Sarepta Therapeutics to Present New Data from its Neuromuscular Portfolio at 27th Annual Neuromuscular Study Group Scientific Meeting

9/24/26

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 24, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, will present new data at the 27th Annual Neuromuscular Study Group (NMSG) Scientific Meeting, taking place Sept. 25-27, 2026, in San Antonio, Texas.

Sarepta will present seven posters at NMSG. Among the analyses are the first results from the Phase 4 ENDURE Study, a multi-center, prospective, observational study in patients receiving delandistrogene moxeparvovec as part of clinical care for Duchenne muscular dystrophy, including safety in patients who received prophylactic immunosuppression with sirolimus.

“The data being presented at NMSG 2026 provide new insights into the real-world use of delandistrogene moxeparvovec and add to the growing body of evidence supporting its clinical profile. Observations from clinical practice are especially valuable because they help us better understand how treatment performs across a broader range of patients and care settings, and we are encouraged that these findings continue to reinforce our understanding of both the safety and effectiveness of delandistrogene moxeparvovec,” said Louise Rodino-Klapac, Ph.D., president, Research & Development and Technical Operations, Sarepta. “Through our portfolio, we are striving to change the course of Duchenne and we’re committed to generating robust evidence throughout the lifecycle of our approved therapies, helping physicians, patients and families make informed treatment decisions while advancing our understanding of treatment in the Duchenne community.”

All presentations will be shared during the NMSG poster session on Friday, Sept. 25, 2026, between 6:00 – 8:00 PM EST / 5:00 – 7:00 PM CST. Poster numbers and titles are as follows:

Duchenne

46	Clinical Validation of the Association Between Micro-Dystrophin Expression and Muscle MRI with Functional Outcomes: A Pooled Analysis of Delandistrogene Moxeparvovec in Patients Treated with Duchenne Muscular Dystrophy (T. Niu, et al)
60	Impact of Delandistrogene Moxeparvovec Treatment Delays for Ambulatory Patients with Duchenne Muscular Dystrophy (L. Sedita, et al)
79	Safety Analysis Following Real-World Use of Delandistrogene Moxeparvovec for Duchenne Muscular Dystrophy (DMD): Interim Analysis of the Phase 4 ENDURE Study (S. Mohammed, et al)
82	PROMIS Parent Proxy Mobility Scores in Patients Treated with Delandistrogene Moxeparvovec in EMBARK Part 2 Compared with External Controls (I. Audhya, et al)
113	Real-World Safety Profile of Delandistrogene Moxeparvovec in the U.S. Postmarketing Setting (V. Pizzo, et al)

DM1 and FSHD

49	Safety and Exposure of SRP-1003, an αvβ6 Integrin-Targeting siRNA Therapy for Patients with Myotonic Dystrophy Type 1: Interim Findings from a Phase 1/2 Study (E. Rodriguez, et al)
50	Safety and Exposure of SRP-1001, an αvβ6 Integrin-Targeting siRNA Therapy for Patients with FSHD1: Interim Findings from a Phase 1/2 Study (R. Roxburgh, et al)

The full agenda for the 2026 NMSG meeting is available online. Sarepta abstracts and presentations will be available on Sarepta.com in the Events & Presentations section following the NMSG embargo.

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**.

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; and ELEVIDYS, including our current understanding based on evidence to date of the potential impact of sirolimus.

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; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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, and bringing our product candidates to market, 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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