



Clinical trial results: An Open-Label, Safety Study for Previously Treated Vatiquinone (PTC743) Subjects With Inherited Mitochondrial Disease Summary

EudraCT number	2022-000375-39
Trial protocol	IT ES FR SE PL
Global end of trial date	15 April 2025

Results information

Result version number	v1 (current)
This version publication date	05 November 2025
First version publication date	05 November 2025

Trial information

Trial identification

Sponsor protocol code	PTC743-CNS-005-LSEP
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05218655
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)	No
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	09 June 2025
Is this the analysis of the primary completion data?	Yes
Primary completion date	15 April 2025
Global end of trial reached?	Yes
Global end of trial date	15 April 2025
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the study was to assess the safety of vatiquinone in participants with inherited mitochondrial disease who participated in a previous vatiquinone clinical study or treatment plan.

Protection of trial subjects:

This study was designed and monitored in accordance with PTC Therapeutics (PTC) procedures, which comply with the ethical principles of Good Clinical Practice 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	22 June 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: 70
Country: Number of subjects enrolled	France: 5
Country: Number of subjects enrolled	Italy: 8
Country: Number of subjects enrolled	Japan: 7
Country: Number of subjects enrolled	Poland: 5
Country: Number of subjects enrolled	Spain: 3
Country: Number of subjects enrolled	United Kingdom: 3
Worldwide total number of subjects	101
EEA total number of subjects	21

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)	39

Adolescents (12-17 years)	30
Adults (18-64 years)	32
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 103 participants were screened, of which 101 participants were enrolled in the study and received at least 1 dose of vatiquinone.

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 vatiquinone oral solution (100 milligrams [mg]/milliliter [mL]), up to 400 mg, administered orally or via feeding tube 3 times daily (TID).

Arm type	Experimental
Investigational medicinal product name	Vatiquinone
Investigational medicinal product code	PTC743
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	101
Received At Least 1 Dose of Study Drug	101
Completed	0
Not completed	101
Sponsor's decision	85
Consent withdrawn by subject	3
Adverse event, non-fatal	6
Death	1
Other than specified	3
Lost to follow-up	3

Baseline characteristics

Reporting groups

Reporting group title	Vatiquinone
Reporting group description:	
Participants received vatiquinone oral solution (100 milligrams [mg]/milliliter [mL]), up to 400 mg, administered orally or via feeding tube 3 times daily (TID).	

Reporting group values	Vatiquinone	Total	
Number of subjects	101	101	
Age Categorical			
Units: Subjects			

Age Continuous			
Units: years			
arithmetic mean	15.5		
standard deviation	± 11.56	-	
Gender Categorical			
Units: Subjects			
Female	49	49	
Male	52	52	
Race			
Units: Subjects			
American Indian/Alaska Native	1	1	
Asian	8	8	
Black/African American	2	2	
White	83	83	
Other	1	1	
Unknown	1	1	
Missing	5	5	
Ethnicity			
Units: Subjects			
Hispanic or Latino	9	9	
Not Hispanic or Latino	86	86	
Not Reported	3	3	
Unknown	2	2	
Missing	1	1	

End points

End points reporting groups

Reporting group title	Vatiquinone
Reporting group description:	
Participants received vatiquinone oral solution (100 milligrams [mg]/milliliter [mL]), up to 400 mg, administered orally or via feeding tube 3 times daily (TID).	

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

End point title	Number of Participants With Treatment-emergent Adverse Events (TEAEs) ^[1]
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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. AEs included both serious adverse events (SAEs) and non-serious AEs. A TEAE was defined as an AE that had an onset date or date of worsening on or after the first dose of study drug and within 30 days of the date of the last dose of treatment. A summary of other non-serious AEs and all SAEs, regardless of causality is located in the 'Reported AE section'. Safety population included all participants who received at least 1 dose of vatiquinone in the study.

End point type	Primary
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End point timeframe:

Baseline (Day 1) up to 30 days after last dose of study drug (956 days)

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 analysis was descriptive in nature.

End point values	Vatiquinone			
Subject group type	Reporting group			
Number of subjects analysed	101			
Units: participants	91			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Baseline (Day 1) up to 30 days after last dose of study drug (956 days)

Adverse event reporting additional description:

Safety population included all participants who received at least 1 dose of vatiquinone in the study.

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 vatiquinone oral solution (100 mg/mL), up to 400 mg, administered orally or via feeding tube TID.

Serious adverse events	Vatiquinone		
Total subjects affected by serious adverse events			
subjects affected / exposed	45 / 101 (44.55%)		
number of deaths (all causes)	7		
number of deaths resulting from adverse events			
Surgical and medical procedures			
Tracheostomy			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Hypothermia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Death			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Generalised oedema			

subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 1		
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	11 / 101 (10.89%)		
occurrences causally related to treatment / all	0 / 16		
deaths causally related to treatment / all	0 / 3		
Acute respiratory distress syndrome			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Asthma			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cough			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hypercapnia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Hypoxia			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Respiratory failure			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Pulmonary amyloidosis			

subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory distress			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Investigations			
SARS-CoV-2 test positive			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Fall			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Postoperative ileus			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Congenital, familial and genetic disorders			
Fanconi syndrome			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Atrial fibrillation			

subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Tachycardia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Cardiomyopathy			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Change in seizure presentation			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Epilepsy			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Autonomic nervous system imbalance			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Myoclonus			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Optic neuritis			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Seizure			

subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Status epilepticus			
subjects affected / exposed	3 / 101 (2.97%)		
occurrences causally related to treatment / all	0 / 4		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Constipation			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Diarrhoea			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Erosive oesophagitis			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal haemorrhage			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Oesophagitis			

subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pancreatitis acute			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pancreatitis			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Pneumoperitoneum			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vomiting			
subjects affected / exposed	4 / 101 (3.96%)		
occurrences causally related to treatment / all	0 / 4		
deaths causally related to treatment / all	0 / 0		
Intestinal obstruction			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Hepatic failure			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Endocrine disorders			

Adrenal insufficiency			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Muscular weakness			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Bronchitis			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
COVID-19			
subjects affected / exposed	3 / 101 (2.97%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Influenza			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Lower respiratory tract infection			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	10 / 101 (9.90%)		
occurrences causally related to treatment / all	0 / 11		
deaths causally related to treatment / all	0 / 0		
Pneumonia aspiration			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Pseudomonas infection			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory tract infection			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Rhinovirus infection			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Sepsis			
subjects affected / exposed	2 / 101 (1.98%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Streptococcal infection			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Urosepsis			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Urinary tract infection			
subjects affected / exposed	3 / 101 (2.97%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Tracheitis			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Metabolism and nutrition disorders			

Hypokalaemia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hypercalcaemia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hyponatraemia			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Mitochondrial cytopathy			
subjects affected / exposed	1 / 101 (0.99%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		

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	59 / 101 (58.42%)		
Investigations			
Gamma-glutamyltransferase increased			
subjects affected / exposed	6 / 101 (5.94%)		
occurrences (all)	7		
Nervous system disorders			
Seizure			
subjects affected / exposed	9 / 101 (8.91%)		
occurrences (all)	9		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	12 / 101 (11.88%)		
occurrences (all)	24		
Gastrointestinal disorders			

Diarrhoea			
subjects affected / exposed	6 / 101 (5.94%)		
occurrences (all)	7		
Vomiting			
subjects affected / exposed	11 / 101 (10.89%)		
occurrences (all)	15		
Infections and infestations			
COVID-19			
subjects affected / exposed	13 / 101 (12.87%)		
occurrences (all)	14		
Nasopharyngitis			
subjects affected / exposed	7 / 101 (6.93%)		
occurrences (all)	9		
Pneumonia			
subjects affected / exposed	6 / 101 (5.94%)		
occurrences (all)	9		
Respiratory tract infection			
subjects affected / exposed	6 / 101 (5.94%)		
occurrences (all)	10		
Upper respiratory tract infection			
subjects affected / exposed	14 / 101 (13.86%)		
occurrences (all)	19		
Urinary tract infection			
subjects affected / exposed	9 / 101 (8.91%)		
occurrences (all)	9		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
11 April 2022	Overall reason for this amendment: All participants rolling over to this study followed the same visit scheme from Screening/Baseline Visit.

Notes:

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.

The study was terminated early as PTC discontinued clinical development of vatiquinone for this indication based on prior clinical study results.

Notes: