



Sarepta Therapeutics Presents New Data at 2026 World Muscle Society Annual Congress Demonstrating Safety and Clinically Meaningful Functional Benefit of ELEVIDYS (delandistrogene moxeparvovec) in Older Ambulatory Patients with Duchenne

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- ***Patients treated with delandistrogene moxeparvovec at ages 8-12 demonstrated durable and clinically meaningful benefits across the North Star Ambulatory Assessment and timed function measures over 2 years compared to a well-matched external control group***
- ***Separate findings in children treated at ages 2 to under 3 demonstrated robust delandistrogene moxeparvovec-dystrophin expression and transduction efficiency at 12 weeks***
- ***In these analyses, the safety profile of delandistrogene moxeparvovec remained consistent and no new signals were identified***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 30, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today presented new data at the 31st Annual Congress of the World Muscle Society (WMS) that provide evidence of clinically meaningful functional benefit of ELEVIDYS (delandistrogene moxeparvovec) in older ambulatory patients with Duchenne muscular dystrophy.

In ambulatory patients treated at ages 8 to 12, an age range in which functional decline becomes even more pronounced, treatment was associated with durable benefits across multiple measures over two years compared to a well-matched external control. These findings suggest that the benefits previously observed in younger ambulatory patients are also being observed in older ambulatory patients. Sarepta also presented an analysis of patients treated at ages 2 and 3 that demonstrated robust delandistrogene moxeparvovec-dystrophin expression and sarcolemmal localization 12 weeks after infusion. Together, the analyses expand the evidence base across distinct ages and stages of ambulant Duchenne patients, while continued follow-up will be important to understand longer-term outcomes.

"The period between 8 and 12 years of age represents a critical phase of Duchenne when many boys transition from relative stability to progressive functional decline," said Louise Rodino-Klapac, Ph.D., president, Research & Development and Technical Operations, Sarepta. "Results presented at WMS 2026 fit within a broader and increasingly consistent body of evidence generated across the clinical development program. Seeing similar patterns emerge across ambulatory Duchenne populations reinforce that timely treatment with delandistrogene moxeparvovec has the potential to meaningfully change the trajectory of the disease. We remain committed to following patients over time and publishing evidence to help inform treatment decisions and advance care for the Duchenne community."

"The older ambulatory patient data demonstrate that delandistrogene moxeparvovec treatment can provide functional benefit that's statistically significant at a stage when decline often becomes more pronounced," said Craig McDonald, M.D., professor and chair of the UC Davis Health Department of Physical Medicine and Rehabilitation, an investigator in the EMBARK and ENDEAVOR studies and an author on both presentations. "In addition, the findings in younger children help us better understand the biological effects of treatment earlier in the disease course where preserving muscle may offer the greater opportunity for long-term benefit. While measures of expression provide important biological evidence, long-term functional outcomes remain the most meaningful way to understand the impact of treatment for patients and families."

Details from the delandistrogene moxeparvovec analyses:

1.103LBP: Functional and safety outcomes in older ambulatory patients with Duchenne muscular dystrophy treated with delandistrogene moxeparvovec (C. McDonald, *et al.*)

This analysis used a prospectively specified approach to compare treated patients with similar patients from several well-characterized DMD natural history datasets. Patients were matched based on the study eligibility criteria, and the groups were further balanced for key factors that can influence disease progression, with the aims of creating a reliable comparison and addressing common limitations of external control (EC) analyses.

Patients from the EMBARK and ENDEAVOR studies who were aged 8-12 years at time of dosing (n=25), were compared to a well-matched EC group (n=99). Treatment with delandistrogene moxeparvovec demonstrated durable and clinically meaningful functional benefits over two years compared to EC. Results on the North Star Ambulatory Assessment (NSAA) and timed function tests as follows:

Outcome measure	LSM* Change Difference vs EC	P=
NSAA	3.32 points (1.24 to 5.41)	0.0022
Time to rise from floor (RFF) velocity	0.041 rise/second (0.016 to 0.066)	0.0019
10-meter walk run (10MWR) velocity	0.195 meters/second (0.046 to 0.345)	0.0114

*least squared means

Clinically meaningful efficacy was observed across NSAA total score, RFF velocity and 10MWR velocity at 1 and 2 years post-infusion, with nominally statistically significant differences between delandistrogene moxeparvovec-treated and EC patients at Years 1 and 2. Results show a sustained treatment effect and slowing of disease progression and support the use of delandistrogene moxeparvovec in ambulatory patients with DMD aged 8-12 years.

Safety in this patient population was manageable and consistent with the known safety profile of delandistrogene moxeparvovec to date, and the most common treatment related adverse events were nausea and vomiting.

2.080P: Delandistrogene moxeparvovec micro-dystrophin expression and safety in 2–3-year-olds with Duchenne muscular dystrophy in ENDEAVOR and ENVOL studies (C. McDonald, *et al.*)

In this analysis, ambulatory patients with Duchenne aged ≥2 to <3 years from the ENDEAVOR and ENVOL clinical studies, results demonstrated robust delandistrogene moxeparvovec-dystrophin expression and sarcolemmal localization 12 weeks after infusion with delandistrogene moxeparvovec.

Week 12 Mean (SD*) (min., max.)	ENDEAVOR Cohort 6 (N=6)	ENVOL Cohort B (N=3)
Vector genome copies number per nucleus	4.5 (1.6) (2.0, 6.6)	5.3 (1.9) (4.0, 7.0)
Western blot, % control	93.9 (38.2) (48.6, 161.0)	72.8 (16.9) (56.0, 90.0)
PDPF %	79.9 (19.8) (43.9, 98.4)	64.7 (8.6) (56.3, 73.5)

*standard deviation

In this patient population, higher overall mean and higher minimum delandistrogene moxeparvovec micro-dystrophin expression was observed compared with previous results in older (≥4 years) ambulatory and non-ambulatory patients. Investigators continue to follow up with patients in order to assess functional outcomes over time and, when available, those results will provide important context for interpreting the clinical benefit in younger patients.

The safety of delandistrogene moxeparvovec in these patients was manageable and consistent with the safety profile to date in ambulatory patients aged 4 years and older.

The use of delandistrogene moxeparvovec in patients under the age of 4 is investigational and its safety and efficacy have not been evaluated by regulatory authorities.

About EMBARK (Study SRP-9001-301)

Study SRP-9001-301, also known as EMBARK, was a multinational, phase 3, randomized, two-part crossover, placebo-controlled study of ELEVIDYS in individuals with Duchenne muscular dystrophy between the ages of 4 to 7 years. The primary endpoint was change from baseline in NSAA Total Score at Week 52 following treatment. Eligible participants received a single dose of ELEVIDYS during either Part 1 or Part 2 of the study.

In Part 1, participants (n=125) were randomized according to age (≥4 to <8 years) or NSAA Total Score at screening (>16 to <29) and received either 1.33 x10¹⁴ vg/kg of ELEVIDYS or placebo with a follow-up period for 52 weeks. In Part 2, participants crossed over - meaning, those who were previously treated with placebo in Part 1 received ELEVIDYS and participants who were previously treated with ELEVIDYS received placebo, with a follow-up period for 52 weeks. All patients remained blinded through Part 1 and Part 2.

Secondary outcome measures in EMBARK included the quantity of shortened dystrophin produced by ELEVIDYS at week 12 as measured by western blot in a subset of participants, timed function tests, stride velocity and validated patient reported outcome measures for mobility and upper limb function. One-year results from the Part 1 placebo-controlled period of the EMBARK study were published in [Nature Medicine](#) in October 2024. Two-year results were published in [Neurology & Therapy](#) in January 2026.

EMBARK was completed at the end of 2024. Following study completion, patients had the option to enroll in EXPEDITION (Study 9001-305), a Phase 3 long-term follow-up study evaluating the safety and efficacy of ELEVIDYS in individuals who previously received the gene therapy. A total of 64 patients received ELEVIDYS in Part 1 of EMBARK, and 52 continue to be followed in EXPEDITION three years after treatment.

About ENDEAVOR (Study 9001-103)

Study SRP-9001-103, also known as ENDEAVOR, is an open-label, Phase 1b study assessing the safety of ELEVIDYS and the expression ELEVIDYS micro-dystrophin in multiple cohorts of male patients with Duchenne. The study has enrolled participants across 8 cohorts and has dosed younger ambulatory individuals aged 2-7 at time of treatment, as well as older ambulant individuals and non-ambulant individuals.

The primary endpoint in ENDEAVOR is the change from baseline in the quantity of ELEVIDYS micro-dystrophin protein expression measured by western blot at 12 weeks. Secondary outcome measures include change from baseline in micro-dystrophin expression measured by percent dystrophin positive fibers at 12 weeks.

About ENVOL (Study 302)

ENVOL is an open-label Phase II study evaluating the safety of Elevidys and expression of Elevidys micro-dystrophin protein in young children. The primary endpoint in ENVOL is safety through five years following treatment, and the secondary outcome measure is change from baseline in the quantity of ELEVIDYS micro-dystrophin protein expression measured by western blot 12 weeks after treatment.

About ELEVIDYS (delandistrogene moxeparvovec-rokl)

ELEVIDYS (delandistrogene moxeparvovec-rokl) is a single-dose, adeno-associated virus (AAV)-based gene transfer therapy for intravenous infusion designed to address the underlying genetic cause of Duchenne muscular dystrophy – mutations or changes in the DMD gene that result in the lack of dystrophin protein – through the delivery of a transgene that codes for the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle.

In the U.S., ELEVIDYS is indicated for the treatment of ambulatory patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the *DMD* gene.

Limitations of Use

ELEVIDYS 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.

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. 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 ELEVIDYS 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 ELEVIDYS 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: ELEVIDYS 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.

WARNINGS AND PRECAUTIONS:

Acute Serious Liver Injury and Acute Liver Failure

See *Boxed Warning*.

- Acute serious liver injury marked by elevations of liver enzymes (e.g., GGT, ALT) and total bilirubin and acute liver failure has occurred with ELEVIDYS. Onset of the liver injury typically begins within 8 weeks of ELEVIDYS administration. In non-ambulatory patients treated with ELEVIDYS, acute liver failure with fatal outcome has occurred in the clinical and post-marketing settings.
- Life-threatening mesenteric vein thrombosis, complicated by bowel ischemia and necrosis, and portal hypertension have been reported following acute liver injury associated with ELEVIDYS in a non-ambulatory patient.
- Patients with preexisting liver impairment, chronic hepatic condition, or acute liver disease (e.g., acute hepatic viral infection) may be at higher risk of acute serious liver injury or acute liver failure. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.
- Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated.

Serious Infections

- Increased susceptibility to serious infections may occur due to concomitant administration of corticosteroid regimen and additional immunosuppressants, and ELEVIDYS. Serious respiratory infections, including with fatal outcomes, have occurred in patients taking immunosuppressant corticosteroids required for ELEVIDYS administration.
- Monitor patients for signs and symptoms of infection before and after ELEVIDYS administration and treat appropriately.
- Administer immunizations according to best clinical practices and immunization guidelines prior to initiation of the corticosteroid regimen required before ELEVIDYS infusion.
- Avoid administration of ELEVIDYS to patients with active infections.

Myocarditis

- Acute, serious, life-threatening myocarditis and troponin-I elevations have been observed within 24 hours to more than 1 year following ELEVIDYS infusion.
- If a patient experiences myocarditis, those with pre-existing left ventricle ejection fraction (LVEF) impairment may be at higher risk of adverse outcomes.
- Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion and continue monitoring if clinically indicated, until results return to near baseline levels or stabilize.
- More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.
- Advise patients to contact a physician immediately if they experience cardiac symptoms.

Infusion-related Reactions

- Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred during or up to several hours following ELEVIDYS administration. Closely monitor patients during and for at least 3 hours after the end of infusion. If

symptoms of infusion-related reactions occur, slow or stop the infusion and give appropriate treatment. Once symptoms resolve, the infusion may be restarted at a lower rate.

- ELEVIDYS should be administered in a setting where treatment for infusion-related reactions is immediately available.
- Discontinue infusion for anaphylaxis.

Immune-mediated Myositis

- Immune-mediated myositis, including serious and life-threatening events, has occurred approximately 1 month following ELEVIDYS infusion. Signs and symptoms include severe muscle weakness, including dysphagia, dyspnea, dysphonia, and hypophonia.
- Severe to life-threatening immune-mediated myositis has been reported in patients with deletions including portions of exons 1-17 and/or exons 59-71 of the *DMD* gene.
- Regardless of genetic mutation, advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea, dysphonia, or hypophonia, as these may be symptoms of myositis. Consider additional immunomodulatory treatment based on patient's clinical presentation and medical history if these symptoms occur.

Preexisting Immunity against AAVrh74

- In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all patients developed anti-AAVrh74 antibodies.
- Perform baseline testing for the presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration.
- ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers $\geq 1:400$.

ADVERSE REACTIONS

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

Report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

Please see the full [Prescribing Information](#) for ELEVIDYS, including [Boxed Warning](#) and [Medication Guide](#).

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold a leadership position in Duchenne muscular dystrophy (Duchenne) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases. For more information, please visit www.sarepta.com or follow us on [LinkedIn](#), [X](#), [Instagram](#) and [Facebook](#).

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Forward-Looking Statements

This press release contains "forward-looking statements." Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements relating to our future operations and business plans; the potential benefits of our technologies and scientific approaches; our clinical trials and our understanding of data to date; and ELEVIDYS, including its potential benefits in older patients.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials are positive, 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 our products; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events, or may cause undesirable side effects or adverse reactions, that result in significant negative consequences following any marketing approval; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans for various reasons, some of which may be outside of our control, including possible limitations of company 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 with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained herein. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.

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