



Clinical trial results:

An Open-label Study to Evaluate Pharmacokinetics, Safety, and Efficacy of Vatiquinone in Children With Friedreich Ataxia Younger Than 7 Years Summary

EudraCT number	2022-003265-38
Trial protocol	Outside EU/EEA
Global end of trial date	29 August 2024

Results information

Result version number	v1 (current)
This version publication date	30 January 2026
First version publication date	30 January 2026

Trial information

Trial identification

Sponsor protocol code	PTC743-NEU-005-FA
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05485987
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	PTC Therapeutics, Inc.
Sponsor organisation address	500 Warren Corp Centre Dr, Warren, United States, NJ 07059
Public contact	Medical Information, PTC Therapeutics, Inc., +011 44 1-866-562-4620, medinfo@ptcbio.com
Scientific contact	Medical Information, PTC Therapeutics International Limited, +353 19068700, medinfo@ptcbio.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMA-001238-PIP03-21
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	25 October 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	29 August 2024
Global end of trial reached?	Yes
Global end of trial date	29 August 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the study was to assess the pharmacokinetics (PK) and safety of vatiquinone administered in participants with Friedreich ataxia (FA) younger than 7 years.

Protection of trial subjects:

This study was designed and monitored in accordance with Sponsor procedures, which comply with the ethical principles of Good Clinical Practice (GCP) as required by the major regulatory authorities, and in accordance with the Declaration of Helsinki.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	13 October 2022
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 5
Worldwide total number of subjects	5
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	5
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Five participants were enrolled and completed the study.

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	Vatiquinone
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Arm description:

Participants received an oral solution (100 milligrams [mg]/milliliter [mL]) of vatiquinone (15 mg/kilogram [kg] if body weight <13 kg and 200 mg if body weight ≥13 kg) 3 times a day (TID) for 72 weeks.

Arm type	Experimental
Investigational medicinal product name	Vatiquinone
Investigational medicinal product code	PTC743, EPI-743
Other name	
Pharmaceutical forms	Oral solution
Routes of administration	Oral use

Dosage and administration details:

Vatiquinone was administered per dose and schedule specified in the arm description.

Number of subjects in period 1	Vatiquinone
Started	5
Received at Least 1 Dose of Study Drug	5
Completed	5

Baseline characteristics

Reporting groups

Reporting group title	Vatiquinone
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Reporting group description:

Participants received an oral solution (100 milligrams [mg]/milliliter [mL]) of vatiquinone (15 mg/kilogram [kg] if body weight <13 kg and 200 mg if body weight ≥13 kg) 3 times a day (TID) for 72 weeks.

Reporting group values	Vatiquinone	Total	
Number of subjects	5	5	
Age Categorical Units: Subjects			
Age Continuous Units: years arithmetic mean standard deviation	5.2 ± 1.79	-	
Gender Categorical Units: Subjects			
Female	2	2	
Male	3	3	
Race Units: Subjects			
White	5	5	
Ethnicity Units: Subjects			
Not Hispanic or Latino	5	5	

End points

End points reporting groups

Reporting group title	Vatiquinone
Reporting group description: Participants received an oral solution (100 milligrams [mg]/milliliter [mL]) of vatiquinone (15 mg/kilogram [kg] if body weight <13 kg and 200 mg if body weight ≥13 kg) 3 times a day (TID) for 72 weeks.	

Primary: Plasma Concentration of Vatiquinone

End point title	Plasma Concentration of Vatiquinone ^[1]
End point description: PK analysis set included all participants who received at least 1 dose of vatiquinone and had evaluable PK data. 'n' signifies participants evaluable at specified timepoint.	
End point type	Primary
End point timeframe: Pre-morning dose (0 hour) at Week 4; 1 to 3 hours and 3 to 6 hours post-morning dose at Weeks 4, 12, and 24	
Notes: [1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point. Justification: The endpoint was descriptive in nature.	

End point values	Vatiquinone			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: nanograms (ng)/mL				
geometric mean (geometric coefficient of variation)				
Week 4 - Pre-dose (n = 5)	173.33 (± 38.37)			
Week 4 - 1-3 Hours Post-dose (n = 4)	347.16 (± 194.95)			
Week 4 - 3-6 Hours Post-dose (n = 5)	417.99 (± 157.26)			
Week 12 - 1-3 Hours Post-dose (n = 5)	674.39 (± 58.77)			
Week 12 - 3-6 Hours Post-dose (n = 5)	1070.60 (± 292.99)			
Week 24 - 1-3 Hours Post-dose (n = 5)	547.63 (± 183.56)			
Week 24 - 3-6 Hours Post-dose (n = 5)	815.52 (± 215.64)			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Treatment-emergent Adverse Events (TEAEs)

End point title	Number of Participants With Treatment-emergent Adverse
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End point description:

An adverse event (AE) was any untoward medical occurrence in a participant who received study drug without regard to possibility of causal relationship. Serious adverse event (SAE) was an AE resulting in any of the following outcomes or deemed significant for any other reason: death; initial or prolonged inpatient hospitalization; life-threatening experience (immediate risk of dying); persistent or significant disability/incapacity; congenital anomaly. AEs included both SAEs and non-serious AEs. A TEAE was defined as an AE that had an onset date on or after the first dose of study drug until 30 days after last dose or occurred prior to first dose of study drug and worsened in severity after first dose of study drug. A summary of other non-serious AEs and all SAEs, regardless of causality is located in the 'Reported AE section'. Safety analysis set included all participants who received at least 1 dose of vatiquinone.

End point type	Primary
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End point timeframe:

Baseline up to Week 76

Notes:

[2] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: The endpoint was descriptive in nature.

End point values	Vatiquinone			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: participants	5			

Statistical analyses

No statistical analyses for this end point

Primary: Area Under the Curve From 0 to 24 Hours Postdose at Steady State (AUC_{0-24h,ss}) of Vatiquinone

End point title	Area Under the Curve From 0 to 24 Hours Postdose at Steady State (AUC _{0-24h,ss}) of Vatiquinone ^[3]
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End point description:

PK analysis set included all participants who received at least 1 dose of vatiquinone and had evaluable PK data.

End point type	Primary
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End point timeframe:

Up to 6 hours postdose on Day 28

Notes:

[3] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: The endpoint was descriptive in nature.

End point values	Vatiquinone			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: ng*hours/mL				
arithmetic mean (confidence interval 90%)	7067.08 (6993.61 to 7140.55)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Baseline up to Week 76

Adverse event reporting additional description:

Safety analysis set included all participants who received at least 1 dose of vatiquinone.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.0
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Reporting groups

Reporting group title	Vatiquinone
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Reporting group description:

Participants received an oral solution (100 milligrams [mg]/milliliter [mL]) of vatiquinone (15 mg/kilogram [kg] if body weight <13 kg and 200 mg if body weight ≥13 kg) 3 times a day (TID) for 72 weeks.

Serious adverse events	Vatiquinone		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 5 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	Vatiquinone		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	5 / 5 (100.00%)		
Nervous system disorders			
Tremor			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences (all)	1		
Headache			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences (all)	1		
General disorders and administration site conditions			

Pyrexia subjects affected / exposed occurrences (all)	2 / 5 (40.00%) 2		
Gastrointestinal disorders Vomiting subjects affected / exposed occurrences (all)	2 / 5 (40.00%) 2		
Abdominal pain subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Skin and subcutaneous tissue disorders Eczema subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Psychiatric disorders Sleep terror subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Infections and infestations Upper respiratory tract infection subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Streptococcal infection subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Otitis media acute subjects affected / exposed occurrences (all)	1 / 5 (20.00%) 1		
Conjunctivitis subjects affected / exposed occurrences (all)	2 / 5 (40.00%) 4		
Ear infection subjects affected / exposed occurrences (all)	2 / 5 (40.00%) 2		
Gastroenteritis subjects affected / exposed occurrences (all)	2 / 5 (40.00%) 3		

Gastroenteritis viral			
subjects affected / exposed	2 / 5 (40.00%)		
occurrences (all)	4		
Viral rhinitis			
subjects affected / exposed	2 / 5 (40.00%)		
occurrences (all)	2		
COVID-19			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences (all)	1		
Herpes zoster			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences (all)	1		
Influenza			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences (all)	1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

No conclusions could be made with regards to efficacy due to the limited sample size of the study population (n=5).

Notes: