



Sarepta Therapeutics Announces Presentations at 2026 World Muscle Society Annual Congress

9/25/26

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 25, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, will present new data from its portfolio of treatments for Duchenne muscular dystrophy at the 31st Annual Congress of the World Muscle Society (WMS), taking place Sept. 29 - Oct. 3, in Hiroshima, Japan.

Data at WMS includes a late-breaking poster presentation on delandistrogene moxeparvovec efficacy and safety in older, ambulatory Duchenne patients in Sarepta's clinical studies who were aged 8-12 years old at the time of treatment.

Poster numbers, titles and session timing are as follows:

1.103LBP	Functional and safety outcomes in older ambulatory patients with Duchenne muscular dystrophy treated with delandistrogene moxeparvovec (C. McDonald, et al)	Wed., Sept. 30 2:30-3:30 PM JST 1:30-2:30 AM EST
2.062P	Delandistrogene moxeparvovec in Duchenne muscular dystrophy: Functional and safety outcomes up to 3 years post-infusion in the EMBARK study (J. Mendell, et al)	Wed., Sept. 30 5:15-6:15 PM JST 4:15-5:15 AM EST
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)	Wed., Sept. 30 5:15-6:15 PM JST 4:15-5:15 AM EST
2.044eP	Pooled safety analysis from Phase 1 to Phase 3 clinical trials of delandistrogene moxeparvovec in Duchenne muscular dystrophy (C. Proud, et al)	Wed., Sept. 30 5:15-6:15 PM JST 4:15-5:15 AM EST
2.050eP	Efficacy and safety of early intervention with delandistrogene moxeparvovec in DMD ^{MDX} mice (N. Pukos, et al)	Wed., Sept. 30 5:15-6:15 PM JST 4:15-5:15 AM EST
2.067eP	Efficacy and safety of golodirsen and casimersen compared with placebo in Duchenne muscular dystrophy (ESSENCE): Phase 3 results and post hoc analysis (F. Muntoni, et al)	Wed., Sept. 30 5:15-6:15 PM JST 4:15-5:15 AM EST

The full WMS 2026 program is available at <https://www.wms2026.com/page/programme>. Sarepta abstracts and presentations will be available on [Sarepta.com](https://www.wms2026.com/page/programme) in the [Events & Presentations](#) section following presentation at the congress.

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 $\geq 1:400$.

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.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20260925573264/en/>

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