Clinical trial results:
A Phase 2B Efficacy and Safety Study of PTC124 in Subjects With Nonsense-Mutation-Mediated Duchenne and Becker Muscular Dystrophy

Summary

EudraCT number	2008-007648-32
Trial protocol	BE FR DE SE GB ES IT
Global end of trial date	31 January 2009

Results information

Result version number	v1 (current)
This version publication date	11 July 2020
First version publication date	11 July 2020

Trial information

Trial identification

Sponsor protocol code	PTC124-GD-007e-DMD

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT00847379
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., +353 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	20 July 2010
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	31 January 2009
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study was to evaluate the long-term safety of ataluren in boys with nonsense mutation Duchenne/Becker muscular dystrophy (nmDBMD), as determined by adverse events and laboratory abnormalities.

Protection of trial subjects:

The study was conducted in accordance with the ethical principles that have their origins in the Declaration of Helsinki (revised version of Edinburgh, Scotland, 2000) and in conformance with the International Conference on Harmonisation (ICH) Good Clinical Practice (GCP) guidance documents.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	31 January 2009
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	Belgium: 5
Country: Number of subjects enrolled	Australia: 11
Country: Number of subjects enrolled	Canada: 10
Country: Number of subjects enrolled	France: 8
Country: Number of subjects enrolled	Germany: 11
Country: Number of subjects enrolled	Israel: 3
Country: Number of subjects enrolled	Italy: 13
Country: Number of subjects enrolled	Spain: 6
Country: Number of subjects enrolled	Sweden: 8
Country: Number of subjects enrolled	United Kingdom: 21
Country: Number of subjects enrolled	United States: 77
Worldwide total number of subjects	173
EEA total number of subjects	72

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)	146
Adolescents (12-17 years)	26
Adults (18-64 years)	1
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

A total of 173 participants were screened for eligibility to enter this extension study after completing the 48-week double-blind study PTC124-GD-007-DMD (NCT00592553). All participants met entry criteria and enrolled in this study.

Pre-assignment

Screening details:

Participants were categorized by the treatment group to which they had been randomly assigned in Study PTC124-GD-007-DMD (NCT00592553).

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	High-Dose Ataluren/High-Dose Ataluren

Arm description:

Participants who were randomized to receive high-dose ataluren in study PTC124-GD-007-DMD, continued to receive ataluren suspension orally 3 times a day (TID), 20 milligrams/kilogram (mg/kg) at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit (Week 48) in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	PTC124
Other name	
Pharmaceutical forms	Oral drops, powder for suspension
Routes of administration	Oral use

Dosage and administration details:

Ataluren was administered as per dose and schedule specified in the arm.

Arm title	Low-Dose Ataluren/High-Dose Ataluren

Arm description:

Participants who were randomized to receive low-dose ataluren in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	PTC124
Other name	
Pharmaceutical forms	Oral drops, powder for suspension
Routes of administration	Oral use

Dosage and administration details:

Ataluren was administered as per dose and schedule specified in the arm.

Arm title	Placebo/High-Dose Ataluren

Arm description:

Participants who were randomized to receive placebo in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of placebo at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	PTC124
Other name	
Pharmaceutical forms	Oral drops, powder for suspension
Routes of administration	Oral use

Dosage and administration details:

Ataluren was administered as per dose and schedule specified in the arm.

Number of subjects in period 1	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren
Started	59	57	57
As-treated population	59	57	57
Completed	0	0	0
Not completed	59	57	57
Study terminated by Sponsor	59	57	57

Baseline characteristics

Reporting groups	
Reporting group title	High-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive high-dose ataluren in study PTC124-GD-007-DMD, continued to receive ataluren suspension orally 3 times a day (TID), 20 milligrams/kilogram (mg/kg) at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit (Week 48) in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Low-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive low-dose ataluren in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Placebo/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive placebo in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of placebo at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren
Number of subjects	59	57	57
Age categorical Units: Subjects			
Age Continuous Units: years			
arithmetic mean	9.4	9.7	9.3
standard deviation	± 2.53	± 2.89	± 2.28
Sex: Female, Male Units: Subjects			
Female	0	0	0
Male	59	57	57

Reporting group values	Total		
Number of subjects	173		
Age categorical Units: Subjects			

Age Continuous Units: years arithmetic mean standard deviation	- 		
Sex: Female, Male Units: Subjects			
Female Male	0 173		

Subject analysis sets

Subject analysis set title	Overall Participants: High-Dose Ataluren
Subject analysis set type	Full analysis

Subject analysis set description:

All participants received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group values	Overall Participants: High-Dose Ataluren		
Number of subjects	173		
Age categorical Units: Subjects			

Age Continuous Units: years arithmetic mean standard deviation	 ±		
Sex: Female, Male Units: Subjects			
Female Male			

End points

End points reporting groups

Reporting group title	High-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive high-dose ataluren in study PTC124-GD-007-DMD, continued to receive ataluren suspension orally 3 times a day (TID), 20 milligrams/kilogram (mg/kg) at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit (Week 48) in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Low-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive low-dose ataluren in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Placebo/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive placebo in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of placebo at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Subject analysis set title	Overall Participants: High-Dose Ataluren
Subject analysis set type	Full analysis

Subject analysis set description:

All participants received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Primary: Number of Participants With Treatment-Emergent Adverse Events (AEs)

End point title	Number of Participants With Treatment-Emergent Adverse Events (AEs)[1]

End point description:

AE: any untoward medical occurrence in a participant who received study drug without regard to possibility of causal relationship. Severity of an AE was classified as: mild (does not interfere with usual function), moderate (interferes to some extent with usual function) and severe (interferes significantly with usual function). Serious AEs: death, a life-threatening AE, inpatient hospitalization or prolongation of existing hospitalization, persistent or significant disability or incapacity, a congenital anomaly or birth defect, or an important medical event that jeopardized participant and required medical intervention. TEAE: AE that occurred or worsened in period extending from first dose of study drug in this study to 6 weeks after last dose of study drug. A summary of other non-serious AEs and all serious AEs, regardless of causality is located in Reported AE section. As-treated population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e.

End point type	Primary

End point timeframe:

Baseline (Week 48 of Study 007) up to Week 102

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: Safety analyses were descriptive in nature.

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	59	57	57	173
Units: participants				
Any AEs	52	49	48	149
Drug-related AEs	10	10	22	42
Serious AEs	0	1	2	3
Mild AEs	34	23	26	83
Moderate AEs	13	22	19	54
Severe AEs	5	4	3	12

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Clinically Significant Abnormal Laboratory Parameters

End point title	Number of Participants With Clinically Significant Abnormal Laboratory Parameters[2]

End point description:

Laboratory parameters tests included hematology, biochemistry assay (hepatic, renal, and serum electrolyte values), adrenal assays, and urinalysis. Clinical significance was defined as per investigator's judgement. As-treated population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e.

End point type	Primary

End point timeframe:

Baseline (Week 48 of Study 007) up to Week 102

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: Safety analyses were descriptive in nature.

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	59	57	57	173
Units: participants	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in 6-Minute Walk Distance (6MWD) at Week 60

End point title	Change From Baseline in 6-Minute Walk Distance (6MWD) at Week 60

End point description:

The 6MWD test was performed in a 30-meters-long flat corridor, where the participant was instructed to walk as far as possible, back and forth around two cones, with the permission to slow down, rest, or stop if needed. Ambulation was assessed via the 6MWD test following standardized procedures by measuring the 6MWD in meters. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: meters				
arithmetic mean (standard deviation)				
Baseline (n=53, 50, 49, 152)	351.60 (± 130.312)	375.95 (± 98.976)	356.25 (± 109.369)	361.11 (± 113.760)
Change at Week 60 (n=46, 46, 47, 139)	-17.23 (± 39.608)	-10.50 (± 41.390)	-7.06 (± 50.121)	-11.56 (± 43.881)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Mean Activity Period/Day/Visit at Week 60, as Assessed by Step Activity Monitoring (SAM)

End point title	Change From Baseline in Mean Activity Period/Day/Visit at Week 60, as Assessed by Step Activity Monitoring (SAM)

End point description:

Participants were instructed to continue to wear SAM for at least 9 consecutive days. SAM was used to record the number of strides/minute following each visit. A stride is the leg motion that begins when the foot with SAM leaves the floor and ends when the same foot touches the floor again. For each day, an active period was defined as the first time after 3:00 AM that greater than (>) 2 strides/minute were recorded to the last time prior to midnight that >2 strides/minute were recorded. Days were deleted on which such an active period was less than (<) 50 percent (%) of the mean active period across all days for that participant's visit. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: meters				
arithmetic mean (standard deviation)				
Baseline (n=52, 49, 48, 149)	759.715 (± 77.5210)	742.223 (± 107.7264)	747.464 (± 102.6531)	750.016 (± 96.1057)
Change at Week 60 (n=41, 38, 40, 119)	1.946 (± 61.2247)	34.283 (± 88.1587)	-1.866 (± 93.0531)	10.991 (± 82.6356)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Mean Total Step Count/Day/Visit During the Active Periods at Week 60, as Assessed by SAM

End point title	Change From Baseline in Mean Total Step Count/Day/Visit During the Active Periods at Week 60, as Assessed by SAM

End point description:

Participants were instructed to continue to wear SAM for at least 9 consecutive days. SAM was used to record the number of strides/minute following each visit. A stride is the leg motion that begins when the foot with SAM leaves the floor and ends when the same foot touches the floor again (that is, a stride generally equals 2 steps). For each day, an active period was defined as the first time after 3:00 AM that >2 strides/minute were recorded to the last time prior to midnight that >2 strides/minute were recorded. Days were deleted on which such an active period was <50% of the mean active period across all days for that participant's visit. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: steps				
arithmetic mean (standard deviation)				
Baseline (n=52, 49, 48, 149)	4934.575 (± 2433.230)	4543.766 (± 2051.300)	5175.330 (± 2307.436)	4883.613 (± 2272.108)

Change at Week 60 (n=41, 38, 40, 119)	-357.679 (± 1232.109)	-215.062 (± 1761.773)	-647.504 (± 1680.522)	-409.558 (± 1566.386)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Mean Total Step Count/Hour During the Active Period at Week 60, as Assessed by SAM

End point title	Change From Baseline in Mean Total Step Count/Hour During the Active Period at Week 60, as Assessed by SAM

End point description:

Participants were instructed to continue to wear SAM for at least 9 consecutive days. SAM was used to record the number of strides/minute following each visit. A stride is the leg motion that begins when the foot with SAM leaves the floor and ends when the same foot touches the floor again (that is, a stride generally equals 2 steps). For each day, an active period was defined as the first time after 3:00 AM that >2 strides/minute were recorded to the last time prior to midnight that >2 strides/minute were recorded. Days were deleted on which such an active period was <50% of the mean active period across all days for that participant's visit. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: steps				
arithmetic mean (standard deviation)				
Baseline (n=52, 49, 48, 149)	395.491 (± 200.7671)	368.672 (± 155.6648)	420.684 (± 188.9124)	394.787 (± 183.0994)
Change at Week 60 (n=41, 38, 40, 119)	-27.650 (± 87.8797)	-34.693 (± 128.0609)	-54.755 (± 122.4142)	-39.010 (± 113.3481)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Maximum Continuous 10-minute, 20-minute, 30-minute, and 60-minute Total Step Count (TSC) at Week 60, as Assessed by SAM

End point title	Change From Baseline in Maximum Continuous 10-minute, 20-minute, 30-minute, and 60-minute Total Step Count

	(TSC) at Week 60, as Assessed by SAM

End point description:

Participants were instructed to continue to wear SAM for at least 9 consecutive days. SAM was used to record the number of strides/minute following each visit. A stride is the leg motion that begins when the foot with SAM leaves the floor and ends when the same foot touches the floor again (that is, a stride generally equals 2 steps). For each day, an active period was defined as the first time after 3:00 AM that >2 strides/minute were recorded to the last time prior to midnight that >2 strides/minute were recorded. Days were deleted on which such an active period was <50% of the mean active period across all days for that participant's visit. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: steps				
arithmetic mean (standard deviation)				
Baseline: 10-minute TSC (n=52,49,48,149)	34.603 (± 12.4886)	32.863 (± 8.8533)	35.256 (± 10.0379)	34.241 (± 10.5911)
Change at Week 60: 10-minute TSC (n=41,38,40,119)	-1.140 (± 6.9584)	-0.607 (± 7.2946)	-2.372 (± 7.1735)	-1.384 (± 7.1170)
Baseline: 20-minute TSC (52,49,48,149)	28.138 (± 11.0629)	26.096 (± 7.9168)	28.323 (± 9.3133)	27.526 (± 9.5426)
Change at Week 60: 20-minute TSC (n=41,38,40,119)	-1.459 (± 6.7165)	-0.662 (± 6.2171)	-2.510 (± 6.4346)	-1.558 (± 6.4549)
Baseline: 30-minute TSC (n=52,49,48,149)	24.222 (± 9.9576)	22.205 (± 7.1045)	24.498 (± 8.4938)	23.648 (± 8.6306)
Change at Week 60: 30-minute TSC (n=41,38,40,119)	-1.400 (± 5.7605)	-0.450 (± 5.6811)	-2.457 (± 5.9579)	-1.452 (± 5.8110)
Baseline: 60-minute TSC (n=52,49,48,149)	18.019 (± 7.8145)	16.453 (± 5.6625)	18.599 (± 6.9509)	17.691 (± 6.8991)
Change at Week 60: 60-minute TSC (n=41,38,40,119)	-0.977 (± 3.7328)	-0.210 (± 4.7035)	-2.257 (± 5.1038)	-1.162 (± 4.5803)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Percentage of Time During Active Period Spent at No Activity (0 steps/minute[min]), Low Activity (Less Than or Equal to [≤]15 steps/min), Medium Activity (16-30 steps/min), and High Activity (Greater Than[>]30 Steps/min) at Week 60

End point title	Change From Baseline in Percentage of Time During Active Period Spent at No Activity (0 steps/minute[min]), Low Activity (Less Than or Equal to [≤]15 steps/min), Medium Activity (16-30 steps/min), and High Activity (Greater Than[>]30 Steps/min) at Week 60

End point description:

Participants were instructed to continue to wear SAM for at least 9 consecutive days. SAM was used to record number of strides/minute following each visit. Percentage of time during active periods spent at no activity(0 steps/min), low activity(≤15 steps/min), medium activity(16-30 steps/min), and high activity(>30 steps/min) were computed for each participant. For each day, an active period was defined as first time after 3:00 AM that >2 strides/min were recorded to the last time prior to midnight that >2 strides/min were recorded. Days were deleted on which such an active period was <50% of the mean active period across all days for that participant's visit. Ambulatory 007e population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: percentage of time				
arithmetic mean (standard deviation)				
Baseline: No activity (n=52,49,48,149)	52.818 (± 14.4129)	53.372 (± 12.2352)	50.006 (± 12.5263)	52.094 (± 13.1188)
Change at Week 60: No activity (n=41,48,40,119)	-0.051 (± 8.1776)	0.830 (± 11.2481)	2.194 (± 7.8652)	0.985 (± 9.1467)
Baseline: Low activity (n=52,49,48,149)	30.677 (± 7.8531)	31.170 (± 7.2431)	32.393 (± 7.2426)	31.392 (± 7.4462)
Change at Week 60: Low activity (n=41,48,40,119)	1.270 (± 5.8178)	1.172 (± 8.3050)	0.252 (± 5.5885)	0.896 (± 6.6058)
Baseline: Medium activity (n=52,49,48,149)	10.248 (± 4.7620)	10.119 (± 4.4344)	10.983 (± 4.6049)	10.442 (± 4.5901)
Change at Week 60:Medium activity(n=41,48,40,119)	-0.542 (± 2.0960)	-1.356 (± 3.7830)	-0.965 (± 3.3763)	-0.944 (± 3.1392)
Baseline: High activity (n=51,49,48,148)	6.404 (± 4.5938)	5.370 (± 3.4243)	6.673 (± 4.5847)	6.149 (± 4.2476)
Change at Week 60:High activity(n=39,38,40,117)	-0.643 (± 2.2656)	-0.520 (± 2.7196)	-1.506 (± 2.9904)	-0.898 (± 2.6913)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Time to Stand From Supine Position at Week 60

End point title	Change From Baseline in Time to Stand From Supine Position at Week 60

End point description:

If the time taken to perform this test exceeded 30 seconds or if a participant could not perform this test due to disease progression, a value of 30 seconds was used. Change from baseline data has been reported. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: seconds				
arithmetic mean (standard deviation)				
Baseline (n=53,50,49,152)	13.736 (± 11.3353)	11.796 (± 10.3544)	12.398 (± 11.6010)	12.666 (± 11.0678)
Change at Week 60 (49,47,48,144)	0.665 (± 3.0080)	-0.187 (± 4.6289)	0.002 (± 4.6322)	0.166 (± 4.1373)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Time to Walk/Run 10 Meters at Week 60

End point title	Change From Baseline in Time to Walk/Run 10 Meters at Week 60

End point description:

If the time taken to perform this test exceeded 30 seconds or if a participant could not perform this test due to disease progression, a value of 30 seconds was used. Change from baseline data has been reported. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation.
'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: seconds				
arithmetic mean (standard deviation)				
Baseline (n=53,50,49,152)	8.143 (± 5.1475)	6.986 (± 2.9995)	6.967 (± 3.5789)	7.384 (± 4.0530)
Change at Week 60 (49,47,48,144)	1.441 (± 4.4822)	0.547 (± 2.2058)	0.758 (± 2.6862)	0.922 (± 3.2905)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Time to Climb 4 Stairs at Week 60

End point title	Change From Baseline in Time to Climb 4 Stairs at Week 60

End point description:

If the time taken to perform this test exceeded 30 seconds or if a participant could not perform this test due to disease progression, a value of 30 seconds was used. Change from baseline data has been reported. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation.
'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: seconds				
arithmetic mean (standard deviation)				
Baseline (n=53,50,49,152)	9.077 (± 9.2543)	6.738 (± 6.4069)	8.071 (± 9.2057)	7.984 (± 8.4076)
Change at Week 60 (n=49,47,48,144)	0.831 (± 2.4664)	0.696 (± 4.0707)	0.194 (± 2.5268)	0.574 (± 3.0898)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Time to Descend 4 Stairs at Week 60

End point title	Change From Baseline in Time to Descend 4 Stairs at Week 60

End point description:

If the time taken to perform this test exceeded 30 seconds or if a participant could not perform this test due to disease progression, a value of 30 seconds was used. Change from baseline data has been reported. Ambulatory 007e population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation.
'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	152
Units: seconds				
arithmetic mean (standard deviation)				
Baseline (n=53,50,49,152)	7.685 (± 8.9934)	5.638 (± 5.5635)	6.641 (± 7.8591)	6.675 (± 7.6319)
Change at Week 60 (n=49,47,48,144)	1.167 (± 4.7624)	0.626 (± 5.2567)	-0.648 (± 4.9202)	0.385 (± 5.0045)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Heart Rate Before, During, and After Each 6MWD Test at Week 60, as Assessed by Heart Rate Monitoring With the Polar® RS400

End point title	Change From Baseline in Heart Rate Before, During, and After Each 6MWD Test at Week 60, as Assessed by Heart Rate Monitoring With the Polar® RS400

End point description:

Heart rate (HR) was measured with a Polar RS400 heart rate monitor, which consists of a transmitter strap worn around the chest and a wristwatch receiver. Monitor produces a digital text file with 1 value per min that represents the mean HR for that min. Mean HR values were collected prior to, during, and after the 6MWT. Participant rested for 5 min in a sitting position prior to 6MWT, and mean HR for the last min of this rest period was collected and documented as the resting HR. During 6MWT, mean HR was collected and documented as active HR. After completing the 6MWT and resting for 3 min, the mean HR for 1 min was collected and documented as the recovery HR. Ambulatory 007e population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and had sufficient baseline and on-treatment data in Study 007e to assess measure of interest, excluding participants who had lost all independent ambulation. 'n'=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	53	50	49	150
Units: beats/minute				
arithmetic mean (standard deviation)				

Baseline: Resting HR (n=52,50,48,150)	105.3 (± 13.29)	108.8 (± 10.71)	103.7 (± 12.98)	105.9 (± 12.48)
Change at Week 60: Resting HR (n=45,46,43,134)	4.0 (± 13.91)	3.7 (± 10.11)	4.8 (± 10.82)	4.2 (± 11.65)
Baseline: Active HR (n=52,48,48,148)	138.7 (± 21.45)	144.8 (± 14.55)	138.6 (± 20.87)	140.6 (± 19.36)
Change at Week 60: Active HR (n=45,44,42,131)	1.6 (± 17.65)	6.1 (± 15.45)	2.4 (± 14.87)	3.4 (± 16.07)
Baseline: Recovery HR (n=52,48,48,148)	110.0 (± 13.32)	112.0 (± 12.17)	109.4 (± 13.21)	110.4 (± 12.88)
Change at Week 60: Recovery HR (n=43,44,44,131)	2.1 (± 12.82)	4.6 (± 12.57)	2.3 (± 11.29)	3.0 (± 12.20)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Number of Digits Recalled Forwards and Backwards on Digit Span Task at Week 60

End point title	Change From Baseline in Number of Digits Recalled Forwards and Backwards on Digit Span Task at Week 60

End point description:

Basic attention and working memory was measured using the digit span task. A series of digits (0-9) were presented to the child in an auditory format only. The task had 2 parts; in the forward condition, the child was requested to repeat back the digits in the order they were presented and in the backward condition, he was requested to reverse the order of presentation. A raw score of the total number of correct responses was converted to an age-scaled-score (z-score) by subtracting the corresponding mean and dividing by the corresponding standard deviation of a reference population for that age. Evaluable population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: z-score				
arithmetic mean (standard deviation)				
Baseline: Forward condition (n=56,54,51,161)	4.1 (± 2.97)	3.6 (± 2.54)	3.2 (± 2.21)	3.6 (± 2.61)
Change at Wk 60:Forward condition (n=52,51,51,154)	0.0 (± 1.53)	0.0 (± 1.19)	0.0 (± 1.22)	0.0 (± 1.32)
Baseline: Backward condition (n=56,54,51,161)	1.4 (± 0.98)	1.3 (± 0.82)	1.2 (± 0.66)	1.3 (± 0.84)
Change at Wk 60:Backward condition(n=49,51,49,149)	0.1 (± 0.86)	-0.1 (± 0.66)	0.0 (± 0.58)	0.0 (± 0.71)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Participant- Reported Health-Related Quality of Life (HRQL) as measured by the Pediatric Quality of Life Inventory (PedsQL) Physical, Emotional, Social, and School Functioning Domain Scores at Week 60

End point title	Change From Baseline in Participant- Reported Health-Related Quality of Life (HRQL) as measured by the Pediatric Quality of Life Inventory (PedsQL) Physical, Emotional, Social, and School Functioning Domain Scores at Week 60

End point description:

PedsQL generic core module (including physical, emotional, social and school functioning scales) comprises 23 questions and fatigue-specific module (including general fatigue, sleep/rest fatigue, and cognitive fatigue scales) comprises an additional 18 questions. Examples of items in each of the generic core module scales include: "It is hard for me to run"; "I feel sad or blue"; "I cannot do things that other kids my age can do;" and "It is hard to pay attention in class." Each of the generic core module items was scored on a 5-point Likert scale from 0 (never a problem) to 4 (almost always a problem). Scores were transformed on a scale from 0 to 100 (0=100, 1=75, 2=50, 3=25, 4=0), with higher scores indicating better HRQL. Evaluable population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline:Physical function(n=54,54,49,157)	63.8145 (± 24.46918)	63.3019 (± 22.72039)	60.4865 (± 22.33484)	62.5995 (± 23.11654)
Change at Wk 60:Physical function(n=50,47,49,146)	-0.5982 (± 14.91137)	-1.2538 (± 14.60384)	1.2755 (± 19.01169)	-0.1804 (± 16.23570)
Baseline:Emotional function(n=54,54,49,157)	76.852 (± 21.1530)	73.241 (± 20.1681)	73.265 (± 22.2568)	74.490 (± 21.1086)
Change at Wk 60:Emotional function(n=50,47,49,146)	-1.675 (± 13.6361)	2.128 (± 14.9181)	-0.995 (± 13.1621)	-0.223 (± 13.9106)
Baseline:Social function(n=54,54,50,158)	72.5000 (± 21.60429)	68.6111 (± 21.37748)	71.3000 (± 21.32858)	70.7911 (± 21.36670)
Change at Wk 60:Social function(n=50,47,50,147)	-2.2000 (± 13.48317)	0.8511 (± 17.36185)	0.3500 (± 15.15237)	-0.3571 (± 15.32468)
Baseline:School function(n=52,54,48,154)	72.019 (± 20.7756)	71.111 (± 18.2918)	69.063 (± 20.3861)	70.779 (± 19.7213)

Change at Wk 60:School function(n=47,46,47,140)	-1.064 (± 16.5153)	-1.630 (± 17.7029)	1.809 (± 16.9214)	-0.286 (± 16.9931)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Parent/Caregiver- Reported HRQL as measured by the PedsQL Physical, Emotional, Social, and School Functioning Domain Scores at Week 60

End point title	Change From Baseline in Parent/Caregiver- Reported HRQL as measured by the PedsQL Physical, Emotional, Social, and School Functioning Domain Scores at Week 60

End point description:

PedsQL generic core module (including physical, emotional, social and school functioning scales) comprises 23 questions and fatigue-specific module (including general fatigue, sleep/rest fatigue, and cognitive fatigue scales) comprises an additional 18 questions. Examples of items in each of the generic core module scales include: "It is hard for me to run"; "I feel sad or blue"; "I cannot do things that other kids my age can do;" and "It is hard to pay attention in class." Each of the generic core module items was scored on a 5-point Likert scale from 0 (never a problem) to 4 (almost always a problem). Scores were transformed on a scale from 0 to 100 (0=100, 1=75, 2=50, 3=25, 4=0), with higher scores indicating better HRQL. Evaluable population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline:Physical function(n=57,55,56,168)	56.8217 (± 23.80718)	52.5162 (± 20.02845)	52.6307 (± 24.35727)	54.0152 (± 22.78661)
Change at Wk 60:Physical function(n=52,49,54,155)	-0.8499 (± 12.75478)	-1.1662 (± 13.27447)	-0.1736 (± 14.75049)	-0.7143 (± 13.55924)
Baseline:Emotional function(n=57,55,56,168)	74.7368 (± 17.73870)	72.5455 (± 16.04025)	68.2813 (± 22.05309)	71.8676 (± 18.87211)
Change at Wk 60:Emotional function(n=52,49,54,155)	-1.9231 (± 11.11289)	-0.3061 (± 13.32323)	-1.9213 (± 16.86642)	-1.4113 (± 13.95342)
Baseline:Social function(n=57,55,56,168)	61.9518 (± 19.07606)	61.6136 (± 15.44605)	59.7545 (± 21.34593)	61.1086 (± 18.71300)
Change at Wk 60:Social function(n=52,49,54,155)	1.5144 (± 13.03356)	-0.7908 (± 11.34994)	0.3472 (± 12.76238)	0.3790 (± 12.38287)
Baseline:School function(n=56,55,55,166)	68.6607 (± 16.05363)	64.0000 (± 15.07389)	65.9091 (± 18.20894)	66.2048 (± 16.50694)
Change at Wk 60:School function(n=50,48,53,151)	0.8000 (± 9.60230)	1.6667 (± 10.93177)	-1.0377 (± 12.60946)	0.4305 (± 11.13464)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Participant-Reported HRQL as measured by the Total Fatigue Scale Score at Week 60

End point title	Change From Baseline in Participant-Reported HRQL as measured by the Total Fatigue Scale Score at Week 60

End point description:

PedsQL fatigue-specific module (including general fatigue, sleep/rest fatigue, and cognitive fatigue scales) comprises an additional 18 questions. Fatigue-specific module obtains information relating to items such as: "I feel too tired to do things that I like to do"; "I spend a lot of time in bed"; and "I have trouble remembering more than one thing at a time;" Each of the fatigue-specific module items was scored on a 5-point Likert scale from 0 (never a problem) to 4 (almost always a problem). Scores were transformed on a scale from 0 to 100 (0=100, 1=75, 2=50, 3=25, 4=0), with higher scores indicating less fatigue. Total score was the sum of all items over the number of items answered on all scales. Evaluable population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline (n=55,53,51,159)	76.7968 (± 18.31310)	72.7833 (± 20.95237)	72.5947 (± 17.32956)	74.1111 (± 18.92041)
Change at Week 60 (n=50,46,49,145)	-2.0484 (± 9.77928)	0.3650 (± 15.94374)	-1.8049 (± 11.12724)	-1.2005 (± 12.42615)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Parent/Caregiver-Reported HRQL as measured by the Total Fatigue Scale Score at Week 60

End point title	Change From Baseline in Parent/Caregiver-Reported HRQL as measured by the Total Fatigue Scale Score at Week 60

End point description:

PedsQL fatigue-specific module (including general fatigue, sleep/rest fatigue, and cognitive fatigue scales) comprises an additional 18 questions. Fatigue-specific module obtains information relating to items such as: "I feel too tired to do things that I like to do"; "I spend a lot of time in bed"; and "I have trouble remembering more than one thing at a time;" Each of the fatigue-specific module items was scored on a 5-point Likert scale from 0 (never a problem) to 4 (almost always a problem). Scores were transformed on a scale from 0 to 100 (0=100, 1=75, 2=50, 3=25, 4=0), with higher scores indicating less fatigue. Total score was the sum of all items over the number of items answered on all scales. Evaluable population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline (n=56,53,56,165)	75.7864 (± 13.88014)	71.6596 (± 12.78488)	71.1062 (± 13.62739)	72.8724 (± 13.53355)
Change at Week 60 (n=53,46,54,153)	-0.3272 (± 6.64218)	1.2219 (± 10.11693)	0.2269 (± 8.55130)	0.3341 (± 8.44319)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Participant and Parent/Caregiver Reported Activities of Daily Living of Participants who Were Unable to Complete the 6MWT (Nonambulatory Participants), as Measured by the Egen Klassifikation (EK) Scale at Week 60

End point title	Change From Baseline in Participant and Parent/Caregiver Reported Activities of Daily Living of Participants who Were Unable to Complete the 6MWT (Nonambulatory Participants), as Measured by the Egen Klassifikation (EK) Scale at Week 60

End point description:

EK scale is an ordinal scale ranging from 0 (highest level of independent function) to 30 points (lowest). Scale consists of 10 categories (each scored 0 to 3), involving different functional domains including 1) ability to use wheelchair, 2) ability to transfer from wheelchair, 3) ability to stand, 4) ability to balance in wheelchair, 5) ability to move the arms, 6) ability to use hands and arms when eating, 7) ability to turn in bed, 8) ability to cough, 9) ability to speak, and 10) physical well-being. The administration of the EK scale consisted of an interview of the participant to capture how he performed the tasks of daily life (as described by Categories 1 to 9) and how he perceived his well-being (as described by Category 10). Non-Ambulatory 007e population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, and who had lost all independent ambulation prior to entering Study 007e. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	5	4	6	15
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline (n=4,4,5,13)	3.0 (± 2.00)	4.3 (± 0.50)	3.2 (± 1.92)	3.5 (± 1.61)
Change at Week 60 (n=4,4,5,13)	1.5 (± 0.58)	0.8 (± 1.26)	-0.8 (± 1.48)	0.4 (± 1.50)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Parent/Caregiver-Reported Treatment Satisfaction Questionnaire for Medication (TSQM) Score at Week 60

End point title	Change From Baseline in Parent/Caregiver-Reported Treatment Satisfaction Questionnaire for Medication (TSQM) Score at Week 60

End point description:

TSQM consisted of 14 questions (Q) in 4 domains: Effectiveness (Q1-3 scored as 1 [extreme dissatisfied] to 7[extreme satisfied]), Side Effects (Q4 as 0 [no] or 1 [yes]; Q5 as 1 [extreme bothersome] to 5 [not at all]; Q6 - 8 as 1 [a great deal] to 5 [not at all]), Convenience (Q9,10 as 1 [extreme difficult] to 7 [extreme easy]; Q11 as 1 [extreme inconvenient] to 5 [extreme convenient]) and Global Satisfaction (Q12 as 1 [not at all confident] to 7 [extreme confident]; Q13 as 1 [not at all certain] to 5 [extreme certain]; Q14 as 1 [extreme dissatisfied] to 5 [extreme satisfied]). Scores of each domains were added together and an algorithm was used to create a score of 0 to 100, where higher scores=better treatment satisfaction. Evaluable population: all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n=participants evaluable for specified categories.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units on a scale				
arithmetic mean (standard deviation)				
Baseline: Effectiveness (n=53,54,52,159)	56.8134 (± 26.72451)	53.8580 (± 21.53292)	50.4274 (± 22.96804)	53.7212 (± 23.82541)
Change at Week 60: Effectiveness (n=50,46,50,146)	2.7778 (± 15.52191)	5.6159 (± 19.53233)	1.7778 (± 14.99307)	3.3295 (± 16.68460)

Baseline: Side-effects (n=55,55,54,164)	96.8182 (± 10.81681)	97.7273 (± 7.64176)	96.8364 (± 8.94695)	97.1291 (± 9.18246)
Change at Week 60: Side-effects (n=51,47,48,146)	0.7353 (± 12.16084)	-1.0638 (± 7.74500)	-1.2587 (± 10.84441)	-0.4994 (± 10.43907)
Baseline: Convenience (n=57,55,54,166)	57.4074 (± 16.10609)	57.9798 (± 19.05889)	60.5967 (± 16.65858)	58.6345 (± 17.26216)
Change at Week 60: Convenience (n=53,47,50,150)	0.8386 (± 12.39372)	-1.6548 (± 11.75175)	1.3333 (± 9.80472)	0.2222 (± 11.38059)
Baseline: Global satisfaction (n=55,55,54,164)	61.4286 (± 25.95857)	60.4870 (± 23.30835)	56.7791 (± 21.25768)	59.5819 (± 23.54109)
Change at Wk60:Global satisfaction(n=53,47,50,150)	-0.2695 (± 13.57882)	1.2538 (± 15.30213)	-0.4643 (± 14.78188)	0.1429 (± 14.45922)

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Serum Concentration of Creatine Kinase (CK) at Week 60

End point title	Change From Baseline in Serum Concentration of Creatine Kinase (CK) at Week 60

End point description:

Blood samples collected for chemistry assays were used to quantify serum CK concentrations. Serum CK was assessed as a potential biomarker for muscle fragility, with a reduction in serum CK considered to be a positive outcome. Evaluable population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e, who had sufficient baseline and on-treatment data in Study 007e to assess the measure of interest. 'n'=participants evaluable at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007), Week 60

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	58	56	56	170
Units: units/liter (U/L)				
arithmetic mean (standard deviation)				
Baseline (n=57,56,54,167)	9163.1 (± 5542.80)	10009.1 (± 5828.26)	9150.7 (± 5657.67)	9442.8 (± 5656.99)
Change at Week 60 (n=49,51,53,153)	-455.1 (± 4962.00)	-520.5 (± 6164.96)	-334.3 (± 4810.45)	-435.1 (± 5310.37)

Statistical analyses

No statistical analyses for this end point

Secondary: Study Drug Compliance

End point title	Study Drug Compliance

End point description:

Study drug compliance was assessed by participant daily diary and quantification of used and unused study drug. Compliance was assessed in terms of the percentage of drug actually taken relative to the amount that should have been taken during the study. As-treated population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e.

End point type	Secondary

End point timeframe:

Baseline (Week 48 of Study 007) to Week 96

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	59	57	57	173
Units: percentage of drug				
median (full range (min-max))	0.00 (0.0 to 45.1)	0.00 (0.0 to 10.5)	0.00 (0.0 to 36.1)	0.00 (0.0 to 45.1)

Statistical analyses

No statistical analyses for this end point

Secondary: Trough Ataluren Plasma Concentration

End point title	Trough Ataluren Plasma Concentration

End point description:

Plasma samples for the determination of ataluren concentrations were analyzed at the bioanalytical laboratory for ataluren parent drug using a validated high performance liquid chromatography with tandem mass spectrometry (HPLC/MS-MS) method with a lower limit of quantitation (LLOQ) of 0.5 micrograms/millilitre (mcg/mL). Values below the LLOQ were set to 0. As-treated population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e. 'n'=participants evaluable at specified timepoints. '99999' signifies 'SD could not be calculated due to single participant'; and '9999' signifies 'no participant was evaluable at this timepoint'.

End point type	Secondary

End point timeframe:

Pre-morning dose (0 hour) at Baseline (Week 48 of 007 study), Weeks 54, 60, 72, 84, and 96

End point values	High-Dose Ataluren/High-Dose Ataluren	Low-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	59	57	57	173
Units: mcg/mL				
arithmetic mean (standard deviation)				
Week 48 (n=58,57,57,172)	12.656 (± 10.8951)	4.541 (± 3.4381)	0.000 (± 0.0000)	5.773 (± 8.4333)

Week 54 (n=58,57,56,171)	10.801 (± 8.4858)	13.941 (± 14.5222)	11.564 (± 9.3312)	12.097 (± 11.1172)
Week 60 (55,54,56,165)	11.547 (± 9.6034)	13.830 (± 11.9096)	13.302 (± 9.4765)	12.890 (± 10.3574)
Week 72 (n=25,21,19,65)	12.279 (± 10.5894)	13.930 (± 10.6393)	10.948 (± 6.3465)	12.424 (± 9.4948)
Week 84 (n=6,7,5,18)	15.232 (± 8.8909)	14.773 (± 10.4404)	11.082 (± 14.6331)	13.901 (± 10.7415)
Week 96 (1,2,0,3)	8.430 (± 99999)	5.685 (± 2.7648)	9999 (± 9999)	6.600 (± 2.5167)

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Baseline (Week 48 of Study 007) up to Week 102

Adverse event reporting additional description:

As-treated population included all participants who completed Study 007 and received ≥1 dose of ataluren in Study 007e.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	11.0

Reporting groups

Reporting group title	High-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive high-dose ataluren in study PTC124-GD-007-DMD, continued to receive ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Placebo/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive placebo in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of placebo at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Overall Participants: High-Dose Ataluren

Reporting group description:

All participants received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Reporting group title	Low-Dose Ataluren/High-Dose Ataluren

Reporting group description:

Participants who were randomized to receive low-dose ataluren in study PTC124-GD-007-DMD, received ataluren suspension orally TID, 20 mg/kg at morning, 20 mg/kg at midday, and 40 mg/kg at evening (total daily dose 80 mg/kg) for up to 96 weeks in this study. Any participant who was receiving a reduced dose of ataluren at the end of treatment visit in study PTC124-GD-007-DMD, was initiated ataluren therapy in this extension study at the 5-, 5-, and 10-mg/kg dose level; dose was increased to 10, 10, and 20 mg/kg at Week 6 and to 20, 20, and 40 mg/kg at Week 12, if the preceding dose level was well tolerated.

Serious adverse events	High-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	3 / 173 (1.73%)

number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Low-Dose Ataluren/High-Dose Ataluren		
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 57 (1.75%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	High-Dose Ataluren/High-Dose Ataluren	Placebo/High-Dose Ataluren	Overall Participants: High-Dose Ataluren
Total subjects affected by non-serious adverse events			
subjects affected / exposed	53 / 59 (89.83%)	51 / 57 (89.47%)	155 / 173 (89.60%)

Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	6 / 173 (3.47%)
occurrences (all)	1	1	7
Melanocytic naevus			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Vascular disorders			
Hot flush			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	2
Hypertension			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	4 / 173 (2.31%)
occurrences (all)	0	2	4
General disorders and administration site conditions			
Disease progression			
subjects affected / exposed	9 / 59 (15.25%)	3 / 57 (5.26%)	19 / 173 (10.98%)
occurrences (all)	9	3	19
Asthenia			
subjects affected / exposed	2 / 59 (3.39%)	2 / 57 (3.51%)	9 / 173 (5.20%)
occurrences (all)	3	2	10
Gait disturbance			
subjects affected / exposed	4 / 59 (6.78%)	2 / 57 (3.51%)	9 / 173 (5.20%)
occurrences (all)	4	2	9
Pyrexia			
subjects affected / exposed	4 / 59 (6.78%)	3 / 57 (5.26%)	9 / 173 (5.20%)
occurrences (all)	4	4	11
Fatigue			
subjects affected / exposed	3 / 59 (5.08%)	1 / 57 (1.75%)	5 / 173 (2.89%)
occurrences (all)	3	1	5
Malaise			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Oedema peripheral			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1

Immune system disorders			
Hypersensitivity			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	1	2
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	6 / 59 (10.17%)	6 / 57 (10.53%)	13 / 173 (7.51%)
occurrences (all)	6	6	13
Oropharyngeal pain			
subjects affected / exposed	4 / 59 (6.78%)	1 / 57 (1.75%)	7 / 173 (4.05%)
occurrences (all)	6	1	9
Nasal congestion			
subjects affected / exposed	2 / 59 (3.39%)	2 / 57 (3.51%)	6 / 173 (3.47%)
occurrences (all)	2	2	6
Epistaxis			
subjects affected / exposed	3 / 59 (5.08%)	0 / 57 (0.00%)	4 / 173 (2.31%)
occurrences (all)	3	0	5
Wheezing			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Asthma			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Dyspnoea			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Pharyngeal erythema			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Postnasal drip			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Productive cough			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Respiratory tract congestion			

subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Rhinitis allergic			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Snoring			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Sinus congestion			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Psychiatric disorders			
Attention deficit/hyperactivity disorder			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	5 / 173 (2.89%)
occurrences (all)	2	1	5
Abnormal behaviour			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	0	0	3
Aggression			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Obsessive-compulsive disorder			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	2 / 173 (1.16%)
occurrences (all)	0	2	2
Tic			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	2	3
Depression			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Frustration			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Oppositional defiant disorder			

subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Investigations			
Blood sodium increased			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	1	2
Weight decreased			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	0	0	2
Blood bicarbonate decreased			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Blood triglycerides increased			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Breath sounds abnormal			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Weight increased			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	5 / 59 (8.47%)	4 / 57 (7.02%)	14 / 173 (8.09%)
occurrences (all)	6	4	15
Contusion			
subjects affected / exposed	2 / 59 (3.39%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	3	0	4
Iliotibial band syndrome			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	1	0	3
Joint sprain			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	1	0	3
Arthropod bite			

subjects affected / exposed	2 / 59 (3.39%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	2	0	2
Femur fracture			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Limb injury			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	1	0	2
Lower limb fracture			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Arthropod sting			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Excoriation			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Facial bones fracture			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Foot fracture			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Head injury			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Humerus fracture			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Incision site erythema			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Lumbar vertebral fracture			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Mouth injury			

subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Muscle strain			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Overdose			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Procedural pain			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Skin laceration			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Spinal compression fracture			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Wound			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Wound dehiscence			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Congenital, familial and genetic disorders			
Kidney malformation			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Cardiac disorders			
Dilatation ventricular			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	1	2
Arrhythmia			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Cardiomyopathy			

subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Left ventricular hypertrophy			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Tachycardia			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Nervous system disorders			
Headache			
subjects affected / exposed	5 / 59 (8.47%)	14 / 57 (24.56%)	34 / 173 (19.65%)
occurrences (all)	48	17	113
Migraine			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	4 / 173 (2.31%)
occurrences (all)	2	1	4
Areflexia			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Dizziness			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Hypertonia			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Hypotonia			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Memory impairment			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Partial seizures			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Sedation			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1

Sinus headache			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	0	2	3
Anaemia			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Microcytic anaemia			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Neutropenia			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Ear and labyrinth disorders			
Deafness			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Ear pain			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Tympanic membrane hyperaemia			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Vertigo			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	5	0	5
Eye disorders			
Cataract			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Myopia			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	1	0	2
Abnormal sensation in eye			

subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Conjunctivitis			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Hypermetropia			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Photophobia			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Strabismus			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Vision blurred			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	11 / 59 (18.64%)	20 / 57 (35.09%)	43 / 173 (24.86%)
occurrences (all)	16	33	72
Diarrhoea			
subjects affected / exposed	3 / 59 (5.08%)	6 / 57 (10.53%)	20 / 173 (11.56%)
occurrences (all)	4	8	25
Abdominal pain			
subjects affected / exposed	6 / 59 (10.17%)	6 / 57 (10.53%)	16 / 173 (9.25%)
occurrences (all)	10	7	21
Abdominal pain upper			
subjects affected / exposed	2 / 59 (3.39%)	4 / 57 (7.02%)	15 / 173 (8.67%)
occurrences (all)	2	5	18
Flatulence			
subjects affected / exposed	7 / 59 (11.86%)	3 / 57 (5.26%)	14 / 173 (8.09%)
occurrences (all)	7	3	14
Nausea			
subjects affected / exposed	5 / 59 (8.47%)	3 / 57 (5.26%)	11 / 173 (6.36%)
occurrences (all)	5	3	12

Dyspepsia			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	5 / 173 (2.89%)
occurrences (all)	1	2	5
Constipation			
subjects affected / exposed	1 / 59 (1.69%)	3 / 57 (5.26%)	4 / 173 (2.31%)
occurrences (all)	1	3	4
Stomach discomfort			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	4 / 173 (2.31%)
occurrences (all)	1	1	5
Aerophagia			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	1	2	3
Gastrooesophageal reflux disease			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Abdominal discomfort			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	2	2
Abdominal pain lower			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Abdominal tenderness			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Abnormal faeces			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Aphthous stomatitis			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Chapped lips			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Duodenogastric reflux			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1

Eructation			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Faecal incontinence			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Gastritis			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Gastrointestinal sounds abnormal			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Gingivitis			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Oral pain			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Tongue disorder			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	2 / 59 (3.39%)	3 / 57 (5.26%)	6 / 173 (3.47%)
occurrences (all)	2	3	6
Dry skin			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	0	2	3
Ecchymosis			
subjects affected / exposed	2 / 59 (3.39%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	2	0	2
Eczema			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	2	0	3
Pruritus			

subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Acne			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Cold sweat			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Hyperkeratosis			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Keratosis pilaris			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Petechiae			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Pityriasis rosea			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Rash maculo-papular			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Rash papular			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Skin chapped			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Skin depigmentation			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Skin discolouration			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Skin exfoliation			

subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Skin odour abnormal			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Swelling face			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Renal and urinary disorders			
Enuresis			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	6 / 173 (3.47%)
occurrences (all)	2	1	6
Urine abnormality			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	1	2	3
Pollakiuria			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	1	0	2
Urinary incontinence			
subjects affected / exposed	2 / 59 (3.39%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	2	0	2
Dysuria			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Haematuria			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Kidney enlargement			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Micturition urgency			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Pyelocaliectasis			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1

Renal cyst			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Ureteric dilatation			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Endocrine disorders			
Cushingoid			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	4 / 59 (6.78%)	4 / 57 (7.02%)	11 / 173 (6.36%)
occurrences (all)	11	7	21
Muscular weakness			
subjects affected / exposed	5 / 59 (8.47%)	2 / 57 (3.51%)	11 / 173 (6.36%)
occurrences (all)	6	2	12
Pain in extremity			
subjects affected / exposed	5 / 59 (8.47%)	1 / 57 (1.75%)	10 / 173 (5.78%)
occurrences (all)	8	1	14
Joint contracture			
subjects affected / exposed	4 / 59 (6.78%)	1 / 57 (1.75%)	9 / 173 (5.20%)
occurrences (all)	4	1	13
Arthralgia			
subjects affected / exposed	3 / 59 (5.08%)	0 / 57 (0.00%)	7 / 173 (4.05%)
occurrences (all)	3	0	7
Muscle tightness			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	6 / 173 (3.47%)
occurrences (all)	2	1	6
Lordosis			
subjects affected / exposed	2 / 59 (3.39%)	2 / 57 (3.51%)	5 / 173 (2.89%)
occurrences (all)	2	2	5
Musculoskeletal chest pain			
subjects affected / exposed	2 / 59 (3.39%)	2 / 57 (3.51%)	5 / 173 (2.89%)
occurrences (all)	2	2	5
Myalgia			

subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	5 / 173 (2.89%)
occurrences (all)	3	1	6
Muscle spasms			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	4 / 173 (2.31%)
occurrences (all)	1	0	4
Osteoporosis			
subjects affected / exposed	3 / 59 (5.08%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	3	0	3
Tendinous contracture			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	3 / 173 (1.73%)
occurrences (all)	1	0	3
Muscle atrophy			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	1	0	2
Scoliosis			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	0	0	2
Tendon disorder			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	1	2
Coccydynia			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Groin pain			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Growth retardation			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Joint instability			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Muscle contracture			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Musculoskeletal pain			

subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Neck pain			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Musculoskeletal stiffness			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Infections and infestations			
Influenza			
subjects affected / exposed	7 / 59 (11.86%)	6 / 57 (10.53%)	18 / 173 (10.40%)
occurrences (all)	7	6	18
Nasopharyngitis			
subjects affected / exposed	3 / 59 (5.08%)	5 / 57 (8.77%)	16 / 173 (9.25%)
occurrences (all)	4	5	19
Upper respiratory tract infection			
subjects affected / exposed	6 / 59 (10.17%)	5 / 57 (8.77%)	14 / 173 (8.09%)
occurrences (all)	8	5	17
Ear infection			
subjects affected / exposed	2 / 59 (3.39%)	4 / 57 (7.02%)	8 / 173 (4.62%)
occurrences (all)	3	5	10
Gastroenteritis viral			
subjects affected / exposed	3 / 59 (5.08%)	3 / 57 (5.26%)	7 / 173 (4.05%)
occurrences (all)	3	3	8
Gastroenteritis			
subjects affected / exposed	2 / 59 (3.39%)	3 / 57 (5.26%)	5 / 173 (2.89%)
occurrences (all)	2	4	6
Otitis media			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	5 / 173 (2.89%)
occurrences (all)	2	1	6
Rhinitis			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	4 / 173 (2.31%)
occurrences (all)	1	2	4
Bronchitis			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	0	2	3

Croup infectious			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	1	2	3
Lower respiratory tract infection			
subjects affected / exposed	0 / 59 (0.00%)	3 / 57 (5.26%)	3 / 173 (1.73%)
occurrences (all)	0	4	4
Paronychia			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	3 / 173 (1.73%)
occurrences (all)	1	1	3
Sinusitis			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	3 / 173 (1.73%)
occurrences (all)	0	4	6
Urinary tract infection			
subjects affected / exposed	0 / 59 (0.00%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	0	2	3
Viral infection			
subjects affected / exposed	1 / 59 (1.69%)	2 / 57 (3.51%)	3 / 173 (1.73%)
occurrences (all)	1	2	3
Enterobiasis			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	1	2
Gastrointestinal infection			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	0	2	3
Molluscum contagiosum			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	1	0	2
Pharyngitis			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	1	2
Pharyngitis streptococcal			
subjects affected / exposed	2 / 59 (3.39%)	0 / 57 (0.00%)	2 / 173 (1.16%)
occurrences (all)	3	0	3
Tinea pedis			
subjects affected / exposed	1 / 59 (1.69%)	1 / 57 (1.75%)	2 / 173 (1.16%)
occurrences (all)	1	3	4

Conjunctivitis infective			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Helicobacter infection			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Localised infection			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Lung infection			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	2
Onychomycosis			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Oral herpes			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Otitis externa			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Pneumonia			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Scarlet fever			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	2 / 59 (3.39%)	1 / 57 (1.75%)	4 / 173 (2.31%)
occurrences (all)	2	1	4
Dehydration			
subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Hypovitaminosis			

subjects affected / exposed	0 / 59 (0.00%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	0	0	1
Insulin resistance			
subjects affected / exposed	1 / 59 (1.69%)	0 / 57 (0.00%)	1 / 173 (0.58%)
occurrences (all)	1	0	1
Iron deficiency			
subjects affected / exposed	0 / 59 (0.00%)	1 / 57 (1.75%)	1 / 173 (0.58%)
occurrences (all)	0	1	1

Non-serious adverse events	Low-Dose Ataluren/High-Dose Ataluren		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	51 / 57 (89.47%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	4 / 57 (7.02%)		
occurrences (all)	5		
Melanocytic naevus			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Vascular disorders			
Hot flush			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	2		
Hypertension			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
General disorders and administration site conditions			
Disease progression			
subjects affected / exposed	7 / 57 (12.28%)		
occurrences (all)	7		
Asthenia			
subjects affected / exposed	5 / 57 (8.77%)		
occurrences (all)	5		
Gait disturbance			

subjects affected / exposed	3 / 57 (5.26%)		
occurrences (all)	3		
Pyrexia			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	3		
Fatigue			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Malaise			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Oedema peripheral			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Immune system disorders			
Hypersensitivity			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Oropharyngeal pain			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Nasal congestion			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Epistaxis			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	2		
Wheezing			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Asthma			

subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Dyspnoea			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Pharyngeal erythema			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Postnasal drip			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Productive cough			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Respiratory tract congestion			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Rhinitis allergic			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Snoring			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Sinus congestion			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Psychiatric disorders			
Attention deficit/hyperactivity disorder			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Abnormal behaviour			
subjects affected / exposed	3 / 57 (5.26%)		
occurrences (all)	3		
Aggression			

subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Obsessive-compulsive disorder			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Tic			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Depression			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Frustration			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Oppositional defiant disorder			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Investigations			
Blood sodium increased			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Weight decreased			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Blood bicarbonate decreased			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Blood triglycerides increased			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Breath sounds abnormal			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Weight increased			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		

Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	5 / 57 (8.77%)		
occurrences (all)	5		
Contusion			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Iliotibial band syndrome			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Joint sprain			
subjects affected / exposed	2 / 57 (3.51%)		
occurrences (all)	2		
Arthropod bite			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Femur fracture			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Limb injury			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Lower limb fracture			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Arthropod sting			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Excoriation			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Facial bones fracture			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Foot fracture			

subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Head injury				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Humerus fracture				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Incision site erythema				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Lumbar vertebral fracture				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Mouth injury				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Muscle strain				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Overdose				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Procedural pain				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Skin laceration				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Spinal compression fracture				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Wound				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Wound dehiscence				

subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Congenital, familial and genetic disorders			
Kidney malformation			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Cardiac disorders			
Dilatation ventricular			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Arrhythmia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Cardiomyopathy			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Left ventricular hypertrophy			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Tachycardia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Nervous system disorders			
Headache			
subjects affected / exposed	15 / 57 (26.32%)		
occurrences (all)	48		
Migraine			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Areflexia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Dizziness			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Hypertonia			

subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Hypotonia			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Memory impairment			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Partial seizures			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Sedation			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Sinus headache			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Anaemia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Microcytic anaemia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Neutropenia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Ear and labyrinth disorders			
Deafness			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Ear pain			

subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Tympanic membrane hyperaemia			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Vertigo			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Eye disorders			
Cataract			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Myopia			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Abnormal sensation in eye			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Conjunctivitis			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Hypermetropia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Photophobia			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Strabismus			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Vision blurred			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Gastrointestinal disorders			
Vomiting			

subjects affected / exposed	12 / 57 (21.05%)			
occurrences (all)	23			
Diarrhoea				
subjects affected / exposed	11 / 57 (19.30%)			
occurrences (all)	13			
Abdominal pain				
subjects affected / exposed	4 / 57 (7.02%)			
occurrences (all)	4			
Abdominal pain upper				
subjects affected / exposed	9 / 57 (15.79%)			
occurrences (all)	11			
Flatulence				
subjects affected / exposed	4 / 57 (7.02%)			
occurrences (all)	4			
Nausea				
subjects affected / exposed	3 / 57 (5.26%)			
occurrences (all)	4			
Dyspepsia				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Constipation				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Stomach discomfort				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	3			
Aerophagia				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Gastrooesophageal reflux disease				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Abdominal discomfort				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Abdominal pain lower				

subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Abdominal tenderness				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Abnormal faeces				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Aphthous stomatitis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Chapped lips				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Duodenogastric reflux				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Eructation				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Faecal incontinence				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Gastritis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Gastrointestinal sounds abnormal				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Gingivitis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Oral pain				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Tongue disorder				

subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Dry skin			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Ecchymosis			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Eczema			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Pruritus			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Acne			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Cold sweat			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Hyperkeratosis			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Keratosis pilaris			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Petechiae			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Pityriasis rosea			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		

Rash maculo-papular subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Rash papular subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Skin chapped subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Skin depigmentation subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Skin discolouration subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Skin exfoliation subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Skin odour abnormal subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Swelling face subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Renal and urinary disorders Enuresis subjects affected / exposed occurrences (all)	3 / 57 (5.26%) 3		
Urine abnormality subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Pollakiuria subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Urinary incontinence			

subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Dysuria			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Haematuria			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Kidney enlargement			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Micturition urgency			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Pyelocaliectasis			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Renal cyst			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Ureteric dilatation			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Endocrine disorders			
Cushingoid			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	3 / 57 (5.26%)		
occurrences (all)	3		
Muscular weakness			
subjects affected / exposed	4 / 57 (7.02%)		
occurrences (all)	4		
Pain in extremity			

subjects affected / exposed	4 / 57 (7.02%)			
occurrences (all)	5			
Joint contracture				
subjects affected / exposed	4 / 57 (7.02%)			
occurrences (all)	8			
Arthralgia				
subjects affected / exposed	4 / 57 (7.02%)			
occurrences (all)	4			
Muscle tightness				
subjects affected / exposed	3 / 57 (5.26%)			
occurrences (all)	3			
Lordosis				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Musculoskeletal chest pain				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Myalgia				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Muscle spasms				
subjects affected / exposed	3 / 57 (5.26%)			
occurrences (all)	3			
Osteoporosis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Tendinous contracture				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Muscle atrophy				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Scoliosis				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Tendon disorder				

subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Coccydynia			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Groin pain			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Growth retardation			
subjects affected / exposed	1 / 57 (1.75%)		
occurrences (all)	1		
Joint instability			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Muscle contracture			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Musculoskeletal pain			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Neck pain			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Musculoskeletal stiffness			
subjects affected / exposed	0 / 57 (0.00%)		
occurrences (all)	0		
Infections and infestations			
Influenza			
subjects affected / exposed	5 / 57 (8.77%)		
occurrences (all)	5		
Nasopharyngitis			
subjects affected / exposed	8 / 57 (14.04%)		
occurrences (all)	10		
Upper respiratory tract infection			
subjects affected / exposed	3 / 57 (5.26%)		
occurrences (all)	4		

Ear infection				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Gastroenteritis viral				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	2			
Gastroenteritis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Otitis media				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	3			
Rhinitis				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Bronchitis				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Croup infectious				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Lower respiratory tract infection				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Paronychia				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Sinusitis				
subjects affected / exposed	2 / 57 (3.51%)			
occurrences (all)	2			
Urinary tract infection				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Viral infection				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			

Enterobiasis				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Gastrointestinal infection				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Molluscum contagiosum				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Pharyngitis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Pharyngitis streptococcal				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Tinea pedis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Conjunctivitis infective				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Helicobacter infection				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Localised infection				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	1			
Lung infection				
subjects affected / exposed	1 / 57 (1.75%)			
occurrences (all)	2			
Onychomycosis				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			
Oral herpes				
subjects affected / exposed	0 / 57 (0.00%)			
occurrences (all)	0			

Otitis externa subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Pneumonia subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Scarlet fever subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Dehydration subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Hypovitaminosis subjects affected / exposed occurrences (all)	1 / 57 (1.75%) 1		
Insulin resistance subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		
Iron deficiency subjects affected / exposed occurrences (all)	0 / 57 (0.00%) 0		

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.

This study was prematurely terminated by Sponsor per Data Monitoring Committee (DMC) recommendation to discontinue ongoing studies of high-dose ataluren in nmDBMD due to lack of efficacy for the high-dose ataluren.

Notes: