Larimar Therapeutics

Phase 1 Topline Data Conference Call

May 11, 2021

Forward Looking Statements

This presentation contains forward-looking statements that are based on the beliefs and assumptions of Larimar Therapeutics, Inc. (the “Company) and on information currently available to management. All statements contained in this presentation other than statements of historical fact are forward-looking statements, including but not limited to statements regarding the expectations and assumptions regarding the future of our business, the Company’s ability to develop and commercialize CTI-1601 and other planned product candidates, the Company’s planned research and development efforts, and other matters regarding the Company’s business strategies, use of capital, results of operations and financial position, and plans and objectives for future operations.

In some cases, you can identify forward-looking statements by the words “may,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “ongoing” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. These statements involve risks, uncertainties and other factors that may cause actual results, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others, the success, cost and timing of the Company’s product development activities, non-clinical studies and clinical trials, including CTI-1601 clinical milestones; that clinical trial results may differ from final clinical trial results, that earlier non-clinical and clinical data and testing of CTI-1601 may not be predictive of the results or success of clinical trials, and that clinical trial data are subject to differing interpretations and assessments; the ongoing impact of the COVID-19 pandemic on the Company’s clinical trials, manufacturing, regulatory and nonclinical study timelines, 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 Company’s periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. These forward-looking statements are based on a combination of facts and factors currently known by the Company and its projections of the future, about which it cannot be certain. As a result, the forward-looking statements may not prove to be accurate. These forward-looking statements are based on information currently available to us, and we assume no obligation to update any forward-looking statements, except as required by law.

Investment Highlights

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

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

Double-blind, placebo-controlled Phase 1 proof-of-concept trials in FA patients complete
Data show dose dependent increases in FXN levels from baseline compared to placebo in all evaluated tissues with daily dosing and that CTI-1601 was generally well tolerated when dosed for up to 13 days

Series A investment by Deerfield in Nov. 2016; went public through a reverse merger/PIPE in May 2020
Shareholder base includes high-quality institutional investors

Strong balance sheet
~$81.4 million in cash as of March 31, 2021, with projected runway into 1H 2022

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FXN: Frataxin

Executive Summary of Phase 1 POC Data

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

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POC: Proof-of-concept

Friedreich's Ataxia (FA)

Rare and Progressive Disease

Caused by genetic defect resulting in low levels of frataxin

- Patients with FA only produce ~20-40% of normal frataxin levels depending on the tissue, sampling technique, and assay considered[1]
- Affects ~5,000 patients in the U.S. & ~20,000 patients in the EU

>70% of patients present before age 14

- Initial symptoms may include unsteady posture, frequent falling and progressive difficulty in walking
- By the time symptoms occur, heart damage may have already occurred
- Progressive disease: Symptoms worsen and patients are eventually confined to a wheelchair with speech becoming hesitant and jerky (often referred to as "scanning of speech")

Life expectancy of 30-50 years

- Early death usually caused by heart disease

No approved therapies available

- Current treatment options are limited to symptom management

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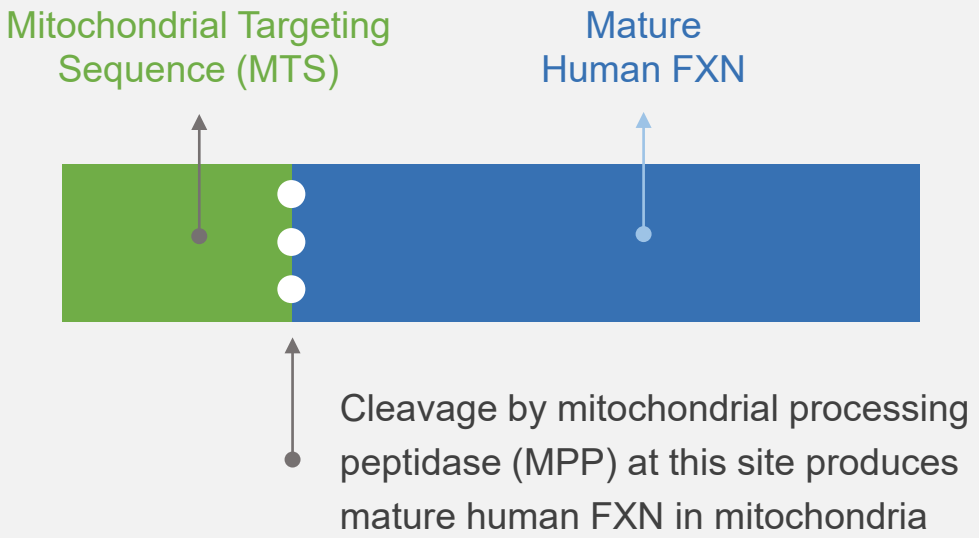
1. E.C. Deutsch et al. Molecular Genetics and Metabolism 101 (2010) 238–245

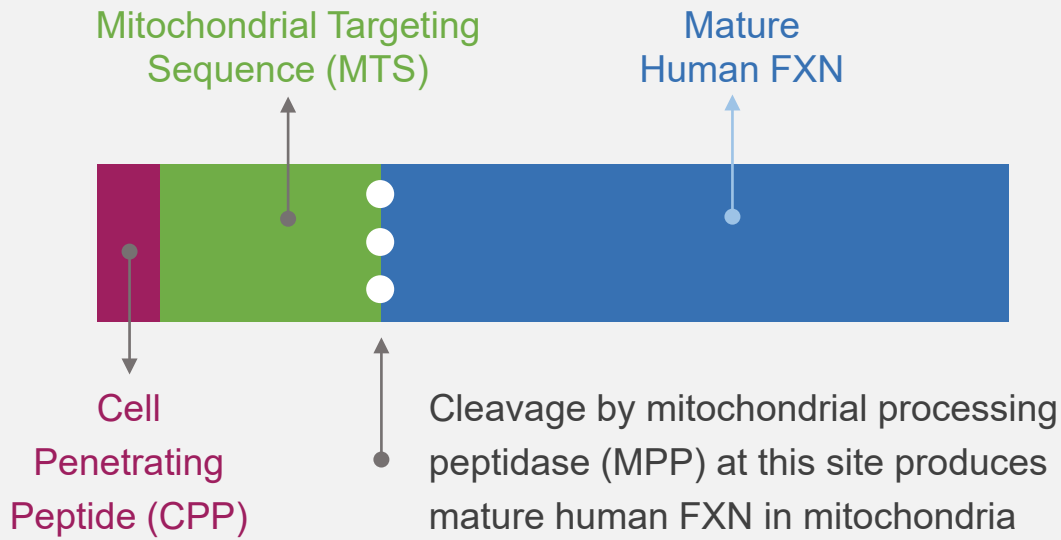
CTI-1601 is Designed to Deliver Additional Frataxin (FXN)

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

STRUCTURE OF ENDOGENOUS FXN



STRUCTURE OF CTI-1601



The maintenance of the cleavage site allows the CPP and MTS to be removed by mitochondrial processing peptidase to produce mature human FXN in the mitochondria

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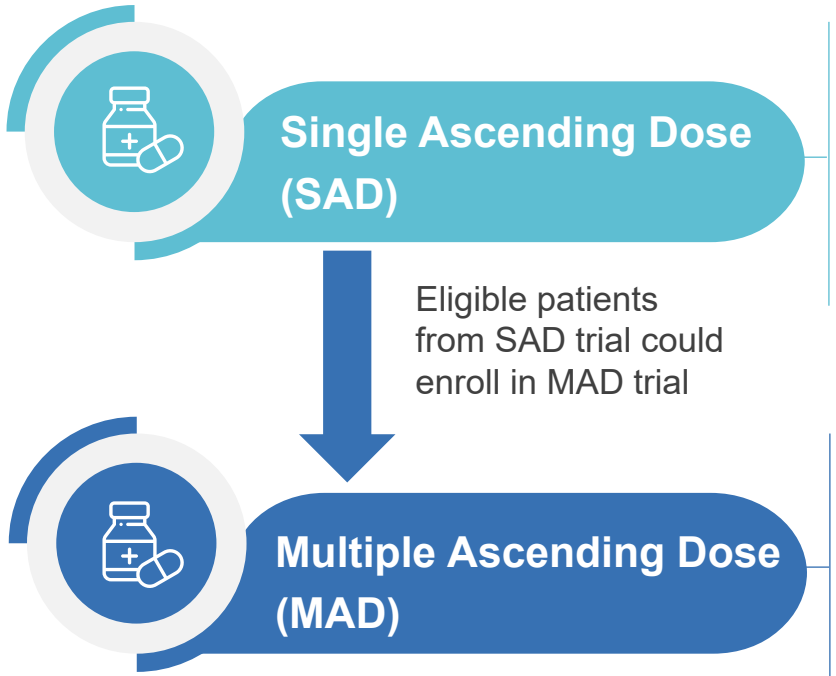
CTI-1601: Phase 1 Clinical Program in Patients with FA

Program consisted of double-blind, placebo controlled single- and multiple-ascending dose trials

Phase 1 Development Plan

- Two double-blind, placebo controlled dosing trials in patients with FA
- Patient dosing began December 2019
- Safety Review Committee assessed all blinded data between each cohort to ensure patient safety



Number of subjects: 28

Dose levels: 25 mg, 50 mg, 75 mg and 100 mg (subcutaneous administration)

Treatment Duration: 1 day

1º Endpoint: Safety and tolerability

2º Endpoints: PK; PD; FXN levels; multiple exploratory

Status: Complete with analysis ongoing

Number of Subjects: 27

Dose Range: 25 mg, 50 mg, 100 mg (subcutaneous administration)

Treatment Regimen: Multiple increasing doses administered subcutaneously over 13 days

1º Endpoint: Safety and tolerability

2º Endpoints: PK; PD; FXN levels (buccal cells, platelets, optional skin biopsies); multiple exploratory

Status: Complete with analysis ongoing

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MAD Trial Patient Enrollment

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

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

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Multiple Ascending Dose Study Design

Treatment Schedules for Each Cohort

Cohort 1 (25 mg; n = 8)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of CTI-1601 or placebo

= No Administration

Cohort 2 (50 mg; n = 9)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of CTI-1601 or placebo

= No Administration

Cohort 3 (100 mg n = 10)

13-day Treatment Period

1	2	3	4	5	6	7	8	9	10	11	12	13	14

= Administration of CTI-1601 or placebo

= No Administration

FXN Level Sampling Days Presented for Each Cohort

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

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

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

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MAD Trial Patient Demographics

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

SD: Standard deviation

MAD Trial Patient Disease Characteristics

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

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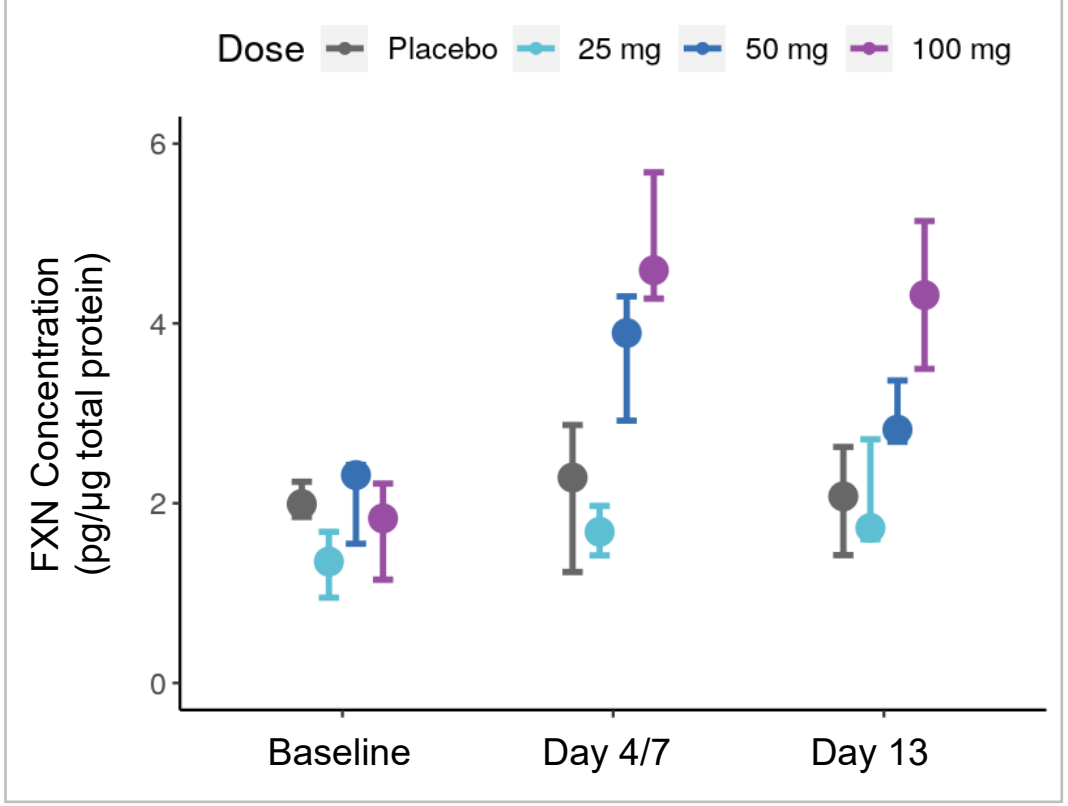
SD: Standard deviation

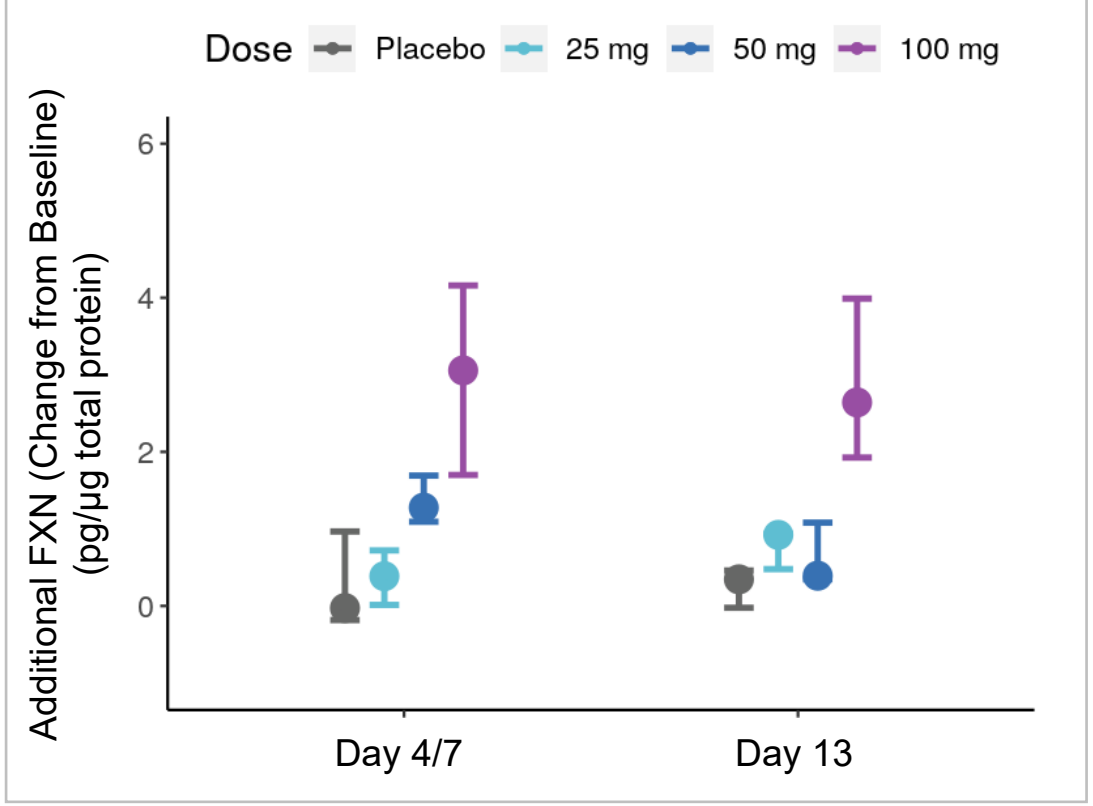
Dose Dependent Increases in FXN Levels Observed in Buccal Cells

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

FXN* Levels By Dose Group (Buccal Cells)



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

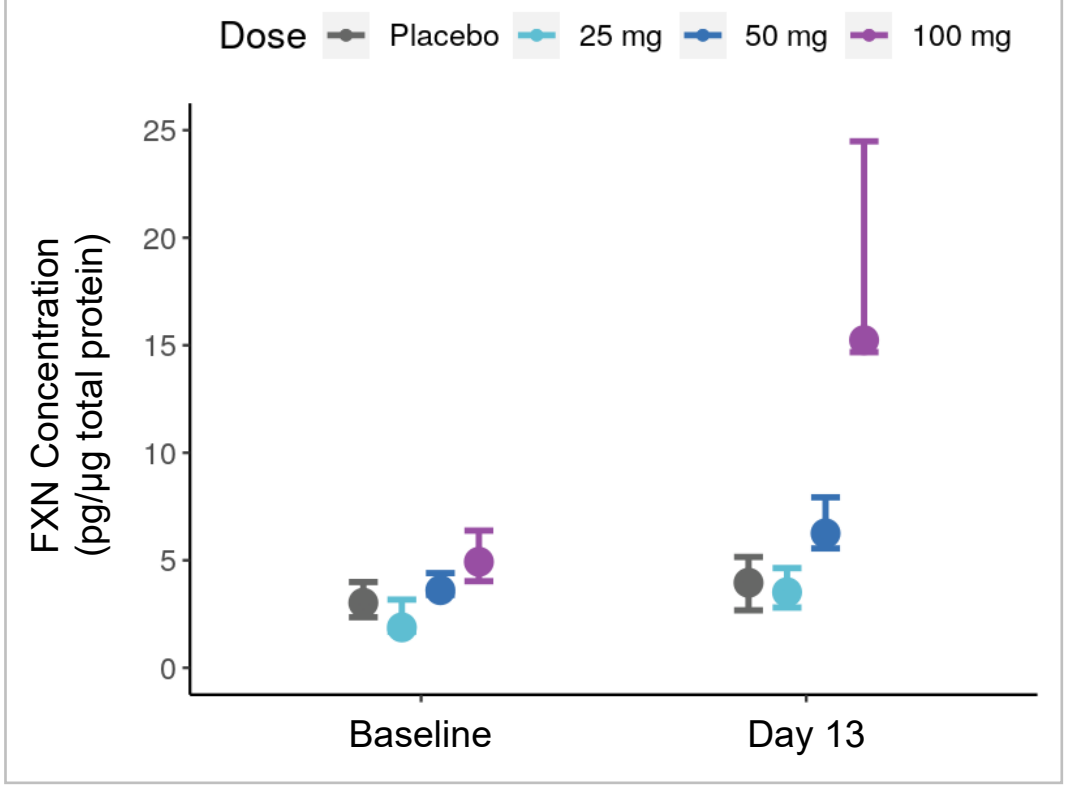


*FXN levels measured via detection of peptide derived from mature FXN; Data represent median and 25th and 75th percentiles; FXN levels from baseline, Day 4, & Day 13 measurements are shown for data derived from the 25 mg cohort; FXN levels from baseline, Day 7 & Day 13 measurements are shown for data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

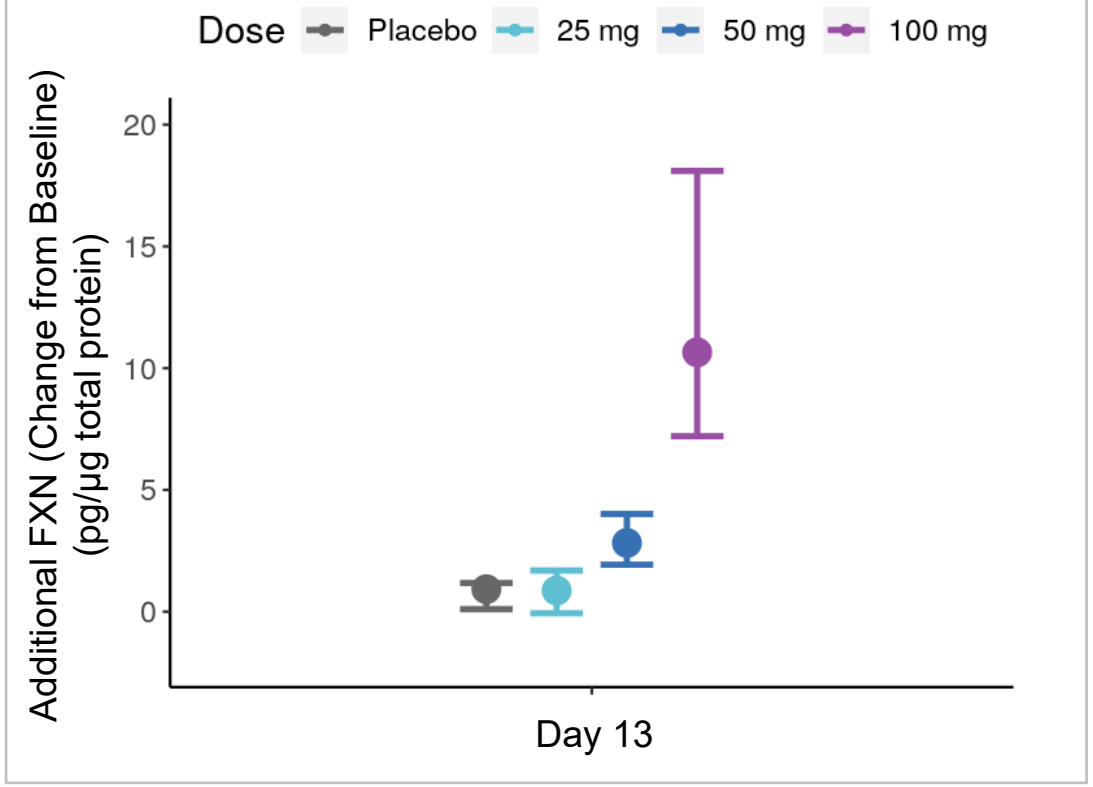
Dose Dependent Increases in FXN Levels Observed in Skin

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

FXN* Levels By Dose Group (Skin Biopsies)



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

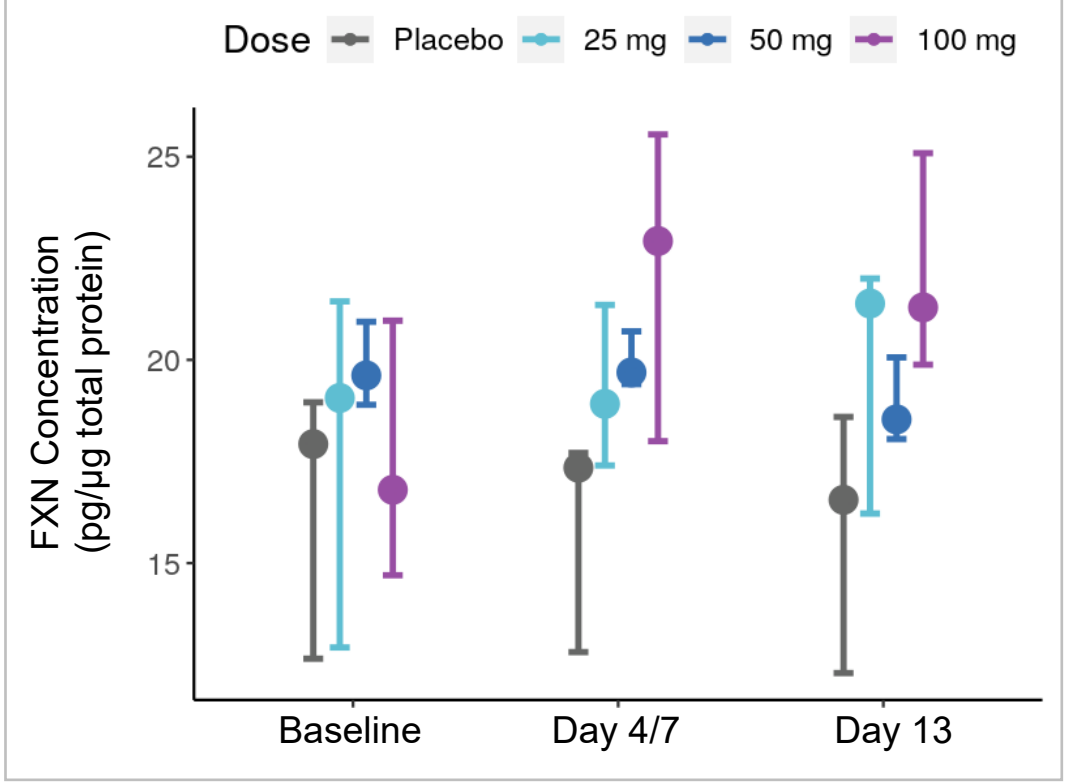


*FXN levels measured via detection of peptide derived from mature FXN; Data represent median and 25th and 75th percentiles

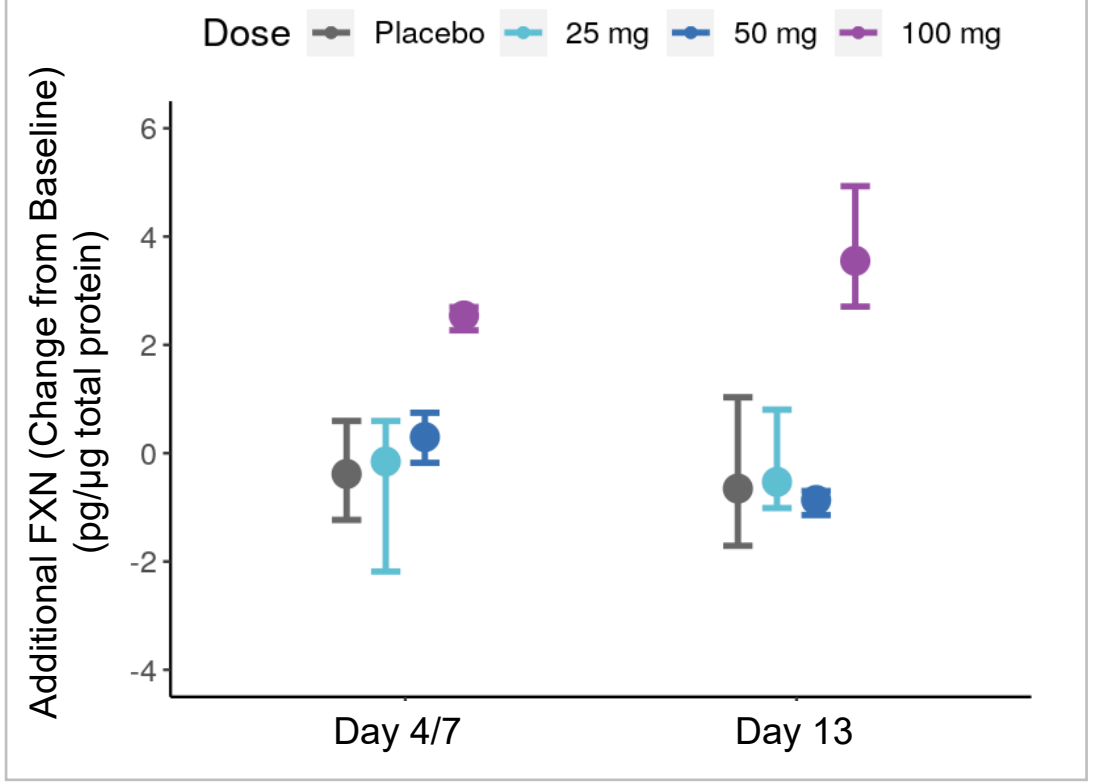
Dose Dependent Increases in FXN Levels Observed in Platelets with Daily Dosing

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

FXN* Levels By Dose Group (Platelets)



FXN* Change from Baseline By Dose Group (Platelets)



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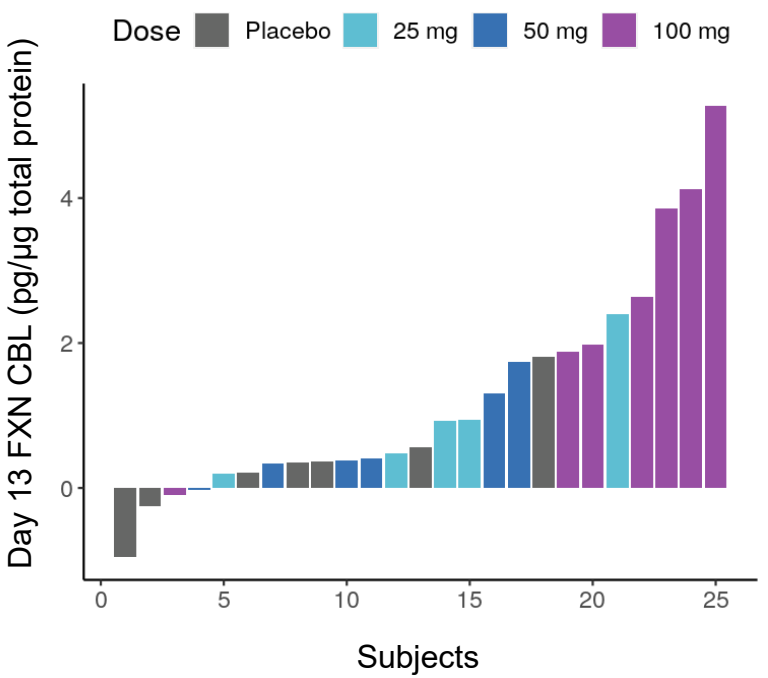
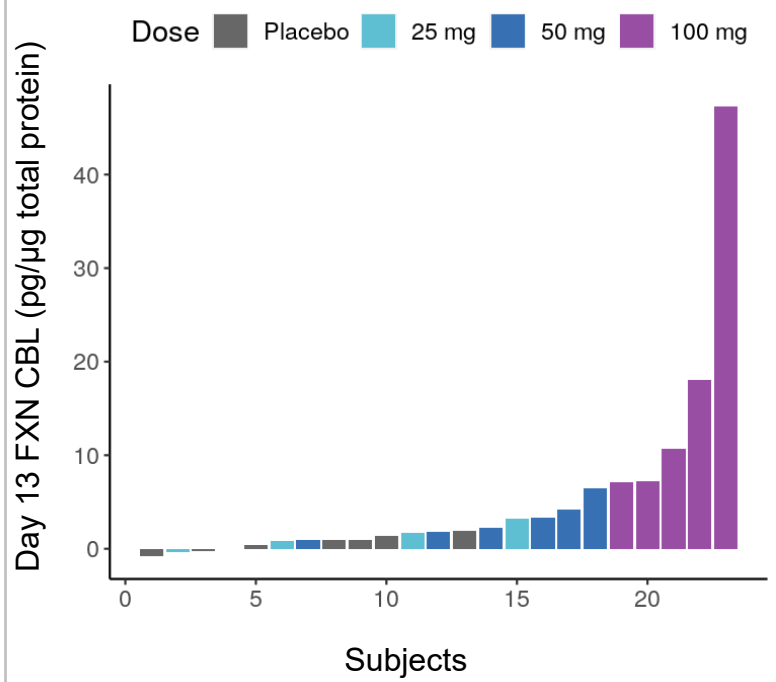
Increases in FXN Correlated with Increasing CTI-1601 Dose

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

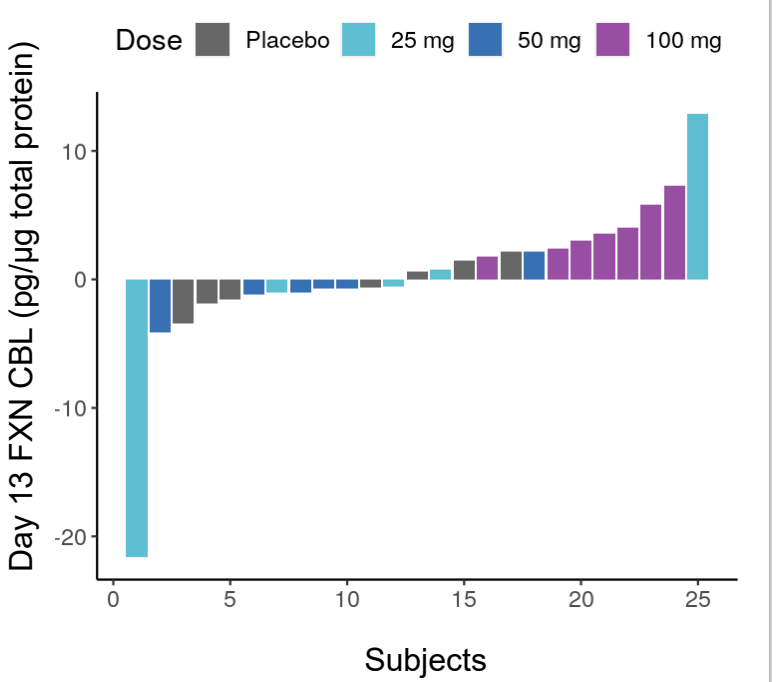
Buccal Cells Day 13 FXN* Change from Baseline



Skin Biopsies# Day 13 FXN* Change from Baseline



Platelets Day 13 FXN* Change from Baseline



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

Data Compare Favorably to FXN Levels Expected in Heterozygous Carriers

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

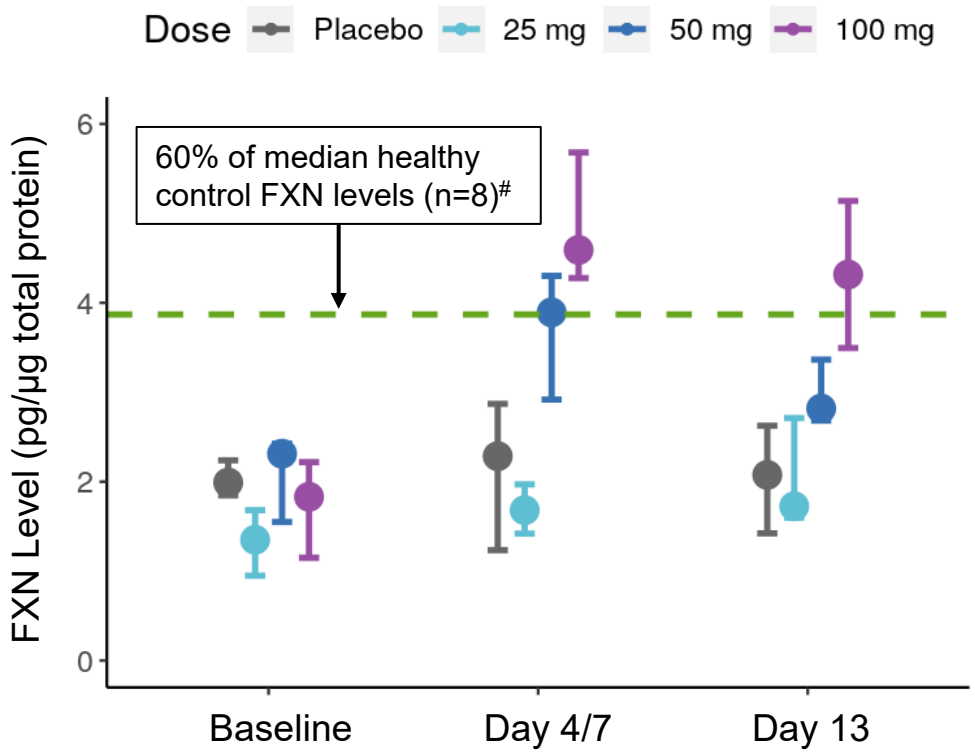
Benchmarking Clinical Relevance

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

Comparison to Healthy Controls

- FXN levels were measured in buccal cells from 8 healthy controls using the same assay and sampling technique employed in the Phase 1 MAD trial
- With daily administration, patients in Cohorts 2 & 3 of the Phase 1 MAD trial achieved median buccal cell FXN levels that were >60% of the median FXN levels observed in healthy controls
- Data from additional healthy control buccal cells, skin, and platelets will be collected in a separate non-interventional study

FXN* Levels By Dose Group (Buccal Cells)



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

Summary of MAD trial safety data:

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

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

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

Summary of PK Analyses

- CTI-1601 was quickly absorbed after subcutaneous administration
- Dose-proportional increases in exposure observed with increasing doses of CTI-1601
- Mean half life of CTI-1601 in plasma was approximately 11 hours

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

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

Safety Data

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

The most common AEs were mild and moderate injection site reactions

No SAEs have been reported

Frataxin Measurements

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

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

Pharmacokinetic Data

CTI-1601 was quickly absorbed after subcutaneous administration

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

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

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

Upcoming Trials and Regulatory Interactions

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

Future Planned Trials and Regulatory Interactions Include:

Continued interactions with FDA regarding clinical trials and non-clinical toxicology studies

Jive open label extension (OLE) trial for eligible patients who participated in SAD or MAD trials (expected initiation 2H 2021)

MAD trial in patients under 18 years of age (expected initiation 2H 2021). Participants eligible to screen for Jive OLE trial

Global double-blind placebo-controlled pivotal trial (expected initiation as early as 2H 2022)

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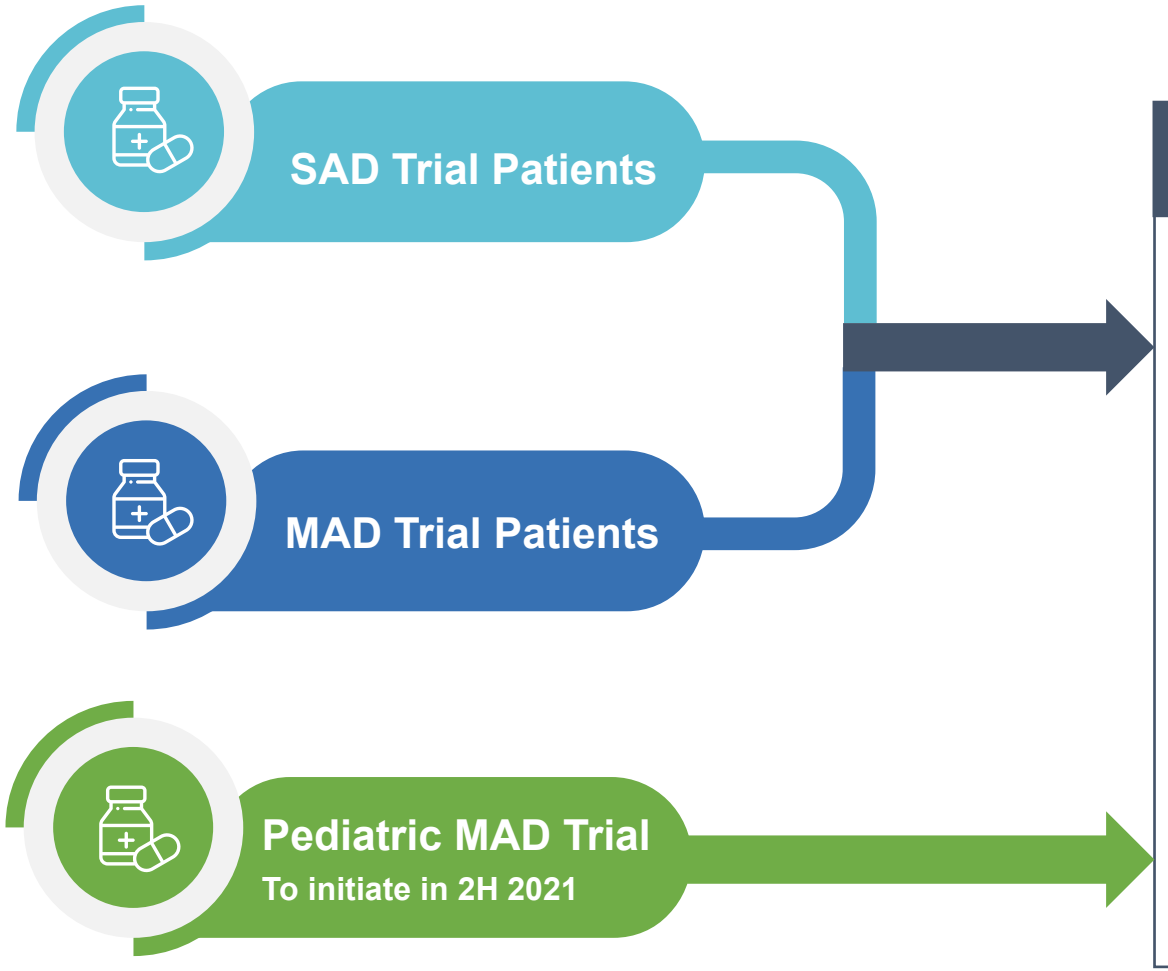
Expect to Initiate Two Additional Trials in 2H 2021

Patients from SAD, MAD, and pediatric trials are eligible to screen for the Jive open label extension trial



Jive OLE Trial

Dose Level: To be determined based on additional analysis of data from SAD and MAD trials

Treatment Duration: Planned for 24 months with any necessary extensions

Number of Subjects: Up to 50

Dose Regimen: Once-daily doses administered subcutaneously

External Control Arm: Derived from Critical Path Institute Data

1º Endpoint: Safety and tolerability

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

Expected Initiation: 2H 2021

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Conclusion

Safety

CTI-1601 appears to be generally well tolerated at doses up to 100 mg administered daily for 13 days

Pharmacodynamics

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

Pharmacokinetics

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

Conclusion

Daily subcutaneous (SC) administration of 50mg and 100mg doses of CTI-1601 resulted in FXN levels in buccal cells that are at, or in excess of, those we would expect to see in phenotypically normal heterozygous carriers (who have FXN levels of ~50% of unaffected healthy persons)

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