Sarepta Therapeutics Presents Data at the American Society of Gene & Cell Therapy Conference, Including Statistically Significant Functional Outcomes for 8- and 9-Year-Old Patients in New Data Analysis of EMBARK Part 2

5/16/25

- ***Significant functional benefits for 8- and 9-year-olds with Duchenne in Part 2 of the EMBARK study, contributing to the evidence of stabilization or slowing of disease progression in later childhood when muscle weakness typically progresses***
- ***Statistically significant differences were observed on all key endpoints including 4.75 points (P=0.0026) on North Star Ambulatory Assessment (NSAA), 6.87 seconds in time-to-rise (TTR) from the floor (P=0.0010), and 4.76 seconds in 10-meter walk/run (10MWR) (P=0.0097) compared to a well-matched external control cohort***
- ***Five abstracts, including two oral presentations at American Society of Gene & Cell Therapy Conference, span Sarepta's portfolio of approved and pipeline therapies across Duchenne and limb-girdle muscular dystrophy***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 16, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today presented new data from Part 2 of the EMBARK study that continue to support the clinical benefits of ELEVIDYS (delandistrogene moxeparvovec-rokl), the only approved gene therapy for patients with Duchenne muscular dystrophy. These data are among other ELEVIDYS data from Sarepta's portfolio presented during the 28th annual meeting of the American Society of Gene & Cell Therapy (ASGCT) Conference.

In the recent analysis of Part 2 of the EMBARK study, participants with Duchenne muscular dystrophy who had received a placebo in Part 1 and were aged 8 to 9 years (n=14) at crossover were included. At one year post ELEVIDYS treatment, there were between-group differences (least square means) on all key endpoints that were statistically significant, including 4.75 points (P=0.0026) on North Star Ambulatory Assessment (NSAA), 6.87 seconds in time-to-rise (TTR) from the floor (P=0.0010), and 4.76 seconds in 10-meter walk/run (10MWR) (P=0.0097) compared to a well-matched external control cohort.

"The latest data from the EMBARK study highlighting motor function improvements in 8- and 9-year-old boys is encouraging and adds to the growing body of evidence supporting ELEVIDYS," said Aravindhan Veerapandiyan, M.D., Associate Professor of Pediatrics at the University of Arkansas for Medical Sciences and Arkansas Children's Hospital. "What stands out is that these patients were treated at an age when motor decline is typically expected in those with Duchenne. Yet, those who received ELEVIDYS demonstrated statistically significant and clinically meaningful functional improvements compared to external controls."

The results presented at ASGCT are from the ongoing analysis of results from Part 2 of EMBARK, which compared two-year outcomes from 63 participants against data from an external control group of untreated individuals with Duchenne. Results at two years post-treatment showed that individuals treated with ELEVIDYS had better outcomes in multiple motor function measures, compared to a well-matched external control group. Additionally, no new safety signals were observed in the EMBARK study over the two-year duration and, in a subset of patients (n=16), micro-dystrophin expression and sarcolemmal localization was sustained from Week 12 to Week 64.

"This has been a significant year for our neuromuscular portfolio, with multiple, ongoing analyses and longer-term data on efficacy and safety presented for ELEVIDYS," said Louise Rodino-Klapac, Ph.D., chief scientific officer and head of research and development, Sarepta Therapeutics. "Building on the topline EMBARK Part 2 data from earlier this year, we're committed to sharing ongoing analyses as fast as possible. The one-year results of patients treated with ELEVIDYS at 8 to 9 years old provide evidence that those treated with gene therapy outperform those who don't receive it at a critical point when more dramatic functional decline is expected."

A full listing of Sarepta's presentations at ASGCT are below.

Abstracts can be found at https://annualmeeting.asgct.org/. Data from presentations are embargoed until 6:00 AM CT on the presentation day for oral abstracts and until 6:00 AM CT on May 13, 2025 for poster abstracts.

Oral Presentations *(*Previously presented at MDA 2025 and supplemented with additional data)*

Title	***Date, Time***
*Long-term Functional Outcomes and Safety Following Delandistrogene Moxeparvovec Treatment in DMD: EMBARK 2-Year Results**	*May 16 4:30 – 4:45 p.m. CST Room 393-396*
Cardiovascular Investigation of SRP-9005 (AAVrh74.MHCK7.hSGCG) in Non-Human Primates: A Gene Therapy for Limb-Girdle Muscular Dystrophy 2C/R5	*May 14 5 – 5:15 p.m. CST New Orleans Theater B*

Poster Presentations *(*Denotes encore presentation)*

Poster #	Title
#1350	3-Year Functional Outcomes of Patients with Duchenne Muscular Dystrophy: Pooled Delandistrogene Moxeparvovec Clinical Trial Data vs. External Controls*
#1353	Assessment of Cardiac Outcomes in Delandistrogene Moxeparvovec Clinical Trials for Duchenne Muscular Dystrophy*
#1422	In Situ Biodistribution and Localization of Bidridistrogene Xeboparvovec (SRP-9003) in LGMD2E/R4 Mice After 1 Year of Follow-up

About EMBARK, Study SRP-9001-301

Study SRP-9001-301, also known as EMBARK, is 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 is 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 1.33×10^{14} vg/kg of ELEVIDYS or placebo with a follow-up period for 52 weeks. In Part 2, participants cross over - meaning, those who were previously treated with placebo in Part 1 receive ELEVIDYS and participants who were previously treated with ELEVIDYS receive placebo, with a follow-up period for 52 weeks. All patients remained blinded through Part 1 and Part 2.

Secondary outcome measures in EMBARK include the quantity of micro-dystrophin produced by ELEVIDYS at week 12 (in a subset of participants) as measured by western blot, 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 and quantitative muscle MR (magnetic resonance) outcomes from part 1 of EMBARK were published in **JAMA Neurology** in May 2025.

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 Duchenne muscular dystrophy (DMD) in individuals at least 4 years of age.

- For patients who are ambulatory and have a confirmed mutation in the *DMD* gene
- For patients who are non-ambulatory and have a confirmed mutation in the *DMD* gene.

The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin in skeletal muscle. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

IMPORTANT SAFETY INFORMATION

CONTRAINDICATION: ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene.

WARNINGS AND PRECAUTIONS:

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 administration 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.

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS, and administration may result in elevations of liver enzymes (such as GGT, GLDH, ALT, AST) or total bilirubin, typically seen within 8 weeks.
- 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. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.
- Prior to ELEVIDYS administration, perform liver enzyme test and monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT, and total bilirubin levels return to near baseline levels).
- Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated. If acute serious liver injury is suspected, consultation with a specialist is recommended.

Immune-mediated Myositis:

- In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the DMD gene. Symptoms of severe muscle weakness, including dysphagia, dyspnea, and hypophonia, were observed.

- Limited data are available for ELEVIDYS treatment in patients with mutations in the DMD gene in exons 1 to 17 and/or exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.
- Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea, or hypophonia, as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

Myocarditis:

- Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.
- 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. 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.

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 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 greater than or equal to 1:400.

Adverse Reactions:

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

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).

For further information, please see the full Prescribing Information.

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 leadership positions in Duchenne muscular dystrophy (Duchenne) and limb-girdle muscular dystrophies (LGMDs) 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 statement 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 future operations, research and development programs, clinical trials and the potential benefits and risks of ELEVIDYS.

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, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; success in clinical trials does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; 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 Annual Report on Form 10-K for the year ended December 31, 2024 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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Investor Contact:
Ian Estepan
617-274-4052
iestepan@sarepta.com

Media Contacts:
Tracy Sorrentino
617-301-8566
tsorrentino@sarepta.com

Kara Hoeger
617-710-3898
KHoeger@sarepta.com

Source: Sarepta Therapeutics, Inc.