxin (FXN)



CTI-1601 similar to frataxin except it has a CPP attached on end to allow it to move into cell and mitochondria

Larimar Therapeutics

CTI-1601 – Delivering Frataxin to the Mitochondria



1. CTI-1601 crosses the cell membrane into cytoplasm
2. CTI-1601 crosses the mitochondrial membrane
3. Mitochondrial Processing Peptide (MPP) cleaves CTI-1601. MTS and CPP leave cell mitochondria
4. Mature human frataxin remains within the mitochondria to function

Larimar Therapeutics

CTI-1601 – POC Achieved Through Multiple Non-Clinical Studies

- ✓ **Extended survival** in a well-characterized nonclinical model of FA
- ✓ **Prevented ataxic gait** in another nonclinical model of FA
- ✓ Demonstrated **capability of delivering sufficient amounts of FXN** to mitochondria
- ✓ Prevented left **ventricle dilation and maintained function**
- ✓ **Safe and well tolerated** in multiple species

Nonclinical efficacy and PD data support of continued development to potentially replace FXN in patients with FA

Larimar Therapeutics

CTI-1601 Extends Survival in FXN-deficient KO Mice

Initial Proof of Concept for FXN Replacement Therapy in FA

TAT-FXN was administered 10 mg/kg SC every other day

✓CTI-1601 extended survival in a well-characterized cardiac mouse model of FRDA

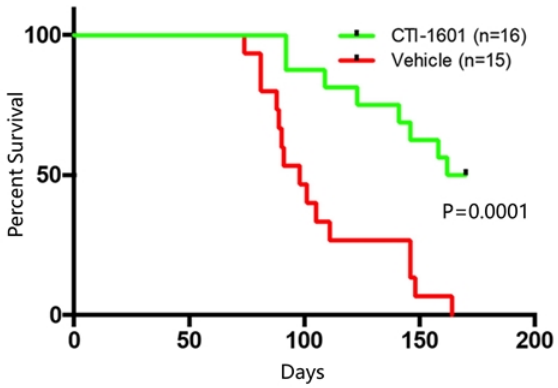
Median Survival of MCK-Cre FXN-KO Mice

- 166 days (CTI-1601) vs 98.0 days (Vehicle)

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, rescuing a severe disease phenotype



Larimar Therapeutics

CTI-1601 Prevents Neurological Phenotype and 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 development of ataxic gait**

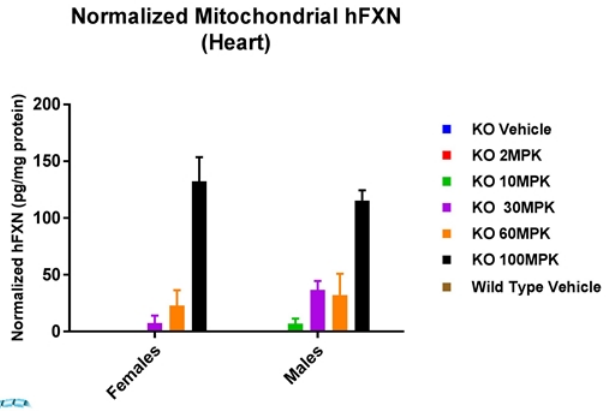
✓ Treated mice **survive longer** than untreated mice

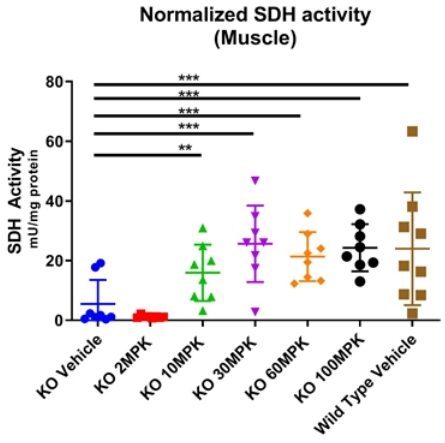
✓ Human frataxin **present in brain, dorsal root ganglia and spinal cord**

Larimar Therapeutics

CTI-1601 Effectively Traffics to Mitochondria; Delivers hFXN

- ✓ hFXN concentration within mitochondria increases in a dose-dependent manner
- ✓ Given subcutaneously, CTI-1601 functionally replaces hFXN in mitochondria of KO mice
- ✓ SDH* activity 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

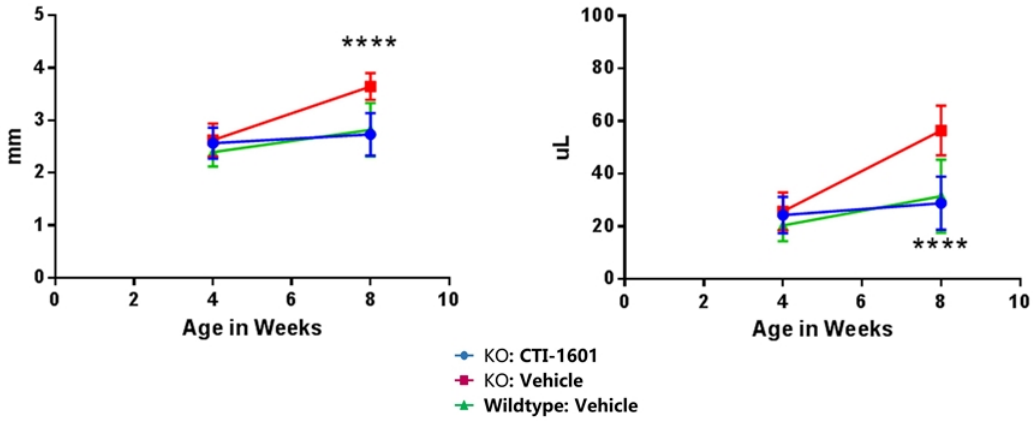




*Succinate dehydrogenase

CTI-1601 Prevents Left Ventricle (LV) *Dilation*

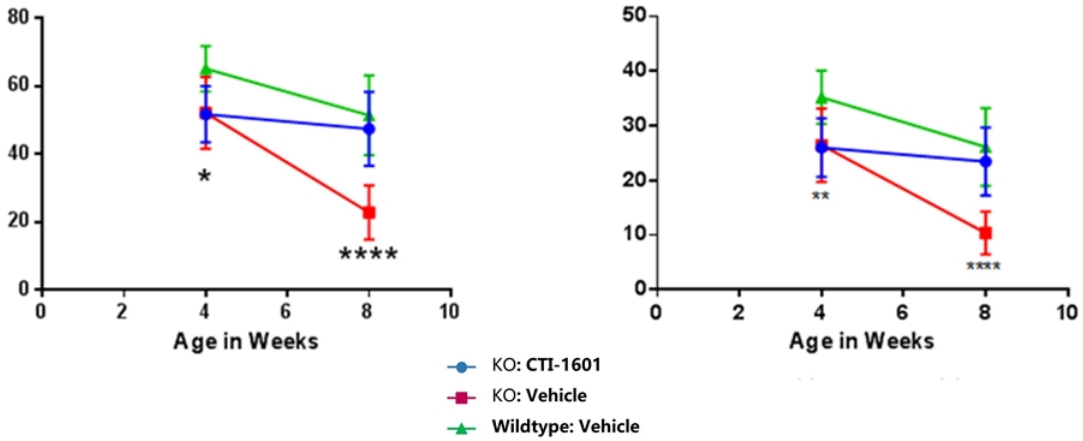
- ✓ Left ventricular volume increases in systole in untreated mice by 8 weeks (after 4 weeks of dosing with vehicle), but remains similar to wildtype when treated with CTI-1601
- ✓ **CTI-1601-treated mice similar to controls**; echocardiogram shows significant differences between KO:Vehicle and KO:CTI-1601 treated mice



Larimar Therapeutics

CTI-1601 Preserves Left Ventricle (LV) *Function*

- ✓ LV function drops significantly in untreated mice by week 8
- ✓ **CTI-1601-treated mice similar to controls**; echocardiogram again shows significant differences between KO:Vehicle and KO:CTI-1601 treated mice



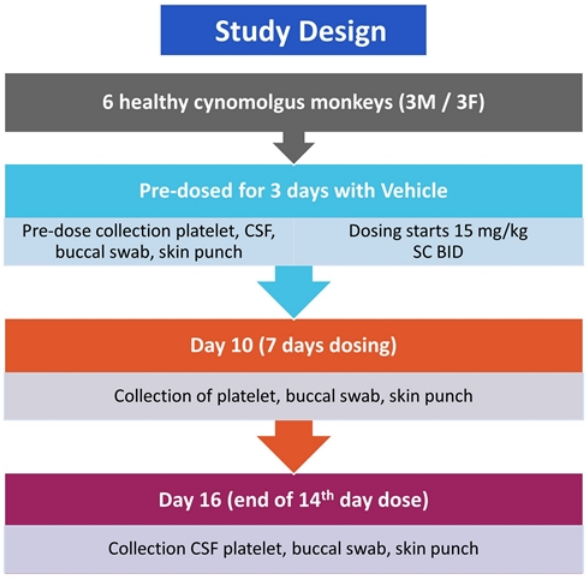
Larimar Therapeutics

Human Frataxin Distributed Into All Tissues Tested

Tissues Examined, By Study	
Study Vehicle	**Human Frataxin Distribution**
Rats	Brain, Heart, Liver
Neuro KO Mice	Brain, Dorsal Root Ganglia, Spinal Cord
Cardiac KO Mice	Mitochondria of Skeletal Muscle and Cardiomyocytes
Cynomolgus Monkey	CSF, Skin, Buccal Cells, Platelets

Larimar Therapeutics

PK/PD Study in Healthy Cynomolgus Monkeys



- ✓ 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 processed hFXN** are found after **14 days** in the cerebrospinal fluid of monkeys, suggesting **CNS penetration**
- ✓ **Preliminary results from 90 Day GLP Toxicology support these findings**

Larimar Therapeutics

CTI-1601: Safe and Well Tolerated in Multiple Animal Models

Cynomolgus Monkey

Injection Site Observations

- Some injection sites raised and firm; increased injection site pathology at higher doses most likely due to local irritation

Systemic Toxicity Analysis

- No other clinical observations or treatment-related changes in food consumption, body weight or organ weight
- No systemic histopathological findings

Sprague Dawley Rat

Injection Site Observations

- Some injection sites showed irritation, firmness, inflammation at higher doses

Systemic Toxicity Analysis

- No significant clinical observations or clinical pathology results
- No systemic histopathological findings

No systemic clinical or pathological observations related to CTI-1601 in early GLP studies

Larimar Therapeutics

CTI-1601: Ongoing Phase 1 Clinical Program in FA Patients

- Dosing regimen: Single Ascending Doses given SC (SAD); Multiple Ascending Doses given SC (MAD)
- Patient dosing began December 2019
  - Three cohorts dosed- SAD Phase 1
- Number of subjects:
  - SAD: approximately 32-34 subjects (currently 4 cohorts planned)
  - MAD: 24-30 subjects (currently 3 cohorts planned)
- Outcome measures:
  - Primary: Safety and tolerability
  - Secondary: PK; PD (hFXN, gene expression in buccal swab and blood); multiple exploratory
- Sufficient drug supply for Phase 1 clinical program (5 GMP batches)
- **Topline results from Phase 1 clinical program expected in 1H 2021**

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Marketing Exclusivity and Accelerated Programs

FDA has granted CTI-1601:

- ✓ Orphan Drug Status
  - Granted July 2017
  - Eligible for 7 years market exclusivity upon approval
- ✓ Fast Track Designation
  - Granted November 2019
  - Actions to expedite development and review
- ✓ Rare Pediatric Disease Designation
  - Granted December 2019
  - Eligible for voucher upon BLA approval

EMA Committee for Orphan Medicinal Products (COMP) recently issued positive opinion on application for orphan drug designation for CTI-1601

Expect European Commission will formally grant the designation this year

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Strong IP Position with Expected Market Exclusivity

- TAT- FXN **composition of matter and methods of use patents** expected to provide protection to 2024 and 2025, with **provisional patents specific to CTI-1601** potentially **extending protection to 2040**
  - Exclusive licenses from Wake Forest and Indiana University
- **Biologic 12 years market exclusivity** expected if CTI-1601 approved
- **Orphan drug designation** that can enable **7 years exclusivity** if CTI-1601 approved
- CTI-1601 **biomarkers, analytical methods and tools** provisional patents
  - PCT and US patent applications for biomarkers of FXN replacement therapy
  - Provisional for method to quantify FXN and FXN fusion proteins
  - Provisional for activity assays and other analytical tools
- Additional pipeline **platform technology provisional patents**

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
  - Assists 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 called The Voice of the Patient

FARA Friedreich's Ataxia Research Alliance

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Summary

- **Novel protein replacement therapy platform**
- **CTI-1601** in **Phase 1 clinical development** for treatment of **Friedreich's ataxia** (FA)
  - **Topline Phase 1 data expected in 1H 2021**
- **Experienced** leadership team
- **Extensive IP**
- **Strong balance sheet** of ~ $114M in cash and investments as of 6/30/20

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