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): November 10, 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 November 10, 2020, Larimar Therapeutics, Inc. (the "***Company***") announced its financial results and operational highlights for the third quarter ended September 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 November 10, 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 November 10, 2020*
99.2	Larimar Therapeutics, Inc. Corporate Presentation, dated November 10, 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: November 10, 2020

Larimar Therapeutics Reports Third Quarter 2020 Operating and Financial Results

Phase 1 trials evaluating CTI-1601 as a treatment for Friedreich's ataxia on track for topline data in 1H 2021

Received orphan drug designation for CTI-1601 from the European Commission

Cash, cash equivalents, and marketable securities of $102.3 million as of September 30, 2020

Bala Cynwyd, PA – November 10, 2020 – Larimar Therapeutics, Inc. (Nasdaq:LRMR), a clinical-stage biotechnology company focused on developing treatments for complex rare diseases, today reported its third quarter 2020 operating and financial results.

"I am very pleased with the progress Larimar has made over the past few months, as we achieved key clinical, regulatory, and corporate milestones that have left us well positioned for continued growth," said Carole Ben-Maimon, MD, President and Chief Executive Officer of Larimar Therapeutics. "In the third quarter, we continued to advance our lead program in development for Friedreich's ataxia (FA), resuming our Phase 1 trials evaluating CTI-1601 in patients with FA. Though the trials were delayed due to the impact of the COVID-19 pandemic, they are now on track for topline results in the first half of 2021."

Dr. Ben-Maimon continued, "Alongside this clinical achievement, we also complemented previous regulatory designations from the U.S. Food and Drug Administration (FDA) with an orphan drug designation from the European Commission and strengthened Larimar's leadership team with the formation of a Scientific Advisory Board (SAB). Members of the SAB are key opinion leaders in the fields of rare disease, pediatrics, and mitochondrial disease who will provide strategic scientific guidance as we build our pipeline."

Third Quarter and Subsequent Highlights

- In July 2020, Larimar resumed its Phase 1 clinical trials to evaluate the safety and tolerability of CTI-1601 for the treatment of FA with the dosing of its third cohort. The trials were previously delayed due to the impact of the COVID-19 pandemic. Topline data from the trials are expected in the first half of 2021.
- In August 2020, the European Commission granted an orphan drug designation for CTI-1601 for the treatment of FA. This designation complements previously received Orphan Drug, Fast Track, and Rare Pediatric Disease designations from the FDA.
- In October 2020, Larimar announced the formation of its SAB. The SAB will provide strategic scientific guidance to company management and is comprised of key opinion leaders in the fields of rare disease, pediatrics, and mitochondrial disease. Members of the SAB include: Russell (Rusty) Clayton, DO; Marni J. Falk, MD; Giovanni Manfredi, MD, PhD; Mark Payne, MD; and Marshall Summar, MD.

Third Quarter 2020 Financial Results

As of September 30, 2020, the Company had cash, cash equivalents, and marketable debt securities totaling $102.3 million.

The Company reported a net loss for the third quarter of 2020 of $10.3 million, or $0.64 per share, compared to a net loss of $8.6 million, or $1.42 per share, for the third quarter of 2019.

Research and development expenses for the third quarter of 2020 were $6.9 million compared to $8.0 million for the third quarter of 2019. The decrease in research and development expenses compared to the prior year period was primarily driven by lower clinical supply manufacturing costs and toxicology studies partially offset by an increase in external clinical trial expenditures, an increase in personnel related costs due to headcount additions in our research and development functions and an increase in stock-based compensation expense associated with stock option grants made in July 2020.

General and administrative expenses for the third quarter of 2020 were $3.4 million, compared to $0.6 million for the third quarter of 2019. The increase in general and administrative expenses as compared to the prior year period was primarily driven by an increase in professional fees and insurance costs that are primarily due to the costs of operating as a public company, an increase in personnel related costs due to increased headcount, an increase in stock-based compensation associated with stock option grants made in July 2020 and an increase in facilities costs.

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 Phase 1 clinical trials 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) and orphan drug designation by the European Commission. 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 (FA). 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 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;; 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 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 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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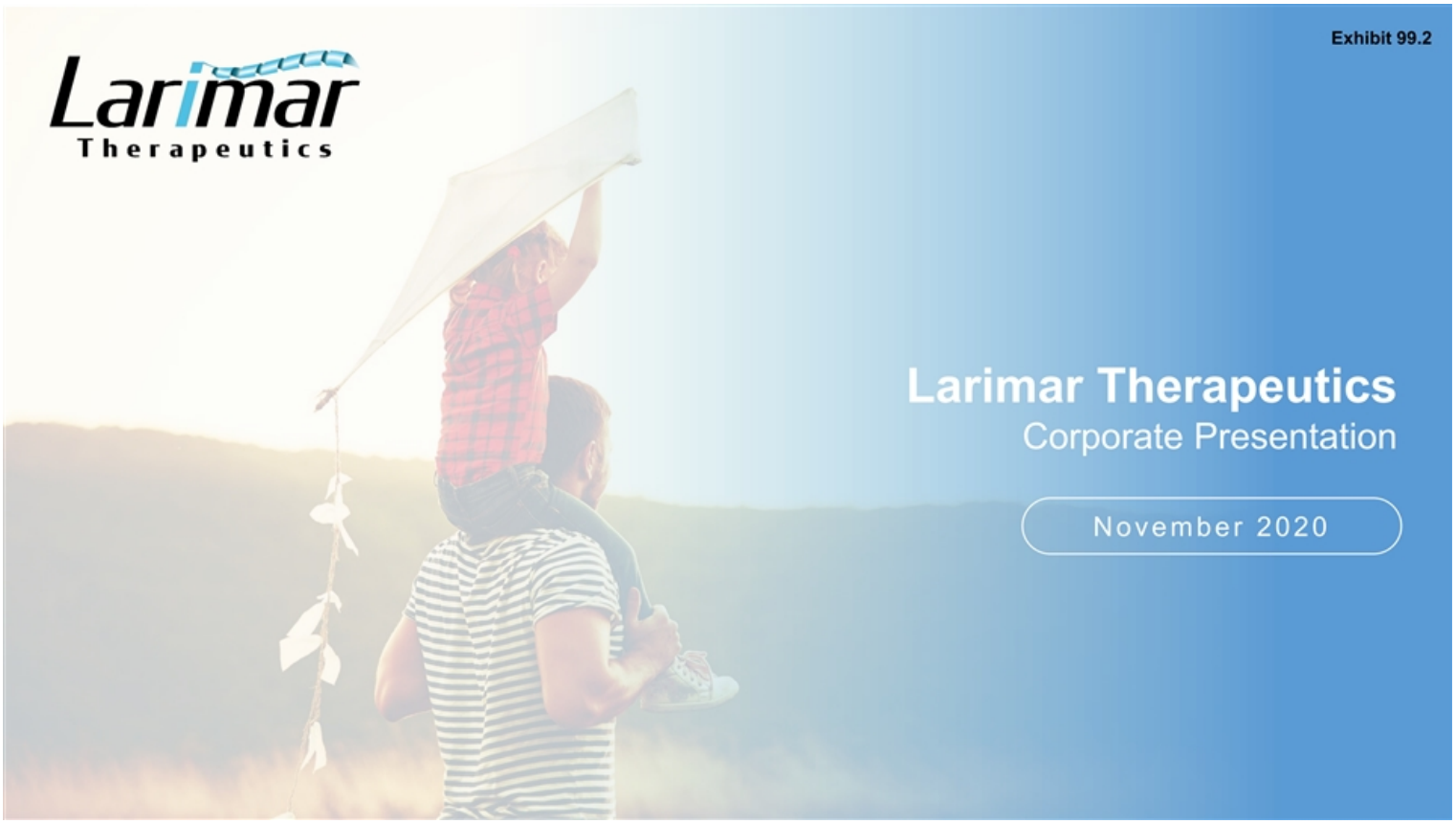
LARIMAR THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 101,308	$ 1,009
Marketable debt securities	1,001	—
Prepaid expenses and other current assets	5,507	3,741
Total current assets	107,816	4,750
Property and equipment, net	630	274
Operating lease right-of-use assets	4,094	87
Restricted cash	1,339	—
Other assets	78	90
Total assets	$ 113,957	$ 5,201
Liabilities and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 1,269	$ 3,539
Accrued expenses	3,384	2,259
Operating lease liabilities, current	525	97
Total current liabilities	5,178	5,895
Operating lease liabilities	6,138	—
Total liabilities	11,316	5,895
Commitments and contingencies (See Note 9)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of September 30, 2020 and December 31, 2019; no shares issued and outstanding as of September 30, 2020 and December 31, 2019	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of September 30, 2020 and December 31, 2019; 15,356,206 and 6,091,250 shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively	15	6
Additional paid-in capital	154,038	22,432
Accumulated deficit	(51,410)	(23,132)
Accumulated other comprehensive loss	(2)	—
Total stockholders' equity (deficit)	102,641	(694)
Total liabilities and stockholders' equity (deficit)	$ 113,957	$ 5,201

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 6,919	$ 8,034	$ 20,833	$ 15,384
General and administrative	3,416	594	7,575	1,672
Total operating expenses	10,335	8,628	28,408	17,056
Loss from operations	(10,335)	(8,628)	(28,408)	(17,056)
Other income, net	61	—	130	—
Net loss	$ (10,274)	$ (8,628)	$ (28,278)	$ (17,056)
Net loss per share, basic and diluted	$ (0.64)	$ (1.42)	$ (2.69)	$ (2.80)
Weighted average common shares outstanding, basic and diluted	15,984,609	6,091,250	10,505,826	6,091,250

Exhibit 99.2



Larimar Therapeutics

Corporate Presentation

November 2020

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 expectations and assumptions regarding the future of our business, 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.

Investment Highlights

Novel protein replacement therapy platform designed to address complex rare diseases

CTI-1601	Lead candidate CTI-1601 is a recombinant fusion protein being developed to deliver human frataxin to the mitochondria for the treatment of Friedreich's ataxia (FA)
Phase 1 clinical development	Placebo-controlled Phase 1 clinical trials in Friedreich's ataxia patients ongoing with topline data expected 1H 2021
Regulatory benefits	Orphan Drug (US & EU), Rare Pediatric Disease, and Fast Track designations; May be eligible for priority review voucher and 12 years of market exclusivity upon approval, if received
Strong balance sheet	~$102M in cash as of 9/30/20 with projected runway into first half of 2022
High quality shareholder base	Includes investors such as Deerfield, Cowen, RA Capital, OrbiMed, Acuta, Vivo, Logos, Altium, Janus and Atlas

Larimar Therapeutics

Scientific Advisory Board

Russell Clayton, DO (Chairman)

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

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

Giovanni Manfredi, MD, PhD

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

Professor of Neuroscience at Weill Cornell Medicine.

Mark Payne, MD

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

Professor of Pediatrics at Indiana University School of Medicine

Marshall Summar, MD

Chief of the Division of Genetics and Metabolism, Director of the Rare Disease Institute, and Margaret O'Malley Chair of Genetic Medicine at Children's National Hospital

Marni J. Falk, MD

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

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

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 considered[1]

>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
- Progressive disease: Symptoms worsen and patients are eventually confined to 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

Market Opportunity

Prevalence

5,000 patients in US and 20,000 patients in EU

Additional affected populations in Australia and Brazil

Highly sophisticated and active advocacy group (FARA) driving quest for treatments

Dosing

Replacement therapy may be needed throughout life to maintain frataxin (FXN) levels

Disease is progressive and irreversible; initiating therapy early and continuing replacement therapy throughout life may be a necessity

Regulatory Benefits

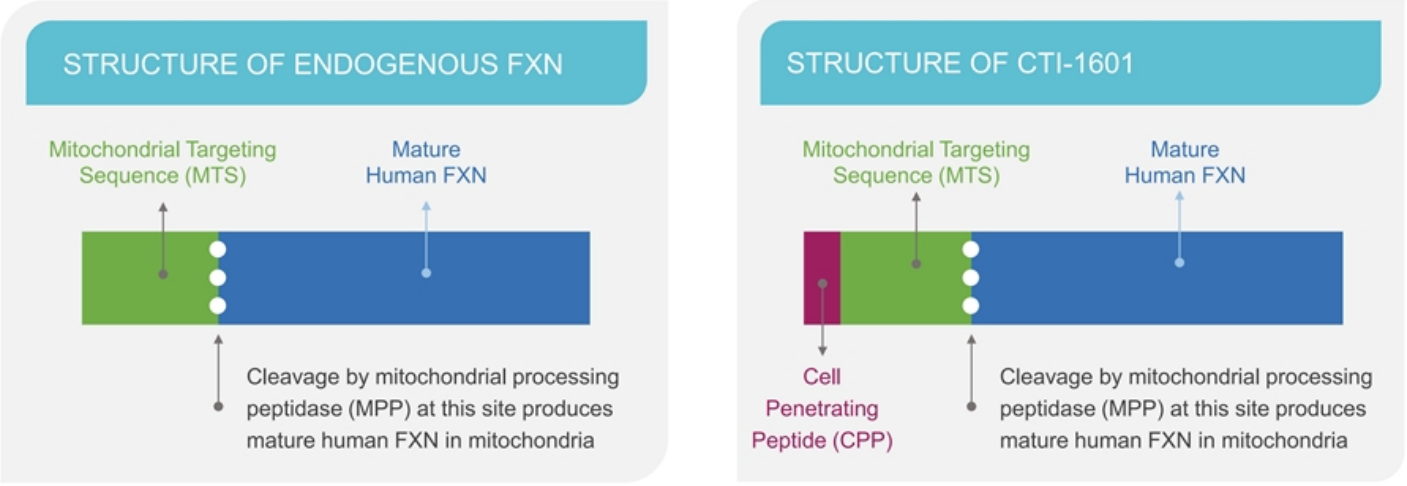
Well known by FDA; "Voice of the Patient" report for FA was released in 2017

Upon Biologics License Application (BLA) approval, CTI-1601, if approved, may be eligible for:

- 12 years market exclusivity
- Rare pediatric disease priority review voucher

CTI-1601 is Being Developed to Deliver Frataxin (FXN)

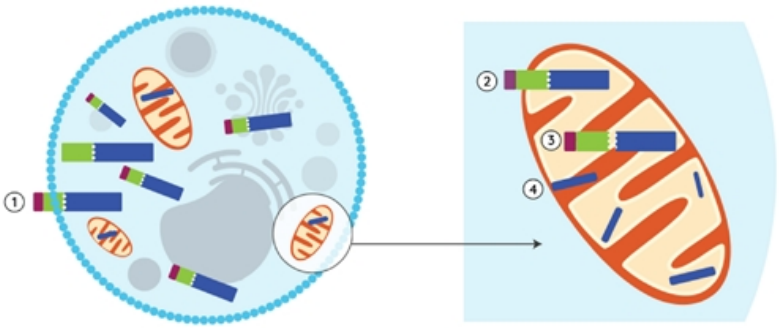
CTI-1601 Maintains the Cleavage Site Between the MTS and Mature Human FXN



The CPP allows CTI-1601 to traverse the cell and mitochondrial membranes where the CPP and MTS are removed by mitochondrial processing peptidase to produce mature human FXN

Larimar Therapeutics

CTI-1601 – Delivering Frataxin to the Mitochondria



01 CPP allows CTI-1601 to traverse the cell membrane into the cytoplasm

02 CPP allows CTI-1601 to traverse the mitochondrial membrane

03 MPP cleaves CTI-1601. MTS and CPP leave cell mitochondria

04 Mature human frataxin remains within the mitochondria to function

Larimar Therapeutics

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

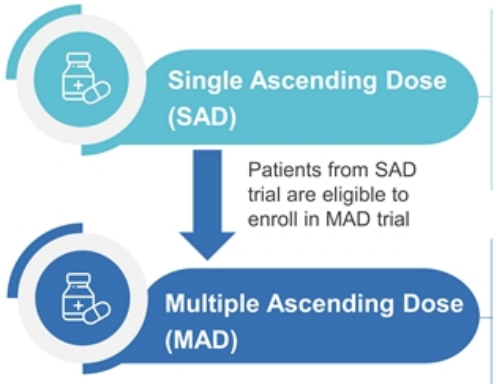
- Assistance with patient recruitment and education
- Access to Global Patient Registry with demographic and clinical information on more than 1,000 FA patients
- Sponsored a Patient-Focused Drug Development Meeting in 2017 resulting in a publication titled "The Voice of the Patient"

FARA Friedreich's Ataxia Research Alliance

CTI-1601: Phase 1 Clinical Program in FA

Phase 1 Development Plan

- Two Double-blind, Placebo Controlled Dosing Studies
- Patient dosing began December 2019
- Safety Review Committee assesses all blinded data between each cohort to ensure patient safety
- **Topline results expected in 1H 2021**



Number of subjects: 32-34
Dose levels: 25mg, 50mg, 75mg and 100mg (subcutaneous administration)
Treatment Duration: 1 day
Status: After dosing 2 cohorts, paused in March due to COVID-19. Restarted in July with 3rd cohort
1º Endpoint: Safety and tolerability
2º Endpoints: PK; PD; hFXN levels (gene expression in buccal swab and blood); multiple exploratory

Number of Subjects: Currently planning for 3 cohorts, 24-30 subjects
Dose Range: To be determined based on SAD data and adjusted continuously based on PK/PD data
Treatment Regimen: Multiple increasing doses administered subcutaneously over 14 days
1º Endpoint: Safety and tolerability
2º Endpoints: PK; PD; hFXN levels (gene expression in buccal swab and blood); multiple exploratory

Larimar Therapeutics

Upcoming Clinical Milestones

1H 2021 Topline Phase 1 Data

Future Planned Studies Include:

Open-label extension (OLE) study for patients who participated in SAD or MAD studies

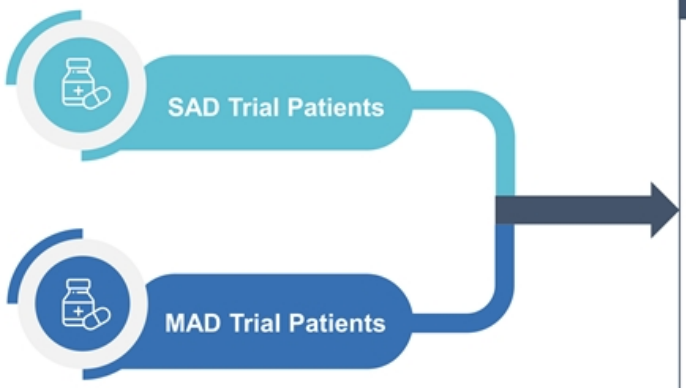
Pediatric MAD Study (followed by an OLE)

Phase 2/3 Double-Blind Placebo-Controlled Study

Larimar Therapeutics

CTI-1601 Open Label Extension Trial

Patients from SAD and MAD trials are eligible to enter an open label extension (OLE)



Multicenter Open Label Extension Trial

Dose Level: To be determined based on PK/PD from SAD and MAD trials

Treatment Duration: Planned for 24 months with any necessary extensions

Number of Subjects: Up to 50

Dose Regimen: To be determined based on PK/PD from SAD and MAD trials

Comparator Arm: Derived from Critical Path Institute Data (includes FACOMS and placebo arms from two FA studies)

1º Endpoint: Safety and tolerability

Key 2º Endpoints: Long-term PD; efficacy assessments

Larimar Therapeutics

CTI-1601: Positive Non-Clinical Data Support Development

Proof-of-Concept Achieved Through Multiple Non-Clinical Studies:

- CTI-1601 extended survival in a well-characterized non-clinical mouse model of FA
- CTI-1601 prevented ataxic gait in another non-clinical mouse model of FA
- Studies demonstrate the ability of CTI-1601 to deliver sufficient amounts of FXN to mitochondria in rodent and non-human primate non-clinical models
- CTI-1601 prevented left ventricle dilation and maintained function in non-clinical mouse models
- CTI-1601 is safe and well tolerated in rats and non-human primates

Larimar Therapeutics

Human Frataxin Distributed Into All Tissues Tested

Tissues Examined	
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	Cerebrospinal fluid, Skin, Buccal Cells, Platelets

CTI-1601 Extends Survival in FXN-deficient KO Mice

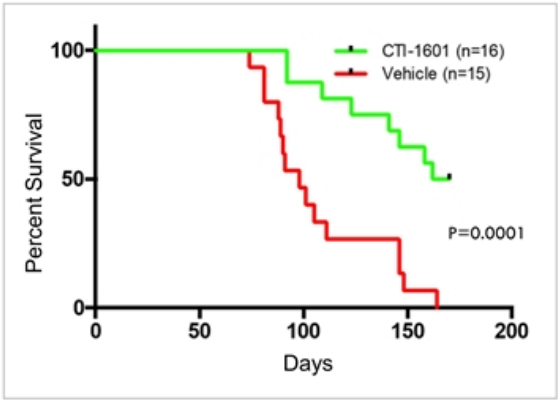
Initial Proof of Concept for FXN Replacement Therapy in Cardiac Mouse Model of FA

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

Larimar Therapeutics

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

In-Vivo Efficacy Data in Neurologic KO Mouse Model

Pvalb-Cre FXN-KO mouse

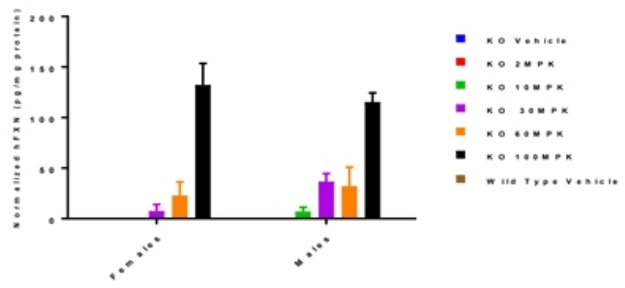
Single dose level: 10 mg/kg CTI-1601 or vehicle given intraperitoneally three times per week

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

CTI-1601 Delivers hFXN to Mitochondria in KO Mice

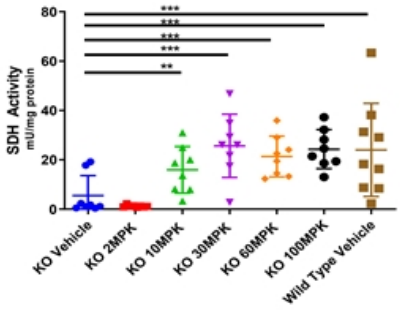
- hFXN concentration within mitochondria increases in a dose-dependent manner
- Given subcutaneously, CTI-1601 functionally replaces hFXN in mitochondria of KO mice
- *Succinate dehydrogenase (SDH) activity, which is indicative of mitochondrial function, increases in a dose-dependent manner after administration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

Normalized Mitochondrial FXN (Heart)



MPK = mg/kg

Normalized SDH Activity (Muscle)



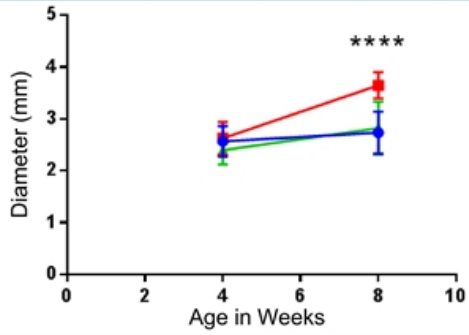
MPK = mg/kg

Larimar Therapeutics

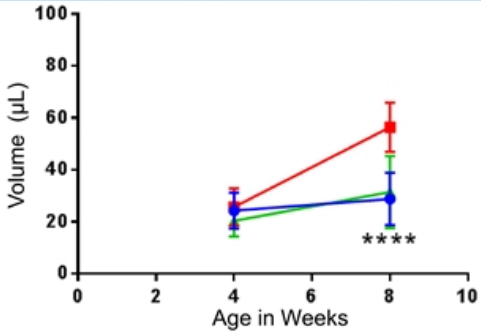
CTI-1601 Prevents Left Ventricle Dilation in KO Mice

- 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 Wildtype: Vehicle

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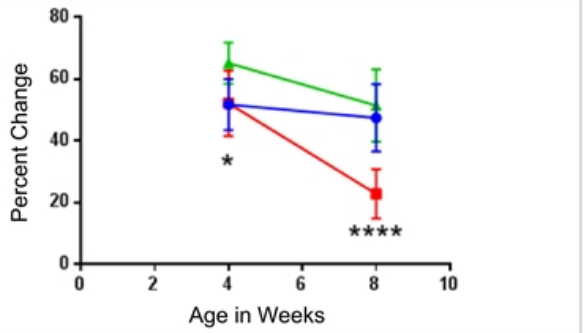
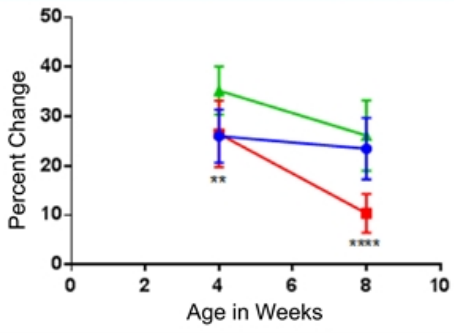
CTI-1601 Preserves Left Ventricle Function in KO Mice

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

Left Ventricle Ejection Function



Left Ventricle Fractional Shortening



KO: CTI-1601 KO: Vehicle Wildtype: Vehicle

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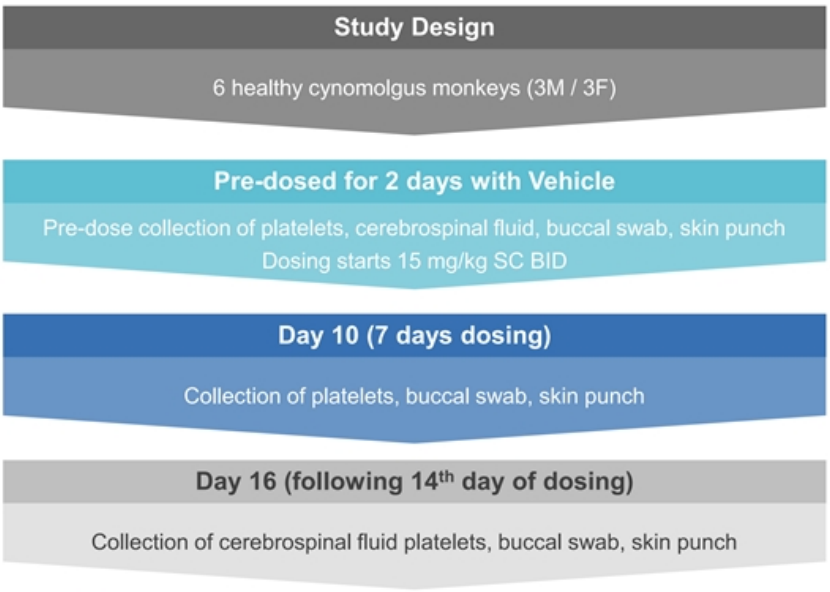
Favorable PK/PD Profile in Healthy Cynomolgus Monkeys



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

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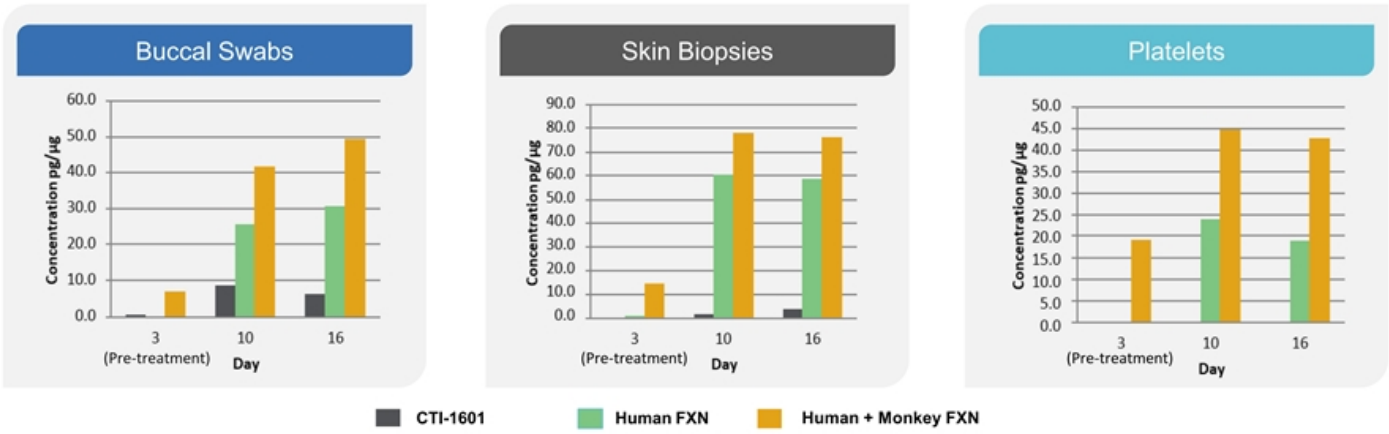
Biodistribution in Healthy Cynomolgus Monkey



- 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

CTI-1601: Safe and Well Tolerated in Early Tox Studies

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 28-day GLP IND-enabling studies

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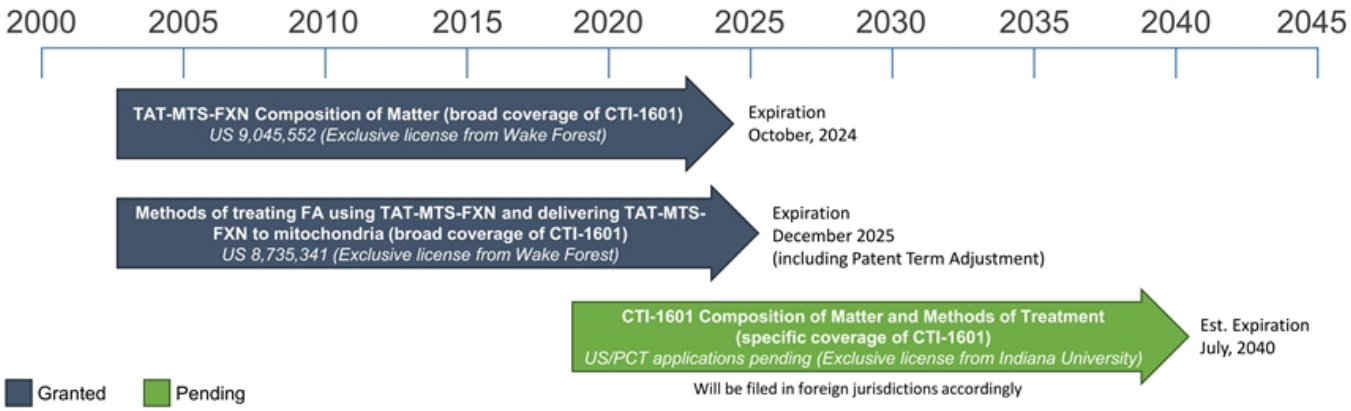
CTI-1601 is Protected by a Strong IP Portfolio

Pending CTI-1601 patent application extends into 2040



Additional intellectual property (IP) protection

- Additional pending applications cover key biomarkers, analytical tools, quantification methods and platform technology
- CTI-1601 is eligible for **12 years of market exclusivity** upon approval in the US (independent of patents)
- CTI-1601 is eligible for at least **10 years of market exclusivity** upon approval in Europe (independent of patents)

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Investment Highlights

Novel protein replacement therapy platform designed to address complex rare diseases

CTI-1601	Lead candidate CTI-1601 is a recombinant fusion protein being developed to deliver human frataxin to the mitochondria for the treatment of Friedreich's ataxia (FA)
Phase 1 clinical development	Placebo-controlled Phase 1 clinical trials in Friedreich's ataxia patients ongoing with topline data expected 1H 2021
Regulatory benefits	Orphan Drug (US & EU), Rare Pediatric Disease, and Fast Track designations; May be eligible for priority review voucher and 12 years of market exclusivity upon approval, if received
Strong balance sheet	~$102M in cash as of 9/30/20 with projected runway into first half of 2022
High quality shareholder base	Includes investors such as Deerfield, Cowen, RA Capital, OrbiMed, Acuta, Vivo, Logos, Altium, Janus and Atlas

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Corporate Presentation
THANK YOU

Leadership Team

Carole Ben-Maimon, MD
Chief Executive Officer

Jennifer Johansson, JD
VP Regulatorv Affairs & Counsel

Michael Celano
Chief Financial Officer

Nancy M. Ruiz, MD, FACP, FIDSA
Chief Medical Officer

David Bettoun, PhD
VP Discovery & Non-clinical R&D

Keith E. Lynch, Jr.
VP, Manufacturing and Supply Chain

John Berman, CPA
VP Finance & Operations

Noreen Scherer
VP, Clinical Operations

Francis Michael Conway
Vice President Controller

Scientific Advisory Board

Russell (Rusty) Clayton, DO, Scientific Advisory Board Chair

- Nearly two decades of executive experience in pharmaceutical, biologics and medical device development and commercialization as a consultant in clinical development, medical affairs and regulatory affairs.
- Prior to consulting, he was chief medical officer at Alcresta Therapeutics, a medical device company; senior vice president of research and development at Discovery Labs, a pharmaceutical and medical device company, where he led the scientific and regulatory efforts leading to the marketing authorization of Discovery's first product.
- Dr. Clayton is a board-certified pediatric pulmonologist who practiced at St. Christopher's Hospital for Children and the Children's Hospital of Philadelphia prior to beginning his career in the pharmaceutical, biologics, and medical device industry. He received his DO from the Philadelphia College of Osteopathic Medicine.

Marni J. Falk, MD

- Dr. Falk is Executive Director of the Mitochondrial Medicine Frontier Program at The Children's Hospital of Philadelphia (CHOP) and Professor in the Division of Human Genetics, Department of Pediatrics at University of Pennsylvania Perelman School of Medicine.
- She also serves as a principal investigator of a National Institutes of Health, pharma and philanthropic-funded translational laboratory group at CHOP that investigates the causes and global metabolic consequences of mitochondrial disease and directs multiple clinical treatment trials in mitochondrial disease patients.
- Dr. Falk received her BS in biology and MD from the George Washington University School of Medicine. In addition, she completed dual specialty training in the Pediatrics and Clinical Genetics residency program at Case Western Reserve University.

Giovanni Manfredi, MD, PhD

- Dr. Manfredi is the Finbar and Marianne Kenny Professor in Clinical and Research Neurology at Weill Cornell Medicine. He is also a Professor of Neuroscience and directs the graduate program in Neuroscience at Weill Cornell Medicine. Dr. Manfredi's lab studies alterations of mitochondrial metabolism in neurodegenerative diseases, particularly amyotrophic lateral sclerosis and primary inherited mitochondrial encephalomyopathies.
- Dr. Manfredi has authored more than 100 publications focused in areas including neurodegenerative and mitochondrial diseases.
- Dr. Manfredi received his MD and PhD in anatomy and cell biology from Catholic University of the Sacred Heart in Rome, where he also completed a residency in neurology.

Mark Payne, MD

- Dr. Payne is a renowned scientist and practicing cardiovascular physician who brings a long-standing scientific focus on protein targeting to mitochondria and a dedication to treating cardiomyopathies of childhood, including Friedreich's ataxia. He is the inventor of the original therapy for frataxin protein replacement in Friedreich's ataxia and co-founded Chondrial Therapeutics, which became Larimar Therapeutics, Inc.
- He holds multiple patents on mitochondrial biology and repair. He is a tenured professor of pediatrics at Indiana University School of Medicine where he directs multiple NIH-funded training, clinical, and research programs as a principal investigator.
- Dr. Payne received his BS in natural sciences from Washington & Lee University, and his MD from the University of Texas at Houston. He performed his postdoctoral clinical and research training at Washington University in St. Louis. He is a Fellow of the American College of Cardiology and the American Academy of Pediatrics.

Marshall Summar, MD

- Dr. Summar serves as Chief of the Division of Genetics and Metabolism, Director of the Rare Disease Institute and is the Margaret O'Malley Chair of Genetic Medicine at Children's National Hospital.
- In addition to guiding clinical research and treatment, he developed and launched the world's first Rare Disease Institute (RDI) at Children's. The RDI is the first Clinical Center of Excellence designated by the National Organization for Rare Diseases (NORD) and focuses on building best clinical practices and diagnostic pathways for patients. With NORD and the FDA, Dr. Summar has worked to develop a patient-driven natural history platform employed by over 35 rare disease advocacy organizations.
- He received his BS in molecular biology from Vanderbilt University and his MD from University of Tennessee Center for Health Sciences.

Friedreich's Ataxia (FA)

Symptoms & Natural History

01

70% of patients present before age 14

Significant asymptomatic period of disease

Age of onset correlated with severity and speed of progression (earlier onset correlated with more drastic progression)

02

Age 10 – 15 years: Initial onset of disease

Symptoms begin to appear and may include unsteady posture, frequent falling and progressive difficulty in walking due to impaired ability to coordinate voluntary movements

By the time symptoms occur, heart damage has occurred

03

Age 10 – 30 years: Progression of disease

Symptoms continue to worsen and may include development of advanced limb ataxia often requiring patient confinement to wheelchair, hypertrophic cardiomyopathy, scoliosis, fatigue, diabetes and hearing loss

04

Age 30 – 50 years: Life expectancy of typical FA patient

Early death usually caused by heart disease due to advanced cardiomyopathy: Most common type is hypertrophic cardiomyopathy, a thickening of the heart muscle