



Clinical trial results:

A Phase 3, Randomised, Double-blind, Placebo-controlled Efficacy and Safety Study of Ataluren in Patients With Nonsense Mutation Duchenne Muscular Dystrophy and Open-label Extension

Summary

EudraCT number	2017-001223-49
Trial protocol	BG PL
Global end of trial date	25 July 2023

Results information

Result version number	v1 (current)
This version publication date	24 January 2026
First version publication date	24 January 2026

Trial information

Trial identification

Sponsor protocol code	PTC124-GD-041-DMD
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03179631
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	PTC Therapeutics, Inc.
Sponsor organisation address	100 Corporate Court, South Plainfield, United States, NJ 07080
Public contact	Medical Information, PTC Therapeutics, Inc., +011 44 1-866-562-4620, medinfo@ptcbio.com
Scientific contact	Medical Information, PTC Therapeutics International Limited, +353 19068700, medinfo@ptcbio.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	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	27 September 2023
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	25 July 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this trial was to determine the effect of ataluren on ambulation and endurance as assessed by the 6-minute walk test (6MWT).

Protection of trial subjects:

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

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	15 August 2017
Long term follow-up planned	Yes
Long term follow-up rationale	Safety, Efficacy
Long term follow-up duration	17 Months
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 360
Worldwide total number of subjects	360
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	340
Adolescents (12-17 years)	20
Adults (18-64 years)	0
From 65 to 84 years	0

85 years and over	0
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Subject disposition

Recruitment

Recruitment details:

The study consisted of a 72-week double-blind (DB) treatment period followed by a 72-week open-label (OL) treatment period.

For confidentiality reasons, all participants have been reported for "United States" instead of their respective countries in "Section F. Population of Trial Subjects".

Pre-assignment

Screening details:

A total of 439 participants were screened for the study. Of these, 363 participants were randomized to DB treatment, and 360 participants received at least 1 dose of study drug (176 participants received placebo and 184 participants received ataluren). Three participants who were randomized in error did not receive treatment.

Period 1

Period 1 title	DB Treatment Period (72 Weeks)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Carer, Assessor

Arms

Are arms mutually exclusive?	Yes
Arm title	Ataluren

Arm description:

Participants received ataluren oral suspension 10 milligrams (mg)/kilogram (kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral suspension
Routes of administration	Oral use

Dosage and administration details:

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

Arm title	Placebo
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Arm description:

Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral suspension
Routes of administration	Oral use

Dosage and administration details:

Placebo matched to ataluren was administered per schedule specified in the arm description.

Number of subjects in period 1	Ataluren	Placebo
Started	184	176
Intent-to-treat (ITT) Population	183	176
Completed	179	172
Not completed	5	4
Consent withdrawn by subject	1	3
Other than specified	1	1
Lost to follow-up	1	-
Protocol deviation	2	-

Period 2

Period 2 title	OL Treatment Period (72 Weeks)
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	Ataluren

Arm description:

Participants received ataluren oral suspension 10 milligrams (mg)/kilogram (kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.

Arm type	Experimental
Investigational medicinal product name	Ataluren
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral suspension
Routes of administration	Oral use

Dosage and administration details:

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

Arm title	Placebo
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Arm description:

Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.

Arm type	Placebo
Investigational medicinal product name	Ataluren
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral suspension
Routes of administration	Oral use

Dosage and administration details:

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

Number of subjects in period 2	Ataluren	Placebo
Started	179	172
Completed	174	165
Not completed	5	7
Consent withdrawn by subject	1	1
Investigator decision	-	1
Receiving commercially available product	1	5
Other than specified	2	-
Protocol deviation	1	-

Baseline characteristics

Reporting groups

Reporting group title	Ataluren
Reporting group description:	
Participants received ataluren oral suspension 10 milligrams (mg)/kilogram (kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.	
Reporting group title	Placebo
Reporting group description:	
Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.	

Reporting group values	Ataluren	Placebo	Total
Number of subjects	184	176	360
Age Categorical Units: Subjects			

Age Continuous Units: years			
arithmetic mean	8.1	8.2	
standard deviation	± 1.91	± 2.10	-
Gender Categorical Units: Subjects			
Female	0	0	0
Male	184	176	360
Race Units: Subjects			
American Indian or Alaska Native	0	1	1
Asian	112	109	221
Black or African American	2	3	5
Native Hawaiian or Other Pacific Islander	2	0	2
White	46	55	101
Other	22	8	30
Ethnicity Units: Subjects			
Hispanic or Latino	18	10	28
Not Hispanic or Latino	166	166	332

End points

End points reporting groups

Reporting group title	Ataluren
Reporting group description: Participants received ataluren oral suspension 10 milligrams (mg)/kilogram (kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.	
Reporting group title	Placebo
Reporting group description: Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.	
Reporting group title	Ataluren
Reporting group description: Participants received ataluren oral suspension 10 milligrams (mg)/kilogram (kg) in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.	
Reporting group title	Placebo
Reporting group description: Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.	
Subject analysis set title	Ataluren/Ataluren
Subject analysis set type	Intention-to-treat
Subject analysis set description: Participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.	
Subject analysis set title	Placebo/Ataluren
Subject analysis set type	Intention-to-treat
Subject analysis set description: Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.	
Subject analysis set title	Ataluren/Ataluren
Subject analysis set type	Safety analysis
Subject analysis set description: Participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.	
Subject analysis set title	Ataluren/Placebo
Subject analysis set type	Safety analysis
Subject analysis set description: Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.	

Primary: DB Period: Change From Baseline in 6-Minute Walk Distance (6MWD) at Week 72 - Modified Intention-to-treat (mITT) Population

End point title	DB Period: Change From Baseline in 6-Minute Walk Distance (6MWD) at Week 72 - Modified Intention-to-treat (mITT) Population
End point description: The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Participants with confirmed loss of ambulation at a particular visit were assigned a 6MWD result of 0. Baseline was defined as the maximum measurement of valid Day 1 and Day 2 6MWD values. Least square (LS) mean and standard error (SE) was calculated using the mixed-model repeated measures (MMRM). The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline.	
End point type	Primary
End point timeframe: Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: meters				
least squares mean (standard error)	-81.83 ($\pm$ 6.461)	-90.09 ($\pm$ 6.377)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.3626
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	8.26
Confidence interval	
level	95 %
sides	2-sided
lower limit	-9.53
upper limit	26.05
Variability estimate	Standard error of the mean
Dispersion value	9.064

Primary: DB Period: Change From Baseline in 6MWD at Week 72 - Intention-to-treat

(ITT) Population

End point title	DB Period: Change From Baseline in 6MWD at Week 72 - Intention-to-treat (ITT) Population
End point description: The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Participants with confirmed loss of ambulation at a particular visit were assigned a 6MWD result of 0. Baseline was defined as the maximum measurement of valid Day 1 and Day 2 6MWD values. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.	
End point type	Primary
End point timeframe: Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: meters				
least squares mean (standard error)	-53.01 ($\pm$ 5.169)	-67.43 ($\pm$ 5.403)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0248
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	14.42
Confidence interval	
level	95 %
sides	2-sided
lower limit	1.83
upper limit	27.01
Variability estimate	Standard error of the mean
Dispersion value	6.42

Primary: DB Period: Average Rate of Change From Baseline in 6MWD at Week 72 - mITT Population

End point title	DB Period: Average Rate of Change From Baseline in 6MWD at Week 72 - mITT Population
End point description:	
The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Participants with confirmed loss of ambulation at a particular visit were assigned a 6MWD result of 0. Baseline was defined as the maximum measurement of valid Day 1 and Day 2 6MWD values. LS mean and SE was calculated using the MMRM. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline.	
End point type	Primary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: meters/week				
least squares mean (standard error)	-1.14 ($\pm$ 0.090)	-1.25 ($\pm$ 0.089)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.3626
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	0.11
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.13
upper limit	0.36
Variability estimate	Standard error of the mean
Dispersion value	0.126

Primary: DB Period: Average Rate of Change From Baseline in 6MWD at Week 72 - ITT Population

End point title	DB Period: Average Rate of Change From Baseline in 6MWD at Week 72 - ITT Population
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End point description:

The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Participants with confirmed loss of ambulation at a particular visit were assigned a 6MWD result of 0. Baseline was defined as the maximum measurement of valid Day 1 and Day 2 6MWD values. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.

End point type	Primary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: meters/week				
least squares mean (standard error)	-0.74 ($\pm$ 0.072)	-0.94 ($\pm$ 0.075)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0248
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	0.2
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.03
upper limit	0.38
Variability estimate	Standard error of the mean
Dispersion value	0.089

Secondary: DB Period: Change From Baseline in Time to Run/Walk 10 Meters at Week 72 - mITT Population

End point title	DB Period: Change From Baseline in Time to Run/Walk 10 Meters at Week 72 - mITT Population
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End point description:

During the test for walking/running 10 meters, the method of walk/run used by the participant was

categorized as follows: 1. Unable to walk independently; 2. Unable to walk independently but can walk with support from a person or with assistive device (full leg calipers [knee-ankle-foot orthoses] or walker); 3. Highly adapted gait, wide-based lordotic gait, cannot increase walking speed; 4. Moderately adapted gait, can pick up speed but cannot run; 5. Able to pick up speed but runs with a double stance phase (that is, cannot achieve both feet off the ground); 6. Runs and gets both feet off the ground (with no double stance phase). Participants who could not perform a timed function test (TFT) within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. mITT population.

End point type	Secondary
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End point timeframe:

Baseline, Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: seconds				
least squares mean (standard error)	3.06 ($\pm$ 0.393)	3.79 ($\pm$ 0.389)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.1877
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.73
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.81
upper limit	0.36
Variability estimate	Standard error of the mean
Dispersion value	0.552

Secondary: DB Period: Change From Baseline in Time to Run/Walk 10 Meters at Week 72 - ITT Population

End point title	DB Period: Change From Baseline in Time to Run/Walk 10 Meters at Week 72 - ITT Population
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End point description:

During the test for walking/running 10 meters, the method of walk/run used by the participant was categorized as follows: 1. Unable to walk independently; 2. Unable to walk independently but can walk with support from a person or with assistive device (full leg calipers [knee-ankle-foot orthoses] or walker); 3. Highly adapted gait, wide-based lordotic gait, cannot increase walking speed; 4. Moderately

adapted gait, can pick up speed but cannot run; 5. Able to pick up speed but runs with a double stance phase (that is, cannot achieve both feet off the ground); 6. Runs and gets both feet off the ground (with no double stance phase). Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.

End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	3.04 ($\pm$ 0.287)	3.82 ($\pm$ 0.297)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0422
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.78
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.53
upper limit	-0.03
Variability estimate	Standard error of the mean
Dispersion value	0.383

Secondary: DB Period: Change From Baseline in Time to Climb 4 Stairs at Week 72 - mITT Population

End point title	DB Period: Change From Baseline in Time to Climb 4 Stairs at Week 72 - mITT Population
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End point description:

During the test for stair-climbing, the method of climbing used by the participant was categorized as follows: 1. Unable to up climb 4 standard stairs; 2. Climbs 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Climbs 4 standard stairs "marking time", using one arm on one handrail; 4. Climbs 4 standard stairs "marking time", not needing handrail; 5. Climbs 4 standard stairs alternating feet, needs handrail for support; 6. Climbs 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or

the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. mITT population.

End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: seconds				
least squares mean (standard error)	5.25 ($\pm$ 0.518)	6.98 ($\pm$ 0.513)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0179
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-1.73
Confidence interval	
level	95 %
sides	2-sided
lower limit	-3.16
upper limit	-0.3
Variability estimate	Standard error of the mean
Dispersion value	0.729

Secondary: DB Period: Change From Baseline in Time to Climb 4 Stairs at Week 72 - ITT Population

End point title	DB Period: Change From Baseline in Time to Climb 4 Stairs at Week 72 - ITT Population
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End point description:

During the test for stair-climbing, the method of climbing used by the participant was categorized as follows: 1. Unable to up climb 4 standard stairs; 2. Climbs 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Climbs 4 standard stairs "marking time", using one arm on one handrail; 4. Climbs 4 standard stairs "marking time", not needing handrail; 5. Climbs 4 standard stairs alternating feet, needs handrail for support; 6. Climbs 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.

End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	4.98 ($\pm$ 0.364)	6.04 ($\pm$ 0.375)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0293
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-1.06
Confidence interval	
level	95 %
sides	2-sided
lower limit	-2.02
upper limit	-0.11
Variability estimate	Standard error of the mean
Dispersion value	0.488

Secondary: DB Period: Change From Baseline in Time to Descend 4 Stairs at Week 72 - mITT Population

End point title	DB Period: Change From Baseline in Time to Descend 4 Stairs at Week 72 - mITT Population
End point description:	During the test for stair-descending, the method of descending used by the participant was categorized as follows: 1. Unable to descend 4 standard stairs; 2. Descends 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Descends 4 standard stairs "marking time", using one arm on one handrail; 4. Descends 4 standard stairs "marking time", not needing handrail; 5. Descends 4 standard stairs alternating feet, needs handrail for support; 6. Descends 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. mITT population.
End point type	Secondary

End point timeframe:

Baseline, Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: seconds				
least squares mean (standard error)	4.58 ($\pm$ 0.545)	4.78 ($\pm$ 0.541)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.7997
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.19
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.7
upper limit	1.31
Variability estimate	Standard error of the mean
Dispersion value	0.768

Secondary: DB Period: Change From Baseline in Time to Descend 4 Stairs at Week 72 - ITT Population

End point title	DB Period: Change From Baseline in Time to Descend 4 Stairs at Week 72 - ITT Population
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End point description:

During the test for stair-descending, the method of descending used by the participant was categorized as follows: 1. Unable to descend 4 standard stairs; 2. Descends 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Descends 4 standard stairs "marking time", using one arm on one handrail; 4. Descends 4 standard stairs "marking time", not needing handrail; 5. Descends 4 standard stairs alternating feet, needs handrail for support; 6. Descends 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.

End point type	Secondary
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End point timeframe:

Baseline, Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	4.96 ($\pm$ 0.384)	5.25 ($\pm$ 0.396)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.5749
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.29
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.3
upper limit	0.72
Variability estimate	Standard error of the mean
Dispersion value	0.516

Secondary: DB Period: Composite of Average Change From Baseline in TFTs at Week 72 - mITT Population

End point title	DB Period: Composite of Average Change From Baseline in TFTs at Week 72 - mITT Population
End point description:	The composite TFT was defined as the average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement (average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs) on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$300 meters and time to stand from supine $\geq$5 seconds at baseline.
End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: seconds				
least squares mean (standard error)	4.15 ($\pm$ 0.435)	5.19 ($\pm$ 0.431)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0892
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-1.04
Confidence interval	
level	95 %
sides	2-sided
lower limit	-2.24
upper limit	0.16
Variability estimate	Standard error of the mean
Dispersion value	0.611

Secondary: DB Period: Composite of Average Change From Baseline in TFTs at Week 72 - ITT Population

End point title	DB Period: Composite of Average Change From Baseline in TFTs at Week 72 - ITT Population
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End point description:

The composite TFT was defined as the average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement (average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs) on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.

End point type	Secondary
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End point timeframe:

Baseline, Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	4.24 ($\pm$ 0.309)	4.93 ($\pm$ 0.319)		

Statistical analyses

Statistical analysis title	Statistical Analysis
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0904
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.7
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.51
upper limit	0.11
Variability estimate	Standard error of the mean
Dispersion value	0.412

Secondary: DB Period: Composite of Average Rate of Change From Baseline in TFTs at Week 72 - mITT Population

End point title	DB Period: Composite of Average Rate of Change From Baseline in TFTs at Week 72 - mITT Population
End point description:	The composite TFT was defined as the average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement (average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs) on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$300 meters and time to stand from supine $\geq$5 seconds at baseline.
End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: seconds/week				
least squares mean (standard error)	0.058 ($\pm$ 0.006)	0.072 ($\pm$ 0.006)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0892
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.014
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.031
upper limit	0.002
Variability estimate	Standard error of the mean
Dispersion value	0.008

Secondary: DB Period: Composite of Average Rate of Change From Baseline in TFTs at Week 72 - ITT Population

End point title	DB Period: Composite of Average Rate of Change From Baseline in TFTs at Week 72 - ITT Population
End point description:	The composite TFT was defined as the average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement (average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs) on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.
End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: seconds/week				
least squares mean (standard error)	0.059 ($\pm$ 0.004)	0.069 ($\pm$ 0.004)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0904
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	-0.01
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.021
upper limit	0.002
Variability estimate	Standard error of the mean
Dispersion value	0.006

Secondary: DB Period: Time to Persistent 10% Worsening in 6MWD at Week 72 - mITT Population

End point title	DB Period: Time to Persistent 10% Worsening in 6MWD at Week 72 - mITT Population
End point description:	The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Time to 10% persistent worsening in 6MWD was defined as the last time that 6MWD was not 10% worse compared with baseline. Participants who did not have 10% 6MWD worsening were censored at the time of the last 6-minute walk test during the DB period. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$300 meters and time to stand from supine $\geq$5 seconds at baseline. Here, 'Overall number of participants analyzed' = participants with 10% persistent worsening by Week 72.
End point type	Secondary
End point timeframe:	
Baseline to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	58	71		
Units: weeks				
median (confidence interval 95%)	36.7 (36.0 to 60.0)	35.6 (23.7 to 48.0)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	129
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0659
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.73
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.52
upper limit	1.03

Secondary: DB Period: Time to Persistent 10% Worsening in 6MWD at Week 72 - ITT Population

End point title	DB Period: Time to Persistent 10% Worsening in 6MWD at Week 72 - ITT Population
End point description:	
6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where participant is instructed to walk as far as possible, back and forth around two cones, with permission to slow down, rest, or stop if needed. Ambulation was assessed via 6MWD test following standardized procedures. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during 6MWD test. Time to 10% persistent worsening in 6MWD was defined as the last time that 6MWD was not 10% worse compared with baseline. Participants who did not have 10% 6MWD worsening were censored at the time of last 6-minute walk test during DB period. ITT population. 'Overall number of participants analyzed' = participants with 10% persistent worsening by Week 72. '99999' = Due to the low number of events, the data could not be estimated.	
End point type	Secondary
End point timeframe:	
Baseline to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	88	109		
Units: weeks				
median (confidence interval 95%)	74.3 (59.1 to 99999)	48.0 (36.0 to 60.9)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	197
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0078
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.69
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.52
upper limit	0.91

Secondary: DB Period: Change From Baseline in NSAA Total Score at Week 72 - ITT Population

End point title	DB Period: Change From Baseline in NSAA Total Score at Week 72 - ITT Population
End point description:	
The NSAA total score in the original scale is the sum of scores from 16 activities (excluding head lift). Each activity was scored as 0 (activity couldn't be performed), 1 (modified method, achieved goal without assistance), or 2 (normal, achieved goal without assistance). The total score ranges from 0 to 32, where higher scores indicate better functioning. If fewer than 13 of the 16 activities were performed, the total score was considered missing. If from 13 to 16 activities were performed, the total score was standardized by (observed total score / number of non-missing activities) x 16. If an activity could not be performed due to disease progression, a score of 0 was assigned. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.	
End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: units on a scale				
least squares mean (standard error)	-3.7 ($\pm$ 0.28)	-4.5 ($\pm$ 0.29)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.0235
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	0.9
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.1
upper limit	1.6
Variability estimate	Standard error of the mean
Dispersion value	0.38

Secondary: DB Period: Change From Baseline in North Start Ambulatory Assessment (NSAA) Total Score at Week 72 - mITT Population

End point title	DB Period: Change From Baseline in North Start Ambulatory Assessment (NSAA) Total Score at Week 72 - mITT Population
End point description:	The NSAA total score in the original scale is the sum of scores from 16 activities (excluding head lift). Each activity was scored as 0 (activity couldn't be performed), 1 (modified method, achieved goal without assistance), or 2 (normal, achieved goal without assistance). The total score ranges from 0 to 32, where higher scores indicate better functioning. If fewer than 13 of the 16 activities were performed, the total score was considered missing. If from 13 to 16 activities were performed, the total score was standardized by (observed total score / number of non-missing activities) x 16. If an activity could not be performed due to disease progression, a score of 0 was assigned. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. mITT population.
End point type	Secondary
End point timeframe:	
Baseline, Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: units on a scale				
least squares mean (standard error)	-5.2 ($\pm$ 0.40)	-6.1 ($\pm$ 0.40)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	185
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.1258
Method	Mixed models analysis
Parameter estimate	LS Mean Difference
Point estimate	0.9
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.2
upper limit	2
Variability estimate	Standard error of the mean
Dispersion value	0.57

Secondary: DB Period: Time to Loss of Ambulation Over 72 Weeks - mITT Population

End point title	DB Period: Time to Loss of Ambulation Over 72 Weeks - mITT Population
End point description:	Time to loss of ambulation was defined as persistent inability to perform the 10-meter run/walk test within 30 seconds at any post-baseline visit and for all remaining visits. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline. Here, 'Overall number of participants analyzed' = participants with loss of ambulation by Week 72. '99999' = Due to the low number of events, the data could not be estimated.
End point type	Secondary
End point timeframe:	
Baseline up to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	9		
Units: weeks				
median (confidence interval 95%)	84.1 (84.1 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	14
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.4803
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.66
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.21
upper limit	2.08

Secondary: DB Period: Time to Loss of Ambulation Over 72 Weeks - ITT Population

End point title	DB Period: Time to Loss of Ambulation Over 72 Weeks - ITT Population
End point description:	
Time to loss of ambulation was defined as persistent inability to perform the 10-meter run/walk test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of ambulation by Week 72. '99999' = Due to the low number of events, the data could not be estimated.	
End point type	Secondary
End point timeframe:	
Baseline up to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	12	20		
Units: weeks				
median (confidence interval 95%)	99999 (99999 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	32
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.1768
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.67
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.32
upper limit	1.39

Secondary: DB Period: Time to Loss of Stair-Climbing Over 72 Weeks - mITT Population

End point title	DB Period: Time to Loss of Stair-Climbing Over 72 Weeks - mITT Population
End point description: Time to loss of stair-climbing was defined as persistent inability to perform the 4-stair climb test within 30 seconds at any post-baseline visit and for all remaining visits. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline. Here, 'Overall number of participants analyzed' = participants with loss of stair-climbing by Week 72. '99999' = Due to the low number of events, the data could not be estimated.	
End point type	Secondary
End point timeframe: Baseline up to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	11	15		
Units: weeks				
median (confidence interval 95%)	84.1 (82.6 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	26
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.5452
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.72
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.33
upper limit	1.59

Secondary: DB Period: Time to Loss of Stair-Descending Over 72 Weeks - mITT Population

End point title	DB Period: Time to Loss of Stair-Descending Over 72 Weeks - mITT Population
End point description: Time to loss of stair-descending was defined as persistent inability to perform the 4-stair descend test within 30 seconds at any post-baseline visit and for all remaining visits. The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline. Here, 'Overall number of participants analyzed' = participants with loss of stair-descending by Week 72. '99999' = Due to the low number of events, the data could not be estimated.	
End point type	Secondary
End point timeframe: Baseline up to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	12	10		
Units: weeks				
median (confidence interval 95%)	84.1 (80.9 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo

Number of subjects included in analysis	22
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.3906
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	1.58
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.65
upper limit	3.84

Secondary: DB Period: Time to Loss of Stair-Climbing Over 72 Weeks - ITT Population

End point title	DB Period: Time to Loss of Stair-Climbing Over 72 Weeks - ITT Population
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End point description:

Time to loss of stair-climbing was defined as persistent inability to perform the 4-stair climb test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of stair-climbing by Week 72. '99999' = Due to the low number of events, the data could not be estimated.

End point type	Secondary
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End point timeframe:

Baseline up to Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	24	31		
Units: weeks				
median (confidence interval 95%)	87.1 (84.1 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	55
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.3055
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.69

Confidence interval	
level	95 %
sides	2-sided
lower limit	0.4
upper limit	1.19

Secondary: DB Period: Time to Loss of Stair-Descending Over 72 Weeks - ITT Population

End point title	DB Period: Time to Loss of Stair-Descending Over 72 Weeks - ITT Population
End point description:	
Time to loss of stair-descending was defined as persistent inability to perform the 4-stair descend test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of stair-descending by Week 72. '99999' = Due to the low number of events, the data could not be estimated.	
End point type	Secondary
End point timeframe:	
Baseline up to Week 72	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	24	26		
Units: weeks				
median (confidence interval 95%)	87.1 (84.1 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren v Placebo
Number of subjects included in analysis	50
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.8165
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.99
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.56
upper limit	1.74

Secondary: DB Period: Number of Participants With Function Loss of NSAA Items at Week 72 - mITT Population

End point title	DB Period: Number of Participants With Function Loss of NSAA Items at Week 72 - mITT Population
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End point description:

Function loss was defined as a drop of score from 1 or 2 at baseline to a score of 0 at the specified post-baseline. The missing assessments at post baseline visits were imputed using last observation carried forward (LOCF). The mITT population included all randomized participants who met the following additional criteria: 7 to 16 years old with 6MWD $\geq$ 300 meters and time to stand from supine $\geq$ 5 seconds at baseline. Here, 'n' = number of participants with baseline score of 2 or 1 and at least one post-baseline assessment for the specific activity.

End point type	Secondary
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End point timeframe:

Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	92	93		
Units: participants				
Stand (n = 92, 93)	6	10		
Walk (10m) (n = 92, 93)	5	10		
Stand up from chair (n = 90, 92)	22	23		
Stand on one leg - right (n = 92, 92)	13	16		
Stand on one leg - left (n = 90, 92)	12	15		
Climb box step - right (n = 76, 81)	22	38		
Descend box step - right (n = 91, 90)	24	25		
Climb box step - left (n = 79, 86)	24	38		
Descend box step - left (n = 90, 89)	25	20		
Lifts head (n = 78, 83)	13	15		
Gets to sitting (n = 92, 92)	6	10		
Rise from floor (n = 92, 92)	32	33		
Stand on heels (n = 56, 54)	26	30		
Jump (n = 82, 76)	26	22		
Hop right (n = 56, 68)	26	30		
Hop left (n = 50, 67)	22	28		
Run (10m) (n = 89, 89)	19	22		

Statistical analyses

No statistical analyses for this end point

Secondary: DB Period: Number of Participants With Function Loss of NSAA Items at Week 72 - ITT Population

End point title	DB Period: Number of Participants With Function Loss of NSAA Items at Week 72 - ITT Population
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End point description:

Function loss was defined as a drop of score from 1 or 2 at baseline to a score of 0 at the specified post-baseline. The missing assessments at post baseline visits were imputed using LOCF. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'n' = number of participants with baseline score of 2 or 1 and at least one post-baseline assessment for the specific activity.

End point type	Secondary
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End point timeframe:

Week 72

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	183	176		
Units: participants				
Stand (n = 182, 176)	14	21		
Walk (10m) (n = 183, 176)	14	21		
Stand up from chair (n = 177, 171)	36	35		
Stand on one leg - right (n = 180, 171)	25	25		
Stand on one leg - left (n = 177, 171)	22	23		
Climb box step - right (n = 155, 154)	28	50		
Descend box step - right (n = 173, 167)	33	39		
Climb box step - left (n = 158, 160)	30	51		
Descend box step - left (n = 170, 164)	32	34		
Lifts head (n = 160, 154)	19	28		
Gets to sitting (n = 183, 175)	13	22		
Rise from floor (n = 182, 173)	50	53		
Stand on heels (n = 130, 112)	40	38		
Jump (n = 160, 143)	32	31		
Hop right (n = 125, 126)	30	37		
Hop left (n = 120, 125)	27	34		
Run (10m) (n = 174, 164)	29	35		

Statistical analyses

No statistical analyses for this end point

Secondary: DB Period: Number of Participants With Treatment-emergent Adverse Events (TEAEs)

End point title	DB Period: Number of Participants With Treatment-emergent Adverse Events (TEAEs)
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End point description:

An adverse event (AE) was any untoward medical occurrence in a participant who received study drug without regard to possibility of causal relationship. Serious adverse event (SAE) was an AE resulting in any of the following outcomes or deemed significant for any other reason: death; initial or prolonged inpatient hospitalization; life-threatening experience (immediate risk of dying); persistent or significant disability/incapacity; congenital anomaly. AEs included both SAEs and non-serious AEs. A TEAE was defined as an AE that occurred or worsened on or after the first dose of study drug and up to 4 weeks after the last dose of double-blind study drug and prior to the first dose of open-label treatment. A summary of other non-serious AEs and all SAEs, regardless of causality is located in the 'Reported AE

section'. The as-treated population included all randomized participants who received study treatment, with treatment assignments designated according to actual treatment received.

End point type	Secondary
End point timeframe:	
Baseline up to Week 76	

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	184	176		
Units: participants	157	149		

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Treatment period: Change From Baseline in 6MWD at Week 144

End point title	Overall Treatment period: Change From Baseline in 6MWD at Week 144
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End point description:

The 6MWD test is a non-encouraged test performed in a 30 meters long flat corridor, where the participant is 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. Participants were not permitted to use assistive devices (walker, long leg braces, or short leg braces) during the 6MWD test. Participants with confirmed loss of ambulation at a particular visit were assigned a 6MWD result of 0. Baseline was defined as the maximum measurement of valid Day 1 and Day 2 6MWD values. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.

End point type	Secondary
End point timeframe:	
Baseline, Week 144	

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: meters				
least squares mean (standard error)	-130.49 ($\pm$ 6.913)	-140.00 ($\pm$ 7.277)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Placebo/Ataluren v Ataluren/Ataluren
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	9.51
Confidence interval	
level	95 %
sides	2-sided
lower limit	-7.49
upper limit	26.51
Variability estimate	Standard error of the mean
Dispersion value	8.671

Secondary: Overall Treatment period: Composite of Average Change From Baseline in TFTs at Week 144

End point title	Overall Treatment period: Composite of Average Change From Baseline in TFTs at Week 144
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End point description:

The composite TFT was defined as the average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement (average in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs) on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized.

End point type	Secondary
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End point timeframe:

Baseline, Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	9.00 (± 0.432)	9.29 (± 0.448)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren

Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	-0.29
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.43
upper limit	0.85
Variability estimate	Standard error of the mean
Dispersion value	0.581

Secondary: Overall Treatment period: Change From Baseline in Time to Run/Walk 10 Meters at Week 144

End point title	Overall Treatment period: Change From Baseline in Time to Run/Walk 10 Meters at Week 144
End point description:	During the test for walking/running 10 meters, the method of walk/run used by the participant was categorized as follows: 1. Unable to walk independently; 2. Unable to walk independently but can walk with support from a person or with assistive device (full leg calipers [knee-ankle-foot orthoses] or walker); 3. Highly adapted gait, wide-based lordotic gait, cannot increase walking speed; 4. Moderately adapted gait, can pick up speed but cannot run; 5. Able to pick up speed but runs with a double stance phase (that is, cannot achieve both feet off the ground); 6. Runs and gets both feet off the ground (with no double stance phase). Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.
End point type	Secondary
End point timeframe:	
Baseline, Week 144	

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	7.99 (± 0.400)	8.16 (± 0.415)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren

Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	-0.16
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.23
upper limit	0.9
Variability estimate	Standard error of the mean
Dispersion value	0.541

Secondary: Overall Treatment period: Change From Baseline in Time to Descend 4 Stairs at Week 144

End point title	Overall Treatment period: Change From Baseline in Time to Descend 4 Stairs at Week 144
End point description:	During the test for stair-descending, the method of descending used by the participant was categorized as follows: 1. Unable to descend 4 standard stairs; 2. Descends 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Descends 4 standard stairs "marking time", using one arm on one handrail; 4. Descends 4 standard stairs "marking time", not needing handrail; 5. Descends 4 standard stairs alternating feet, needs handrail for support; 6. Descends 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.
End point type	Secondary
End point timeframe:	
Baseline, Week 144	

End point values	Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	9.56 (± 0.512)	10.15 (± 0.530)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren

Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	-0.59
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.96
upper limit	0.77
Variability estimate	Standard error of the mean
Dispersion value	0.696

Secondary: Overall Treatment period: Change From Baseline in Time to Climb 4 Stairs at Week 144

End point title	Overall Treatment period: Change From Baseline in Time to Climb 4 Stairs at Week 144
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End point description:

During the test for stair-climbing, the method of climbing used by the participant was categorized as follows: 1. Unable to up climb 4 standard stairs; 2. Climbs 4 standard stairs "marking time" (climbs one foot at a time, with both feet on a step before moving to next step), using both arms on one or both handrails; 3. Climbs 4 standard stairs "marking time", using one arm on one handrail; 4. Climbs 4 standard stairs "marking time", not needing handrail; 5. Climbs 4 standard stairs alternating feet, needs handrail for support; 6. Climbs 4 standard stairs alternating feet, not needing handrail support. Participants who could not perform a TFT within 30 seconds, including those who loss of ambulation or the TFT was above 30 seconds, a value of 30 seconds was used. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using the MMRM. ITT population.

End point type	Secondary
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End point timeframe:

Baseline, Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: seconds				
least squares mean (standard error)	9.89 (± 0.480)	10.18 (± 0.496)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren

Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	-0.29
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.56
upper limit	0.98
Variability estimate	Standard error of the mean
Dispersion value	0.649

Secondary: Overall Treatment period: Time to Loss of Ambulation Over 144 Weeks

End point title	Overall Treatment period: Time to Loss of Ambulation Over 144 Weeks
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End point description:

Time to loss of ambulation was defined as persistent inability to perform the 10-meter run/walk test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of ambulation by Week 144. '99999' = Due to the low number of events, the data could not be estimated.

End point type	Secondary
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End point timeframe:

Baseline up to Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	40	42		
Units: weeks				
median (confidence interval 95%)	99999 (99999 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren
Number of subjects included in analysis	82
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	Hazard ratio (HR)
Point estimate	0.89

Confidence interval	
level	95 %
sides	2-sided
lower limit	0.57
upper limit	1.39

Secondary: Overall Treatment period: Change From Baseline in NSAA Total Score at Week 144

End point title	Overall Treatment period: Change From Baseline in NSAA Total Score at Week 144
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End point description:

The NSAA total score in the original scale is the sum of scores from 16 activities (excluding head lift). Each activity was scored as 0 (activity couldn't be performed), 1 (modified method, achieved goal without assistance), or 2 (normal, achieved goal without assistance). The total score ranges from 0 to 32, where higher scores indicate better functioning. If fewer than 13 of the 16 activities were performed, the total score was considered missing. If from 13 to 16 activities were performed, the total score was standardized by (observed total score / number of non-missing activities) x 16. If an activity could not be performed due to disease progression, a score of 0 was assigned. Baseline was defined as the last observed measurement on or prior to the first dose of study drug. LS mean and SE was calculated using analysis of covariation (ANCOVA) with multiple imputation. ITT population.

End point type	Secondary
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End point timeframe:

Baseline, Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: units on a scale				
least squares mean (standard error)	-7.0 (± 0.54)	-7.2 (± 0.56)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren
Number of subjects included in analysis	359
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	LS Mean Difference
Point estimate	0.3
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.1
upper limit	1.6
Variability estimate	Standard error of the mean
Dispersion value	0.68

Secondary: Overall Treatment period: Time to Loss of Stair-Climbing Over 144 Weeks

End point title	Overall Treatment period: Time to Loss of Stair-Climbing Over 144 Weeks
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End point description:

Time to loss of stair-climbing was defined as persistent inability to perform the 4-stair climb test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of stair-climbing by Week 144. '99999' = Due to the low number of events, the data could not be estimated.

End point type	Secondary
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End point timeframe:

Baseline up to Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	53	59		
Units: weeks				
median (confidence interval 95%)	99999 (99999 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren
Number of subjects included in analysis	112
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	Hazard ratio (HR)
Point estimate	0.75
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.51
upper limit	1.11

Secondary: Overall Treatment period: Number of Participants With Function Loss of NSAA Items at Week 144

End point title	Overall Treatment period: Number of Participants With Function Loss of NSAA Items at Week 144
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End point description:

Function loss was defined as a drop of score from 1 or 2 at baseline to a score of 0 at the specified post-baseline. The missing assessments at post baseline visits were imputed using LOCF. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'n' = number of participants with baseline score of 2 or 1 and at least one post-baseline assessment for the specific activity.

End point type	Secondary
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End point timeframe:

Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	183	176		
Units: participants				
Stand (n = 182, 176)	49	49		
Walk (10m) (n = 183, 176)	49	48		
Stand up from chair (n = 177, 171)	64	73		
Stand on one leg - right (n = 180, 171)	58	52		
Stand on one leg - left (n = 177, 171)	57	50		
Climb box step - right (n = 155, 154)	68	72		
Descend box step - right (n = 173, 167)	72	69		
Climb box step - left (n = 158, 160)	60	76		
Descend box step - left (n = 170, 164)	69	70		
Lifts head (n = 160, 154)	46	57		
Gets to sitting (n = 183, 175)	53	49		
Rise from floor (n = 182, 173)	85	91		
Stand on heels (n = 130, 112)	57	49		
Jump (n = 160, 143)	63	56		
Hop right (n = 125, 126)	48	58		
Hop left (n = 120, 125)	46	56		
Run (10m) (n = 174, 164)	60	60		

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Treatment period: Time to Loss of Stair- Descending Over 144 Weeks

End point title	Overall Treatment period: Time to Loss of Stair- Descending Over 144 Weeks
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End point description:

Time to loss of stair-descending was defined as persistent inability to perform the 4-stair descend test within 30 seconds at any post-baseline visit and for all remaining visits. The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants with loss of stair-descending by Week 144. '99999' = Due to the low number of events, the data could not be estimated.

End point type	Secondary
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End point timeframe:
Baseline up to Week 144

End point values	Ataluren/Ataluren	Placebo/Ataluren		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	53	59		
Units: weeks				
median (confidence interval 95%)	99999 (99999 to 99999)	99999 (99999 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Ataluren/Ataluren v Placebo/Ataluren
Number of subjects included in analysis	112
Analysis specification	Pre-specified
Analysis type	other
Parameter estimate	Hazard ratio (HR)
Point estimate	0.85
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.58
upper limit	1.25

Secondary: OL Period: Plasma Pharmacokinetic (PK) Concentration of Ataluren

End point title	OL Period: Plasma Pharmacokinetic (PK) Concentration of Ataluren
End point description:	The ITT population included all participants who were randomized, with treatment assignments designated according to initial randomization, regardless of whether participants received a different study treatment from the one randomized. Here, 'Overall number of participants analyzed' = participants evaluable for this outcome measure. 'n' = participants evaluable at specified timepoint.
End point type	Secondary
End point timeframe:	Predose and 2 hours postdose at Week 144

End point values	Ataluren	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	165	157		
Units: micrograms (µg)/milliliter (mL)				
geometric mean (geometric coefficient of variation)				
Predose (n = 161, 157)	4.45 (± 120.5)	4.11 (± 102.7)		
2 hours postdose (n = 165, 156)	12.16 (± 57.2)	12.21 (± 57.3)		

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Treatment period: Number of Participants With TEAEs

End point title	Overall Treatment period: Number of Participants With TEAEs
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End point description:

An AE was any untoward medical occurrence in a participant who received study drug without regard to possibility of causal relationship. An SAE was an AE resulting in any of the following outcomes or deemed significant for any other reason: death; initial or prolonged inpatient hospitalization; life-threatening experience (immediate risk of dying); persistent or significant disability/incapacity; congenital anomaly. AEs included both SAEs and non-serious AEs. A TEAE was defined as an AE that occurs or worsens on or after the first dose of ataluren (regardless of double-blind or open-label) and up to 4 weeks after the last dose of ataluren. A summary of other non-serious AEs and all SAEs, regardless of causality is located in the 'Reported AE section'. The as-treated-OA population included all randomized participants who received at least 1 dose of ataluren anytime during the study.

End point type	Secondary
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End point timeframe:

Baseline up to Week 148

End point values	Ataluren/Ataluren	Ataluren/Placebo		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	184	172		
Units: participants	170	110		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Baseline up to Week 148

Adverse event reporting additional description:

As-treated population included all randomized participants who received study treatment, with treatment assignments designated according to actual treatment received. AEs were summarized separately for DB period and for the overall ataluren experience, which included all participants who received ataluren throughout the study (DB and OL Period).

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	24.1
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Reporting groups

Reporting group title	DB Period: Ataluren
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Reporting group description:

Participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period.

Reporting group title	Ataluren/Placebo
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Reporting group description:

Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period. After completion of DB treatment period, participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in OL treatment period.

Reporting group title	Ataluren/Ataluren
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Reporting group description:

Participants received ataluren oral suspension 10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening each day for 72 weeks in DB treatment period and for an additional 72 weeks in OL treatment period.

Reporting group title	DB Period: Placebo
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Reporting group description:

Participants received placebo matched to ataluren oral suspension for 72 weeks in DB treatment period.

Serious adverse events	DB Period: Ataluren	Ataluren/Placebo	Ataluren/Ataluren
Total subjects affected by serious adverse events			
subjects affected / exposed	13 / 184 (7.07%)	11 / 172 (6.40%)	22 / 184 (11.96%)
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 / 184 (0.00%)	1 / 172 (0.58%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Humerus fracture			

subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Spinal compression fracture			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Lower limb fracture			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Tibia fracture			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Compression fracture			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Femoral neck fracture			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Lumbar vertebral fracture			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hip fracture			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Congenital, familial and genetic disorders			
Sebaaceous naevus			

subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Myocarditis			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Atrial fibrillation			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac failure chronic			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiomyopathy			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	2 / 184 (1.09%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Surgical and medical procedures			
Medical device removal			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Seizure			

subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Acute disseminated encephalomyelitis			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic gastritis			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Inguinal hernia			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	2 / 184 (1.09%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vomiting			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intussusception			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Abdominal pain			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Enteritis			

subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Rhabdomyolysis			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pain in extremity			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Back pain			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Appendicitis			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Influenza			
subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis			
subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Pneumonia mycoplasmal subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Upper respiratory tract infection subjects affected / exposed	1 / 184 (0.54%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Tracheitis subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis yersinia subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	0 / 184 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hand-foot-and-mouth disease subjects affected / exposed	0 / 184 (0.00%)	0 / 172 (0.00%)	1 / 184 (0.54%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Viral infection subjects affected / exposed	0 / 184 (0.00%)	1 / 172 (0.58%)	1 / 184 (0.54%)
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	DB Period: Placebo		
Total subjects affected by serious adverse events			
subjects affected / exposed	12 / 176 (6.82%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Femur fracture			

subjects affected / exposed	3 / 176 (1.70%)			
occurrences causally related to treatment / all	0 / 3			
deaths causally related to treatment / all	0 / 0			
Humerus fracture				
subjects affected / exposed	1 / 176 (0.57%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Spinal compression fracture				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Lower limb fracture				
subjects affected / exposed	1 / 176 (0.57%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Tibia fracture				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Compression fracture				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Femoral neck fracture				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Lumbar vertebral fracture				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Hip fracture				

subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Congenital, familial and genetic disorders			
Sebacous naevus			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Myocarditis			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Atrial fibrillation			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac failure chronic			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiomyopathy			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Surgical and medical procedures			
Medical device removal			

subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Seizure			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Acute disseminated encephalomyelitis			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Chronic gastritis			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Inguinal hernia			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Vomiting			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Intussusception			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Abdominal pain			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Enteritis			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Rhabdomyolysis			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pain in extremity			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Back pain			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Appendicitis			
subjects affected / exposed	0 / 176 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Influenza			
subjects affected / exposed	1 / 176 (0.57%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Gastroenteritis				
subjects affected / exposed	1 / 176 (0.57%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Pneumonia mycoplasmal				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Upper respiratory tract infection				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Tracheitis				
subjects affected / exposed	1 / 176 (0.57%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Gastroenteritis yersinia				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Hand-foot-and-mouth disease				
subjects affected / exposed	0 / 176 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Viral infection				
subjects affected / exposed	0 / 176 (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: 5 %

Non-serious adverse events	DB Period: Ataluren	Ataluren/Placebo	Ataluren/Ataluren
Total subjects affected by non-serious adverse events			
subjects affected / exposed	157 / 184 (85.33%)	107 / 172 (62.21%)	170 / 184 (92.39%)
Injury, poisoning and procedural complications			
Ligament sprain			
subjects affected / exposed	17 / 184 (9.24%)	5 / 172 (2.91%)	20 / 184 (10.87%)
occurrences (all)	17	6	22
Fall			
subjects affected / exposed	19 / 184 (10.33%)	6 / 172 (3.49%)	25 / 184 (13.59%)
occurrences (all)	35	7	48
Nervous system disorders			
Headache			
subjects affected / exposed	19 / 184 (10.33%)	6 / 172 (3.49%)	22 / 184 (11.96%)
occurrences (all)	38	29	56
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	19 / 184 (10.33%)	3 / 172 (1.74%)	25 / 184 (13.59%)
occurrences (all)	23	3	29
Disease progression			
subjects affected / exposed	11 / 184 (5.98%)	24 / 172 (13.95%)	47 / 184 (25.54%)
occurrences (all)	11	24	47
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	13 / 184 (7.07%)	4 / 172 (2.33%)	16 / 184 (8.70%)
occurrences (all)	22	4	27
Diarrhoea			
subjects affected / exposed	15 / 184 (8.15%)	2 / 172 (1.16%)	18 / 184 (9.78%)
occurrences (all)	17	2	20
Vomiting			
subjects affected / exposed	30 / 184 (16.30%)	4 / 172 (2.33%)	33 / 184 (17.93%)
occurrences (all)	46	4	52
Constipation			
subjects affected / exposed	0 / 184 (0.00%)	4 / 172 (2.33%)	11 / 184 (5.98%)
occurrences (all)	0	4	12
Respiratory, thoracic and mediastinal disorders			

Rhinorrhoea subjects affected / exposed occurrences (all)	12 / 184 (6.52%) 16	3 / 172 (1.74%) 4	16 / 184 (8.70%) 20
Cough subjects affected / exposed occurrences (all)	18 / 184 (9.78%) 23	6 / 172 (3.49%) 7	25 / 184 (13.59%) 35
Musculoskeletal and connective tissue disorders			
Back pain subjects affected / exposed occurrences (all)	0 / 184 (0.00%) 0	6 / 172 (3.49%) 8	11 / 184 (5.98%) 13
Pain in extremity subjects affected / exposed occurrences (all)	0 / 184 (0.00%) 0	3 / 172 (1.74%) 4	12 / 184 (6.52%) 17
Infections and infestations			
Upper respiratory tract infection subjects affected / exposed occurrences (all)	27 / 184 (14.67%) 50	17 / 172 (9.88%) 32	33 / 184 (17.93%) 70
Nasopharyngitis subjects affected / exposed occurrences (all)	27 / 184 (14.67%) 42	7 / 172 (4.07%) 11	34 / 184 (18.48%) 70
Influenza subjects affected / exposed occurrences (all)	8 / 184 (4.35%) 9	1 / 172 (0.58%) 1	10 / 184 (5.43%) 11
Gastroenteritis subjects affected / exposed occurrences (all)	0 / 184 (0.00%) 0	2 / 172 (1.16%) 2	14 / 184 (7.61%) 17
COVID-19 subjects affected / exposed occurrences (all)	0 / 184 (0.00%) 0	12 / 172 (6.98%) 13	8 / 184 (4.35%) 8

Non-serious adverse events	DB Period: Placebo		
Total subjects affected by non-serious adverse events subjects affected / exposed	148 / 176 (84.09%)		
Injury, poisoning and procedural complications Ligament sprain			

subjects affected / exposed occurrences (all) Fall subjects affected / exposed occurrences (all)	6 / 176 (3.41%) 6 23 / 176 (13.07%) 30		
Nervous system disorders Headache subjects affected / exposed occurrences (all)	15 / 176 (8.52%) 22		
General disorders and administration site conditions Pyrexia subjects affected / exposed occurrences (all) Disease progression subjects affected / exposed occurrences (all)	9 / 176 (5.11%) 10 18 / 176 (10.23%) 18		
Gastrointestinal disorders Abdominal pain subjects affected / exposed occurrences (all) Diarrhoea subjects affected / exposed occurrences (all) Vomiting subjects affected / exposed occurrences (all) Constipation subjects affected / exposed occurrences (all)	14 / 176 (7.95%) 15 12 / 176 (6.82%) 15 9 / 176 (5.11%) 10 0 / 176 (0.00%) 0		
Respiratory, thoracic and mediastinal disorders Rhinorrhoea subjects affected / exposed occurrences (all) Cough subjects affected / exposed occurrences (all)	5 / 176 (2.84%) 9 20 / 176 (11.36%) 32		

Musculoskeletal and connective tissue disorders Back pain subjects affected / exposed occurrences (all) Pain in extremity subjects affected / exposed occurrences (all)	0 / 176 (0.00%) 0 0 / 176 (0.00%) 0		
Infections and infestations Upper respiratory tract infection subjects affected / exposed occurrences (all) Nasopharyngitis subjects affected / exposed occurrences (all) Influenza subjects affected / exposed occurrences (all) Gastroenteritis subjects affected / exposed occurrences (all) COVID-19 subjects affected / exposed occurrences (all)	44 / 176 (25.00%) 77 12 / 176 (6.82%) 17 9 / 176 (5.11%) 9 0 / 176 (0.00%) 0 0 / 176 (0.00%) 0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
01 August 2019	It included the following changes: - Added collection of blood samples for determination of ataluren plasma concentrations. Addition of secondary objectives of ataluren PK profile evaluation and correlation between ataluren plasma concentrations and functional outcomes. - Facilitated enrolment of the mITT Population by limiting enrolment to participants aged ≥ 7 to ≤ 16 years who met the mITT criteria after approximately 270 participants were enrolled in the study. - Increased the sample size from approximately 250 participants to a maximum of 340 participants to achieve up to 162 participants in the mITT Population.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported