UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

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

Date of Report (Date of earliest event reported): **May 20, 2023**

PTC THERAPEUTICS, INC.
(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

100 Corporate Court **South Plainfield, NJ** (Address of Principal Executive Offices)	**07080** (Zip Code)

Registrant's telephone number, including area code: **(908) 222-7000**

Not applicable
(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, $0.001 par value per share	PTCT	Nasdaq Global Select 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 2.05 Costs Associated with Exit or Disposal Activities.

On May 23, 2023, PTC Therapeutics, Inc. (the "Company") announced a strategic pipeline prioritization following a review of its preclinical and clinical development programs. Based on this review, the Company has determined to discontinue its preclinical and early research programs in its gene therapy platform. In connection with the strategic pipeline prioritization, on May 22, 2023, the Company committed to a reduction in workforce of approximately 8%, which will primarily affect employees in the United States. As a result of the strategic pipeline prioritization and associated reduction in workforce, the Company expects to realize residual 2023 operating expense savings of approximately 15% for the year ending December 31, 2023 (without giving effect to any non-cash, stock-based compensation expenses).

The Company plans to complete the reduction in workforce by August 31, 2023. Affected employees will be offered separation benefits, including severance payments along with temporary healthcare coverage assistance and other benefits. The Company estimates that the employee severance and benefit costs along with required pre-termination associated payments and benefits will be approximately $7.0 million, substantially all of which are expected to be cash expenditures. The estimate of costs that the Company expects to incur, and the timing thereof, are subject to a number of assumptions and actual results may differ. The Company may also incur other charges or cash expenditures not currently contemplated due to events that may occur as a result of, or associated with, the reduction in workforce.

Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.

On May 20, 2023, the Company informed Emily Hill, its Chief Financial Officer, that her employment will be terminated effective as of August 20, 2023, and effective immediately she will cease to serve as the Company's Chief Financial Officer. Pursuant to Ms. Hill's employment agreement, a copy of which is attached as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the period ended on June 30, 2019, as Ms. Hill's employment is being terminated without cause (as such term is defined in Ms. Hill's employment agreement), Ms. Hill will be entitled to certain severance benefits in exchange for the execution of a general separation and release agreement.

Item 7.01. Regulation FD Disclosure.

On May 23, 2023, the Company issued a press release regarding the strategic pipeline prioritization and associated reduction in workforce, furnished as Exhibit 99.1 to this Current Report on Form 8-K (this "Report").

On May 23, 2023, the Company issued a press release announcing the results from its Phase 3 registration-directed trial of vatiquinone in children and young adults with Friedreich ataxia, furnished as Exhibit 99.2 to this Report.

The Company will host a conference call on May 23, 2023 at 5:00 PM Eastern time. Directions on how to access the conference call are included in the press releases attached to this Report. A copy of the slide deck that will be presented during the conference call is furnished as Exhibit 99.3 to this Report.

The information in this Item 7.01 of this Report, including Exhibits 99.1, 99.2 and 99.3 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 8.01. Other Events.

On May 23, 2023, the Company announced that the primary endpoint in its Phase 3 registration-directed trial of vatiquinone in children and young adults with Friedreich ataxia was not achieved.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated May 23, 2023 issued by PTC Therapeutics, Inc.
99.2	Press Release, dated May 23, 2023 issued by PTC Therapeutics, Inc.
99.3	Corporate Presentation – Move FA Topline Results
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: May 23, 2023

By: /s/ Mark E. Boulding
Name: Mark E. Boulding
Title: Executive Vice President and Chief Legal Officer

Exhibit 99.1

PTC Therapeutics Announces Strategic Pipeline Prioritization

- Preclinical and early research gene therapy programs discontinued -
- Expected reductions of approximately 15% in residual 2023 OPEX -

SOUTH PLAINFIELD, N.J., May 23, 2023 -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today the discontinuation of preclinical and early research programs in gene therapy as part of a strategic portfolio prioritization. PTC will continue its development and global commercialization of Upstaza, the first-ever approved gene therapy directly administered to the brain. As a result of the prioritization, estimated reductions of approximately fifteen percent in residual 2023 operating expenses (OPEX) are expected. Additional 2023 OPEX guidance will be provided as part of second quarter earnings.

“We are incredibly proud of PTC’s accomplishments in the field of gene therapy, including the pioneering work associated with the approval of Upstaza. We believe that the decision to discontinue our pipeline gene therapy programs enables PTC to focus R&D efforts on our other innovative and differentiated scientific platforms and strongly positions us for long-term growth and success,” said Chief Executive Officer Dr. Matthew Klein. “Where possible, we will work to ensure that the discontinued gene therapy programs can be developed by other parties so that the therapies have the potential to benefit patients.”

The decision to deprioritize early-stage gene therapy programs reflects the company’s desire to focus resources on areas of the pipeline likely to deliver significant return on investment and transformative therapies for patients with high unmet medical need. The discontinuation of the gene therapy programs will also result in a reduction in workforce which will be conducted in accordance with appropriate labor regulations.

The discontinued gene therapy programs include preclinical stage programs in Friedreich ataxia and Angelman syndrome as well as several other programs targeting rare CNS and ophthalmological disorders of high unmet medical need at various stages of preclinical development. The prioritization decision will allow for additional focus on PTC’s proprietary splicing platform as well as additional CNS and metabolic disorders that leverage its differentiated and innovative scientific expertise.

In addition, PTC Therapeutics also announced today that PTC’s Chief Financial Officer Emily Hill has been relieved of her responsibilities and will be leaving the organization.

Conference Call and Webcast Details:

PTC will hold a conference call at 5:00 pm EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About PTC Therapeutics, Inc.

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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Forward-Looking Statement:

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this press release, other than statements of historic fact, are forward-looking statements, including statements regarding the extent, timing and financial aspects of the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce; the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters, future strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward -looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; the timing of and actual expenses incurred in connection with the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce, which may be in different periods and may be materially higher than we estimate; the savings that may result from the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce, which may be materially less than we expect; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10 -K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

Exhibit 99.2

PTC Therapeutics Announces Topline Results from Vatiquinone MOVE-FA Registration-Directed Trial

- Conference call and webcast to be held at 5:00 pm EDT -

SOUTH PLAINFIELD, N.J., May 23, 2023 -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today reported topline results from the MOVE-FA trial of vatiquinone in patients with Friedreich ataxia. The study did not meet its primary endpoint of statistically significant change in mFARS score at 72 weeks in the primary analysis population. However, vatiquinone treatment did demonstrate significant benefit on key disease subscales and secondary endpoints. In addition, in the population of subjects that completed the study protocol, significance was reached in the mFARS endpoint and several secondary endpoints.

“While we are disappointed that the study did not achieve its primary endpoint, we are encouraged by the findings of meaningful impact on several different aspects of FA disease progression and morbidity over 72 weeks,” said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. “Given the signals of clinical benefit, vatiquinone's well-established safety profile in children, and the unmet medical need for pediatric patients with FA, we look forward to discussing a potential path to registration with regulatory authorities.”

The MOVE-FA trial enrolled 146 pediatric and adult patients, the majority of which were under 18 years of age. The mean placebo corrected change in the mFARS score in the primary analysis population was 1.6 (p=0.14). Notably, there was significant benefit recorded in the bulbar and upright stability subscales (nominal p values of 0.044 and 0.021, respectively) which are regarded as reflective of key aspects of disease morbidity and predictive of loss of time to loss of ambulation. In addition, a statistically significant difference was recorded on the Modified Fatigue Scale, which captures one of the most impactful sources of disease morbidity (nominal p value of 0.025). On a prespecified sensitivity analysis of subjects who completed 72 weeks on assigned therapy, the placebo corrected difference was 2.31, which represents a 75% slowing in disease progression over 72 weeks. Overall, vatiquinone was demonstrated to be well tolerated, adding to the large volume of safety data collected in other pediatric clinical studies.

Conference Call and Webcast Details:
PTC will hold a conference call at 5:00 pm EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About the MOVE-FA Clinical Trial
The Phase 3 registration-directed trial in Friedreich Ataxia patients, called MOVE-FA, is a randomized, placebo-controlled 72-week trial with the primary endpoint being change in the modified Friedreich Ataxia Rating Scale (mFARS) score. The mFARS is a clinical assessment that measures disease progression, namely swallowing and speech, upper and lower limb coordination, and upright stability. The key secondary endpoint is the change from baseline in activities of daily living as assessed by the FA-Activities of Daily Living (ADL) scale. Patients who completed the placebo portion of the trial will be trial are eligible to enroll in an open label 24-week extension.

About Vatiquinone
PTC is developing vatiquinone, a potential treatment for Friedreich ataxia based on our Bio-e platform. Vatiquinone is a small molecule, first-in-class selective inhibitor of 15-Lipoxygenase (15-LO), an enzyme that is a key regulator of the energetic and oxidative stress pathways that are disrupted in Friedreich ataxia. Inhibition of 15-LO helps to alleviate the consequences of mitochondrial dysfunction and oxidative stress, ultimately preventing ferroptosis and aiding neuronal survival.[1,2,3] Vatiquinone has been evaluated in a number of clinical studies and has demonstrated an impact on mortality risk and a number of neurological and neuromuscular disease symptoms.

About Friedreich Ataxia

Friedreich ataxia (FA) is a rare, physically debilitating, life-shortening, neuromuscular disorder that mainly affects the central nervous system and the heart.[4] It is the most common hereditary ataxia (abnormal, uncoordinated movements) and is usually caused by a single genetic defect in the frataxin (FXN) gene that leads to reduced production of frataxin, a mitochondrial protein that is important for cellular metabolism and energy production.[4,5] Decreased frataxin levels are associated with mitochondrial iron accumulation and increased oxidative stress, which can lead to cell death through ferroptosis.[6,7,8]

Symptoms include progressive loss of coordination and muscle strength leading to poor balance and coordination, difficulty speaking, swallowing, and breathing, curvature of the spine, serious heart conditions, diabetes, and hearing and vision impairment.[9,10] The severity of symptoms and speed of progression varies between people and some symptoms may not be evident in all. Friedreich ataxia is usually diagnosed in childhood or adolescence.[5,11] Approximately 25,000 people have Friedreich ataxia globally.

About PTC Therapeutics, Inc.

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Forward-Looking Statement

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References:

1. Hinman A, et al. PLoS one. 2018;13:e0201369.
2. PTC Therapeutics. EPI-743 Pre-Clinical Data Deck.
3. Shrader WD, et al. Bioorg Med Chem Lett. 2011;21:3693–3698.
4. Lynch DR, Farmer JM, Balcer LJ, et al. Arch Neurol 2002;59(5):743–747.
5. Campuzano V, Montermini L, Lutz Y, et al. Hum Mol Genet 1997;11(6):1771–1780.
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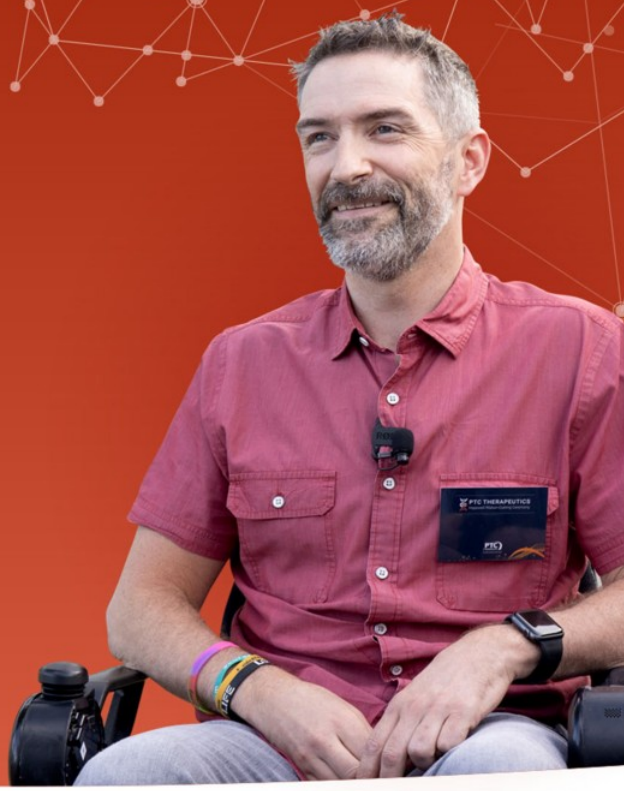
7. Cook A, Giunti P. Br Med Bull. 2017;124:19–30.
8. Pandolfo M, Hausmann L. J Neurochem. 2013;126:142–146.
9. Bürk K. Cerebellum Ataxias 2017;4:4.
10. Cook A, Giunti P. Br Med Bull 2017;124(1):19–30.
11. Delatycki MB, Williamson R, Forrest SM. J Med Genet 2000;37(1):1–8.

MOVE-FA Topline Results

Matthew B. Klein, MD
CEO



Forward-Looking Statements

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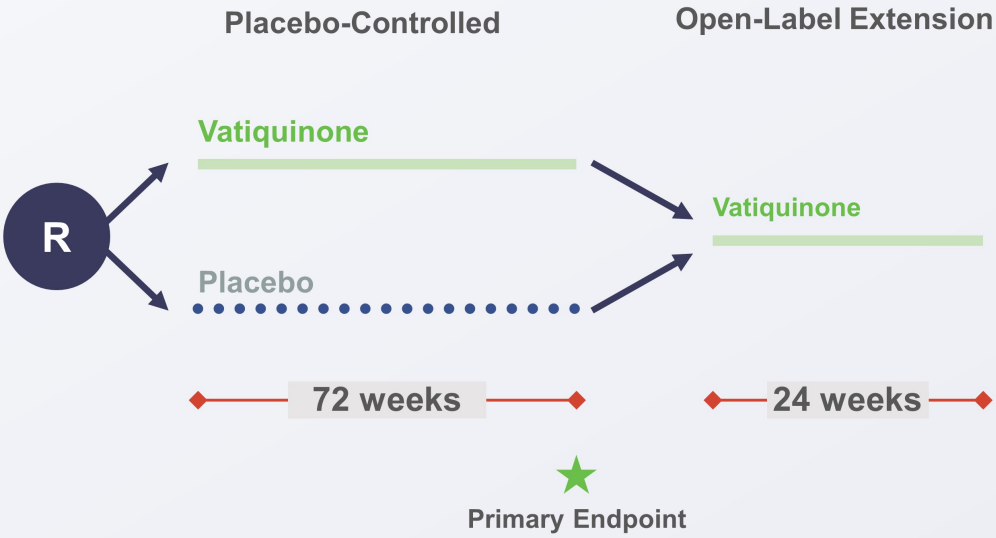
MOVE-FA Is a Global Registration-Directed Trial of Vatiquinone in Friedreich Ataxia Patients



Primary Analysis
7-21 years

Overall Enrolled
included patients

Primary En
Change in mFA

Other End
FARS-ADL
Upright Stability
Modified Fatigue

mFARS Disease Rating Scale Measures Disease Progression Across Four Domains

Bulbar

Upright stability

Lower limb coordination

Upp
coor

mFARS scores worsen (increase) on average by 2 to 3 points per yea in pediatric patients based on natural history studies[1]

[1] Rummey. Neurology, 2022.

Baseline Patient Characteristics: Primary Analysis & Overall Enrolled Populations

	Primary Analysis Population			Overall Enrolled P	
	Placebo n (%)	Vatiquinone n (%)	Total n (%)	Placebo n (%)	Vatiquinone n (%)
Subject Number	62	61	123	73	70
Mean Age at Baseline [min,max]	14.3 [8,21]	15.0 [9,21]	14.6 [8,21]	18.2 [8,68]	19.1 (9,68)
Age at Onset					
<14	58 (93.5)	53 (86.9)	111 (90.2)	62 (84.9)	55 (78.6)
>=14	4 (6.5)	8 (13.1)	12 (9.8)	11 (15.1)	15 (21.4)
mFARS at Baseline [min, max]	43.3 [20, 68]	41.6 [22, 69]	42.5 [20, 69]	43.3 [20, 68]	42.5 [22, 69]
Region – n (%)					
Asia Pacific	3 (4.8)	5 (8.2)	8 (6.5)	3 (4.1)	5 (7.1)
European Union	19 (30.6)	16 (26.2)	35 (28.5)	19 (26)	16 (22.9)
North America	31 (50)	33 (54.1)	64 (52)	42 (57.5)	42 (60)
Latin America	9 (14.5)	7 (11.5)	16 (13)	9 (12.3)	7 (10)

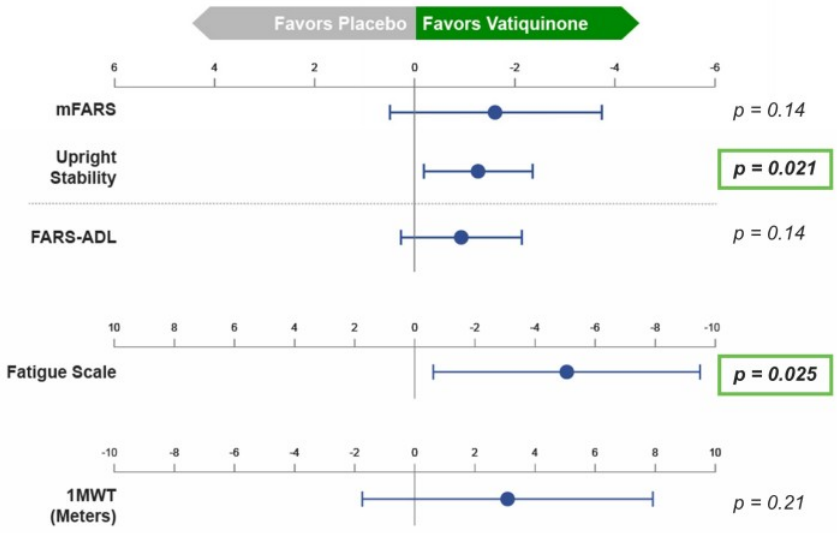
Vatiquinone Treatment Demonstrated Slowing of Disease Progression on mFARS with Nominal Significance in Key Subscales

	Primary Analysis Population Change from Baseline to Week 72				Overall Enrolled Popu Change from Baseline to		
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**	**Placebo**	**Vatiquinone**	**Differen**
mFARS Total*	2.83	1.22	-1.61	0.14	2.56	0.90	-1.66
Bulbar	0.22	0.033	-0.18	**0.044**	0.18	0.033	-0.15
Upright Stability	2.99	1.73	-1.26	**0.021**	2.49	1.38	-1.11
Lower Limb	0.40	-0.11	-0.51	0.23	0.36	-0.11	-0.47
Upper Limb	-0.51	-0.18	0.32	0.58	-0.64	-0.35	0.29

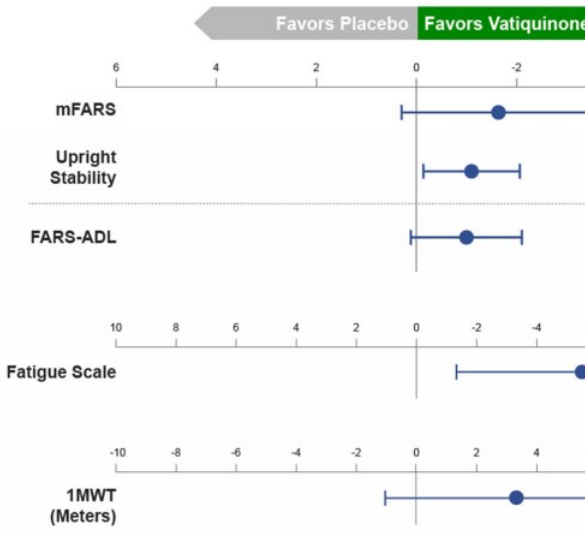
*Primary endpoint which did not meet statistical significance

Vatiquinone Treatment Resulted in Meaningful Slowing of Disease Symptom Progression

Primary Analysis Population



Overall Study Populatio



Pre-Specified Completers Sensitivity Analysis

As the study was conducted during the COVID-19 pandemic, a pre-specified sensitivity analysis was included for subjects that completed the study protocol on assigned treatment

The total number of subjects completing the study without treatment assignment disruption was 96 in the primary analysis population and 110 in the overall study population

Excluded from this analysis were subjects that discontinued due to COVID-related issues, non-compliance, dose disruptions and withdrawal for other reasons

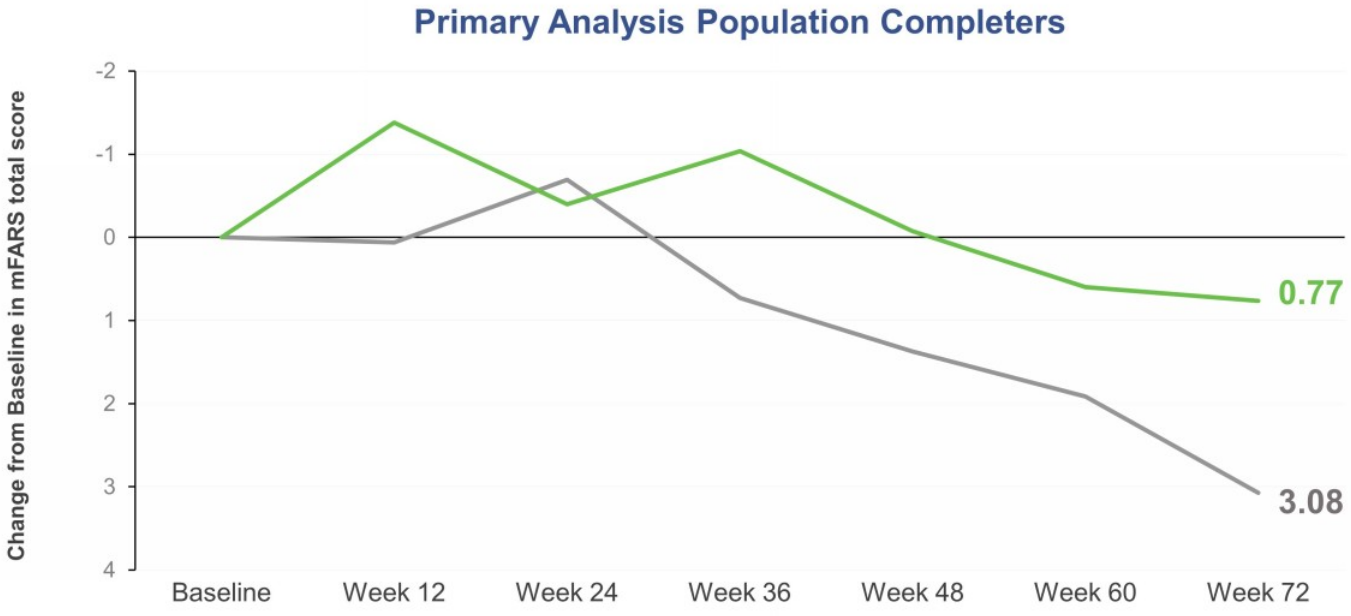
Completer Sensitivity Analysis Population

	Primary Analysis Population			Overall Enrolled P	
	Placebo n (%)	Vatiquinone n (%)	Total n (%)	Placebo n (%)	Vatiquinon n (%)
Randomized	48	48	96	56	54
Mean Age at Baseline [min,max]	14.2 [8,20]	15.4 [9,21]	14.8 [8,21]	17.6 [8,68]	19.0 (9,68)
Age at Onset					
<14	45 (93.8)	40 (83.3)	85 (88.5)	48 (85.7)	41 (75.9)
>=14	3 (6.3)	8 (16.7)	11 (11.5)	8 (14.3)	13 (24.1)
Region – n (%)					
Asia Pacific	3 (6.3)	4 (8.3)	7 (7.3)	3 (5.4)	4 (7.4)
European Union	17 (35.4)	12 (25)	29 (30.2)	17 (30.4)	12 (22.2)
North America	21 (43.8)	26 (54.2)	47 (49.0)	29 (51.8)	32 (59.3)
Latin America	7 (14.6)	6 (12.5)	13 (13.5)	7 (12.5)	6 (11.1)

Vatiquinone Treatment Demonstrated Greater Magnitudeof Effect on Disease Progression in Completers Sensitivity Analysis

	Primary Analysis Population Completers Change from Baseline to Week 72				Overall Enrolled Population Change from Baseline to		
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**	**Placebo**	**Vatiquinone**	**Differen**
mFARS Total	3.08	0.77	-2.31	0.054	2.72	0.57	-2.15
Bulbar	0.17	.0003	-0.17	**0.030**	0.19	0.030	-0.16
Upright Stability	3.16	1.78	-1.38	**0.026**	2.69	1.45	-1.23
FARS-ADL	1.35	0.66	-0.69	0.29	1.30	0.75	-0.55
Fatigue Scale (MFIS)	4.14	-0.59	-4.73	**0.042**	3.88	-1.62	-5.50

Vatiquinone Treatment Slowed Disease Progression by 75% in Completers Sensitivity Analysis at Week 72



Vatiquinone Demonstrated to Be Well Tolerated

Similar Adverse Event Profile Between Vatiquinone and Placebo Subjects

Most Common Treatment-Related Adverse Events Were GI Symptoms

MOVE-FA Safety Profile Consistent with Other Vatiquinone Pediatric Studies

Overview of Treatment-Emergent Adverse Events in Overall Study Population

Category	Placebo (N=73) N (%)	Vat (N=
Subjects with at least one TEAE	73 (100)	
Subjects with TEAEs by maximum severity		
Mild	31 (42.5)	2
Moderate	32 (43.8)	3
Severe	9 (12.3)	
Life-Threatening/Fatal	1 (1.4)	
Subjects with treatment-related TEAEs		
Probable	4 (5.5)	1
Possible	28 (38.4)	3
Subjects with at least one TESAE	8 (11.0)	
Subjects with treatment-related TESAES		
Probable	0	
Possible	0	
Subjects discontinued study drug due to treatment-related TEAE	3 (4.1)	

MOVE-FA Results Support Discussions With Regulatory Authorities

Evidence of meaningful clinical benefit on key aspects of FA disease

High unmet need for pediatric FA patients

Discuss potentia to registration regulatory autho