



Clinical trial results:

A Phase 3 Multinational, Randomized, Double-Blind, Placebo Controlled Systemic Gene Delivery Study to Evaluate the Safety and Efficacy of SRP 9001 in Subjects With Duchenne Muscular Dystrophy (EMBARK)

Summary

EudraCT number	2019-003374-91
Trial protocol	FR BE ES DE IT
Global end of trial date	25 October 2024

Results information

Result version number	v1 (current)
This version publication date	09 May 2025
First version publication date	09 May 2025

Trial information

Trial identification

Sponsor protocol code	SRP-9001-301
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05096221
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Sarepta Therapeutics Inc.
Sponsor organisation address	215 First Street,, Cambridge, MA, United States, 02142
Public contact	Medical Director, Sarepta Therapeutics, Inc., +1 888-727-3782, SareptAlly@sarepta.com
Scientific contact	Medical Director, Sarepta Therapeutics Inc., +1 888-727-3782, SareptAlly@sarepta.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMA-002677-PIP01-19
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

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

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the trial was to evaluate the effect of SRP 9001 on physical function as assessed by the North Star Ambulatory Assessment (NSAA) score.

Protection of trial subjects:

This study was conducted in compliance with the Institutional Review Board (IRB)/Independent Ethics Committee (IEC) regulations, Good Clinical Practice (GCP) Guidelines described in the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) E6 (R2) and 21 Code of Federal Regulations, and applicable national regulations and directives including the European Union (EU) Clinical Practice Directive 2005/28/EC, EU No 536/2014, and Japanese GCP Regulation.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	27 October 2021
Long term follow-up planned	Yes
Long term follow-up rationale	Safety, Efficacy
Long term follow-up duration	5 Years
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 80
Country: Number of subjects enrolled	Spain: 15
Country: Number of subjects enrolled	Taiwan: 5
Country: Number of subjects enrolled	Italy: 12
Country: Number of subjects enrolled	United Kingdom: 3
Country: Number of subjects enrolled	Japan: 4
Country: Number of subjects enrolled	Hong Kong: 3
Country: Number of subjects enrolled	Belgium: 2
Country: Number of subjects enrolled	Germany: 1
Worldwide total number of subjects	125
EEA total number of subjects	30

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)	125
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

126 participants were enrolled in the study. 1 of the 126 participants was enrolled via a country-specific protocol addendum. Data were collected but not analyzed for the participant enrolled via the country-specific protocol addendum. Data are presented below for the 125 participants enrolled via the global protocol.

Period 1

Period 1 title	Part 1
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	Delandistrogene Moxeparvovec followed by Placebo

Arm description:

Participants received a single intravenous (IV) infusion of delandistrogene moxeparvovec on Day 1 of Part 1. Then, participants received a single IV infusion of matching placebo on Day 1 in Part 2.

Arm type	Experimental
Investigational medicinal product name	Delandistrogene moxeparvovec
Investigational medicinal product code	
Other name	SRP-9001
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administration per details specified in the arm description.

Arm title	Placebo followed by Delandistrogene Moxeparvovec
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Arm description:

Participants received a single IV infusion of matching placebo on Day 1 of Part 1. Then, participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.

Arm type	Experimental
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	Saline, 0.9% sodium chloride solution
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administration per details specified in the arm description.

Number of subjects in period 1	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec
Started	63	62
Received at Least 1 Dose in Part 1	63	62
Completed	63	62

Period 2

Period 2 title	Part 2
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	Delandistrogene Moxeparvovec followed by Placebo

Arm description:

Participants received a single intravenous (IV) infusion of delandistrogene moxeparvovec on Day 1 of Part 1. Then, participants received a single IV infusion of matching placebo on Day 1 in Part 2.

Arm type	Experimental
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	Saline, 0.9% sodium chloride solution
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administration per details specified in the arm description.

Arm title	Placebo followed by Delandistrogene Moxeparvovec
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Arm description:

Participants received a single IV infusion of matching placebo on Day 1 of Part 1. Then, participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.

Arm type	Experimental
Investigational medicinal product name	Delandistrogene moxeparvovec
Investigational medicinal product code	
Other name	SRP-9001
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administration per details specified in the arm description.

Number of subjects in period 2^[1]	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec
Started	63	60
Received at Least 1 Dose in Part 2	62 ^[2]	60
Completed	63	59
Not completed	0	1
Physician decision	-	1

Notes:

[1] - The number of subjects starting the period is not consistent with the number completing the preceding period. It is expected the number of subjects starting the subsequent period will be the same as the number completing the preceding period.

Justification: Two participants who completed Part 1 discontinued prior to Part 2.

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: Number of participants who received at least 1 dose in Part 2.

Baseline characteristics

Reporting groups

Reporting group title	Delandistrogene Moxeparvovec followed by Placebo
Reporting group description:	
Participants received a single intravenous (IV) infusion of delandistrogene moxeparvovec on Day 1 of Part 1. Then, participants received a single IV infusion of matching placebo on Day 1 in Part 2.	
Reporting group title	Placebo followed by Delandistrogene Moxeparvovec
Reporting group description:	
Participants received a single IV infusion of matching placebo on Day 1 of Part 1. Then, participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.	

Reporting group values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec	Total
Number of subjects	63	62	125
Age categorical Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	63	62	125
Adolescents (12-17 years)	0	0	0
Adults (18-64 years)	0	0	0
From 65-84 years	0	0	0
85 years and over	0	0	0
Age Continuous Units: years			
arithmetic mean	5.98	6.08	
standard deviation	± 1.06	± 1.05	-
Sex: Female, Male Units: participants			
Female	0	0	0
Male	63	62	125
Race/Ethnicity, Customized Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	8	11	19
Native Hawaiian or Other Pacific Islander	0	0	0
Black of African American	0	2	2
White	49	46	95
More than One Race	1	0	1
Other or Not Reported	5	3	8
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	15	8	23
Not Hispanic or Latino	47	53	100

Unknown or Not Reported	1	1	2
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End points

End points reporting groups

Reporting group title	Delandistrogene Moxeparvovec followed by Placebo
Reporting group description: Participants received a single intravenous (IV) infusion of delandistrogene moxeparvovec on Day 1 of Part 1. Then, participants received a single IV infusion of matching placebo on Day 1 in Part 2.	
Reporting group title	Placebo followed by Delandistrogene Moxeparvovec
Reporting group description: Participants received a single IV infusion of matching placebo on Day 1 of Part 1. Then, participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.	
Reporting group title	Delandistrogene Moxeparvovec followed by Placebo
Reporting group description: Participants received a single intravenous (IV) infusion of delandistrogene moxeparvovec on Day 1 of Part 1. Then, participants received a single IV infusion of matching placebo on Day 1 in Part 2.	
Reporting group title	Placebo followed by Delandistrogene Moxeparvovec
Reporting group description: Participants received a single IV infusion of matching placebo on Day 1 of Part 1. Then, participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.	

Primary: Part 1: Change From Baseline in North Star Ambulatory Assessment (NSAA) Total Score at Week 52

End point title	Part 1: Change From Baseline in North Star Ambulatory Assessment (NSAA) Total Score at Week 52
End point description: The NSAA is a healthcare provider administered scale. During assessment, participants were asked to perform 17 functional activities graded as: 2-"Normal" - no obvious modification of activity; 1-Modified method but achieves goal independent of assistance; 0-Unable to achieve independently. The NSAA total score is the sum of all 17 items, ranging from 0(worst) to 34(best). The response vector consists of the change from baseline in NSAA total score at the post-baseline visit. The model includes the covariates of treatment group, visit, treatment group by visit interaction, age group, baseline NSAA total score, and baseline NSAA total score by visit interaction. An increase in score=improvement in motor function. Modified Intent-to-Treat (mITT) Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Number of Subjects Analyzed= number of participants evaluable for this outcome measure.	
End point type	Primary
End point timeframe: Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	63	61		
Units: scores on a scale				
least squares mean (standard error)	2.57 (± 0.39)	1.92 (± 0.39)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2441
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	0.65
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.45
upper limit	1.74
Variability estimate	Standard error of the mean
Dispersion value	0.55

Secondary: Part 1: Quantity of Delandistrogene Moxeparvovec Dystrophin Protein Expression at Week 12 as Measured by Western Blot Adjusted by Muscle Content

End point title	Part 1: Quantity of Delandistrogene Moxeparvovec Dystrophin Protein Expression at Week 12 as Measured by Western Blot Adjusted by Muscle Content
End point description: Quantity of delandistrogene moxeparvovec dystrophin protein levels (in muscle biopsy samples) were determined by Western blot at Part 1, Week 12. 2 blocks of tissues were analyzed by Western blot, each with 2 technical replicates to determine the delandistrogene moxeparvovec dystrophin protein level (percent control). The block average value from 2 technical replicates was computed. The overall average was calculated as the mean of the block average values. The overall average values were used for the analysis. Dystrophin protein was measured and then adjusted based on the percentage of muscle content in the biopsy sample. An increase in protein expression =production of the delandistrogene moxeparvovec dystrophin protein. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable.	
End point type	Secondary
End point timeframe: Week 12	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	17	14		
Units: Percent control				
arithmetic mean (standard deviation)	34.29 (\pm 41.04)	0.00 (\pm 0.00)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec
Number of subjects included in analysis	31
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Re-randomization test

Secondary: Part 1: Change From Baseline in Time to Rise From the Floor at Week 52

End point title	Part 1: Change From Baseline in Time to Rise From the Floor at Week 52
End point description:	
The time to rise from the floor test quantifies the time required for the participant to stand in an upright position with arms by sides, starting from the supine position with arms by sides. Data is presented for the change from baseline to Week 52 in the time taken (in seconds) to rise from the floor. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Participants Subjects"= number of participants evaluable for this outcome measure.	
End point type	Secondary
End point timeframe:	
Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	63	61		
Units: Seconds				
least squares mean (standard error)	-0.27 (\pm 0.15)	0.37 (\pm 0.15)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0025
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	-0.64
Confidence interval	
level	95 %
sides	2-sided
lower limit	-1.06
upper limit	-0.23
Variability estimate	Standard error of the mean
Dispersion value	0.21

Secondary: Part 1: Change from Baseline in Time to Complete 10 Meter Walk/Run (10MWR) at Week 52

End point title	Part 1: Change from Baseline in Time to Complete 10 Meter Walk/Run (10MWR) at Week 52
End point description: The timed 10MWR quantifies the time required for the participant to run or walk 10 meters (on a straight walkway) from a standing position. Data is presented for change from baseline to Week 52 in the time (in seconds) taken to complete the 10MWR. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.	
End point type	Secondary
End point timeframe: Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	63	61		
Units: Seconds				
least squares mean (standard error)	-0.34 (± 0.10)	0.08 (± 0.10)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0048
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	-0.42
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.71
upper limit	-0.13
Variability estimate	Standard error of the mean
Dispersion value	0.15

Secondary: Part 1: Change from Baseline in Time to Complete 100 Meter Walk/Run (100MWR) at Week 52

End point title	Part 1: Change from Baseline in Time to Complete 100 Meter Walk/Run (100MWR) at Week 52
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End point description:

The timed 100MWR quantifies the time required for the participant to run or walk 100 meters (on a straight walkway) from a standing position. Data is presented for change from baseline to Week 52 in the time (in seconds) taken to complete the 100MWR. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Participants Subjects"= number of participants evaluable for this outcome measure.

End point type	Secondary
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End point timeframe:

Baseline, Week 52 (Part 1)

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	59	57		
Units: Seconds				
least squares mean (standard error)	-6.57 (± 1.76)	-3.28 (± 1.80)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo

	followed by Delandistrogene Moxeparvovec
Number of subjects included in analysis	116
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1942
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	-3.29
Confidence interval	
level	95 %
sides	2-sided
lower limit	-8.28
upper limit	1.7
Variability estimate	Standard error of the mean
Dispersion value	2.52

Secondary: Part 1: Change from Baseline in the Timed Stair Ascend 4 Steps Test at Week 52

End point title	Part 1: Change from Baseline in the Timed Stair Ascend 4 Steps Test at Week 52
End point description:	
The timed stair ascend 4 steps test quantifies the time required for the participant to ascend 4 standard steps (each step was 6 inches in height). Data presented is the change from baseline to Week 52 in the time (in seconds) to ascend 4 steps. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.	
End point type	Secondary
End point timeframe:	
Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	62	60		
Units: Seconds				
least squares mean (standard error)	-0.44 (± 0.12)	-0.08 (± 0.13)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec

Number of subjects included in analysis	122
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0412
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	-0.36
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.71
upper limit	-0.01
Variability estimate	Standard error of the mean
Dispersion value	0.18

Secondary: Part 1: Change From Baseline in Stride Velocity 95th Centile (SV95C) at Week 52

End point title	Part 1: Change From Baseline in Stride Velocity 95th Centile (SV95C) at Week 52
End point description:	
Each participant was provided with wearable devices to collect data on stride velocity. Participants wore a device on each ankle. SV95C data was derived based on stride velocity. Data is presented for change from baseline to Week 52 in SV95C. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.	
End point type	Secondary
End point timeframe:	
Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	57	61		
Units: meters (m)/second (s)				
least squares mean (standard error)	0.06 (± 0.03)	-0.03 (± 0.03)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec

Number of subjects included in analysis	118
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0402
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	0.1
Confidence interval	
level	95 %
sides	2-sided
lower limit	0
upper limit	0.19
Variability estimate	Standard error of the mean
Dispersion value	0.05

Secondary: Part 1: Change from Baseline in Patient-Reported Outcomes Measurement Information System (PROMIS) Score in Mobility to Week 52

End point title	Part 1: Change from Baseline in Patient-Reported Outcomes Measurement Information System (PROMIS) Score in Mobility to Week 52
End point description:	PROMIS is a family of instruments developed and validated to assess health-related quality of life, including mobility. Mobility function scores ranged from 1 (worst mobility function) to 5 (best mobility function), where higher scores indicate a better clinical outcome. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.
End point type	Secondary
End point timeframe:	Baseline, Week 52 (Part 1)

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	57	59		
Units: Scores on a scale				
least squares mean (standard error)	0.05 (± 0.05)	-0.01 (± 0.05)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec

Number of subjects included in analysis	116
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4272
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	0.05
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.08
upper limit	0.19
Variability estimate	Standard error of the mean
Dispersion value	0.07

Secondary: Part 1: Change from Baseline in PROMIS Score in Upper Extremity Function to Week 52

End point title	Part 1: Change from Baseline in PROMIS Score in Upper Extremity Function to Week 52
End point description:	
PROMIS is a family of instruments developed and validated to assess health-related quality of life, including upper extremity function. Upper extremity function scores ranged from 1 (not able to do so [worst upper extremity function]) to 5 (no trouble [best upper extremity function]), where higher scores indicate a better clinical outcome. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.	
End point type	Secondary
End point timeframe:	
Baseline, Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	57	58		
Units: scores on a scale				
least squares mean (standard error)	0.19 (± 0.07)	0.23 (± 0.07)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec

Number of subjects included in analysis	115
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7324
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	-0.04
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.24
upper limit	0.17
Variability estimate	Standard error of the mean
Dispersion value	0.1

Secondary: Part 1: Number of Skills Gained or Improved at Week 52 as Measured by the NSAA

End point title	Part 1: Number of Skills Gained or Improved at Week 52 as Measured by the NSAA
End point description:	
<p>The NSAA is a clinician-administered scale that rates performance on various functional activities. As measured by the NSAA, data was collected for the number of skills gained (the average item score was 0 at Baseline and > 0 at Part 1 Week 52) or improved (the average item score at Baseline was > 0 but less than the average item score at Part 1 Week 52). As pre-specified, data are presented for the combined number of skills gained or improved at Week 52. Measured in the mITT Population, which included all randomized participants who received study treatment, with treatment group designated according to randomization. Here, "Number of Subjects Analyzed"= number of participants evaluable for this outcome measure.</p>	
End point type	Secondary
End point timeframe:	
Week 52 (Part 1)	

End point values	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	63	61		
Units: number of skills				
least squares mean (standard error)	4.18 (± 0.31)	3.99 (± 0.31)		

Statistical analyses

Statistical analysis title	Delandistrogene Moxeparvovec vs Placebo
Comparison groups	Delandistrogene Moxeparvovec followed by Placebo v Placebo followed by Delandistrogene Moxeparvovec

Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6554
Method	Mixed model of repeated measures
Parameter estimate	Least squares mean change difference
Point estimate	0.19
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.67
upper limit	1.06
Variability estimate	Standard error of the mean
Dispersion value	0.44

Secondary: Parts 1 and 2: Number of Participants with Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs)

End point title	Parts 1 and 2: Number of Participants with Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs)
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End point description:

TEAEs were defined as an adverse event (AE) that emerged during treatment, having been absent pre-treatment, or worsened relative to the pretreatment state. SAEs were defined as any adverse event that resulted in death, was life threatening, required or prolonged inpatient hospitalization, persistent or significant disability/incapacity, congenital anomaly/birth defect, or was an important medical event. A Summary of all SAEs and nonserious AEs ("Other"), regardless of causality and regardless of the AESI category, is located in the "Reported Adverse Events" section. Measured in the Safety Population in Part 1, which included all participants who received study treatment, with treatment group designated according to the treatment they received. Measured in the SRP-treated population in Part 2, which included all participants who received investigational study treatment (delandistrogene moxeparvec) in the study.

End point type	Secondary
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End point timeframe:

Up to 104 weeks

End point values	Delandistrogene Moxeparvec followed by Placebo	Delandistrogene Moxeparvec followed by Placebo	Placebo followed by Delandistrogene Moxeparvec	Placebo followed by Delandistrogene Moxeparvec
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	63	63	62	60
Units: participants				
TEAEs	62	53	57	56
SAEs	14	5	5	8

Statistical analyses

Secondary: Parts 1 and 2: Number of Participants with Adverse Events of Special Interest (AESI)

End point title	Parts 1 and 2: Number of Participants with Adverse Events of Special Interest (AESI)
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End point description:

AESIs defined as adverse events (AEs) of special interest, based on prespecified criteria and pertain to categories labeled hepatotoxicity, immune-mediated myositis, thrombotic microangiopathy (TMA), hypersensitivity, thrombocytopenia, rhabdomyolysis, and troponin elevations. Data represent number of participants who experienced an event within specified AESI category as observed by principal investigator (PI), after adjudication. Per prespecified analysis, AESI data were only collected based on specified AESI categories. Summary of all SAEs and nonserious AEs ("Other"), regardless of causality and regardless of the AESI category, is located in the "Reported Adverse Events" section. Measured in Safety Population in Part 1, which included all participants who received study treatment, with treatment group designated according to treatment received. Measured in SRP-treated population in Part 2, which included all participants who received delandistrogene moxeparvovec in the study.

End point type	Secondary
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End point timeframe:

Up to 104 weeks

End point values	Delandistrogene Moxeparvovec followed by Placebo	Delandistrogene Moxeparvovec followed by Placebo	Placebo followed by Delandistrogene Moxeparvovec	Placebo followed by Delandistrogene Moxeparvovec
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	63	63	62	60
Units: participants				
Hepatotoxicity	12	0	0	14
Immune-mediated Myositis	0	0	0	0
TMA	0	0	0	0
Hypersensitivity	1	1	0	0
Thrombocytopenia	2	1	0	0
Rhabdomyolysis	1	0	1	0
Troponin I Elevations	2	14	1	8

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Up to approximately 104 weeks

Adverse event reporting additional description:

Part 1: Safety Population, which included all participants who received study treatment, with treatment group designated according to the treatment they received. Part 2: SRP-treated population, which included all participants who received investigational study treatment (delandistrogene moxeparvovec) in the study.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.0
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Reporting groups

Reporting group title	Part 1: Delandistrogene Moxeparvovec
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Reporting group description:

Participants received a single IV infusion of delandistrogene moxeparvovec on Day 1 of Part 1.

Reporting group title	Part 2: Delandistrogene Moxeparvovec
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Reporting group description:

Participants who received matching placebo on Day 1 in Part 1 received a single IV infusion of delandistrogene moxeparvovec on Day 1 in Part 2.

Reporting group title	Part 2: Placebo
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Reporting group description:

Participants who received delandistrogene moxeparvovec on Day 1 of Part 1 received a single IV infusion of matching placebo on Day 1 in Part 2.

Reporting group title	Part 1: Placebo
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Reporting group description:

Participants received a single IV infusion of matching placebo on Day 1 of Part 1.

Serious adverse events	Part 1: Delandistrogene Moxeparvovec	Part 2: Delandistrogene Moxeparvovec	Part 2: Placebo
Total subjects affected by serious adverse events			
subjects affected / exposed	14 / 63 (22.22%)	8 / 60 (13.33%)	5 / 63 (7.94%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events			
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	1 / 63 (1.59%)	1 / 60 (1.67%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Transaminases increased			

subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 63 (1.59%)	1 / 60 (1.67%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Craniocerebral injury			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Prescription drug used without a prescription			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Arterial injury			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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
Upper limb fracture			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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
Forearm fracture			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	1 / 63 (1.59%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Left ventricular dysfunction			

subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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
Myocarditis			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	1 / 63 (1.59%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Haemorrhage intracranial			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Headache			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	1 / 63 (1.59%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	1 / 63 (1.59%)	2 / 60 (3.33%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	2 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	2 / 63 (3.17%)	2 / 60 (3.33%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 2	2 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Abdominal pain			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nausea			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Faecaloma			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	1 / 63 (1.59%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Hepatotoxicity			
subjects affected / exposed	1 / 63 (1.59%)	2 / 60 (3.33%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	4 / 4	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Liver injury			
subjects affected / exposed	1 / 63 (1.59%)	1 / 60 (1.67%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	1 / 1	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Rhabdomyolysis			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	1 / 63 (1.59%)
occurrences causally related to treatment / all	1 / 2	0 / 0	2 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Pneumonia			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Appendicitis			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
COVID-19			
subjects affected / exposed	2 / 63 (3.17%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Rotavirus infection			

subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Toxic shock syndrome streptococcal			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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
Anal abscess			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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
Influenza			
subjects affected / exposed	0 / 63 (0.00%)	0 / 60 (0.00%)	0 / 63 (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			
subjects affected / exposed	0 / 63 (0.00%)	1 / 60 (1.67%)	0 / 63 (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

Serious adverse events	Part 1: Placebo		
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 62 (8.06%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Transaminases increased			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gamma-glutamyltransferase			

increased			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Craniocerebral injury			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Prescription drug used without a prescription			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Arterial injury			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Upper limb fracture			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Forearm fracture			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Left ventricular dysfunction			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Myocarditis			

subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Haemorrhage intracranial			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Headache			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Abdominal pain			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nausea			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Faecaloma			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

Hepatobiliary disorders			
Hepatotoxicity			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Liver injury			
subjects affected / exposed	0 / 62 (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	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Pneumonia			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Appendicitis			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
COVID-19			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Rotavirus infection			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Toxic shock syndrome streptococcal			

subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Anal abscess			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Influenza			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastroenteritis			
subjects affected / exposed	0 / 62 (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	Part 1: Delandistrogene Moxeparvovec	Part 2: Delandistrogene Moxeparvovec	Part 2: Placebo
Total subjects affected by non-serious adverse events			
subjects affected / exposed	62 / 63 (98.41%)	55 / 60 (91.67%)	52 / 63 (82.54%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	1 / 63 (1.59%)	3 / 60 (5.00%)	0 / 63 (0.00%)
occurrences (all)	1	3	0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	19 / 63 (30.16%)	12 / 60 (20.00%)	5 / 63 (7.94%)
occurrences (all)	24	14	8
Fatigue			
subjects affected / exposed	9 / 63 (14.29%)	10 / 60 (16.67%)	1 / 63 (1.59%)
occurrences (all)	10	10	1
Immune system disorders			

Seasonal allergy subjects affected / exposed occurrences (all)	3 / 63 (4.76%) 3	0 / 60 (0.00%) 0	4 / 63 (6.35%) 4
Respiratory, thoracic and mediastinal disorders			
Cough subjects affected / exposed occurrences (all)	12 / 63 (19.05%) 15	20 / 60 (33.33%) 29	12 / 63 (19.05%) 15
Oropharyngeal pain subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	2 / 60 (3.33%) 2	2 / 63 (3.17%) 3
Nasal congestion subjects affected / exposed occurrences (all)	2 / 63 (3.17%) 3	6 / 60 (10.00%) 8	4 / 63 (6.35%) 7
Rhinorrhoea subjects affected / exposed occurrences (all)	5 / 63 (7.94%) 13	6 / 60 (10.00%) 9	7 / 63 (11.11%) 8
Epistaxis subjects affected / exposed occurrences (all)	2 / 63 (3.17%) 2	3 / 60 (5.00%) 4	1 / 63 (1.59%) 3
Psychiatric disorders			
Aggression subjects affected / exposed occurrences (all)	1 / 63 (1.59%) 1	6 / 60 (10.00%) 6	2 / 63 (3.17%) 2
Irritability subjects affected / exposed occurrences (all)	9 / 63 (14.29%) 10	3 / 60 (5.00%) 3	2 / 63 (3.17%) 2
Attention deficit hyperactivity disorder subjects affected / exposed occurrences (all)	4 / 63 (6.35%) 4	1 / 60 (1.67%) 1	3 / 63 (4.76%) 3
Investigations			
Blood creatine phosphokinase increased subjects affected / exposed occurrences (all)	1 / 63 (1.59%) 1	1 / 60 (1.67%) 1	1 / 63 (1.59%) 1
Gamma-glutamyltransferase increased			

subjects affected / exposed	5 / 63 (7.94%)	12 / 60 (20.00%)	2 / 63 (3.17%)
occurrences (all)	6	17	2
Glutamate dehydrogenase increased			
subjects affected / exposed	18 / 63 (28.57%)	12 / 60 (20.00%)	1 / 63 (1.59%)
occurrences (all)	22	13	1
Troponin I increased			
subjects affected / exposed	2 / 63 (3.17%)	5 / 60 (8.33%)	6 / 63 (9.52%)
occurrences (all)	3	7	10
Blood bilirubin increased			
subjects affected / exposed	0 / 63 (0.00%)	3 / 60 (5.00%)	0 / 63 (0.00%)
occurrences (all)	0	5	0
Injury, poisoning and procedural complications			
Skin abrasion			
subjects affected / exposed	1 / 63 (1.59%)	1 / 60 (1.67%)	1 / 63 (1.59%)
occurrences (all)	3	1	1
Fall			
subjects affected / exposed	5 / 63 (7.94%)	6 / 60 (10.00%)	5 / 63 (7.94%)
occurrences (all)	14	7	10
Contusion			
subjects affected / exposed	7 / 63 (11.11%)	5 / 60 (8.33%)	5 / 63 (7.94%)
occurrences (all)	13	5	7
Ligament sprain			
subjects affected / exposed	3 / 63 (4.76%)	6 / 60 (10.00%)	0 / 63 (0.00%)
occurrences (all)	4	7	0
Joint injury			
subjects affected / exposed	0 / 63 (0.00%)	3 / 60 (5.00%)	0 / 63 (0.00%)
occurrences (all)	0	3	0
Nervous system disorders			
Headache			
subjects affected / exposed	8 / 63 (12.70%)	10 / 60 (16.67%)	9 / 63 (14.29%)
occurrences (all)	12	16	17
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	1 / 63 (1.59%)	4 / 60 (6.67%)	1 / 63 (1.59%)
occurrences (all)	1	4	1
Gastrointestinal disorders			

Nausea subjects affected / exposed occurrences (all)	24 / 63 (38.10%) 30	23 / 60 (38.33%) 38	5 / 63 (7.94%) 7
Abdominal pain upper subjects affected / exposed occurrences (all)	10 / 63 (15.87%) 14	11 / 60 (18.33%) 15	1 / 63 (1.59%) 1
Diarrhoea subjects affected / exposed occurrences (all)	6 / 63 (9.52%) 8	8 / 60 (13.33%) 9	0 / 63 (0.00%) 0
Vomiting subjects affected / exposed occurrences (all)	39 / 63 (61.90%) 93	43 / 60 (71.67%) 103	9 / 63 (14.29%) 11
Abdominal pain subjects affected / exposed occurrences (all)	5 / 63 (7.94%) 6	8 / 60 (13.33%) 13	0 / 63 (0.00%) 0
Constipation subjects affected / exposed occurrences (all)	5 / 63 (7.94%) 5	6 / 60 (10.00%) 6	4 / 63 (6.35%) 4
Skin and subcutaneous tissue disorders			
Ecchymosis subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	4 / 60 (6.67%) 5	1 / 63 (1.59%) 1
Rash subjects affected / exposed occurrences (all)	6 / 63 (9.52%) 8	8 / 60 (13.33%) 9	2 / 63 (3.17%) 2
Renal and urinary disorders			
Proteinuria subjects affected / exposed occurrences (all)	1 / 63 (1.59%) 1	2 / 60 (3.33%) 3	4 / 63 (6.35%) 4
Ketonuria subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	3 / 60 (5.00%) 3	0 / 63 (0.00%) 0
Endocrine disorders			
Cushingoid subjects affected / exposed occurrences (all)	4 / 63 (6.35%) 4	3 / 60 (5.00%) 3	0 / 63 (0.00%) 0
Musculoskeletal and connective tissue			

disorders			
Back pain			
subjects affected / exposed	4 / 63 (6.35%)	5 / 60 (8.33%)	5 / 63 (7.94%)
occurrences (all)	7	5	7
Arthralgia			
subjects affected / exposed	7 / 63 (11.11%)	8 / 60 (13.33%)	3 / 63 (4.76%)
occurrences (all)	8	21	3
Pain in extremity			
subjects affected / exposed	7 / 63 (11.11%)	10 / 60 (16.67%)	8 / 63 (12.70%)
occurrences (all)	15	23	8
Myalgia			
subjects affected / exposed	4 / 63 (6.35%)	4 / 60 (6.67%)	1 / 63 (1.59%)
occurrences (all)	4	4	3
Muscle spasms			
subjects affected / exposed	2 / 63 (3.17%)	2 / 60 (3.33%)	2 / 63 (3.17%)
occurrences (all)	2	2	5
Rhabdomyolysis			
subjects affected / exposed	1 / 63 (1.59%)	0 / 60 (0.00%)	0 / 63 (0.00%)
occurrences (all)	1	0	0
Infections and infestations			
Nasopharyngitis			
subjects affected / exposed	9 / 63 (14.29%)	5 / 60 (8.33%)	4 / 63 (6.35%)
occurrences (all)	11	6	7
Ear infection			
subjects affected / exposed	6 / 63 (9.52%)	4 / 60 (6.67%)	2 / 63 (3.17%)
occurrences (all)	6	6	3
Enterobiasis			
subjects affected / exposed	5 / 63 (7.94%)	1 / 60 (1.67%)	5 / 63 (7.94%)
occurrences (all)	6	1	7
Viral infection			
subjects affected / exposed	5 / 63 (7.94%)	2 / 60 (3.33%)	5 / 63 (7.94%)
occurrences (all)	6	2	8
Gastroenteritis viral			
subjects affected / exposed	4 / 63 (6.35%)	1 / 60 (1.67%)	1 / 63 (1.59%)
occurrences (all)	6	1	1
Conjunctivitis			

subjects affected / exposed occurrences (all)	2 / 63 (3.17%) 2	1 / 60 (1.67%) 1	1 / 63 (1.59%) 1
COVID-19 subjects affected / exposed occurrences (all)	15 / 63 (23.81%) 15	6 / 60 (10.00%) 6	6 / 63 (9.52%) 6
Influenza subjects affected / exposed occurrences (all)	9 / 63 (14.29%) 9	5 / 60 (8.33%) 5	2 / 63 (3.17%) 2
Upper respiratory tract infection subjects affected / exposed occurrences (all)	13 / 63 (20.63%) 16	13 / 60 (21.67%) 30	10 / 63 (15.87%) 21
Gastroenteritis subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	2 / 60 (3.33%) 2	4 / 63 (6.35%) 5
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all)	20 / 63 (31.75%) 21	15 / 60 (25.00%) 19	1 / 63 (1.59%) 1
Vitamin D deficiency subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	3 / 60 (5.00%) 3	0 / 63 (0.00%) 0
Dehydration subjects affected / exposed occurrences (all)	0 / 63 (0.00%) 0	3 / 60 (5.00%) 3	1 / 63 (1.59%) 1

Non-serious adverse events	Part 1: Placebo		
Total subjects affected by non-serious adverse events subjects affected / exposed	57 / 62 (91.94%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps) Skin papilloma subjects affected / exposed occurrences (all)	1 / 62 (1.61%) 1		
General disorders and administration site conditions Pyrexia subjects affected / exposed occurrences (all)	14 / 62 (22.58%) 19		

Fatigue subjects affected / exposed occurrences (all)	6 / 62 (9.68%) 8		
Immune system disorders Seasonal allergy subjects affected / exposed occurrences (all)	2 / 62 (3.23%) 2		
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all) Oropharyngeal pain subjects affected / exposed occurrences (all) Nasal congestion subjects affected / exposed occurrences (all) Rhinorrhoea subjects affected / exposed occurrences (all) Epistaxis subjects affected / exposed occurrences (all)	19 / 62 (30.65%) 30 4 / 62 (6.45%) 4 7 / 62 (11.29%) 11 7 / 62 (11.29%) 9 3 / 62 (4.84%) 3		
Psychiatric disorders Aggression subjects affected / exposed occurrences (all) Irritability subjects affected / exposed occurrences (all) Attention deficit hyperactivity disorder subjects affected / exposed occurrences (all)	4 / 62 (6.45%) 4 4 / 62 (6.45%) 6 1 / 62 (1.61%) 1		
Investigations Blood creatine phosphokinase increased			

subjects affected / exposed	4 / 62 (6.45%)		
occurrences (all)	4		
Gamma-glutamyltransferase increased			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences (all)	0		
Glutamate dehydrogenase increased			
subjects affected / exposed	2 / 62 (3.23%)		
occurrences (all)	2		
Troponin I increased			
subjects affected / exposed	2 / 62 (3.23%)		
occurrences (all)	3		
Blood bilirubin increased			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences (all)	0		
Injury, poisoning and procedural complications			
Skin abrasion			
subjects affected / exposed	4 / 62 (6.45%)		
occurrences (all)	5		
Fall			
subjects affected / exposed	7 / 62 (11.29%)		
occurrences (all)	9		
Contusion			
subjects affected / exposed	9 / 62 (14.52%)		
occurrences (all)	17		
Ligament sprain			
subjects affected / exposed	1 / 62 (1.61%)		
occurrences (all)	1		
Joint injury			
subjects affected / exposed	2 / 62 (3.23%)		
occurrences (all)	2		
Nervous system disorders			
Headache			
subjects affected / exposed	8 / 62 (12.90%)		
occurrences (all)	8		
Blood and lymphatic system disorders			

Thrombocytopenia subjects affected / exposed occurrences (all)	0 / 62 (0.00%) 0		
Gastrointestinal disorders			
Nausea subjects affected / exposed occurrences (all)	8 / 62 (12.90%) 10		
Abdominal pain upper subjects affected / exposed occurrences (all)	9 / 62 (14.52%) 14		
Diarrhoea subjects affected / exposed occurrences (all)	13 / 62 (20.97%) 15		
Vomiting subjects affected / exposed occurrences (all)	11 / 62 (17.74%) 14		
Abdominal pain subjects affected / exposed occurrences (all)	7 / 62 (11.29%) 9		
Constipation subjects affected / exposed occurrences (all)	5 / 62 (8.06%) 6		
Skin and subcutaneous tissue disorders			
Ecchymosis subjects affected / exposed occurrences (all)	1 / 62 (1.61%) 1		
Rash subjects affected / exposed occurrences (all)	3 / 62 (4.84%) 5		
Renal and urinary disorders			
Proteinuria subjects affected / exposed occurrences (all)	0 / 62 (0.00%) 0		
Ketonuria subjects affected / exposed occurrences (all)	0 / 62 (0.00%) 0		
Endocrine disorders			

Cushingoid subjects affected / exposed occurrences (all)	4 / 62 (6.45%) 4		
Musculoskeletal and connective tissue disorders			
Back pain subjects affected / exposed occurrences (all)	4 / 62 (6.45%) 5		
Arthralgia subjects affected / exposed occurrences (all)	3 / 62 (4.84%) 5		
Pain in extremity subjects affected / exposed occurrences (all)	12 / 62 (19.35%) 20		
Myalgia subjects affected / exposed occurrences (all)	1 / 62 (1.61%) 1		
Muscle spasms subjects affected / exposed occurrences (all)	4 / 62 (6.45%) 4		
Rhabdomyolysis subjects affected / exposed occurrences (all)	4 / 62 (6.45%) 4		
Infections and infestations			
Nasopharyngitis subjects affected / exposed occurrences (all)	12 / 62 (19.35%) 15		
Ear infection subjects affected / exposed occurrences (all)	6 / 62 (9.68%) 7		
Enterobiasis subjects affected / exposed occurrences (all)	0 / 62 (0.00%) 0		
Viral infection subjects affected / exposed occurrences (all)	5 / 62 (8.06%) 5		
Gastroenteritis viral			

subjects affected / exposed	1 / 62 (1.61%)		
occurrences (all)	1		
Conjunctivitis			
subjects affected / exposed	4 / 62 (6.45%)		
occurrences (all)	4		
COVID-19			
subjects affected / exposed	10 / 62 (16.13%)		
occurrences (all)	11		
Influenza			
subjects affected / exposed	3 / 62 (4.84%)		
occurrences (all)	3		
Upper respiratory tract infection			
subjects affected / exposed	17 / 62 (27.42%)		
occurrences (all)	27		
Gastroenteritis			
subjects affected / exposed	2 / 62 (3.23%)		
occurrences (all)	2		
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	3 / 62 (4.84%)		
occurrences (all)	4		
Vitamin D deficiency			
subjects affected / exposed	2 / 62 (3.23%)		
occurrences (all)	2		
Dehydration			
subjects affected / exposed	0 / 62 (0.00%)		
occurrences (all)	0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
02 August 2021	Added a blinded crossover design.
30 August 2021	Updated/refined the exon language for inclusion criterion #2; and safety monitoring and adverse events of special interest language.
29 August 2022	Updated the randomization language.
28 May 2024	Added mITT as the analysis population. Updated safety information for delandistrogene moxeparvovec. Removed glutamate dehydrogenase (GLDH) from safety monitoring and reporting.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported