Sarepta Therapeutics to Present New Data from its Neuromuscular Portfolio at 2025 World Muscle Society Congress

10/3/25

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 3, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, will present at the 30th Annual Congress of the World Muscle Society (WMS) 2025 Congress, taking place Oct. 7-11, 2025, in Vienna, Austria.

Sarepta presentations will include results from several studies in the delandistrogene moxeparvovec clinical development program, as well as a real-world evidence study of pulmonary function in advanced-stage patients with Duchenne muscular dystrophy treated with casimersen. Further, Sarepta will present results from the EMERGENE phase 3 study on the expression of SGCB and safety following bidridistrogene xeboparvovec treatment in patients with LGMD2E/R4.

In addition to studies to be presented by Sarepta, multiple independent studies on delandistrogene moxeparvovec will be presented, including an abstract on the exploratory use of sirolimus prophylaxis to help mitigate the potential for acute liver injury in patients receiving delandistrogene moxeparvovec. The results presented in this abstract add to our understanding of how prophylactic use of sirolimus could potentially be part of an enhanced immunosuppressive regimen intended to reduce the risk of liver injury associated with infusion of AAV-mediated gene therapy.

“Our presentations at WMS span important milestones across our programs, including safety and micro-dystrophin expression in a larger cohort of 3- to 4-year-olds with Duchenne when treated with gene therapy, a late-breaker on considerations from an interdisciplinary expert committee in mitigating and managing acute liver injury in patients treated with delandistrogene moxeparvovec, and evidence of pulmonary stabilization in patients treated with the PMO casimersen,” said Louise Rodino-Klapac, Ph.D., president of research & development and technical operations, Sarepta. “As leaders in genetic medicine for neuromuscular conditions, these data expand our understanding of the safety, efficacy and transformative potential of our portfolio, with the goal of ultimately improving patient care.”

Sarepta Podium Presentations (*Denotes encore presentation):

21O: Assessment of cardiac outcomes in delandistrogene moxeparvovec clinical trials for Duchenne muscular dystrophy*	Oct. 11, 2025 2:00-2:15 a.m. EDT (8:00-8:15 CET)
06LBO: Acute liver injury mitigation and management in patients with Duchenne muscular dystrophy following administration of delandistrogene moxeparvovec: expert considerations **(late-breaker)**	Oct. 11, 2025 6:45-6:57 a.m. EDT (12:45-12:57 CET)

Sarepta Poster Presentations (*Denotes encore presentation):

424P: Delandistrogene moxeparvovec micro-dystrophin expression and safety in 3–4-year-olds with Duchenne muscular dystrophy in ENDEAVOR and ENVOL studies	**Poster:** Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET) **Oral:** Oct. 8, 2025 9:30-10 a.m. EDT (15:30-16:00 CET)
176P: 3-year functional outcomes of patients with Duchenne muscular dystrophy: Pooled delandistrogene moxeparvovec clinical trial data vs external controls*	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)
659P: Pulmonary function in advanced-stage patients with Duchenne muscular dystrophy treated with casimersen	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)
80P: Delandistrogene moxeparvovec in Duchenne muscular dystrophy: Long-term EMBARK 2-year functional outcomes, safety, and micro-dystrophin expression*	Oct. 10, 2025 8:15-9:15 a.m. EDT (14:15-15:15 CET)
734LBP: Expression of SGCB and safety following bidridistrogene xeboparvovec treatment in patients with LGMD2E/R4: results from the EMERGENE phase 3 study **(late-breaker)**	Oct. 10, 2025 9:45-10:45 a.m. EDT (15:45-16:45 CET)

Presentations of Independent Studies (†Denotes investigator-initiated study):

668P: Using muscle homing peptide CyPep10 to improve delivery of phosphorodiamidate morpholino oligomers in the mdx mouse†	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)
181P: AAV gene therapy with delandistrogene moxeparvovec in two-year-old patients with Duchenne muscular dystrophy: clinical efficacy measured by digital endpoints	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)

660P: Delandistrogene moxeparvovec in Duchenne muscular dystrophy: experience from a single center	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)
676P: One-year motor function outcomes following treatment with delandistrogene moxeparvovec in young boys with Duchenne muscular dystrophy: a single center experience	Oct. 8, 2025 8:30-9:30 a.m. EDT (14:30-15:30 CET)
732LBP: Preliminary analysis of safety, tolerability, and efficacy of a prophylactic sirolimus protocol for patients receiving delandistrogene moxeparvovec-rokl gene therapy **(late-breaker)**	Oct. 10, 2025 9:45-10:45 a.m. EDT (15:45-16:45 CET)

The full WMS 2025 program is available at https://www.wms2025.com/page/programme. Sarepta abstracts and presentations will be available on Sarepta.com in the Events & Presentations section following the WMS 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 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 (noted hereafter as “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 for ELEVIDYS

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 of ELEVIDYS may result in elevations of liver enzymes (e.g., 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, a 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 between exons 1 to 17 and 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.

Pre-existing 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 subjects 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 greater than or equal to 1:400.

Adverse Reactions:

- The most common adverse reactions (incidence ≥ 5%) reported in clinical studies were vomiting, nausea, liver function test increased, 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** for ELEVIDYS (delandistrogene moxeparvovec-rokl).

About AMONDYS 45 (casimersen)
AMONDYS 45 (casimersen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

AMONDYS 45 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 45 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

AMONDYS 45 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for AMONDYS 45

CONTRAINDICATION: AMONDYS 45 is contraindicated in patients with a known serious hypersensitivity to casimersen or any of the inactive ingredients in AMONDYS 45. Instances of hypersensitivity including angioedema and anaphylaxis have occurred.

WARNINGS AND PRECAUTIONS

Hypersensitivity: Hypersensitivity reactions, including angioedema and anaphylaxis, have occurred in patients who were treated with AMONDYS 45. If a hypersensitivity reaction occurs, institute appropriate medical treatment, and consider slowing the infusion, interrupting, or discontinuing the AMONDYS 45 infusion and monitor until the condition resolves. AMONDYS 45 is contraindicated in patients with known serious hypersensitivity to casimersen or to any of the inactive ingredients in AMONDYS 45.

Kidney Toxicity: Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse Reactions: Adverse reactions occurring in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were (AMONDYS 45, placebo): upper respiratory infections (65%, 55%), cough (33%, 26%), pyrexia (33%, 23%), headache (32%, 19%), arthralgia (21%, 10%), and oropharyngeal pain (21%, 7%).

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were: ear pain, nausea, ear infection, post-traumatic pain, and dizziness and light-headedness.

Other adverse events may occur.

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or **www.fda.gov/medwatch**.

For further information, please see the full US **Prescribing Information** for AMONDYS 45 (casimersen).

About SRP-9003 (bidridistrogene xeboparvovec)
SRP-9003 (bidridistrogene xeboparvovec) is an investigational gene therapy that uses the AAVrh74 vector, which is designed to be systemically and robustly delivered to skeletal, diaphragm and cardiac muscle, making it an ideal candidate to treat neuromuscular diseases. SRP-9003 is intended to deliver a full-length beta-sarcoglycan transgene and uses the MHCK7 promoter, chosen for its ability to robustly express in the heart[1,2,3] which is critically important for patients with limb-girdle muscular dystrophy Type 2E (LGMD2E), also known as beta-sarcoglycanopathy and LGMDR4, many of whom die from pulmonary or cardiac complications.

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 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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements related to our research and development programs, technologies, scientific approaches, product and product candidates, including potential benefits related to safety and efficacy; the potential benefits of sirolimus and our understanding of the independent findings to date; and our mission and expected plans and milestones, including presenting certain data and findings at WMS.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; 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; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and 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; 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 expected or planned regulatory milestones and timelines, clinical development plans, and bringing products to markets for various reasons including possible limitations of 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 Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2024, and 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 in this press release. 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 Contacts:
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Ryan Wong, 617-800-4112
rwong@sarepta.com

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

Kara Hoeger, 617-710-3898
khoeger@sarepta.com

Source: Sarepta Therapeutics, Inc.