Sarepta Announces Positive Topline Three-Year EMBARK Results Showing ELEVIDYS Significantly Slows Disease Progression on Key Functional Measures in Ambulatory Duchenne Patients

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- ***At a mean age of 9 years old, ELEVIDYS-treated patients achieved mean North Star Ambulatory Assessment (NSAA) scores above baseline three years after treatment (n=52)***
- ***ELEVIDYS gene therapy demonstrated 70% or greater reduction in the rate of decline relative to the propensity-weighted external control group, as measured by Time to Rise (TTR) and 10-meter walk/run (10MWR). ELEVIDYS-treated patients showed an increasing treatment effect over time, with the functional gap versus the external control group significantly widening between Year 2 and Year 3***
- ***No new treatment-related safety signals were observed, consistent with the manageable safety profile observed with ELEVIDYS in ambulatory patients to-date***
- ***Investor webcast to be held today at 8:30 a.m. ET***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 26, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive topline three-year functional results from Part 1-treated patients in EMBARK (Study SRP-9001-301), the global, randomized placebo-controlled Phase 3 study evaluating ELEVIDYS (delandistrogene moxeparvovec-rokl) in ambulatory individuals with Duchenne muscular dystrophy who were aged four to seven at time of treatment and at time of last assessment were on average over nine years of age.

Three years after treatment, patients who received ELEVIDYS in Part 1 of EMBARK demonstrated statistically significant, clinically meaningful and durable efficacy across all key motor function measures, North Star Ambulatory Assessment (NSAA), Time to Rise (TTR) and 10-meter walk/run (10MWR), when compared to a pre-specified propensity-weighted untreated external control group (EC)*. The mean NSAA score remained above baseline at Year 3 for the ELEVIDYS-treated group (n=52) while the EC group (n=73) continued to show the expected age-related decline below their baseline score. The ELEVIDYS group showed a 73% slowing of disease progression as measured by TTR and 70% slowing of disease progression as measured by 10MWR when compared to the EC group.**

Patients treated with ELEVIDYS in Part 1 maintained significantly higher levels of motor function three years after treatment compared to the EC group. Topline efficacy results are summarized in the table below:

Functional Outcomes	LSM Change Difference vs EC	p-Value
NSAA	+4.39 points (improvement)	p=0.0002
TTR	-6.05 seconds (improvement)	p<0.0001
10MWR	-2.70 seconds (improvement)	p=0.0039

“ELEVIDYS is the first gene therapy for Duchenne to show a dramatic shift in disease trajectory out to three years consistent with earlier long-term data extending up to five years. This is long-term data in a robust, controlled clinical dataset that demonstrates the power of a disease-modifying therapy targeting the underlying cause of Duchenne,” said Louise Rodino-Klapac, Ph.D., president of research & development and technical operations, Sarepta. “At an age when functional decline is typically accelerating, ELEVIDYS-treated patients showed a 70 percent or greater reduction in the rate of decline on key functional measures such as time to rise and the 10-meter walk/run. These statistically significant benefits not only persist but continue to strengthen over time, creating a sustained and growing separation from the expected disease trajectory.”

“As a pediatric neurologist, I spend time with families who are doing everything they can to help their children stay strong in the face of Duchenne,” said Crystal Proud, M.D., chief of Neurology and director of Neuromuscular Medicine at Children’s Hospital of The King’s Daughters, and an investigator in the EMBARK study. “The EMBARK results give us a clearer picture of how treatment with ELEVIDYS can make a meaningful difference over time, and they reflect what I see in clinical practice – helping boys perform everyday movements, such as standing, walking and running with greater strength and speed than what we expect as Duchenne progresses without a disease-modifying treatment.”

No new treatment-related safety signals were observed, reinforcing the consistent and manageable safety profile seen in ambulatory patients treated with ELEVIDYS to date. Analysis of the three-year data is ongoing and includes functional results from crossover-treated patients two years after treatment. The Company plans to share results at upcoming medical meetings and in publication. Two-year EMBARK results were published in *Neurology & Therapy* this month.

ELEVIDYS is the only approved gene therapy for Duchenne. To date, ELEVIDYS has been administered to over 1,200 patients globally in clinical and real-world settings. ELEVIDYS is available as appropriate to ambulatory individuals ages 4 and over per an updated FDA label announced end of 2025.

As part of a collaboration agreement signed in 2019, Sarepta is working with Roche to transform the future for the Duchenne community, with the goal of enabling those living with the disease to maintain and protect their muscle function. Sarepta is responsible for regulatory approval and commercialization of ELEVIDYS in the U.S., as well as manufacturing. Roche is responsible for regulatory approvals and bringing ELEVIDYS to patients across the rest of the world. Commercialization of ELEVIDYS in Japan is through Chugai Pharmaceuticals, a member of the Roche Group.

*The pre-specified external control analysis included data from three separate studies in Duchenne, comprising DMD controls from one randomized

trial and two natural history cohorts who met predefined matching criteria. Comparison of treated and control patients was based on a pre-specified, propensity score weighting approach using age, height, BMI, steroid usage, baseline NSAA and timed function tests in order to balance key prognostic factors between the groups.

**Percent slowing is calculated as the ELEVIDYS group change from baseline divided by the EC group change from baseline (TTR EC n=78; 10MWR EC n=73).

Sarepta Investor Call Details

At 8:30 a.m. ET on Jan. 26, 2026, Sarepta will host a conference call and webcast to discuss these results.

The event will be webcast live under the investor relations section of Sarepta's website at https://investorrelations.sarepta.com/events-presentations and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using this online form. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

About EMBARK (Study 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 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.

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 ≥1:400.

ADVERSE REACTIONS

- The most common adverse reactions (incidence ≥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**.

Forward-Looking Statements

This press release 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, without limitation, statements relating to our technologies, strategies, and priorities; future operations; ELEVIDYS; and our clinical trials, including Study 9001-301.

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; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; we may observe adverse reactions in our clinical trials or in patients who receive our approved products; our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our business; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; we may not be able to comply with all FDA requests in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; 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.

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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20260126453576/en/

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