Sarepta Therapeutics ELEVIDYS (Delandistrogene moxeparvovec-rokl)

Parent Project Muscular Dystrophy

Data disclaimer

- Delandistrogene moxeparvovec is not approved outside the United States of America, Bahrain, Brazil, Israel, Japan, the State of Kuwait, Oman, Qatar and the United Arab Emirates.

- This presentation and discussion are for educational purposes only and solely intended to provide scientific information on Sarepta's therapies. Nothing in this presentation and discussion should be construed as seeking to promote Sarepta's therapies or to recruit patients in relation to Sarepta's clinical development programs. Nor should the data presented or discussed be considered suggestive of any clinical benefits of Sarepta's therapies or as providing any safety data in respect of Sarepta's therapies. This presentation is being provided upon an unsolicited request of Dr. Omer Abdul Hamid as an aid for the preparation of their own presentation in the United States for the annual Parent Project Muscular Dystrophy (PPMD) Annual Conference. It is Dr. Hamid 's responsibility to extract the contents of this material for the creation of their own presentation in compliance with local regulations.

- Published or presented materials are protected by copyright. The slides have been provided for your use in response to your unsolicited request. You are responsible for complying with all appropriate copyright laws with your use of these materials, including required rights for figures used from publications as well as adapted figures from publications.

- This presentation includes scientific information from post-hoc analysis, which are exploratory and not definitive, and information generated outside controlled clinical settings that therefore should be interpreted with appropriate caution.

- Only an authorized healthcare professional may determine if a specific treatment is appropriate for any individual person.

- Nothing in in this slide deck should be disclosed externally without Sarepta's prior agreement. This presentation should be treated confidentially and its distribution is prohibited.

PPMD

Slides in this presentation may contain off-label information which has not been reviewed or approved by the any regulatory authorities.

Forward Looking Statements

This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward- looking statements. Words such as “believe,” “anticipate,” “plan,” “expect,” “will,” “may,” “intend,” “prepare,” “look,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our opportunities in the rare disease space; market opportunities with respect to our RNA technologies, gene therapy and gene editing; the potential benefits of our technologies and scientific approaches; the potential of our collaborations and partnerships; our goal to develop precision genetic medicine to treat 100% of individuals with Duchenne; and expected timelines and milestones.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control and are based on our current beliefs, expectations, and assumptions regarding our business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties and could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: our data for our different programs may not be sufficient for obtaining regulatory approval; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons, including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, for various reasons including possible limitations of our financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office; and those risks identified under the heading “Risk Factors” in Sarepta’s most recent Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in its other SEC filings.

For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's filings with the SEC. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. The forward-looking statements in this presentation are made as of the date of this presentation only and, other than as required under applicable law, Sarepta does not undertake any obligation to publicly update its forward-looking statements.

Eteplirsen is not approved outside of the United States, Israel, the State of Kuwait, Libya, and Georgia. Golodirsen and casimersen are not approved outside of the United States, the State of Kuwait, Libya, and Georgia. Slides in this presentation may contain off-label information which has not been reviewed or approved by the US FDA. Delandistrogene moxeparvovec is not approved outside the United States of America, Bahrain, Brazil, Israel, Japan, the State of Kuwait, Oman, Qatar and the United Arab Emirates.

Current FDA Approved Indication for Delandistrogene Moxeparvovec

Delandistrogene moxeparvovec is indicated for the treatment of patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who are ambulatory and have a confirmed mutation in the *DMD* gene *[see Clinical Pharmacology (12.2), Clinical Studies (14)]*.

Limitations of Use

Delandistrogene moxeparvovec is not recommended in patients with:

- **Preexisting liver impairment (defined as gamma-glutamyl transferase [GGT] > 2 x upper limit of normal or total bilirubin > the upper limit of normal not due to Gilbert's syndrome) or active hepatic viral infection due to the high risk of acute serious liver injury and acute liver failure.**
- **Recent vaccination (within 4 weeks of treatment) due to immunogenicity and potential safety concerns.**
- **Active or recent (within 4 weeks) infections due to safety concerns.**

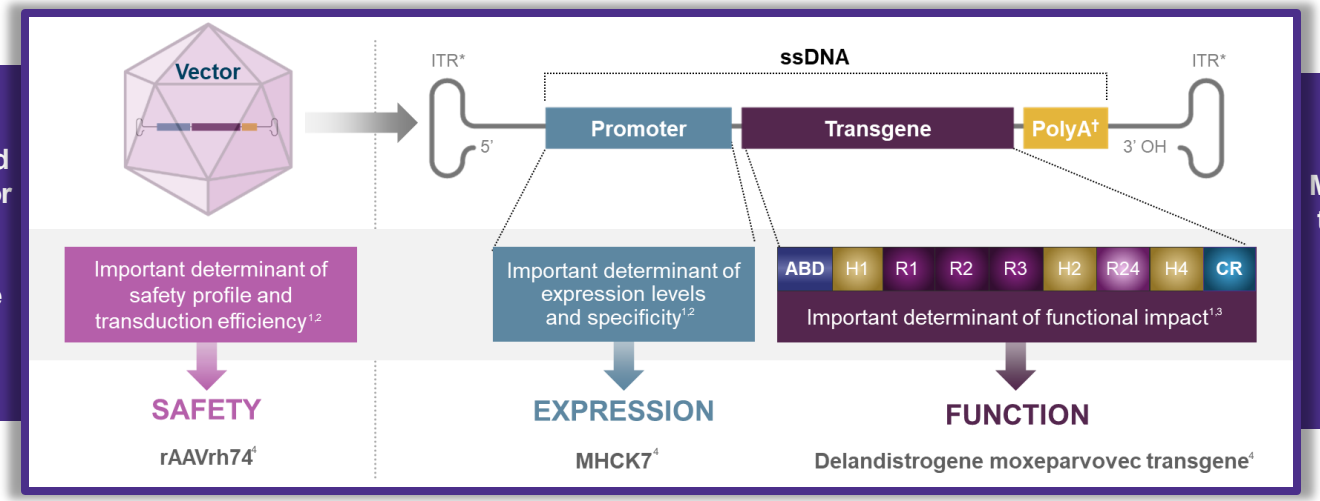
DMD, Duchenne muscular dystrophy; *DMD*, the gene that encodes dystrophin; GGT, gamma-glutamyl transferase.

Delandistrogene moxeparvovec-rokl suspension [package insert]. Sarepta Therapeutics, November 2025.

Delandistrogene moxeparvovec is approved in the United States of America, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.

FDA does not consider structurally distinct versions of micro-dystrophin to be functionally equivalent[5]

The choices of vector, promoter, and transgene are a major determinant for the safety, expression, and function of gene therapies, respectively



The combination of the AAVrh74 vector, MHCK7 promoter, and the micro-dystrophin transgene result in a distinct safety and efficacy profile for delandistrogene moxeparvovec

*ITRs are required for genome replication and packaging; †PolyA signals the end of the transgene to the cellular machinery that transcribes (i.e., copies) it.

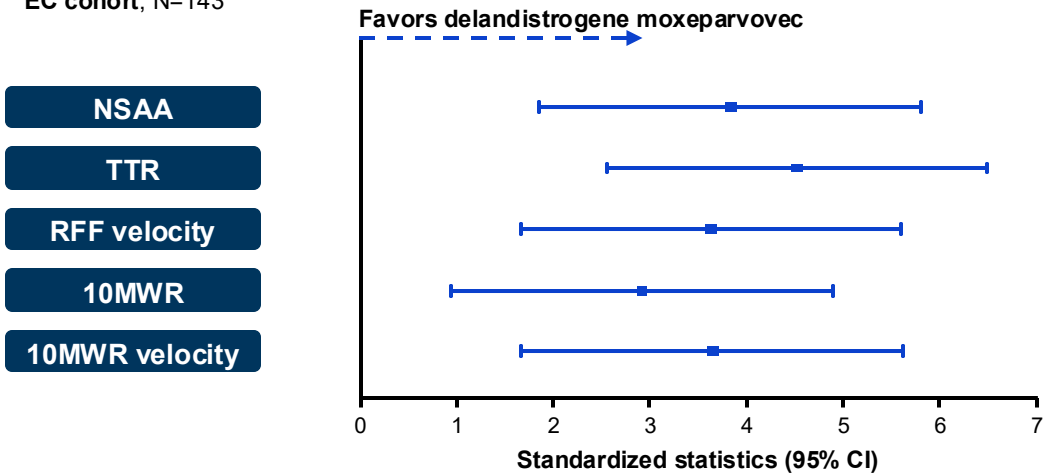
AAV, adeno-associated virus; DNA, deoxyribonucleic acid; ITR, information transfer rate; MHCK7, myosin heavy chain creatine kinase 7; rAAVrh74, recombinant adeno-associated virus rhesus serotype 74; ssDNA, single-stranded DNA. 1. Asher DR, *et al*. *Expert Opin Biol Ther*. 2020;20(3):263–74. 2. Zheng C and Baum BJ. *Methods Mol Biol*. 2008;434:205–19. 3. Chandler RJ and Venditti CP. Version 2. *Transl Sci Rare Dis*. 2016;1(1):73–89. 4. Mendell JR, *et al*. *JAMA Neurol*. 2020;77(9):1122–31. 5. Clinical Decision Memo. BLA 125781. June 2023. Accessed January 14, 2025. https://www.fda.gov/media/169707/download?attachment

Delandistrogene moxeparvovec is approved in the United States of America, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.

EMBARK Part 1: Functional Outcomes at 3 Years

At 3 years, Part 1-treated patients demonstrated **clinically meaningful, durable, and statistically significant functional benefit** versus a propensity-score-weighted EC cohort

Delandistrogene moxeparvovec, N=64*
EC cohort, N=143*



	LSM mean change difference (95% CI)†	*P*-value
NSAA	Δ 4.39 points (2.13 to 6.65)	*P*=0.0002
TTR	Δ –6.05 seconds (–8.69 to –3.41)	*P*<0.0001
RFF velocity	Δ 0.06 rise/second (0.028 to 0.097)	*P*=0.0004
10MWR	Δ –2.70 seconds (–4.51 to –0.88)	*P*=0.0039
10MWR velocity	Δ 0.36 meters/second (0.164 to 0.550)	*P*=0.0003

While the primary endpoint of change from baseline in NSAA total score versus placebo was not met at Year 1, both 2- and 3-year follow-up of Part 1-treated patients showed slowing of DMD disease progression versus a matched EC cohort[1,2]

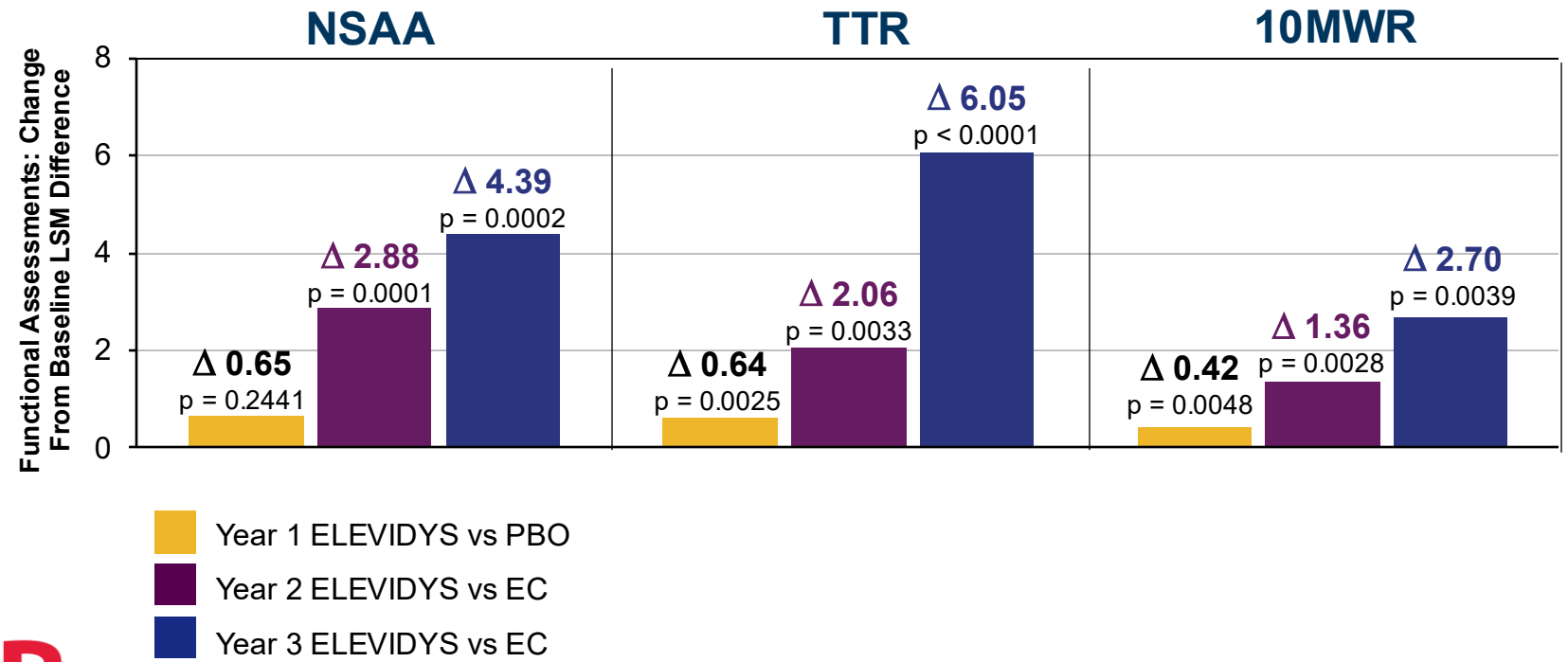
*All 64 patients treated with delandistrogene moxeparvovec and all 143 patients in the EC cohort were included in the analyses; MMRM methods account for missing data in these analyses. Twelve patients treated with delandistrogene moxeparvovec in Part 1 did not have 3-year follow-up data. In the EC cohort, 70 patients had missing Year 3 data for NSAA, 10MWR, and 10MWR velocity assessments, and 65 patients had missing Year 3 data for TTR and RFF velocity assessments. †LSMs (of change from baseline) and CIs were standardized in the forest plot by dividing by the SE. Negative values for timed function tests (TTR and 10MWR) show an improvement in the time taken to achieve these endpoints. LSM differences are on original scale (without SE adjustment). Signs of timed function tests were reversed in the forest plot to align favorable directions among endpoints. Numerical results of LSM difference kept the original signs. All *P*-values are nominal and have not been adjusted for multiple comparisons.
10MWR, 10-meter Walk/Run; CI, confidence interval; DMD, Duchenne muscular dystrophy; EC, external control; LSM, least-squares mean; MMRM, Mixed Models for Repeated Measures; NSAA, North Star Ambulatory Assessment; RFF, rise from floor; SE, standard error; TTR, Time to Rise.

1. Mendell JR, et al. *Nat Med*. 2025; 31:332–341; 2. Mendell JR, et al. *Neurol Ther*. 2026; doi:10.1007/s40120-025-00879-8 (Online ahead of print).

Delandistrogene moxeparvovec is approved in the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan

Slides in this presentation may contain off-label information which has not been reviewed or approved by the any regulatory authorities.

Elevidys Key Functional Data over 3 Years



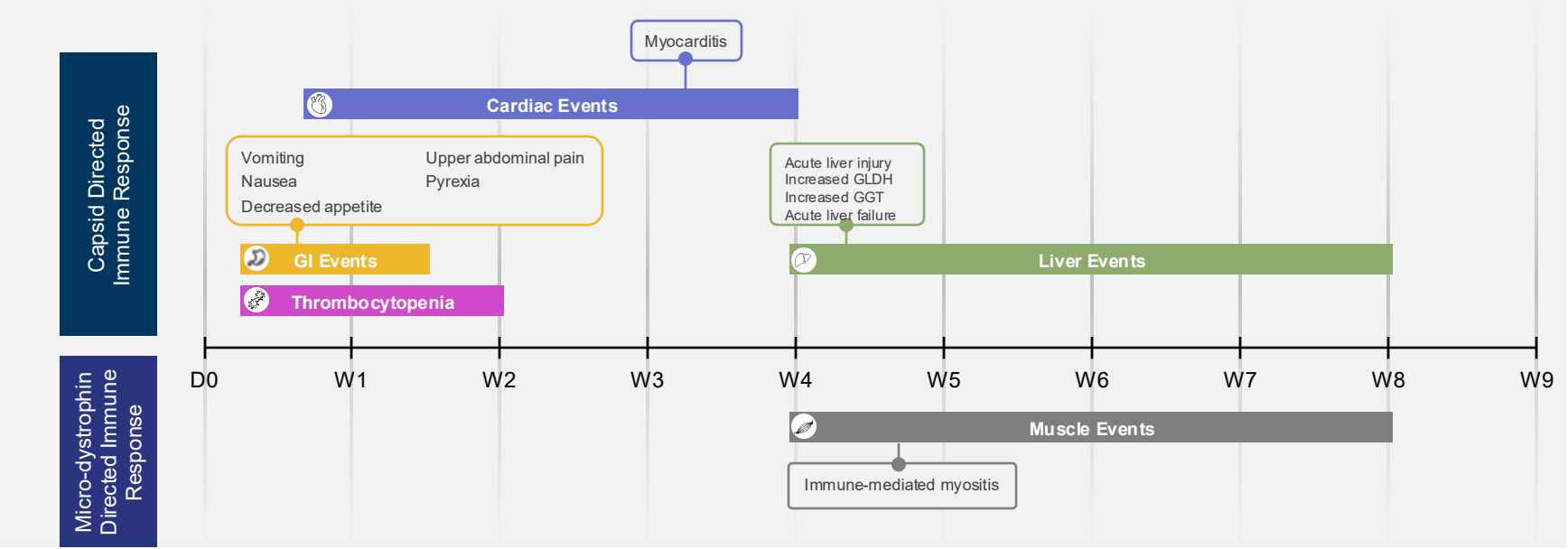
Year 2 and 3 data are exploratory.
All P-values are nominal and have not been adjusted for multiple comparisons.
EC, external control; LSM, least-squares mean; NSAA, North Star Ambulatory Assessment; TTR: time to rise; MWR: Meter Walk Test

1. Mendell JR, et al. Neurol Ther. 2026; doi:10.1007/s40120-025-00879-8 (Online ahead of print).

Delandistrogene moxeparvovec is approved in the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.

Slides in this presentation may contain off-label information which has not been reviewed or approved by the any regulatory authorities.

Timeline of Adverse Events Following Treatment With Elevidys[1-4]



Mendell JR, *et al. Nat Med*. 2025;31(1):332–41.

The most common adverse reactions (incidence ≥5%) reported in clinical studies were vomiting, nausea, liver injury, pyrexia, thrombocytopenia, and troponin-I increased

EMBARK 3-year safety overview[3,4]

- **No new safety concerns** were observed between Year 2 and Year 3
- There was a **lower** number of **treatment-related side effects** between Year 2 and Year 3, than during the first 2 years
- No treatment-related deaths

1. Proud C, et al. Presented at MDA 2026 (Poster 478LB); 2. Mendell JR, et al. *Nat Med* 2025; 31:332–41; 3. Mendell JR, et al. *Neurol Ther* 2026; doi:10.1007/s40120-025-00879-8 (Online ahead of print); 4. Mendell J, et al. Presented at MDA 2026 (Oral#480LB).

Delandistrogene moxeparvovec is approved in the United States, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.

Important Safety Information

BOXED WARNING: ACUTE SERIOUS LIVER INJURY AND ACUTE LIVER FAILURE

- **Acute serious liver injury, including life-threatening and fatal acute liver failure, has occurred with delandistrogene moxeparvovec.**
- **Patients with preexisting liver impairment may be at higher risk.**
- **Prior to infusion, assess liver function by clinical examination and laboratory testing. Administer systemic corticosteroids before and after delandistrogene moxeparvovec infusion. Continue to monitor liver function weekly for the first 3 months after infusion and continue until results are unremarkable.**
- **Instruct patients to maintain proximity to an appropriate healthcare facility, as determined by the healthcare provider, for at least 2 months following delandistrogene moxeparvovec infusion.**
- **Obtain prompt consultation with a specialist (e.g., gastroenterologist or hepatologist) if acute serious liver injury or impending acute liver failure is suspected.**

CONTRAINDICATION

- Delandistrogene moxeparvovec is contraindicated in patients with any deletion in exon 8 and/or exon 9, including a deletion of any portion or the entirety of these exons, in the *DMD* gene.

DMD, the gene that encodes dystrophin.

Delandistrogene moxeparvovec-rokl suspension [package insert]. Sarepta Therapeutics, November 2025.

Delandistrogene moxeparvovec is approved in the United States of America, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Brazil, and Japan.

SRP-9001-103 ENDEAVOR Cohort 8

Cohort 8 Design and Study Information

An Open-Label, Systemic Gene Delivery Study Using ELEVIDYS (delandistrogene moxeparvovec-rokl) to Evaluate the Safety and Expression in Subjects with Duchenne Muscular Dystrophy[1]

Participants: Approximately 25[2]

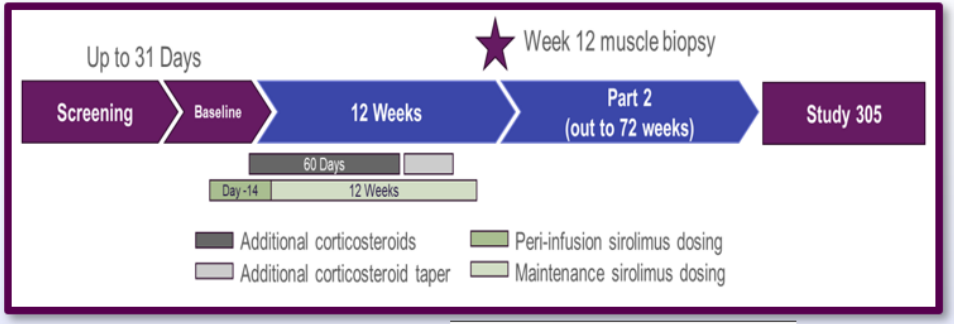
Primary endpoints
- Quantity of micro-dystrophin protein expression at Week 12 (Part 1) as measured by Western blot[1]
- To evaluate the effectiveness of sirolimus on acute liver injury[2]

Key Inclusion Criteria*
- Non-ambulatory for a minimum of 3 months[2]
- Has a PUL entry item score ≥3[1]

Key Exclusion Criteria*[1]
- Any confounding factors that would prevent the use of oral sirolimus including a known hypersensitivity to sirolimus or any of its excipients

Participating Sites[1]

Additional sites to activate within the US.
- Standford University
- UC Davis
- Washington University
- Children's Hospital of The King's Daughters
- Arkansas Children's Hospital
- Neurology Rare Disease Center



ClinicalTrials.gov ID: NCT04626674

*Other inclusion and exclusion criteria may apply

1. ClinicalTrials.gov Identifier: NCT04626674. Updated December 29, 2025. Accessed March 5, 2026. https://clinicaltrials.gov/study/NCT04626674
2. Sarepta Therapeutics. Data on file.