MOVE-FA Topline Results

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Patient Living with FA

Forward-Looking Statements

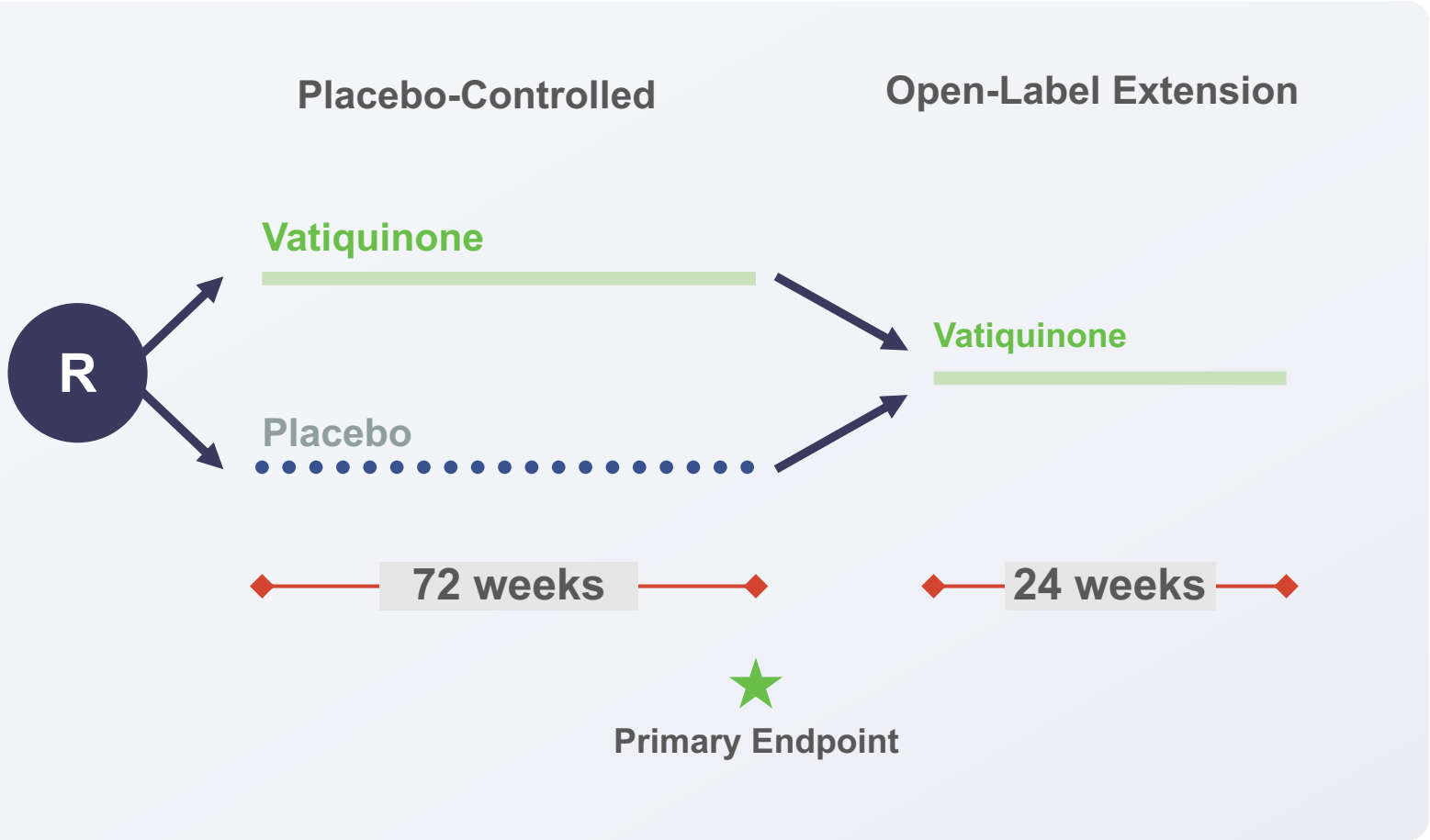
This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements with respect to the future expectations, 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 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; 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 presentation 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 presentation except as required by law.

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



Primary Analysis Population:
7-21 years old

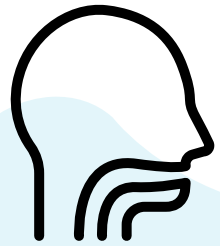
Overall Enrolled Population:
included patients over age 21

Primary Endpoint
Change in mFARS Score

Other Endpoints
FARS-ADL Scale
Upright Stability Subscale
Modified Fatigue Impact Scale

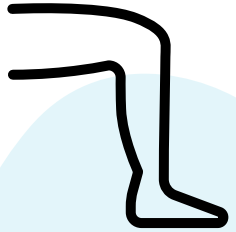
mFARS Disease Rating Scale Measures Disease Progression Across Four Domains

MOVE-FA



Bulbar

Upright stability



Lower limb coordination

Upper limb coordination

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

[1] Rummey. Neurology, 2022.

PTC THERAPEUTICS 25 YEARS

Baseline Patient Characteristics: Primary Analysis & Overall Enrolled Populations

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	Primary Analysis Population			Overall Enrolled Population		
	Placebo n (%)	Vatiquinone n (%)	Total n (%)	Placebo n (%)	Vatiquinone n (%)	Total n (%)
Subject Number	62	61	123	73	70	143
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)	18.7 (8,68)
Age at Onset						
<14	58 (93.5)	53 (86.9)	111 (90.2)	62 (84.9)	55 (78.6)	117 (81.8)
>=14	4 (6.5)	8 (13.1)	12 (9.8)	11 (15.1)	15 (21.4)	26 (18.2)
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]	42.9 [20,69]
Region – n (%)						
Asia Pacific	3 (4.8)	5 (8.2)	8 (6.5)	3 (4.1)	5 (7.1)	8 (5.6)
European Union	19 (30.6)	16 (26.2)	35 (28.5)	19 (26)	16 (22.9)	35 (24.5)
North America	31 (50)	33 (54.1)	64 (52)	42 (57.5)	42 (60)	84 (58.8)
Latin America	9 (14.5)	7 (11.5)	16 (13)	9 (12.3)	7 (10)	16 (11.2)

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

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	Primary Analysis Population Change from Baseline to Week 72				Overall Enrolled Population Change from Baseline to Week 72			
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**	**Placebo**	**Vatiquinone**	**Difference**	**P-value**
mFARS Total*	2.83	1.22	-1.61	0.14	2.56	0.90	-1.66	0.098
Bulbar	0.22	0.033	-0.18	**0.044**	0.18	0.033	-0.15	0.069
Upright Stability	2.99	1.73	-1.26	**0.021**	2.49	1.38	-1.11	**0.025**
Lower Limb	0.40	-0.11	-0.51	0.23	0.36	-0.11	-0.47	0.23
Upper Limb	-0.51	-0.18	0.32	0.58	-0.64	-0.35	0.29	0.59

*Primary endpoint which did not meet statistical significance

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Vatiquinone Treatment Resulted in Meaningful Slowing of Disease Symptom Progression

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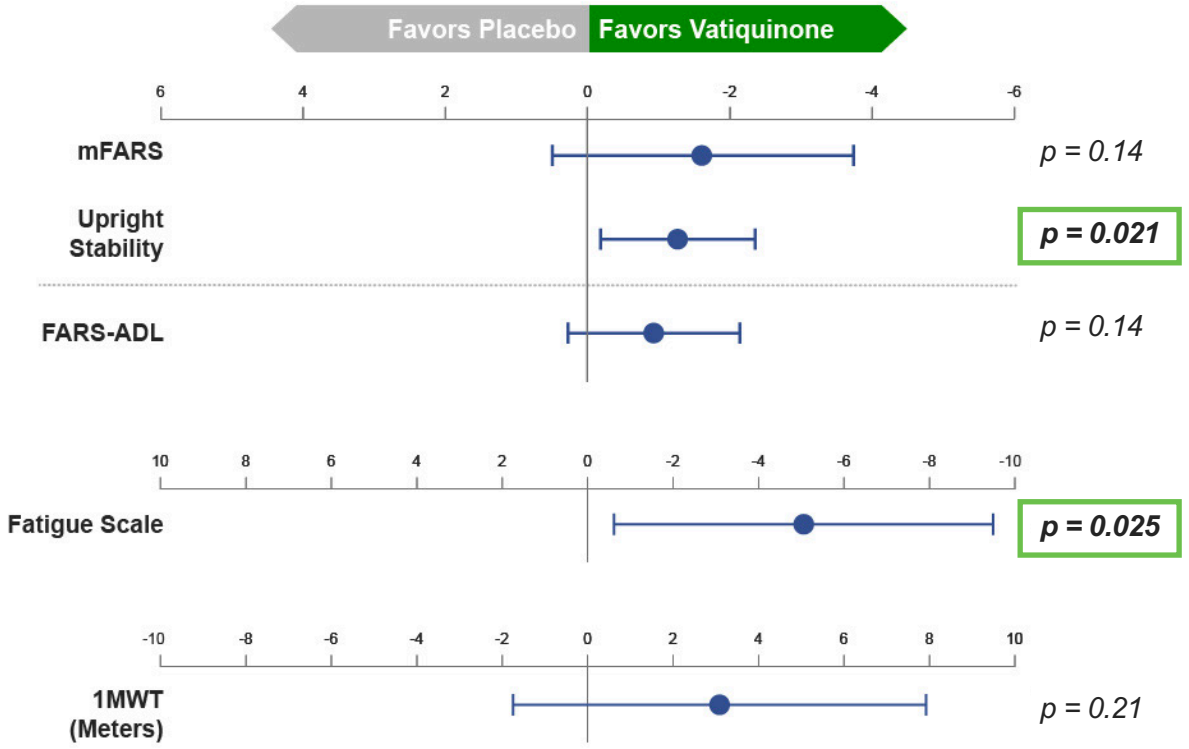
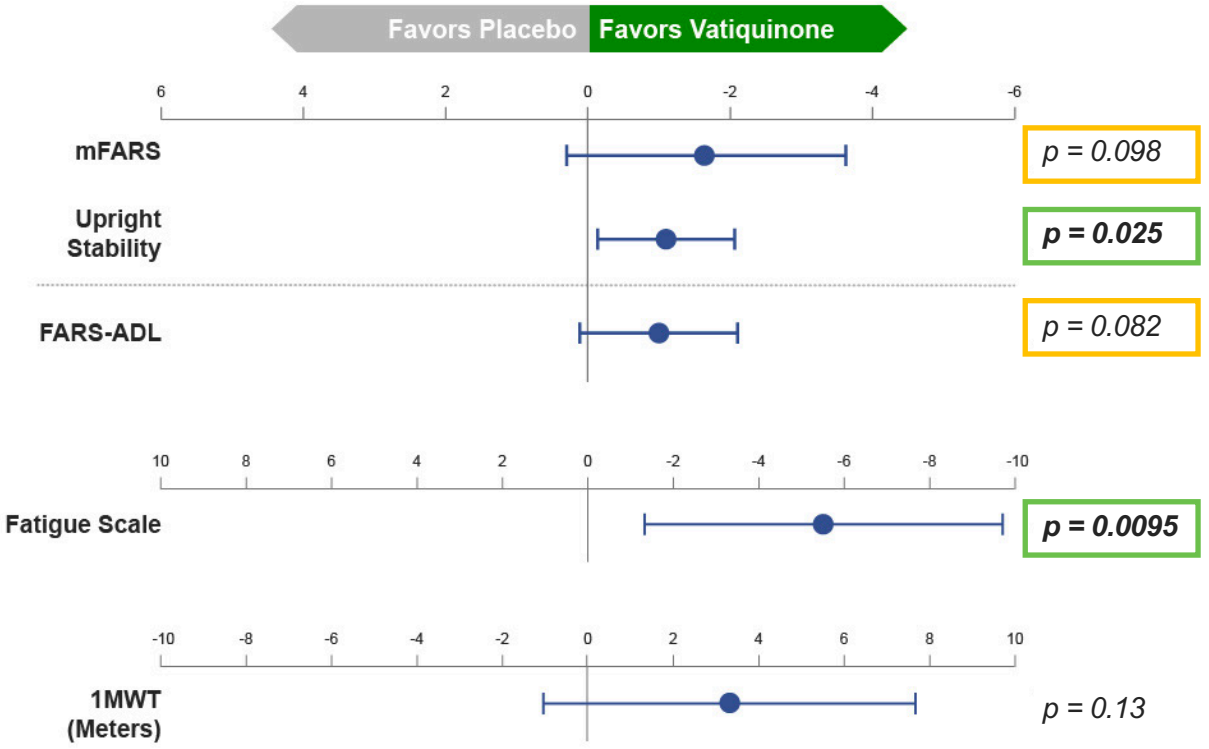
Primary Analysis Population



Overall Study Population



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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 Population		
	Placebo n (%)	Vatiquinone n (%)	Total n (%)	Placebo n (%)	Vatiquinone n (%)	Total n (%)
Randomized	48	48	96	56	54	110
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)	18.3 (8,68)
Age at Onset						
<14	45 (93.8)	40 (83.3)	85 (88.5)	48 (85.7)	41 (75.9)	89 (80.9)
>=14	3 (6.3)	8 (16.7)	11 (11.5)	8 (14.3)	13 (24.1)	21 (19.1)
Region – n (%)						
Asia Pacific	3 (6.3)	4 (8.3)	7 (7.3)	3 (5.4)	4 (7.4)	7 (6.4)
European Union	17 (35.4)	12 (25)	29 (30.2)	17 (30.4)	12 (22.2)	29 (26.4)
North America	21 (43.8)	26 (54.2)	47 (49.0)	29 (51.8)	32 (59.3)	61 (55.5)
Latin America	7 (14.6)	6 (12.5)	13 (13.5)	7 (12.5)	6 (11.1)	13 (11.8)

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

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	Primary Analysis Population Completers Change from Baseline to Week 72				Overall Enrolled Population Completers Change from Baseline to Week 72			
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**	**Placebo**	**Vatiquinone**	**Difference**	**P-value**
mFARS Total	3.08	0.77	-2.31	0.054	2.72	0.57	-2.15	**0.047**
Bulbar	0.17	.0003	-0.17	**0.030**	0.19	0.030	-0.16	0.065
Upright Stability	3.16	1.78	-1.38	**0.026**	2.69	1.45	-1.23	**0.029**
FARS-ADL	1.35	0.66	-0.69	0.29	1.30	0.75	-0.55	0.35
Fatigue Scale (MFIS)	4.14	-0.59	-4.73	**0.042**	3.88	-1.62	-5.50	**0.014**

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

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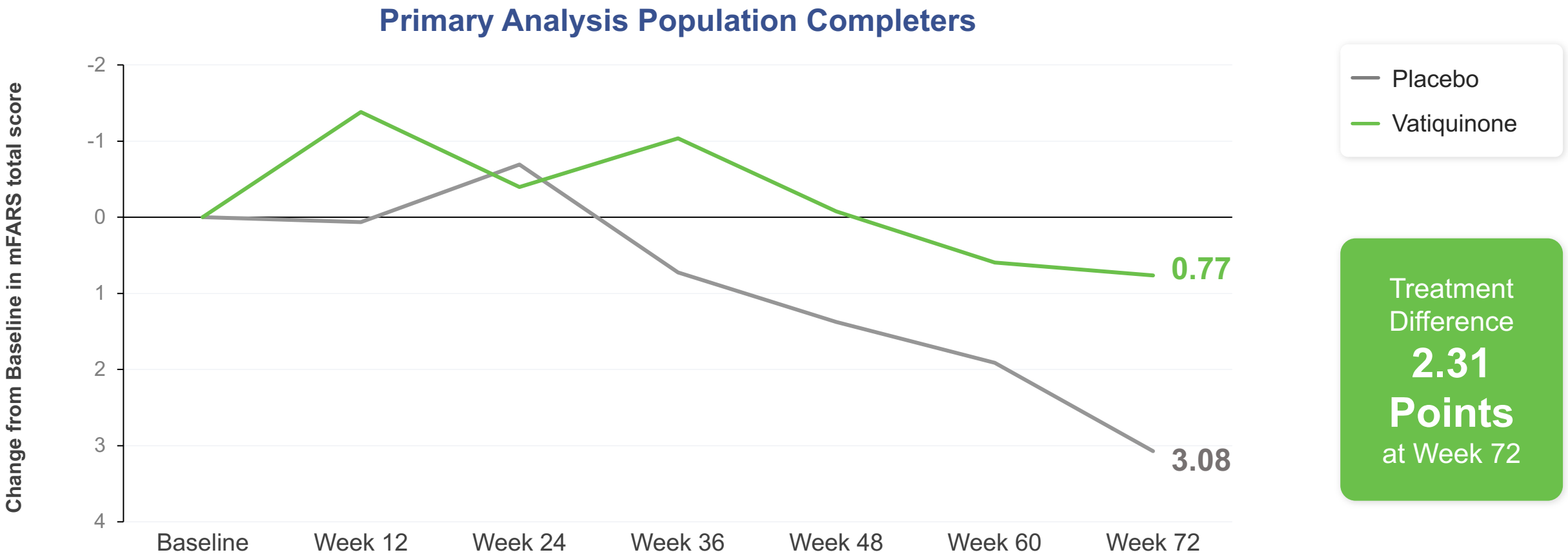

Treatment Difference **2.31 Points** at Week 72

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Vatiquinone Demonstrated to Be Well Tolerated

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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

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Overview of Treatment-Emergent Adverse Events in Overall Study Population

Category	Placebo (N=73) N (%)	Vatiquinone (N=73) N (%)
Subjects with at least one TEAE	73 (100)	71 (97)
Subjects with TEAEs by maximum severity		
Mild	31 (42.5)	26 (35.6)
Moderate	32 (43.8)	37 (50.7)
Severe	9 (12.3)	7 (9.6)
Life-Threatening/Fatal	1 (1.4)	1 (1.4)
Subjects with treatment-related TEAEs		
Probable	4 (5.5)	12 (16.4)
Possible	28 (38.4)	32 (43.8)
Subjects with at least one TESAE	8 (11.0)	8 (11.0)
Subjects with treatment-related TESAES		
Probable	0	0
Possible	0	1 (1.4)
Subjects discontinued study drug due to treatment-related TEAE	3 (4.1)	2 (2.7)

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 potential path to registration with regulatory authorities

Strategic Portfolio Prioritization

- Discontinuation of preclinical and early research programs in gene therapy
- Reduction in workforce related to deprioritized programs and SG&A
- Estimated reductions in residual 2023 OPEX of approximately 15%
- Updated 2023 full-year OPEX to be shared at Q2 earnings