Protocol C3391011

LONG-TERM FOLLOW-UP SAFETY AND EFFICACY STUDY IN PARTICIPANTS WITH DUCHENNE MUSCULAR DYSTROPHY WHO HAVE RECEIVED FORDADISTROGENE MOVAPARVOVEC IN A PRECEDING CLINICAL STUDY

Statistical Analysis Plan
(SAP)

Version: <1>

Date: 28Mar2023

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1. VERSION HISTORY

Table 1. Summary of Changes

Version/ Date	Associated Protocol Amendment	Rationale	Specific Changes
1 28 Mar 2023	Original 28 Oct 2022	N/A	N/A

2. INTRODUCTION

This SAP provides the detailed methodology for summary and statistical analyses of the data collected in Study C3391011. This document may modify the plans outlined in the protocol; however, any major modifications of the primary endpoint definition or its analysis will also be reflected in a protocol amendment.

Statistical analyses will be performed using cleaned case report form (CRF) data as well as non-CRF data (i.e., blood samples data and banked biospecimens).

2.1. Modifications to the Analysis Plan Described in the Protocol

Not applicable.

2.2. Study Objectives and Endpoints

Type	Objective	Endpoint
Primary	**Primary**	**Primary**
Safety	Characterize the long-term safety of treatment with fordadistrogene movaparvovec	• Incidence and severity of: o SAEs o AEs considered related to previous treatment with fordadistrogene movaparvovec o Clinically significant abnormalities in cardiac troponin I, echocardiogram and ECG parameters • Incidence of malignancy
Secondary	**Secondary**	**Secondary**

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Efficacy	Characterize the long-term efficacy of treatment with fordadistrogene movaparvovec	• Change from pre-fordadistrogene movaparvovec dosing (from interventional study), or end of interventional study, or change from first collection in: o Ability to walk 10 m unassisted o Ability to climb stairs o PUL 2.0 entry score o NSAA total score (subset of participants) o Pulmonary function test parameters %pFVC and %pPEF o LVEF on echocardiography • Age (years) of participants when %pFVC is <30% • Age at loss of ambulation • Age at death and cause of death • Glucocorticoid use (dose and frequency)
Efficacy	Characterize the long-term health-related quality of life after treatment with fordadistrogene movaparvovec	• Change from pre-fordadistrogene movaparvovec dosing (from interventional study) or change from first collection in: o Modified PODCI o UL-PROM (non-ambulatory participants only)

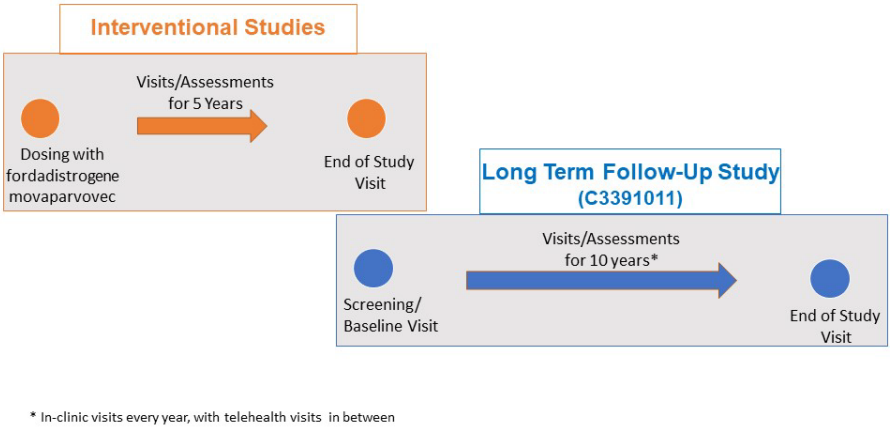
2.3. Study Design

This is a multi-center, international, low-interventional, long-term follow-up study that will enroll patients with DMD who have received fordadistrogene movaparvovec in any interventional Pfizer study. Approximately 250 patients will be invited to enter this study, and if enrolled will be followed for safety and efficacy for 10 years after the end of their interventional study. Post-dose follow-up in the interventional studies will be about 5 years; therefore, the fordadistrogene movaparvovec total follow-up period for patients who enter and complete this study will be 15 years.

Participants in this study will have safety & efficacy assessments performed at the investigative clinic on a yearly basis, and check-ins at least every 6 months via a telehealth visit.

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Figure 1. Study Schema



2.3.1 Reporting Periods for Analysis

The reporting period will include all visits, assessments, and AEs that occur on or after screening/baseline visit through end of study (Year 10).

Screening/baseline refers to screening/baseline visit for C3391011 study.

3. ENDPOINTS AND BASELINE VARIABLES: DEFINITIONS AND CONVENTIONS

3.1. Primary Endpoint(s)

- Incidence and severity of:
  - SAEs
  - AEs considered related to previous treatment with fordadistrogene movaparvovec
  - Clinically significant abnormalities in cardiac troponin I, echocardiogram and ECG parameters
- Incidence of malignancy (as collected on the CRF)

3.2. Secondary Endpoint(s)

- Change from pre-fordadistrogene movaparvovec dosing (from interventional study), from end of interventional study, or change from first collection in:
  - Ability to walk 10 m unassisted
  - Ability to climb stairs

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- PUL 2.0 entry score
- NSAA total score (subset of participants)
- Pulmonary function test parameters %pFVC and %pPEF
- LVEF on echocardiography

- Age (years) of participants when %pFVC is <30%
- Age at loss of ambulation

  Ambulatory is defined as participants who are able to walk 10 m unassisted. The age at loss of ambulation is defined as the age (years) of participants who are non-ambulatory, unable to walk 10 m unassisted.

- Age (years) at death and cause of death
- Glucocorticoid use (dose and frequency)
- Change from pre-fordadistrogene movaparvovec dosing (from interventional study) or change from first collection in:
  - Modified PODCI
  - UL-PROM (non-ambulatory participants only)

3.3. Other Endpoint(s)

Not applicable.

3.4. Baseline Variables

For demographic and baseline characteristic summaries, baseline is defined as the screening visit within this study. Where appropriate, select analyses (e.g. secondary endpoints) will consider change from pre-fordadistrogene movaparvovec from the prior interventional study.

The following baseline variables will be summarized:

- Demographic characteristics including age, race, and ethnicity, where age is calculated as age in years at the Screening visit.
- Physical measurements at baseline including height (cm), weight (kg) and BMI (kg/m^2)=weight (kg)/[height (m)]^2.

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3.5. Safety Endpoints

3.5.1. Adverse Events

MedDRA will be used to code all AEs (non-serious and serious) with respect to system organ class (SOC) and preferred term (PT). All AEs will be considered as treatment-emergent as follow-up within this study is post-fordadistrogene movaparvovec dosing.

If any findings on the physical or neurological examination are considered by the investigator to be 'clinically significant', the events are to be recorded as an AE, as appropriate.

3.5.2. Laboratory Data

The following safety laboratory tests (Table 2) will be collected. Analyses for banked biospecimens are out of scope for this SAP.

Table 2. Protocol-Required Safety Laboratory Assessments

Hematology	Chemistry	Urinalysis	Other
Hemoglobin	BUN and creatinine	pH	Cardiac troponin I
Hematocrit	Glucose (random)	Glucose (qual)	
RBC count and morphology	Calcium	Protein (qual)	
Platelet count	Sodium	Blood (qual)	
WBC count (and morphology as applicable)	Potassium	Ketones	
Neutrophils (Abs)	Chloride	Nitrites	
Eosinophils (Abs)	Total CO_2 (Bicarbonate)	Leukocyte esterase (qual)	
Monocytes (Abs)	Total, direct, and indirect bilirubin	Microscopy and culture[a]	
Basophils (Abs)	Alkaline phosphatase		
Lymphocytes (Abs)	Albumin		
	Creatine kinase		
	Total protein		
	Serum phosphorus		
	GGT		
	ALT, AST		
	Cystatin C		
	GLDH		

a) Only if urine dipstick is positive for blood, protein, nitrites, or leukocyte esterase.

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3.5.3. Other Safety Endpoints

- Electrocardiogram (ECG): a single 12-Lead ECG produces the following parameters, as assessed by a central reader: heart rate; PR, QT, and corrected QT intervals (ie, QTcF corrected using Fridericia's formula), and QRS complex duration.

- Echocardiogram: the parameters from the echocardiogram include LVEF: %

4. ANALYSIS SETS (POPULATIONS FOR ANALYSIS)

For purposes of analysis, the following analysis sets are defined:

Population	Description
Safety Analysis Set	All participants who signed the informed consent document and received a single dose of fordadistrogene movaparvovec in a previous interventional study.

All analyses will be performed on the Safety Analysis Set.

5. GENERAL METHODOLOGY AND CONVENTIONS

5.1. Hypotheses and Decision Rules

No hypotheses or decision rules are specified for this study and hence no inferential analyses will be performed.

5.2. General Methods

The unscheduled and early termination visits will be excluded from by visit analysis.

5.2.1. Analyses for Binary Endpoints

Descriptive statistics including the number of participants (N) and percentage (%) will be provided.

5.2.2. Analyses for Continuous Endpoints

Descriptive statistics including the number of participants (N), mean, standard deviation (SD), median, first quartile (Q1), third quartile (Q3), minimum, and maximum will be provided.

5.2.3. Analyses for Time-to event Endpoints

Time-to-event endpoints will be summarized using the Kaplan-Meier method and estimated survival curves will be displayed graphically when appropriate. Graphs will describe the number of participants at risk over time. The median, quartiles, and probabilities of an event at particular points in time will be estimated by the Kaplan-Meier method. Confidence intervals for medians and quartiles will be based on the Brookmeyer-Crowley method.

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Confidence intervals for the estimated probability of an event at a particular time point will be generated using the Greenwood formula.

5.3. Methods to Manage Missing Data

No imputation for missing data will be performed.

6. ANALYSES AND SUMMARIES

6.1. Primary Endpoint(s)

Endpoints:

- Incidence and severity of:
  - SAEs
  - AEs considered related to previous treatment with fordadistrogene movaparvovec
  - Clinically significant abnormalities in cardiac troponin I, echocardiogram and ECG parameters
- Incidence of malignancy

Analyses:

See Section 6.5 for details on the analyses of the above endpoints.

6.2. Secondary Efficacy Endpoint(s)

The analysis and population for all secondary endpoints are specified in the Appendix 1.

6.2.1. Functional assessments

Endpoint:

Change from pre-fordadistrogene movaparvovec dosing (from interventional study) in ability to walk 10 m unassisted.

Population:

Participants who are in Safety Analysis Set and are ambulatory at pre-fordadistrogene movaparvovec Analysis:

- Number (%) of non-ambulatory participants will be provided as described in Section 5.2.1 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total by visit.
- For each participant, time to non-ambulatory from pre-fordadistrogene movaparvovec dosing (from interventional study) will be analyzed by Kaplan-Meier method as described in Section 5.2.3. by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

Endpoint:

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Change from screening/baseline in ability to climb stairs.

Population:

Participants who are in Safety Analysis Set and are able to climb stairs at screening/baseline visit.

Analysis:

- Number (%) of participants who are unable to climb stairs will be provided as described in Section 5.2.1 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.
- For each participant, time to unable to climb stairs from screening/baseline will be analyzed by Kaplan-Meier method as described in Section 5.2.3 by age <6, ≥ 6 (years) at fordadistrogene movaparvovec and total.

6.2.2. NSAA total score

Endpoint:

Change from pre-fordadistrogene movaparvovec dosing in NSAA total score.

Population:

Participants who are from study C3391001 and are ambulatory at pre-fordadistrogene movaparvovec.

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec dosing (from Study C3391001) will be summarized descriptively as described in Section 5.2.2 by visit and by age <8, ≥ 8 (years) at pre-fordadistrogene movaparvovec and total.

6.2.3. PUL 2.0 and pulmonary test parameters

Endpoint:

Change from pre-fordadistrogene movaparvovec in PUL 2.0 entry score.

Population:

Participants who are in Safety Analysis Set and are from studies C3391001 and C3391002.

Analysis:

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Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit and by ambulatory status at pre-fordadistrogene movaparvovec and total.

Endpoint:

Change from screening/baseline in PUL 2.0 entry score.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Observed values and changes from screening/baseline will be summarized descriptively as described in Section 5.2.2 by visit and by ambulatory status at pre-fordadistrogene movaparvovec and total.

Endpoint:

Change from pre-fordadistrogene movaparvovec in pulmonary function test parameters %pFVC and %pPEF

Population:

Participants who are from studies C3391001, C3391002 and C3391003 and are >=6 years of age at pre-fordadistrogene movaparvovec

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit.

Endpoint:

Change from screening/baseline in pulmonary function test parameters %pFVC and %pPEF.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Observed values and changes from screening/baseline will be summarized descriptively as described in Section 5.2.2 by visit and by age.

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6.2.4. Echocardiography

Endpoint:

Change from pre-fordadistrogene movaparvovec in LVEF (%) on echocardiography.

Population:

Participants who are in Safety Analysis Set and had a non-missing LVEF (%) assessment at pre- fordadistrogene movaparvovec.

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

Endpoint:

Change from screening/baseline in LVEF (%) on echocardiography.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Observed values and changes from screening/baseline will be summarized descriptively as described in Section 5.2.2 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

6.2.5. Age (years) of participants when %FVC is <30%

Endpoint:

Age (years) of participants when %FVC is < 30%.

Population:

Participants who are from studies C3391001, C3391002 and C3391003 and were >=6 years of age (years) at pre-fordadistrogene movaparvovec.

Analysis:

Summary descriptive analysis for age (years) of participants when %FVC < 30% as described in Section 5.2.2 will be provided by visit.

Endpoint:

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Age (years) of participants when %FVC is < 30%.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Summary descriptive analysis for age (years) of participants when %FVC < 30% as described in Section 5.2.1 will be provided by visit and by age <6, ≥ 6 at pre-fordadistrogene movaparvovec and total.

6.2.6. Age (years) at loss of ambulation

Endpoint:

Age (years) at loss of ambulation.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Summary descriptive analysis for age (years) of loss ambulation as described in Section 5.2.1 will be provided by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

6.2.7. Age (years) of death and cause of death

Endpoint:

Age (years) of death.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Summary descriptive analysis for age (years) of death as described in Section 5.2.2 will be provided by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

Endpoint:

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Cause of death.

Population:

Participants who are in Safety Analysis Set.

Analysis:

The cause of death will be summarized and listed.

6.2.8. Glucocorticoid use

Endpoint:

Glucocorticoid use (dose and frequence)

Population:

Participants who are in Safety Analysis Set

Analysis:

Summary descriptive analysis as described in Section 5.2.2 in glucocorticoid dose and frequency will be provided by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

6.2.9. Modified PODCI and Clinician Global Impression of Severity-Ambulatory Motor Function (CGIS)

Endpoint:

Change from pre-fordadistrogene movaparvovec in Modified PODCI - Transfer and Basic Mobility Core Scale.

Population:

Participants who are in Safety Analysis Set and had a non-missing Modified PODCI: Transfer and Basic Mobility assessment at pre- fordadistrogene movaparvovec.

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit and 1) by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total and 2) by ambulatory status at pre-fordadistrogene movaparvovec.

Endpoint:

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Change from screening/baseline in Modified PODCI - Transfer and Basic Mobility Core Scale.

Population:

Participants who are in Safety Analysis Set.

Analysis:

Observed values and changes from screening/baseline will be summarized descriptively as described in Section 5.2.2 by visit and 1) by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total and 2) by ambulatory status at pre-fordadistrogene movaparvovec.

Endpoint:

Change from pre-fordadistrogene movaparvovec in Modified PODCI - Sports and Physical Functioning Core Scale.

Population:

Participants who are ambulatory and had a non-missing Modified PODCI: Sports and Physical Functioning assessment at pre- fordadistrogene movaparvovec.

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

Endpoint:

Change from screening/baseline in Modified PODCI - Sports and Physical Functioning Core Scale.

Population:

Participants who are in Safety Analysis Set and are ambulatory at screening/baseline.

Analysis:

Observed values and changes from screening/baseline will be summarized descriptively as described in Section 5.2.2 by visit and by age <6, ≥ 6 (years) at pre-fordadistrogene movaparvovec and total.

Endpoint:

CGIS.

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Population:

Participants who are from study C3391001 and are ambulatory at pre-fordadistrogene movaparvovec.

Analysis:

- Summary descriptive analysis as described in Section 5.2.2 will be provided by visit where NSAA assessed and by age <8, ≥ 8 (years) at pre-fordadistrogene movaparvovec and total.

6.2.10. UL-PROM

Endpoint:

Change from pre-fordadistrogene movaparvovec in UL-PROM (including UL-PROM-GC).

Population:

Participants who are in Safety Analysis Set and are non-ambulatory at pre-fordadistrogene movaparvovec.

Analysis:

Observed values and changes from pre-fordadistrogene movaparvovec will be summarized descriptively as described in Section 5.2.2 by visit.

Endpoint:

Change from the first assessment in this study in UL-PROM (including UL-PROM-GC).

Population:

Participants who are in Safety Analysis Set and have first assessment in UL-PROM in this study.

Analysis:

Observed values and changes from first assessment will be summarized descriptively as described in Section 5.2.2 by visit.

6.3. Subset Analyses

NA.

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6.4. Baseline and Other Summaries and Analyses

6.4.1. Baseline Summaries

1. Demographic characteristics will be summarized.
2. Baseline physical measurements (including height and weight) will be summarized.
3. The below will be summarized using number (%) of participants:
    - Dystrophin mutation type; and
    - Enrollment country.
4. General medical history will be summarized from interventional studies.
5. Prior medications will be summarized.
6. Ambulatory status.
7. NSAA total scores.
8. PUL 2.0 entry score.

6.4.2. Study Conduct and Participant Disposition

Participant disposition (eg, discontinuation from study, reason for discontinuation, completed study, and ongoing) will be summarized.

6.4.3. Concomitant Medications and Nondrug Treatments

The World Health Organization (WHO)-Drug coding dictionary will be used to classify concomitant medications.

The number (%) of participants who took each concomitant medication will be provided.

6.5. Safety Summaries and Analyses

6.5.1. Adverse Events

Incidence of TEAEs (treatment-related) in the reporting period will be summarized by SOC, PT, and severity.

Deaths and SAEs will be listed and incidence of SAEs (all causalities and treatment-related) will be summarized by SOC and PT.

6.5.2. Laboratory Data

Incidence of laboratory test abnormalities will be summarized without regard to pre-fordadistrogene movaparvovec abnormality, for normal pre-fordadistrogene movaparvovec , and for abnormal pre-fordadistrogene movaparvovec.

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6.5.3. Vital Signs

Observed values and changes from pre-fordadistrogene movaparvovec in vital sign parameters will be summarized descriptively by visit. If there are multiple records at any timepoint, the last one at each specific timepoint will be included in the analysis.

Maximum increases (only in blood pressure), and maximum decreases (only in blood pressure) from pre-fordadistrogene movaparvovec will be provided by visit.

6.5.4. Electrocardiograms

Changes from pre-fordadistrogene movaparvovec (QTcF, PR and QRS) in ECG parameters will be summarized descriptively by visit using categories as defined in Appendix 2.2 (for QTc these correspond to ICH E14). Numbers and percentages of participants meeting the categorical criteria will be provided. Additionally, the number (%) of participants with ECG findings of potential clinical concern (defined in protocol Appendix 7) will be summarized.

6.5.5. Neurological and Physical Examinations

No specific data summaries will be provided for neurological and physical examinations. Any findings considered by the investigator to be 'clinically significant' will be recorded as AEs and will be included in the AE summaries.

7. INTERIM ANALYSES

The data will be summarized throughout the course of the study.

8. REFERENCES

None.

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9. APPENDICES

Appendix 1. Summary of Efficacy Analyses

Endpoint	Population	Analysis	Strata analysis
Change in ability to walk 10 meters unassisted	Participants who are in Safety Analysis Set and are ambulatory at pre- fordadistrogene movaparvovec	• Numbers (%) of non-ambulatory participants by visit • Time to non-ambulatory	By age <6, >=6 (years) pre-fordadistrogene movaparvovec and total
Change in ability to climb stairs	Participants who are in Safety Analysis Set and are able to climb stairs at screening/baseline visit	• Numbers (%) of participants who are unable to climb stair by visit • Time to unable to climb stairs	By age <6, >=6 (years) pre-fordadistrogene movaparvovec and total
PUL 2.0 (entry score)	1) Participants who are in Safety Analysis Set and are from studies C3391001 and C3391002	1) Change from pre-fordadistrogene movaparvovec by visit	1) By ambulatory, non-ambulatory at pre-fordadistrogene movaparvovec and total
	2) Participants who are in Safety Analysis Set	2) Change from screening/baseline by visit	2) By ambulatory, non-ambulatory at screening/baseline and total
NSAA total score (1001)	Participants who are from study C3391001 and are ambulatory at pre-fordadistrogene movaparvovec	Change from pre-fordadistrogene movaparvovec by visit	By age <8, >=8 (years) at pre-fordadistrogene movaparvovec and total
CGIS (1001)	Participants who are from study C3391001 and are ambulatory at pre-fordadistrogene movaparvovec	Change from pre-fordadistrogene movaparvovec by visit	By age <8, >=8 (years) at pre-fordadistrogene movaparvovec and total
%FVC and %PEF	1) Participants who are from studies C3391001, C3391002 and C3391003 and are >=6 years of age at pre-fordadistrogene movaparvovec	1) Change from pre-fordadistrogene movaparvovec by visit	1) Total

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	2) Participants who are in Safety Analysis Set	2) change from screening/baseline by visit	2) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total
LVEF (echo)	1) Participants who are in Safety Analysis Set and had a non-missing LVEF (%) assessment at pre-fordadistrogene movaparvovec	1) Change from pre-fordadistrogene movaparvovec by visit	1) By age < 6 and >=6 at pre-fordadistrogene movaparvovec and total
	2) Participants who are in Safety Analysis Set	2) Change from screening/baseline by visit	2) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total
Age (years) of participants when %pFVC is <30%	1) Participants who are from studies C3391001, C3391002 and C3391003 and were >=6 years of age at pre-fordadistrogene movaparvovec	Descriptive summary statistics	1) Total
	2) Participants who are in Safety Analysis Set		2) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total
Age at loss of ambulation	Participants who are in Safety Analysis Set	Descriptive summary statistics	By age <6, >=6 (years) pre-fordadistrogene movaparvovec and total
Age at death	Participants who are in Safety Analysis Set	Descriptive summary statistics	By age <6, >=6 (years) pre-fordadistrogene movaparvovec and total
GC use (dose and frequency)	Participants who are in Safety Analysis Set	Descriptive summary statistics by visit	By age <6, >=6 (years) pre-fordadistrogene movaparvovec and total

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Modified PODCI: Transfer and Basic Mobility	1) Participants who are in Safety Analysis Set and had a non-missing Modified PODCI: Transfer and Basic Mobility assessment at pre-fordadistrogene movaparvovec	1) Change from pre-fordadistrogene movaparvovec by visit	1.1) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total 1.2) Ambulatory status at pre-fordadistrogene movaparvovec
	2) Participants who are in Safety Analysis Set	2) Change from screening/baseline by visit	2.1) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total 2.2) Ambulatory status at pre-fordadistrogene movaparvovec
Modified PODCI: Sport and Physical Function	1) Participants who are ambulatory and had a non-missing Modified PODCI: Transfer and Basic Mobility assessment at pre-fordadistrogene movaparvovec	1) Change from pre-fordadistrogene movaparvovec by visit	1) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total
	2) Participants who are in Safety Analysis Set and are ambulatory at screening/baseline	2) Change from screening/baseline by visit	2) By age < 6 and >=6 (years) at pre-fordadistrogene movaparvovec and total
UL-PROM (including UL-PROM CG)	1) Participants who are in Safety Analysis Set and are non-ambulatory at pre-fordadistrogene movaparvovec	1) Change from pre-fordadistrogene movaparvovec by visit	Total
	2) Participants who are in Safety Analysis Set and have first assessment in UL-PROM in this study	2) Change from first assessment by visit	

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Appendix 2. Data Derivation Details

Appendix 2.1. Definition and Use of Visit Windows in Reporting

The below analysis windows will be used for all secondary efficacy endpoints. If multiple values are observed within a window, the value closest (and prior) to the target day will be included in the analysis.

Visit label	Window lower limit[1]	Window upper limit[1]
Year 1	2	365 + 182
Year 2	365+183	2*365 + 182
Year 3	2*365 + 183	3*365 + 182
Year 4	3*365 + 183	4*365 + 182
Year 5	4*365 + 183	5*365 + 182
Year 6	5*365 + 183	6*365 + 182
Year 7	6*365 + 183	7*365 + 182
Year 8	7*365 + 183	8*365 + 182
Year 9	8*365 + 183	9*365 + 182
Year 10	9*365 + 183	No upper limit
1: Days related to Screening/Baseline		

Appendix 2.2. Categorical Classes for ECG of Potential Clinical Concern

Categories for QTcF

	Borderline	Prolonged	
QTcF (ms)	450≤ max. <480	480≤ max.<500	max. ≥500
QTcF (ms) increase from baseline	30≤ max. <60	max. ≥60	

Categories for PR and QRS

PR (ms)	max. ≥300	
PR (ms) increase from baseline	Baseline >200 and max. ≥25% increase	Baseline ≤200 and max. ≥50% increase
QRS (ms)	max. ≥140	

Appendix 3. Data Set Descriptions

None.

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Appendix 4. Statistical Methodology Details

None.

Appendix 5. List of Abbreviations

Abbreviation	Term
AE	adverse event
ALT	alanine aminotransferase
AST	aspartate aminotransferase
BLQ	below the limit of quantitation
BMI	body mass index
BP	blood pressure
BUN	blood urea nitrogen
CDARS	Clinical Data Analysis and Reporting System (of US Food and Drug Administration)
CRF	case report form
CSR	clinical study report
DMD	Duchenne muscular dystrophy
ECG	electrocardiogram
GGT	gamma glutamyl transferase
GLDH	glutamate dehydrogenase
LLoQ	lower limit of quantitation
LVEF	left ventricular ejection fraction
MedDRA	Medical Dictionary for Regulatory Activities
N/A	not applicable
NSAA	North Star Ambulatory Assessment
%pFVC	percent predicted forced vital capacity
%pPEF	percent predicted preserved ejection fraction
PODCI	pediatric Outcomes Data Collection Instrument
PR	pulse rate
PT	preferred term
Q1	first quartile
Q3	third quartile
QRS	Q wave, R wave and S wave
QTc	corrected QT
QTcF	corrected QT (Fridericia method)
RBC	red blood cell
SAE	serious adverse event
SAP	statistical analysis plan
SD	standard deviation
SOC	system organ class
SOP	standard operating procedure

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Abbreviation	Term
TEAE	treatment-emergent adverse event
UL-PROM	upper limb patient reported outcome measure
ULN	upper limit of normal
WBC	white blood cell
WHO	world health organization

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