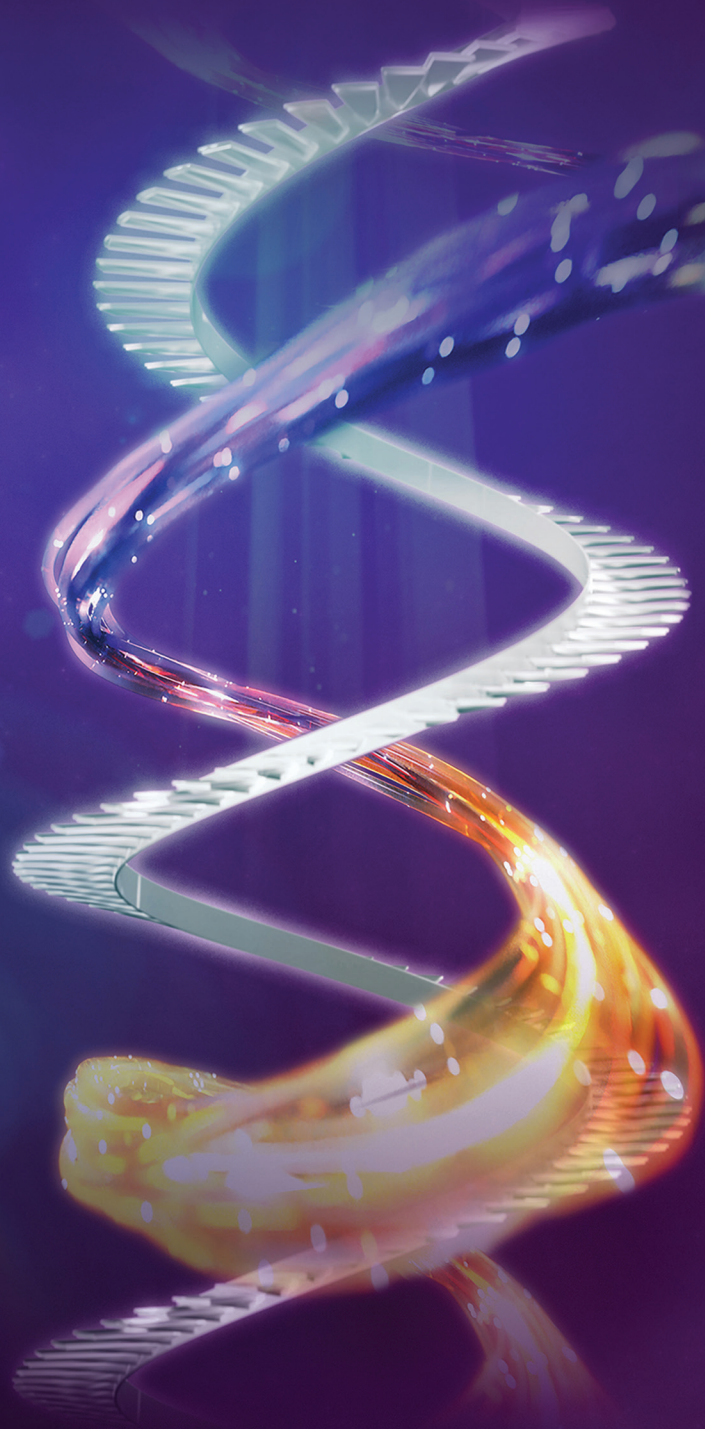
SAREPTA THERAPEUTICS

Clinical Programs

May 2026



ONGOING SAREPTA CLINICAL DEVELOPMENT PROGRAMS

	TREATMENT (MOA)	PHASE 1/2	PHASE 3	PHASE 4	RWE
DUCHENNE MUSCULAR DYSTROPHY (DMD)	**ETEPLIRSEN** (EXON 51 SKIPPING)		**MIS51ON:** Randomized, double-blind, dose-finding comparison study[1] N=160 (actual)/Age: 4-13 years Active, not recruiting	**EVOLVE:** Post-marketing observational study[2] N=~300 (target)/Age: All ages Enrolling by invitation	
	GOLODIRSEN (EXON 53 SKIPPING)				
	CASIMERSEN (EXON 45 SKIPPING)				
	DELANDISTROGENE MOXEPARVOVEC-ROKL[a] SRP-9001 (GENE THERAPY)	**ENDEAVOR:** Open-label, single-dose study[3] N=~83 (target)/Age: ≥2 years (ages vary by cohort) Recruiting	**ENVISION:** Randomized, double blind, placebo-controlled study[4] N=~148 (target)/Ages vary by cohort Active, not recruiting **EXPEDITION:** Long-term follow-up study[5] N=~400 (target)/All ages Enrolling by invitation	**ENDURE:** Long-term, multicenter, prospective, observational study[6] N=~500 (target)/Age: ≥4 years Enrolling by invitation **ENHANCE:** Post-marketing, open-label, single-dose study[7] N=~20 (target)/Age: ≥4 years Not yet recruiting	
LIMB-GIRDLE MUSCULAR DYSTROPHIES (LGMDs)	**SRP-9003** (GENE THERAPY)	**VOYAGENE:** Open-label, single-dose study[8] N=6 (actual)/ Age: 4-50 years Active, not recruiting	**EMERGENE:** Open-label, single-dose study[9] N=17 (actual)/Age: ≥4 years Active, not recruiting		
	OTHER				**JOURNEY:** A longitudinal natural history study[10] N=205 (actual)/Age: ≥4 years Active, not recruiting

Eteplirsen is not approved in any other country outside the United States of America, Kuwait, Israel, Georgia, and Libya. Golodirsen is not approved in any other country outside the United States of America, Kuwait, Georgia, and Libya. Casimersen is not approved in any other country outside the United States of America, Kuwait, Georgia, and Libya. Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan. SRP-9003 is investigational only and not approved by any regulatory authority in the world.

[a]Accompanying correspondence uses the generic biological term "delandistrogene moxeparvovec" throughout this document. The FDA applies a "distinguishing suffix" naming convention for original and reference biological products in compliance with Section 351(a) of the Public Health Service Act. Delandistrogene moxeparvovec is identified in the FDA-approved Prescribing Information as delandistrogene moxeparvovec-rokl. [b]The intent-to-treat population for study analysis includes 125 patients.[11]

FDA, US Food and Drug Administration; MOA, mechanism of action; RWE, real-world evidence.

1. ClinicalTrials.gov Identifier: NCT03992430. Updated February 27, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT03992430 2. ClinicalTrials.gov Identifier: NCT06606340. Updated September 10, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06606340 3. ClinicalTrials.gov Identifier: NCT04626674. Updated April 6, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT04626674 4. ClinicalTrials.gov Identifier: NCT05881408. Updated June 22, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05881408 5. ClinicalTrials.gov Identifier: NCT05967351. Updated February 6, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05967351 6. ClinicalTrials.gov Identifier: NCT06270719. Updated December 2, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06270719 7. ClinicalTrials.gov Identifier: NCT07542314. Updated April 21, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT07542314 8. ClinicalTrials.gov Identifier: NCT05876780. Updated February 19, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05876780 9. ClinicalTrials.gov Identifier: NCT06246513. Updated March 2, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06246513 10. ClinicalTrials.gov Identifier: NCT04475926. Updated November 6, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT04475926 11. Sarepta Therapeutics. Data on file.

Information updated as of April 23, 2026.

MIS51ON (Study 402): A randomized, double-blind, dose-finding and comparison study of the safety and efficacy of high doses of eteplirsen, preceded by an open-label dose escalation, in individuals with DMD with deletion mutations amenable to exon 51 skipping[1]

FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT03992430

EudraCT number: 2018-001762-42

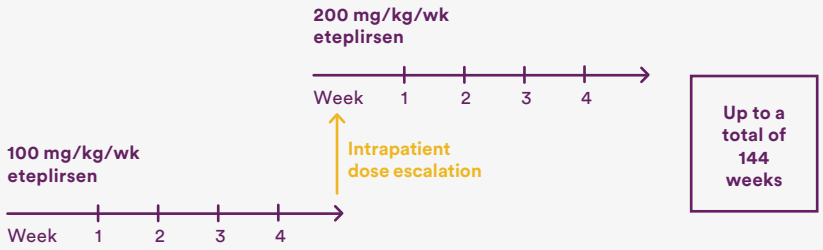
Other study ID number: SRP-4658-402

Study start date: July 2020

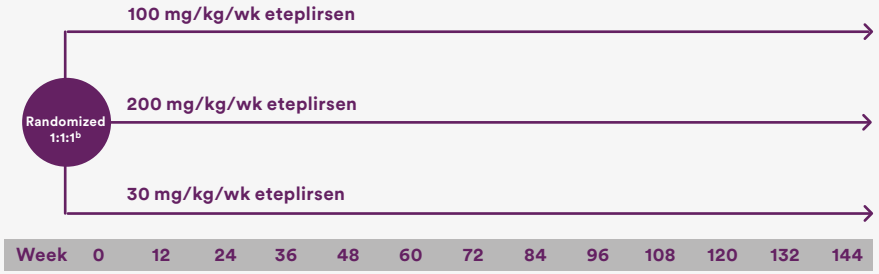
STUDY DESIGN[1,2]

N=160 (actual)

PART 1: Open-label, dose-escalation[a]



PART 2: Double-blind, dose-finding, and comparison



OUTCOME MEASURES[1]

PRIMARY

PART 1

- **Incidences of AEs** (up to week 148)

PART 2

- **Final analysis: Change from baseline in NSAA Total Score** (week 144)
- **Conditional Efficacy Interim Analysis: Change from baseline in NSAA Total Score** (week 72 or week 96)

SECONDARY

PART 2

- **Change from baseline in TTR, 10MWR, 6MWT, 4SC, FVC%p** (week 144)
- **Time to LOA (baseline** up to week 144)
- **Change from baseline in skeletal muscle dystrophin expression** (weeks 24, 48, or 144)
- **Incidence of AEs (baseline** up to week 148)
- **PK parameters** (predose to 2 hours postdose up to week 144)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[c]

- Male with an out-of-frame deletion mutation of the *DMD* gene amenable to exon 51 skipping
- Aged 4-13 years
- Ambulatory, able to perform TTR ≤10 seconds
- Able to walk independently without assistive devices
- Have intact right and left biceps muscles or an alternative upper arm muscle group
- On a stable dose or dose equivalent of oral corticosteroids for at least 12 weeks prior to randomization
- If ≥7 years, FVC%p ≥50% and no requirement for nocturnal ventilation; if 4-6 years, no support from ventilator or non-invasive ventilation at screening

KEY EXCLUSION CRITERIA[c]

- Use of any pharmacologic treatment (other than corticosteroids) within 12 weeks prior to randomization[d]
- Current or previous treatment with experimental pharmacologic treatment for DMD or any prior antisense oligonucleotide, gene therapy, or gene editing[e]
- Major surgery within 3 months prior to randomization
- Presence of any other significant neuromuscular or genetic disease other than DMD
- Presence of any known impairment of renal function and/or other clinically significant illness
- Evidence of cardiomyopathy[f]

Eteplirsen is not approved in any other country outside the United States of America, Kuwait, Israel, Georgia, and Libya.
[a]If eteplirsen IV weekly at a dose of 100 mg/kg is deemed safe by the SRC. A minimum of 2 SRCs are planned at each dose level.[2] [b]On a stable dose or dose equivalent of oral corticosteroids for at least 12 weeks prior to randomization. [c]Other criteria apply.[1] [d]Eligible individuals previously treated with, or currently using eteplirsen are not excluded from enrollment.[1] [e]Some exceptions apply.[1] [f]Left ventricular ejection fraction <50% based on the screening echocardiogram or QTcF ≥450 ms based on the screening electrocardiogram.[1]
4SC, time to ascend 4 stairs; 6MWT, 6-minute walk test; 10MWR, 10-meter walk/run; AE, adverse event; DMD, Duchenne muscular dystrophy; *DMD*, the gene that encodes dystrophin; FVC%p, percentage predicted forced vital capacity; IV, intravenous; kg, kilogram; LOA, loss of ambulation; mg, milligram; ms, millisecond; NSAA, North Star Ambulatory Assessment; PK, pharmacokinetics; QTcF, QT interval Fridericia's correction formula; SRC, safety review committee; TTR, time to rise; wk, week.
1. ClinicalTrials.gov Identifier: NCT03992430. Updated February 27, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT03992430
2. Sarepta Therapeutics. Data on file.
Information updated as of April 23, 2026.

EVOLVE: Prospective, observational study, evaluating the long-term effect of eteplirsen, golodirsen, and casimersen in individuals with DMD amenable to exon 51, 53, and 45 skipping, respectively, in routine clinical practice[1,2]

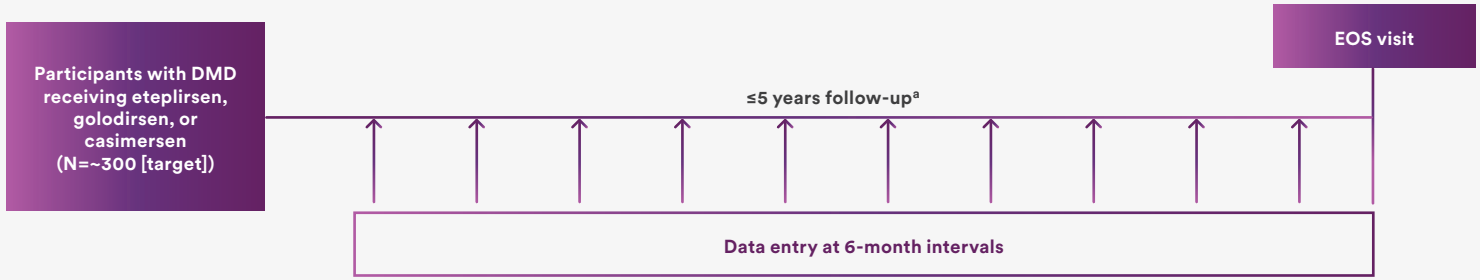
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT06606340

Other study ID number: SRP-4658-403

Study start date: January 2019

STUDY DESIGN[1,2]



OUTCOME MEASURES[1,2]

- **Safety**
- **LOA** (up to 5 years)
- **TTR** (up to 5 years)
- **10MWR** (up to 5 years)
- **PUL 2.0** (up to 5 years)
- **Pulmonary function as measured by FVC%p** (up to 5 years)
- **Cardiac function, as assessed by LVEF measured through ECHO** (up to 5 years)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[b]

- Male (child, adult, or older adult) with an established clinical diagnosis of DMD as documented prior to screening by a genetic report
- Willing to provide informed consent to comply with the study data collection procedures
- Receiving or initiating treatment with eteplirsen, golodirsen, or casimersen at the time of enrollment. Note: Participants with a prescription for eteplirsen, golodirsen, or casimersen at enrollment must initiate the exon-skipping therapy within 6 months of the date of enrollment or will no longer be eligible for this study. Note: Enrollment of eteplirsen participants has been completed, no additional participants will be enrolled

KEY EXCLUSION CRITERIA[b]

- Participating in any DMD interventional study at the time of observational study enrollment
- Declining to provide consent for collection of their genetic data
- Has a medical condition or confounding circumstances that, in the opinion of the Investigator, might compromise the ability of the participant to comply with the protocol-required procedures, the participant's wellbeing or safety, and/or the clinical interpretability of the data collected from the participant

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The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Enrolled individuals will be followed for the duration of the study, or until individual withdrawal of consent or death, whichever occurs first.
[b]Other criteria apply.
10MWR, 10-meter walk/run; DMD, Duchenne muscular dystrophy; ECHO, echocardiogram; EOS, end of study; FVC%p, percentage predicted forced vital capacity; LOA, loss of ambulation; LVEF, left ventricular ejection fraction; PUL, performance of upper limb; TTR, time to rise.
1. ClinicalTrials.gov Identifier: NCT06606340. Updated September 10, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06606340 2. Tian C, et al. *J Comp Eff Res.* 2026;10:e250108
Information updated as of April 23, 2026.

ENDEAVOR: An open-label study using intended commercially representative material to evaluate the safety of and expression from delandistrogene moxeparvovec (SRP-9001) in individuals with DMD[1]

FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT04626674

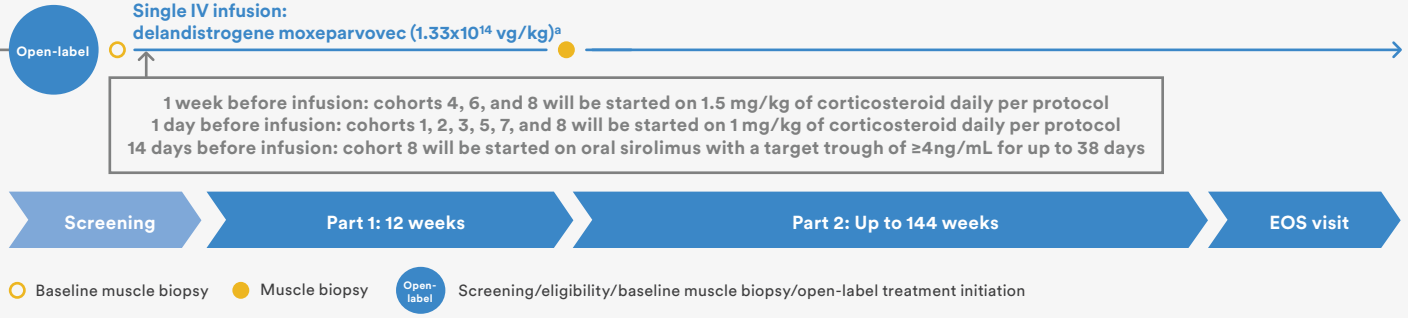
Other study ID number: SRP-9001-103

Study start date: November 2020

STUDY DESIGN[1-3]

Eight cohorts (N=~83 [target]):

- **1:** Male ambulatory individuals with DMD ≥4 to <8 years of age
- **2:** Male ambulatory individuals with DMD ≥8 to <18 years of age
- **3, 5b, and 7:** Male non-ambulatory individuals with DMD
- **4:** Male ambulatory individuals with DMD ≥3 to <4 years of age
- **5a:** Male ambulatory individuals with DMD ≥4 to <9 years of age with TTR from floor ≤7 seconds at screening
- **6:** Male ambulatory individuals with DMD ≥2 to <3 years of age
- **8:** Male non-ambulatory individuals with DMD with a PUL entry item score ≥3 and a total PUL score of ≥20 and ≤40 at screening



OUTCOME MEASURES[1]

PRIMARY

PART 1

- **Cohorts 1 to 5: Change from baseline in quantity of delandistrogene moxeparvovec dystrophin expression as measured by western blot** (week 12)
- **Cohorts 6 to 8: Quantity of delandistrogene moxeparvovec dystrophin expression as measured by western blot** (week 12)
- **Cohort 8: Number of participants with ALI** (baseline up to week 72)

SECONDARY

- **Number of participants with TEAEs, SAEs, and AESIs** (baseline up to week 156)
- **Change from baseline in delandistrogene moxeparvovec dystrophin protein expression as measured by IF fiber intensity and PDPF** (week 12)

SECONDARY (continued)

- **Number of participants with TEAEs, SAEs, and AESIs** (baseline up to week 156)
- **Change from baseline in delandistrogene moxeparvovec dystrophin protein expression as measured by IF fiber intensity and PDPF** (week 12)
- **Immunogenicity of delandistrogene moxeparvovec as determined by level of antibody titers to rAAVrh74** (day 2 up to week 156)
- **Vector shedding as measured in urine, saliva, and stool samples post-infusion** (day 1 up to week 104)
- **Quantity of delandistrogene moxeparvovec dystrophin protein expression as measured by IF fiber intensity and PDPF** (week 12)

Cohort 8

- **Number of participants with infection, edema, wound-healing complications, hyperlipidemia, angioedema, and intestinal lung disease/non-infectious pneumonitis** (baseline up to week 72)
- **Number of participants with hepatic AEs, hepatic biomarkers, and lab assessments indicative of either acute hepatocellular injury or acute liver dysfunction** (baseline up to week 72)
- **Number of participants with severe ALI** (baseline up to week 72)
- **Duration of steroid administered** (baseline up to week 72)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[b]

- **Cohort 1: Ambulatory, ≥4 to <8 years of age**
- **Cohort 2: Ambulatory, ≥8 to <18 years of age**
- **Cohort 3: Non-ambulatory per protocol-specified criteria**
- **Cohort 4: Ambulatory, ≥3 to <4 years of age, do not require chronic steroids for treatment of DMD**
- **Cohort 5a: Ambulatory, ≥4 to <9 years of age with TTR from floor ≤7 seconds at screening**
- **Cohort 5b: Non-ambulatory per protocol-specified criteria**
- **Cohort 6: Ambulatory, ≥2 to <3 years of age, do not require chronic steroids for treatment of DMD**
- **Cohort 7: Non-ambulatory per protocol-specified criteria**
- **Cohort 8: Non-ambulatory per protocol-specified criteria, has a PUL entry item score ≥3 and a total PUL score of ≥20 and ≤40 at screening**
- **Definitive diagnosis of DMD based on documented clinical findings and prior genetic testing**
- **Stable dose equivalent of oral glucocorticoids for at least 12 weeks for cohorts 1-3, 5, 7, and 8 only**

KEY EXCLUSION CRITERIA[b]

- **Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression within protocol-specified time limits**
- **Abnormality in protocol-specified diagnostic evaluations or laboratory tests**
- **Has a concomitant illness, autoimmune disease, chronic drug treatment, and/or cognitive delay/impairment that in the opinion of the Investigator creates unnecessary risks for gene transfer**
- **Cohort 8: Any confounding factors or known hypersensitivity that would prevent the use of oral sirolimus**

Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]1.33×10^{14} vg/kg (linear qPCR). 2×10^{14} vg/kg supercoiled qPCR equivalent.[2] [b]Other criteria apply.[1]
AE, adverse event; AESI, adverse event of special interest; ALI, acute liver injury; DMD, Duchenne muscular dystrophy; EOS, end of study; IF, immunofluorescence; IV, intravenous; kg, kilogram; mg, milligram; mL, milliliter; ng, nanogram; PDPF, percent dystrophin positive fibers; PUL, performance upper limb; qPCR, quantitative polymerase chain reaction; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; SAE, serious adverse event; TEAE, treatment-emergent adverse event; TTR, time to rise; vg, vector genome.

1. ClinicalTrials.gov Identifier: NCT04626674. Updated April 6, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT04626674
2. Zaidman CM, et al. *Ann Neurol.* 2023;94(5):955-968. 3. Sarepta Therapeutics. Data on file.

Information updated as of April 23, 2026.

ENVISION: A Phase 3, multinational, randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in non-ambulatory and ambulatory individuals with DMD[1]

FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

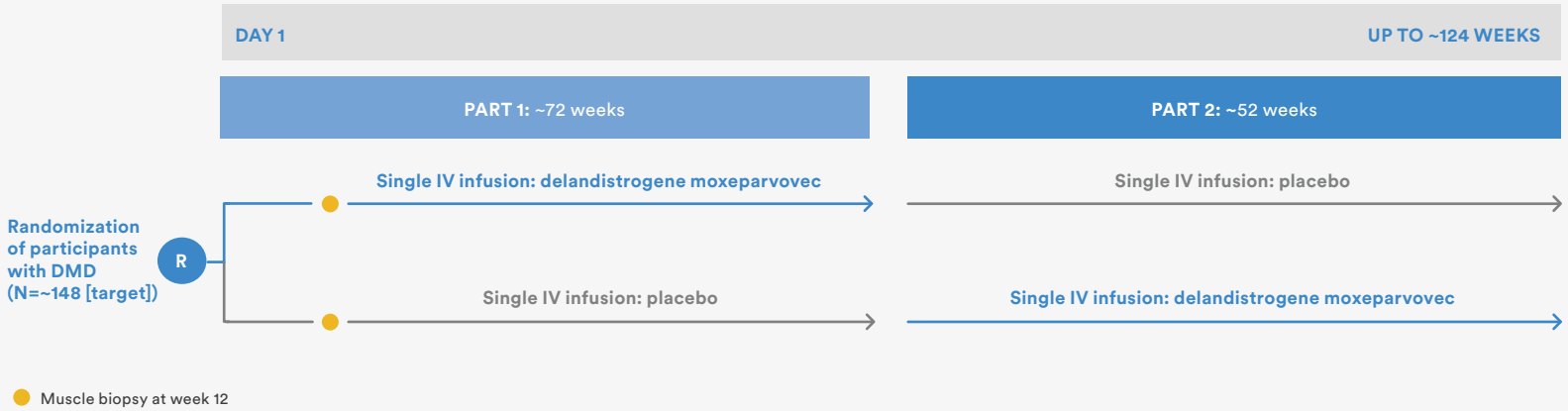
ClinicalTrials.gov identifier: NCT05881408

EudraCT number: 2020-002372-13

Other study ID number: SRP-9001-303

Study start date: May 2023

STUDY DESIGN[1,2]



OUTCOME MEASURES[1]

PRIMARY

PART 1

- **Change from baseline in PUL Version 2.0** (week 72)

SECONDARY

- **Number of participants with TEAEs, AESIs, and SAEs** (baseline up to week 124)

PART 1 ONLY

- **Change from baseline in FVC%p and PEF%p** (week 72)
- **Quantity of delandistrogene moxeparvovec dystrophin expression by western blot** (week 12)
- **Change from baseline PROMIS score in upper extremity function** (week 72)
- **Change from baseline in global circumferential strain as measured by cardiac MRI** (week 72)
- **Change from baseline in the Middle Domain Score of PUL Version 2.0** (week 72)
- **Cohort 2 only: Change from baseline NSAA total score** (week 72)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[a]

- **Definitive diagnosis of DMD based on documented clinical findings and prior genetic testing**
- **Cohort 1 only: Non-ambulatory per protocol-specified criteria**
- **Cohort 2 only: Ambulatory per protocol-specified criteria, and ≥8 to <18 years of age**
- **Ability to cooperate with motor assessment testing**
- **Stable daily dose of oral corticosteroids for at least 12 weeks prior to screening**
- **rAAVrh74 antibody titers not elevated as per protocol-specified requirements**
- **A pathogenic frameshift mutation or premature stop codon in the *DMD* gene, except for any deletion mutations in exon 8 and/or 9**

KEY EXCLUSION CRITERIA[a]

- **Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression within protocol-specified time limits**
- **Abnormality in protocol-specified diagnostic evaluations or laboratory tests**
- **Presence of any other clinically significant illness, medical condition, or requirement for chronic drug treatment that in the opinion of the Investigator creates unnecessary risk for gene transfer**

Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Other criteria may apply.[1]
AESI, adverse event of special interest; DMD, Duchenne muscular dystrophy; *DMD*, the gene that encodes dystrophin; FVC%p, percent predicted forced vital capacity; IV, intravenous; MRI, magnetic resonance imaging; NSAA, North Star Ambulatory Assessment; PEF%p, percent predicted peak expiratory flow; PROMIS, Patient-Reported Outcomes Measurement Information System; PUL, performance of upper limb; R, randomization; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; SAE, serious adverse event; TEAE, treatment-emergent adverse event.
1. ClinicalTrials.gov Identifier: NCT05881408. Updated June 22, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05881408 2. Muntoni F, et al. Poster presented at: 28th Annual International Congress of the World Muscle Society (WMS); October 3-7, 2023; Charleston, SC, USA. Poster 47.
Information updated as of April 23, 2026.

EXPEDITION: A Phase 3, multinational, long-term follow-up study to evaluate safety and efficacy in individuals who have previously received delandistrogene moxeparvovec in a clinical study[1]

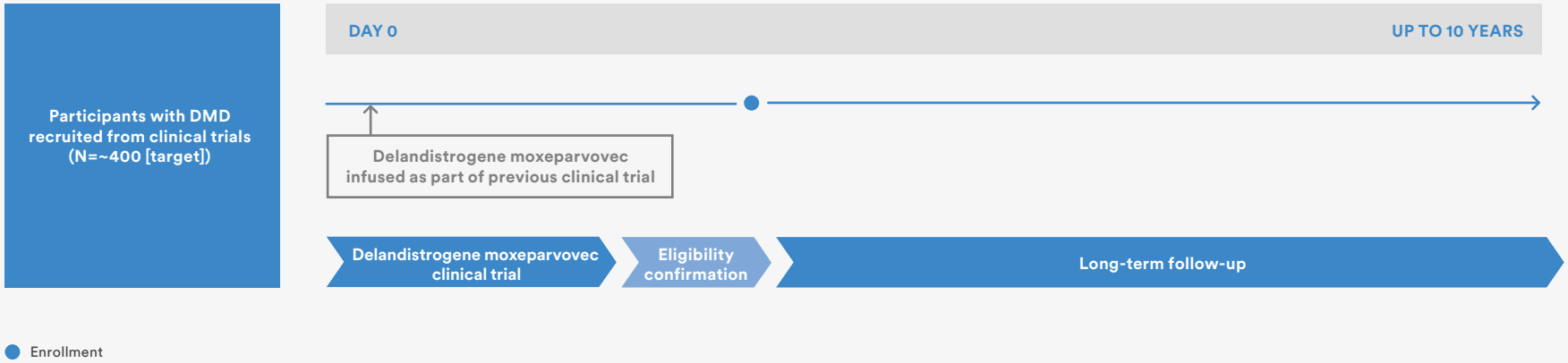
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT05967351

Other study ID number: SRP-9001-305

Study start date: September 2023

STUDY DESIGN[1,2]



OUTCOME MEASURES[1]

PRIMARY

- **Number of participants with a TEAE, SAE, and AESI** (up to 10 years after dosing)

SECONDARY

- **Change from pre-infusion baseline in NSAA total score, TTR, 10MWR, PUL Version 2.0 total score, and PUL Version 2.0 domain-specific scores** (up to 10 years)
- **Change from pre-infusion baseline in FVC%p and PEF%p** (up to 10 years)
- **Change from pre-infusion baseline in cardiac MRI and musculoskeletal MRI** (up to 10 years)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[a]

- **Received delandistrogene moxeparvovec for DMD in a previous clinical study**
- **Has (a) parent(s) or legal caregiver(s) or is ≥18 years of age and able to understand and comply with the study visit schedule and all other protocol requirements**

KEY EXCLUSION CRITERIA[a]

- **Not applicable**

Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Other criteria may apply.[1]
10MWR, 10-meter walk/run; AESI, adverse event of special interest; DMD, Duchenne muscular dystrophy; FVC%p, percent predicted forced vital capacity; MRI, magnetic resonance imaging; NSAA, North Star Ambulatory Assessment; PEF%p, percent predicted peak expiratory flow; PUL, performance of upper limb; SAE, serious adverse event; TEAE, treatment-emergent adverse event; TTR, time to rise.
1. ClinicalTrials.gov Identifier: NCT05967351. Updated February 6, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05967351 2. Sarepta Therapeutics. Data on file.
Information updated as of April 23,2026.

ENDURE: A long-term multicenter prospective observational study evaluating the comparative effectiveness and safety of Sarepta gene transfer therapy vs standard of care in patients with DMD under conditions of routine clinical practice[1]

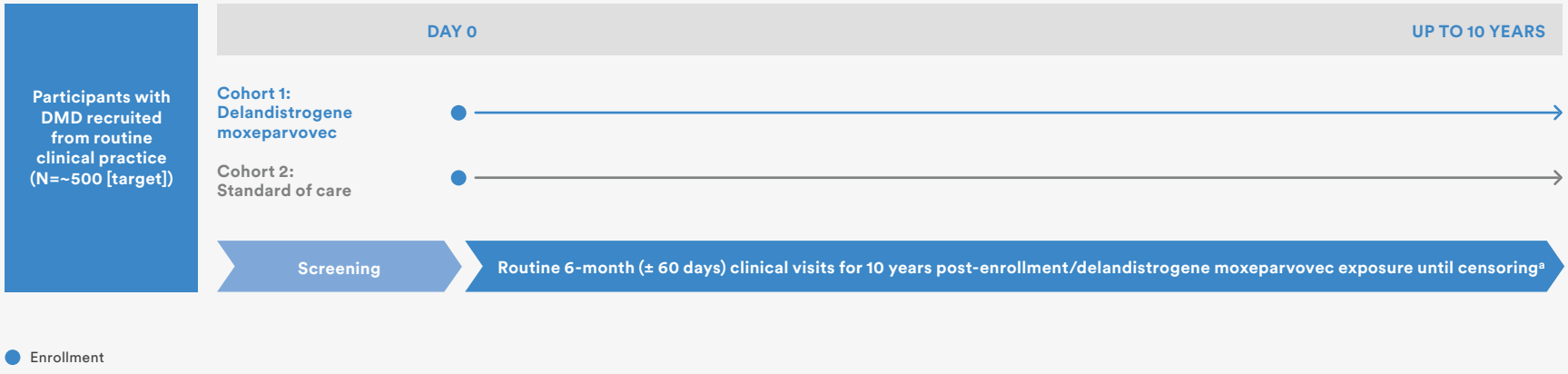
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT06270719

Other study ID number: SRP-9001-401

Study start date: February 2024

STUDY DESIGN[1,2]



OUTCOME MEASURES[1]

PRIMARY

- **Mean change from baseline in 10MWR** (baseline, month 12)

SECONDARY

- **Time to rise from floor, time to LOA, and PUL 2.0 entry item score** (up to 10 years)
- **PROMIS mobility, upper extremity, and fatigue** (up to 10 years)
- **Pulmonary function, as measured by FVC%p** (up to 10 years)
- **Echocardiogram, including assessment of LVEF** (up to 10 years)
- **Number of participants with a TEAE** (up to 10 years)
- **Time to 10MWR velocity** (up to 10 years)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[b]

- **Male and ≥4 years with a definitive diagnosis of DMD prior to screening based on documentation of clinical findings and confirmatory genetic testing, and is currently receiving or has been prescribed to start chronic glucocorticoid therapy at the time of enrollment**
- **Cohorts 1a or 2 only: Ambulatory per protocol-specified criteria**
- **Cohort 1b only: Non-ambulatory per protocol-specified criteria**
- **For delandistrogene moxeparvovec participants: will initiate usual-care treatment with delandistrogene moxeparvovec at time of enrollment**
- **Comparator: unexposed to *DMD* gene therapy at time of enrollment**

KEY EXCLUSION CRITERIA[b]

- **Has any deletion of exon 8 and/or 9 in the *DMD* gene**
- **Is currently participating in any DMD interventional study at the time of study enrollment**
- **Has a medical condition or confounding circumstance (for example, prior traumatic limitation for mobility or significant behavioral comorbidity) that, in the opinion of the Investigator, might compromise the participant's ability to comply with the protocol-required procedures, well-being or safety, and/or the clinical interpretability of the data collected from the participant**

Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Censoring is earliest of: exposure to another DMD gene therapy, new participation in DMD clinical trial (not applicable for post-trial participants), death, disenrollment, loss to follow-up, 3,681 days of follow-up from baseline or dosing with delandistrogene moxeparvovec (10 years plus 28-day visit window), or the end of the study period.[2] [b]Other criteria may apply.[1]
10MWR, 10-meter walk/run; DMD, Duchenne muscular dystrophy; *DMD*, the gene that encodes dystrophin; FVC%p, percent predicted forced vital capacity; LOA, loss of ambulation; LVEF, left ventricular ejection fraction; PROMIS, Patient-Reported Outcomes Measurement Information System; PUL, performance of upper limb; TEAE, treatment-emergent adverse event.
1. ClinicalTrials.gov Identifier: NCT06270719. Updated December 2, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06270719 2. Ricchetti-Masterson KL, et al. Poster presented at Muscular Dystrophy Association 2025 Conference. March 16-19, 2025; Dallas, TX, USA. Poster 91.
Information updated as of April 23, 2026.

ENHANCE: Phase 4 study to evaluate the safety and effectiveness of delandistrogene moxeparvovec (SRP-9001) in patients with Duchenne Muscular Dystrophy treated in a post-marketing setting

FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT07542314

Other study ID number: SRP-9001-402

Study start date: April 2026 (estimated)

STUDY DESIGN

Two cohorts (N=~20 [target])

Cohort 1: Male at birth, ambulatory, ≥4 years of age at the time of dosing, and eligible for commercial SRP-9001

Cohort 2: Male at birth and previously received SRP-9001 in the commercial setting after pre-treatment with sirolimus and corticosteroids

Cohort 1 will receive SRP-9001 in a commercial setting on Day 1. They will also receive sirolimus, glucocorticoids, and antibiotics orally

Cohort 2 will receive no intervention and will participate in one study visit during which a muscle biopsy will be performed

→ **Cohort 1 will be followed until week 24 after treatment**

OUTCOME MEASURES[a]

PRIMARY

- Number of Patients with ALI (week 12)

SECONDARY

- Number of patients with TEAEs, AESIs, and SAEs (day 1 up to week 24)
- Number of patients with infections, edema, wound-healing complications, hyperlipidemia, angioedema, and interstitial lung disease/non-infectious pneumonitis (day 1 up to week 24)
- Number of patients with hepatic AEs, hepatic biomarkers, and laboratory assessments indicative of either acute hepatocellular injury or acute liver dysfunction (day 1 up to week 24)
- Number of patients with severe ALI (day 1 up to week 24)
- Number of patients with ALI (day 1 up to week 24)
- Duration of ALI (day 1 up to week 24)
- Amount of steroid use (day 1 up to week 24)
- Duration of steroid use (day 1 up to week 24)
- Quantity of SRP-9001 dystrophin protein expression as measured by western blot (week 12)

KEY INCLUSION/EXCLUSION CRITERIA

KEY INCLUSION CRITERIA[b]

- Male at birth, ambulatory, and ≥4 years of age at the time of dosing (Cohort 1)
- Eligible for commercial SRP-9001 (Cohort 1)
- Male at birth and has previously received SRP-9001 in a commercial setting after pre-treatment with sirolimus and corticosteroids (Cohort 2)

KEY EXCLUSION CRITERIA[b]

- Contraindicated to receive SRP-9001 per the USPI (Cohort 1)
- Has serological evidence of current, chronic, or active human immunodeficiency virus, hepatitis C, or hepatitis B infection (Cohort 1)
- Has a symptomatic infection (eg, upper respiratory tract infection, pneumonia, pyelonephritis, meningitis) within 4 weeks prior to day 1 (Cohort 1)
- Has received a live virus vaccine within 4 weeks or inactive vaccine within 2 weeks of the day 1 visit or expects to receive a vaccination during the first 3 months after day 1 (Cohort 1)

Delandistrogene moxeparvovec is not approved in any country other than the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan. The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.

[a]All outcomes were only measured in Cohort 1.[b]Other criteria apply.

AE, adverse event; AESI, adverse event of special interest; ALI, acute liver injury; SAE, serious adverse event; TEAE, treatment-emergent adverse event; USPI, United States Package Insert.

ClinicalTrials.gov Identifier: NCT07542314. Updated April 21, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT07542314.

Information updated as of April 23, 2026

VOYAGENE: Multicenter, single-dose, open-label study of the safety, tolerability, and efficacy of SRP-9003 (bidridistrogene xeboparvovec) in individuals with LGMD2E/R4 (β-sarcoglycanopathy)[1]

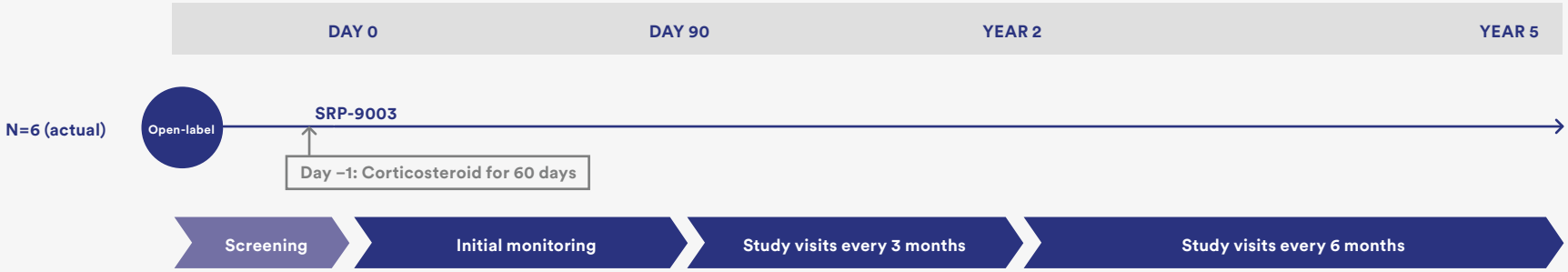
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT05876780

Other study ID number: SRP-9003-102

Study start date: December 2022

STUDY DESIGN[1,2]



Muscle biopsies will be carried out per protocol-specified requirements.

OUTCOME MEASURES[1]

PRIMARY

- **TEAEs and TE SAEs** (baseline up to month 60)
- **Change from baseline in β-SG protein expression quantity as assessed by IF fiber intensity, IF PPF, and western blot** (day 60)

SECONDARY

- **Change from baseline in β-SG protein expression quantity as assessed by IF fiber intensity, IF PPF, and western blot** (month 24)
- **Change from baseline in NSAD score** (month 60)
- **Change from baseline in TTR, 100MWR, 10MWR, 4SC** (month 60)
- **Change from baseline in PUL Version 2.0 score** (month 60)
- **Change from baseline in ACTIVE seated workspace volume task** (month 60)
- **Cohort 1 (ambulatory): Change from baseline in stride velocity (95%) and stair-climbing velocity (95%), stride length (95%), number of stairs climbed per hour, and distance walked per hour as assessed by a wearable device** (month 60)
- **Change from baseline in upper extremity activity and angular wrist velocity** (month 60)
- **Change from baseline in vector genome copies using ddPCR in target muscle tissue** (day 60 and month 24)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[a]

- **Cohort 1 only: Ambulatory**
- **Cohort 2 only: Non-ambulatory and aged 4-50 years**
- **Possesses 1 homozygous or 2 heterozygous pathogenic and/or likely pathogenic *SGCB* mutations**
- **Ability to cooperate with muscle testing**

KEY EXCLUSION CRITERIA[a]

- **Presence of any other clinically significant illness or medical condition**
- **Exposure to gene therapy, investigational medication, or other protocol-specified treatment within protocol-specified time limits**
- **Any contraindication to use of corticosteroid**

SRP-9003 is investigational only and is not approved by any regulatory authority in the world.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Other criteria apply.[1]
β-SG, beta-sarcoglycan; 4SC, time to ascend 4 stairs; 10MWR, 10-meter walk/run; 100MWR, 100-meter walk/run; ACTIVE, ability captured through interactive video evaluation; ddPCR, droplet digital polymerase chain reaction; IF, immunofluorescence; LGMD, limb-girdle muscular dystrophy; NSAD, North Star Assessment for Dysferlinopathy; PPF, percent protein fibers; PUL 2.0, Performance of Upper Limb Version 2.0; *SGCB*, beta-sarcoglycan gene; TEAE, treatment-emergent adverse event; TE SAE, treatment-emergent serious adverse event; TTR, time to rise.
1. ClinicalTrials.gov Identifier: NCT05876780. Updated February 19, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT05876780 2. Connolly AM, et al. Poster presented at 2025 Neuromuscular Study Group (NMSG) Annual Scientific Meeting; September 26-28, 2025; Stresa, Lake Maggiore, Italy. Poster 1212.
Information updated as of April 23, 2026.

EMERGENE: A Phase 3, multinational, open-label study to evaluate the safety and efficacy of SRP-9003 (bidridistrogene xeboparvovec) in individuals with LGMD2E/R4 (β-sarcoglycanopathy)[1]

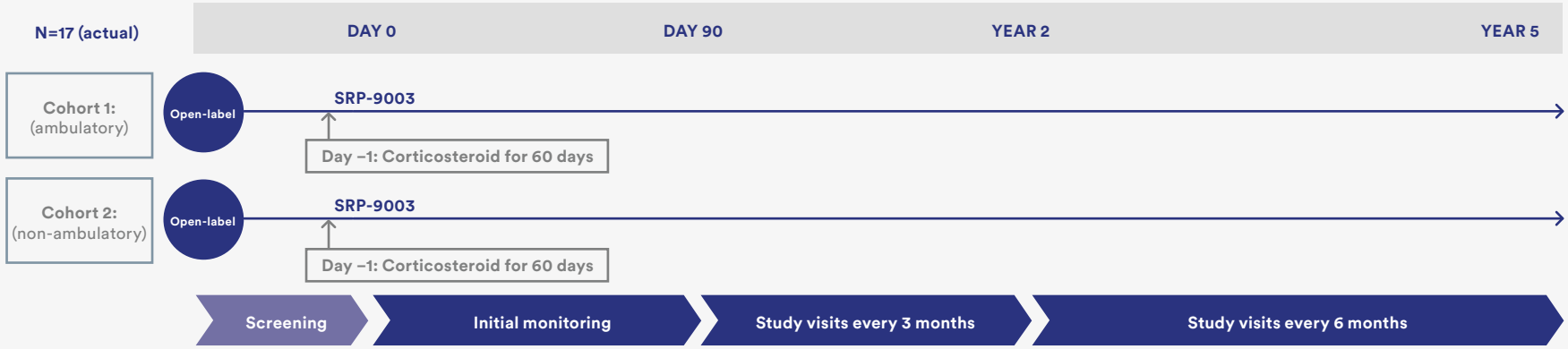
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT06246513

Other study ID number: SRP-9003-301

Study start date: January 2024

STUDY DESIGN[1,2]



Muscle biopsies will be carried out per protocol-specified requirements.

OUTCOME MEASURES[1]

PRIMARY

- **Cohort 1 (ambulatory): Change from baseline in β-SG protein expression as measured by IF PPF** (day 60)

SECONDARY

- **Cohorts 1 and 2:**
  - **Change from baseline in β-SG expression post-dose, as measured by western assay and IF PFI** (day 60)
  - **Change from baseline in NSAD Total Score** (month 60)
  - **Change from baseline in PUL 2.0 Total Score** (month 60)
- **Cohort 1 (ambulatory): Change from baseline in TTR, 10MWR, 4SC, 100MWR, and TUG** (month 60)
- **Cohort 2 (non-ambulatory): Change from baseline in β-SG expression as measured by IF PPF** (day 60)
- **Cohorts 1 and 2:**
  - **TEAEs, treatment-emergent SAEs, and AESIs (month 60)**
  - **Change from baseline in creatine kinase level** (month 60)
  - **Time to change of LOA** (month 60)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA[a]

- **Cohort 1 (ambulatory):**
  - **Able to walk without assistive aid**
  - **10MWR <30 seconds**
  - **NSAD ≥25**
- **Cohort 2 (non-ambulatory):**
  - **10MWR ≥30 seconds or unable to perform**
  - **PUL 2.0 entry scale score ≥3**
- **≥4 years of age and possesses 1 homozygous or 2 heterozygous pathogenic and/or likely pathogenic *SGCB* mutations**
- **Ability to cooperate with muscle testing**
- **AAVrh74 antibody titers <1:400 (ie, not elevated), as determined by AAVrh74 antibody enzyme-linked immunosorbent assay**

KEY EXCLUSION CRITERIA[a]

- **LVEF <40% or clinical signs and/or symptoms of cardiomyopathy**
- **FVC ≤40% of predicted value and/or requirement for nocturnal ventilation**
- **Diagnosis of (or ongoing treatment for) an autoimmune disease and is on active immunosuppressant treatment**
- **Presence of any other clinically significant illness or medical condition (other than LGMD2E/R4)**

SRP-9003 is investigational only and is not approved by any regulatory authority in the world.
The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.
[a]Other criteria apply.[1]
β-SG, beta-sarcoglycan; 4SC, time to ascend 4 stairs; 10MWR, 10-meter walk/run; 100MWR, 100-meter walk/run; AAVrh74, adeno-associated virus rhesus isolate serotype 74; AESI, adverse event of special interest; FVC, forced vital capacity; IF, immunofluorescence; LGMD, limb-girdle muscular dystrophy; LOA, loss of ambulation; LVEF, left ventricular ejection fraction; NSAD, North Star Assessment for Limb-girdle Muscular Dystrophies; PFI, percent fluorescent intensity; PPF, percent positive fibers; PUL 2.0, Performance of Upper Limb Version 2.0; SAE, serious adverse event; *SGCB*, beta-sarcoglycan gene; TEAE, treatment-emergent adverse event; TTR, time to rise; TUG, timed up and go.
1. ClinicalTrials.gov Identifier: NCT06246513. Updated March 2, 2026. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT06246513 2. Sarepta Therapeutics. Data on file.
Information updated as of April 23, 2026.

JOURNEY: A global, multicenter, longitudinal study of the natural history of individuals with LGMD type 2E/R4 (β-sarcoglycanopathy), 2D/R3 (α-sarcoglycanopathy), 2C/R5 (γ-sarcoglycanopathy), and 2A/R1 (calpainopathy)[1]

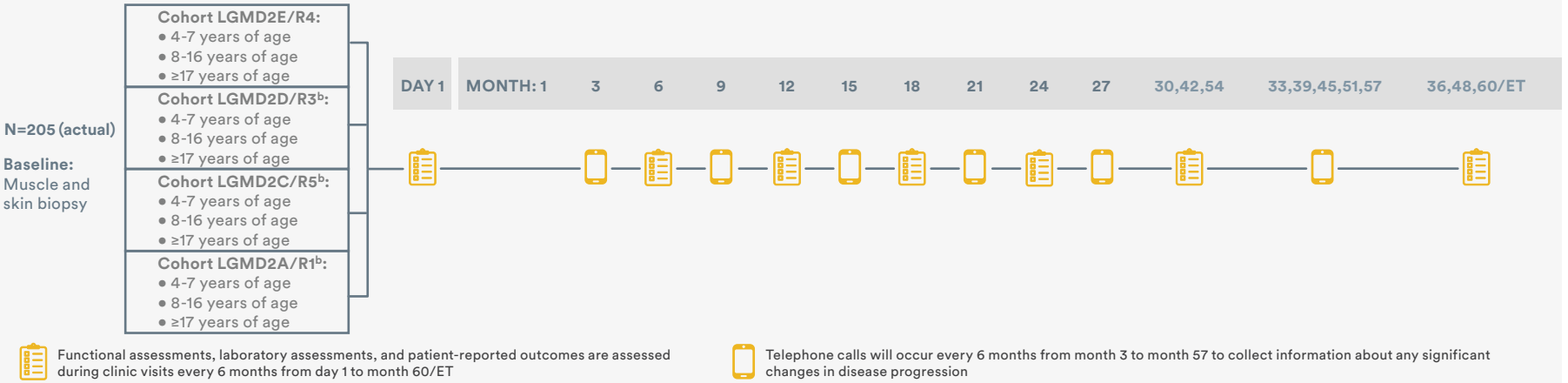
FOR A LIST OF PARTICIPATING SITES, PLEASE SEE CLINICALTRIALS.GOV

ClinicalTrials.gov identifier: NCT04475926

Other study ID number: SRP-LGMD-501-NHS

Study start date: April 2021

STUDY DESIGN[1,2,a]



Functional assessments, laboratory assessments, and patient-reported outcomes are assessed during clinic visits every 6 months from day 1 to month 60/ET

Telephone calls will occur every 6 months from month 3 to month 57 to collect information about any significant changes in disease progression

OUTCOME MEASURES[1]

- **NSAD total score** (baseline up to month 60)
- **TTR** (baseline up to month 60)
- **10MWR** (baseline up to month 60)
- **4SC** (baseline up to month 60)
- **Ankle ROM** (baseline up to month 60)
- **Dimension of the PUL** (baseline up to month 60)
- **TUG** (baseline up to month 60)
- **100MWR** (baseline up to month 60)
- **Pulmonary function test: FVC** (baseline up to month 60)

KEY INCLUSION/EXCLUSION CRITERIA[1]

KEY INCLUSION CRITERIA

- **Male or female patients, aged ≥4 years with confirmed clinical and genetic diagnosis and who demonstrates symptoms of LGMD2E/R4, LGMD2D/R3, LGMD2C/R5, or LGMD2A/R1**

KEY EXCLUSION CRITERIA

- **Demonstrates cognitive delay or impairment that could confound motor development, in the opinion of the Investigator**
- **Has a medical condition, in the opinion of the Investigator, that might compromise patient's ability to comply with study requirements**
- **Is participating in other interventional study(ies) at the time of enrollment in this study**

The projected timelines in this material might be impacted by multiple factors that may not be under Sarepta's control.

[a]The enrolled participants will be followed to evaluate mobility and pulmonary function for up to 5 years after enrollment for participants with LGMD2C/R5, LGMD2D/R3, and LGMD2E/R4 with a NSAD ≥25 at baseline, up to 3 years for participants with LGMD2C/R5, LGMD2D/R3, and LGMD2E/R4 with a NSAD <25 at baseline, and up to 3 years for participants with LGMD2A/R1.[1] [b]Study cohort has been terminated.

4SC, time to ascend 4 stairs; 10MWR, 10-meter walk/run; 100MWR, 100-meter walk/run; ET, early termination; FVC, forced vital capacity; LGMD, limb-girdle muscular dystrophy; NSAD, North Star Assessment for Dysferlinopathy; PUL, performance of upper limb; ROM, range of motion; RWE, real-world evidence; TTR, time to rise; TUG, timed up and go.

1. ClinicalTrials.gov Identifier: NCT04475926. Updated November 6, 2025. Accessed April 23, 2026. https://clinicaltrials.gov/study/NCT04475926 2. Lowes LP, et al. Poster presented at: 28th Annual International Congress of the World Muscle Society (WMS); October 3-7, 2023; Charleston, SC, USA.Poster 304.