Clinical trial results:
A Phase 2 Study of the Safety, Pharmacokinetics, and Pharmacodynamics of Ataluren (PTC124®) in Patients Aged 2 to <5 Years Old with Nonsense Mutation Dystrophinopathy

Summary

EudraCT number	2016-001764-11
Trial protocol	Outside EU/EEA
Global end of trial date	09 February 2018

Results information

Result version number	v1 (current)
This version publication date	03 October 2020
First version publication date	03 October 2020

Trial information

Trial identification

Sponsor protocol code	PTC124-GD-030-DMD

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02819557
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	PTC Therapeutics, Inc.
Sponsor organisation address	100 Corporate Court, South Plainfield, United States, NJ 07080
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)	EMEA-000115-PIP01-07
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	29 March 2018
Is this the analysis of the primary completion data?	Yes
Primary completion date	09 February 2018
Global end of trial reached?	Yes
Global end of trial date	09 February 2018
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Evaluate the safety of ataluren as measured by type, frequency, severity, timing, and relationship to study drug of treatment emergent adverse events (TEAEs), laboratory abnormalities, and electrocardiogram (ECGs).

Protection of trial subjects:

This study was conducted in accordance with International Conference on Harmonisation (ICH) Good Clinical Practice, and the principles of the Declaration of Helsinki, in addition to following the laws and regulations of the country or countries in which a study is conducted.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	09 June 2016
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 14
Worldwide total number of subjects	14
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)	14
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:

Participants aged ≥2 to <5 years old with Duchenne muscular dystrophy (DMD) caused by a nonsense mutation in the dystrophin gene were recruited for this study.

Pre-assignment

Screening details:

Participants in the Evaluable Population included all participants who received at least 1 dose of ataluren and had a baseline and at least 1 postbaseline measurement for Timed Function Test (TFT), North Star Ambulatory Assessment (NSAA), or response to the palatability of ataluren questions.

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	Ataluren

Arm description:

Participants were administered ataluren orally at a dose of 10 milligrams/kilograms (mg/kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening (for a total of 40 mg/kg/day) for up to 52 weeks. Dose was provided based upon the weight of each participant, which was assessed every 12 weeks.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	PTC124
Other name	Translarna
Pharmaceutical forms	Powder for oral suspension
Routes of administration	Oral use

Dosage and administration details:

White to off-white powder for oral suspension.

Number of subjects in period 1	Ataluren
Started	14
Safety Population	14
Pharmacokinetics (PK) Population	14
Evaluable Population	14
Completed	14

Baseline characteristics

Reporting groups	
Reporting group title	Ataluren
Reporting group description:	
Participants were administered ataluren orally at a dose of 10 milligrams/kilograms (mg/kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening (for a total of 40 mg/kg/day) for up to 52 weeks. Dose was provided based upon the weight of each participant, which was assessed every 12 weeks.	

Reporting group values	Ataluren	Total	
Number of subjects	14	14	
Age categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	14	14	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	0	0	
From 65-84 years	0	0	
85 years and over	0	0	
Age Continuous Units: years			
arithmetic mean	3.4		
standard deviation	± 0.76	-	
Sex: Female, Male Units: participants			
Female	0	0	
Male	14	14	
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0	0	
Asian	3	3	
Native Hawaiian or Other Pacific Islander	0	0	
Black or African American	0	0	
White	11	11	
More than one race	0	0	
Unknown or Not Reported	0	0	
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	3	3	
Not Hispanic or Latino	11	11	
Unknown or Not Reported	0	0	

End points

End points reporting groups

Reporting group title	Ataluren

Reporting group description:

Participants were administered ataluren orally at a dose of 10 milligrams/kilograms (mg/kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening (for a total of 40 mg/kg/day) for up to 52 weeks. Dose was provided based upon the weight of each participant, which was assessed every 12 weeks.

Primary: Number of Participants With Treatment Emergent Adverse Events (TEAEs), TEAEs Leading to Discontinuation, and Serious Adverse Events (SAEs)

End point title	Number of Participants With Treatment Emergent Adverse Events (TEAEs), TEAEs Leading to Discontinuation, and Serious Adverse Events (SAEs)[1]

End point description:

A TEAE was any untoward medical occurrence or undesirable event that begins or worsens following administration of study drug, whether or not considered related to study drug by Investigator. An SAE was an adverse event (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) or persistent or significant disability/incapacity not related to dystrophinopathy. An event was not reported as an SAE, if event was exclusively a relapse or expected change or progression of baseline dystrophinopathy. AEs included both SAEs and nonserious AEs. AEs classified according to NCI CTCAE v4.0 and coded using MedDRA. Population included all participants who received at least 1 dose of ataluren (Safety Population). A summary of SAEs and all non-serious AEs, regardless of causality, is located in the Reported Adverse Events section.

End point type	Primary

End point timeframe:

Baseline up to Week 56

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: Statistical analyses not applicable for this endpoint.

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: participants				
At least 1 TEAE	14			
Mild TEAE	5			
Moderate TEAE	8			
Severe TEAE	1			
TEAE Related to Study Drug	5			
TEAE Leading to Participant Study Discontinuation	0			
Serious TEAE	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With a Clinically Meaningful Abnormal Clinical Laboratory (Biochemistry, Hematology, and Urinalysis) Parameter

End point title	Number of Participants With a Clinically Meaningful Abnormal Clinical Laboratory (Biochemistry, Hematology, and Urinalysis) Parameter[2]

End point description:

Clinical laboratory results that were considered clinically meaningful were determined by the Investigator and Sponsor. Biochemistry parameters: sodium, potassium, chloride, bicarbonate, blood urea nitrogen, creatinine, magnesium, calcium, phosphorus, uric acid, glucose, total protein, albumin, bilirubin (total, direct, and indirect), AST, ALT, gamma-glutamyl transferase, creatine kinase, lactate dehydrogenase, ALP, total cholesterol, high-density lipoprotein, low-density lipoprotein, triglycerides, and cystatin C. Hematology parameters: WBC count with differential, hemoglobin, hematocrit, other red cell parameters, and platelet count. Urinalysis parameters: pH, specific gravity, glucose, ketones, blood, protein, urobilinogen, bilirubin, nitrite, and leukocyte esterase. Population included all participants who received at least 1 dose of ataluren (Safety Population). A summary of other non-serious AEs and all serious AEs, regardless of causality is located in Reported AE section.

End point type	Primary

End point timeframe:

Baseline up to Week 56

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: Statistical analyses not applicable for this endpoint.

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With a Clinically Meaningful Abnormal ECG Test Results

End point title	Number of Participants With a Clinically Meaningful Abnormal ECG Test Results[3]

End point description:

ECG results that were considered clinically meaningful were to be determined by the Investigator. Population included all participants who received at least 1 dose of ataluren (Safety Population). A summary of other non-serious AEs and all serious AEs, regardless of causality is located in Reported AE section.

End point type	Primary

End point timeframe:

Baseline up to Week 56

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: Statistical analyses not applicable for this endpoint.

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants with a Dose-Limiting Toxicity as Measured by Hepatic and Renal Toxicity

End point title	Number of Participants with a Dose-Limiting Toxicity as Measured by Hepatic and Renal Toxicity[4]

End point description:

Dose-limiting toxicity was measured through clinical evaluations for potential hepatic and renal toxicities. The clinical evaluations included the following:
• Hepatic: The participant's medical history, hepatitis screening results, all clinical blood values (particularly serum bilirubin, gamma-glutamyl transferase [GGT], aspartate aminotransferase [AST], and alanine aminotransferase [ALT] values), and all concomitant medications were reviewed.
• Renal: The participant's medical history, all clinical blood and urine renal values, serum electrolytes, medications, and potential pre- or post-renal conditions were reviewed.
Population included all participants who received at least 1 dose of ataluren (Safety Population).

End point type	Primary

End point timeframe:

Baseline up to Week 56

Notes:

[4] - 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: Statistical analyses not applicable for this endpoint.

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics: Maximum Observed Plasma Concentration From Time Zero up to 6 Hours After the Morning Dose (Cmax0-6hr)

End point title	Pharmacokinetics: Maximum Observed Plasma Concentration From Time Zero up to 6 Hours After the Morning Dose (Cmax0-6hr)

End point description:

Ataluren concentrations in plasma were analyzed using a validated high performance liquid chromatography-tandem mass spectrometry (HPLC-MS/MS) method. Population included all participants who received at least 1 dose of ataluren and had at least 1 PK concentration datum (PK Population).

End point type	Secondary

End point timeframe:

0 (predose), 1, 2, 4, and 6 (postdose) hours on Days 1 and 28

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: microgram/milliliter (µg/mL)				
arithmetic mean (standard deviation)				
Day 1	15.95 (± 9.51)			
Day 28	12.54 (± 4.43)			

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics: Time to Reach Maximum Observed Plasma Concentration From Time Zero up to 6 Hours After the Morning Dose (Tmax0-6hr)

End point title	Pharmacokinetics: Time to Reach Maximum Observed Plasma Concentration From Time Zero up to 6 Hours After the Morning Dose (Tmax0-6hr)

End point description:

Ataluren concentrations in plasma were analyzed using a validated HPLC-MS/MS method. Population included all participants who received at least 1 dose of ataluren and had at least 1 PK concentration datum (PK Population).

End point type	Secondary

End point timeframe:

0 (predose), 1, 2, 4, and 6 (postdose) hours on Days 1 and 28

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: hours				
arithmetic mean (standard deviation)				
Day 1	3.84 (± 1.82)			
Day 28	2.72 (± 1.98)			

Statistical analyses

No statistical analyses for this end point

Secondary: Area Under the Plasma Concentration Time Curve From Time Zero up to 10 Hours After the Morning Dose (AUC0-10hr)

End point title	Area Under the Plasma Concentration Time Curve From Time Zero up to 10 Hours After the Morning Dose (AUC0-10hr)

End point description:

Ataluren concentrations in plasma were analyzed using a validated HPLC-MS/MS method. AUC0-10hr was measured using the linear trapezoidal rule during the ascending portion of the curve and the log-trapezoidal rule during the descending portion of the curve. Population included all participants who received at least 1 dose of ataluren and had at least 1 PK concentration datum (PK Population).

End point type	Secondary

End point timeframe:

0 (predose), 1, 2, 4, 6, 8, and 10 (postdose) hours on Days 1 and 28

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: hour*µg/mL				
arithmetic mean (standard deviation)				
Day 1	101.64 (± 58.52)			
Day 28	82.13 (± 27.43)			

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics: Concentration at the End of the First (Morning) Dose Interval (Ctrough6hr)

End point title	Pharmacokinetics: Concentration at the End of the First (Morning) Dose Interval (Ctrough6hr)

End point description:

Ataluren concentrations in plasma were analyzed using a validated HPLC-MS/MS method. Population included all participants who received at least 1 dose of ataluren and had at least 1 PK concentration datum (PK Population).

End point type	Secondary

End point timeframe:

0 (predose), 1, 2, 4, and 6 (postdose) hours on Days 1 and 28

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: microgram per milliliter (µg/ml)				
arithmetic mean (standard deviation)				
Day 1	9.12 (± 8.88)			
Day 28	5.43 (± 3.15)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Proximal Muscle Function as Assessed by Speed During TFTs

End point title	Change From Baseline in Proximal Muscle Function as Assessed by Speed During TFTs

End point description:

TFTs included time to stand from supine position (rise to standing), time to run/walk 10 meters (m), and time to ascend/descend 4 stairs. A decrease from baseline reflects faster completion of the functional task and, thus, better muscle function. If the time taken to perform a test exceeded 30 seconds or if a participant could not perform the test due to disease progression (PD), a value of 30 seconds was used. Population included all participants who received at least 1 dose of ataluren and had a baseline and at least 1 postbaseline measurement for TFT, NSAA, or response to the palatability of ataluren questions (Evaluable Population) and had evaluable data for the applicable timed function test.

End point type	Secondary

End point timeframe:

Baseline, Week 28 (W28) and Week 52 (W52)

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: seconds				
arithmetic mean (standard deviation)				
Rise to Standing, Baseline	7.2 (± 7.21)			
Rise to Standing Change from Baseline at W28, n=13	-3.1 (± 6.47)			
Rise to Standing, Change from Baseline at Week 52	-3.1 (± 6.50)			
Walk/Run 10 m, Baseline	6.6 (± 2.37)			
Walk/Run 10 m, Change from Baseline at W28, n=13	-0.8 (± 1.54)			
Walk/Run 10 m, Change from Baseline at Week 52	-1.1 (± 1.35)			
Ascend 4 Stairs, Baseline	7.1 (± 6.95)			
Ascend 4 Stairs, Change from Baseline at W28, n=13	-1.8 (± 4.85)			
Ascend 4 Stairs, Change from Baseline at Week 52	-2.6 (± 5.00)			
Descend 4 Stairs, Baseline, n=13	7.5 (± 3.95)			
Descend 4 Stairs Change from Baseline at W28, n=12	-0.6 (± 1.93)			
Descend 4 Stairs Change from Baseline at W52, n=13	-2.2 (± 2.58)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Physical Function as Measured by the NSAA

End point title	Change From Baseline in Physical Function as Measured by the NSAA

End point description:

NSAA: 17 activities including items assessing abilities necessary to remain functionally ambulant (ie, ability to rise from floor, to go from lying to sitting/sitting to standing, and that are known to progressively deteriorate); items that can be partly present in DMD early stages (ie, assessing head raise and standing on heels); and a number of activities such as hopping, jumping and running. Since the boys were <5 years old, revised 16, 8, and 3-point (pt), scales were used over the 17 pt scale. Scores for evaluations=0 (Unable to achieve independently), 1 (Modified method but achieved goal independent of physical assistance) or 2 (Normal, no obvious modification of activity). Maximum total score for the 16-pt scale=32, 8-pt scale=16 and 3-pt scale=6. If an activity couldn't be performed due to PD/loss of ambulation, a score of 0 was assigned. Change from Baseline=subtracting Baseline value from Week 28 and Week 52 values. Population=Evaluable Population with evaluable NSAA data.

End point type	Secondary

End point timeframe:

Baseline, Week 28 and Week 52

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: units on a scale				
arithmetic mean (standard deviation)				
16-Point Scale, Baseline	16.0 (± 4.66)			
16-Point Scale Change from Baseline at Wk 28, n=13	3.5 (± 3.43)			
16-Point Scale, Change from Baseline at Week 52	5.5 (± 4.43)			
8-Point Scale, Baseline	10.5 (± 2.56)			
8-Point Scale, Change from Baseline at Wk 28, n=13	1.5 (± 1.39)			
8-Point Scale, Change from Baseline at Week 52	2.3 (± 2.13)			
3-Point Scale, Baseline	5.4 (± 0.63)			
3-Point Scale, Change from Baseline at Wk 28, n=13	0.5 (± 0.78)			
3-Point Scale, Change from Baseline at Week 52	0.3 (± 0.73)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Height of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point title	Change From Baseline in Height of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point description:

Population included all participants who received at least 1 dose of ataluren (Safety Population) and had evaluable height data.

End point type	Secondary

End point timeframe:

Baseline, Weeks 4, 16, 28, 40, 52, and 56

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: centimeters				
arithmetic mean (standard deviation)				
Baseline	99.43 (± 5.278)			
Change at Week 4	0.83 (± 1.595)			
Change at Week 16	1.84 (± 1.466)			
Change at Week 28	3.11 (± 1.386)			
Change at Week 40, n=13	3.82 (± 1.506)			
Change at Week 52	5.95 (± 2.096)			
Change at Week 56	6.04 (± 2.075)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Weight of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point title	Change From Baseline in Weight of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point description:

Population included all participants who received at least 1 dose of ataluren (Safety Population) and had evaluable weight data.

End point type	Secondary

End point timeframe:

Baseline, Weeks 4, 16, 28, 40, 52, and 56

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: kg				
arithmetic mean (standard deviation)				
Baseline	16.99 (± 3.257)			
Change at Week 4	-0.04 (± 0.502)			
Change at Week 16	0.73 (± 0.974)			
Change at Week 28	1.16 (± 0.940)			
Change at Week 40, n=13	1.39 (± 0.882)			
Change at Week 52	1.90 (± 1.259)			
Change at Week 56	2.13 (± 1.340)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Body Mass Index of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point title	Change From Baseline in Body Mass Index of Participants at Weeks 4, 16, 28, 40, 52, and 56

End point description:

Body mass index is an estimate of body fat based on body weight divided by height squared. Population included all participants who received at least 1 dose of ataluren (Safety Population) and had evaluable body mass index data.

End point type	Secondary

End point timeframe:

Baseline, Weeks 4, 16, 28, 40, 52, and 56

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: kilograms per square meter (kg/m^2)				
arithmetic mean (standard deviation)				
Baseline	17.094 (± 2.2196)			
Change at Week 4	-0.346 (± 0.6804)			
Change at Week 16	0.026 (± 0.3822)			
Change at Week 28	0.030 (± 0.5567)			
Change at Week 40, n=13	0.008 (± 0.6399)			
Change at Week 52	-0.186 (± 0.9237)			

Change at Week 56	0.015 (± 1.2161)			

Statistical analyses

No statistical analyses for this end point

Secondary: Ataluren Palatability Characteristics as Determined by a Parent/Caregiver Questionnaire

End point title	Ataluren Palatability Characteristics as Determined by a Parent/Caregiver Questionnaire

End point description:

To assess palatability characteristics, participants/parents or guardians were asked to provide a response of "Strongly disagree", "Disagree", "Neither agree or disagree", "Agree", or "Strongly Agree" to the following 3 questions: Question 1. "Is the medicine palatable?" Question 2. "On the basis of reaction / facial expression of your child, do you think that the medication is pleasant?" Question 3."You sometimes have problems in giving the medication to your child because he/she refuses to take it or throws it up?" Population included all participants who received at least 1 dose of ataluren and had a baseline and at least 1 postbaseline measurement for TFT, NSAA, or response to the palatability of ataluren questions (Evaluable Population).

End point type	Secondary

End point timeframe:

Baseline up to Week 28

End point values	Ataluren			
Subject group type	Reporting group			
Number of subjects analysed	14			
Units: Count of participants				
Question 1, Strongly disagree	0			
Question 1, Disagree	0			
Question 1, Neither Agree nor Disagree	2			
Question 1, Agree	0			
Question 1, Strongly agree	0			
Question 1, No Response	12			
Question 2, Strongly disagree	0			
Question 2, Disagree	2			
Question 2, Neither Agree nor Disagree	2			
Question 2, Agree	6			
Question 2, Strongly Agree	4			
Question 2, No response	0			
Question 3, Strongly Disagree	5			
Question 3, Disagree	7			
Question 3, Neither agree or disagree	0			
Question 3, Agree	2			
Question 3, Strongly Agree	0			
Question 3, No response	0			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Baseline up to Week 56	
Adverse event reporting additional description:	
Adverse events were collected from all participants who received at least 1 dose of ataluren (Safety Population).	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	19.0

Reporting groups	
Reporting group title	Ataluren
Reporting group description:	
Participants were administered ataluren orally at a dose of 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening (for a total of 40 mg/kg/day) for up to 52 weeks. Dose was provided based upon the weight of each participant, which was assessed every 12 weeks.	

Serious adverse events	Ataluren		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 14 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Ataluren		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	14 / 14 (100.00%)		
Investigations			
Hepatic Enzyme Increased			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	3		
Contusion			

subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Arthropod Bite			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	2		
Tibia Fracture			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Gait Disturbance			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Pyrexia			
subjects affected / exposed	6 / 14 (42.86%)		
occurrences (all)	14		
Gastrointestinal disorders			
Constipation			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	2		
Abdominal Pain Upper			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	3		
Flatulence			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	2		
Nausea			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Stomatitis			

subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	3 / 14 (21.43%)		
occurrences (all)	6		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	3 / 14 (21.43%)		
occurrences (all)	6		
Epistaxis			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Rhinorrhoea			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Tonsillar Hypertrophy			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Hypertrichosis			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Rash			
subjects affected / exposed	3 / 14 (21.43%)		
occurrences (all)	3		
Rash Generalised			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Urticaria			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Psychiatric disorders			
Abnormal Behaviour			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Enuresis			

subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Insomnia			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Renal and urinary disorders			
Chromaturia			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	2		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Pain In Extremity			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	3		
Infections and infestations			
Bronchitis Viral			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Conjunctivitis			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Croup Infectious			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Ear Infection			
subjects affected / exposed	5 / 14 (35.71%)		
occurrences (all)	5		
Gastroenteritis			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Gastroenteritis Norovirus			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Gingival Abscess			

subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Hordeolum			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Influenza			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	3		
Nasopharyngitis			
subjects affected / exposed	4 / 14 (28.57%)		
occurrences (all)	8		
Pharyngitis Streptococcal			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	2		
Respiratory Tract Infection			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Rhinitis			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Upper Respiratory Tract Infection			
subjects affected / exposed	2 / 14 (14.29%)		
occurrences (all)	2		
Viral Rash			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Metabolism and nutrition disorders			
Decreased Appetite			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Hyperlipidaemia			
subjects affected / exposed	1 / 14 (7.14%)		
occurrences (all)	1		
Metabolic Acidosis			
subjects affected / exposed	1 / 14 (7.14%)		
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

None reported