Sarepta Therapeutics Announces Approval in Japan of ELEVIDYS, a Gene Therapy to Treat Duchenne Muscular Dystrophy

5/13/25

- ***Approval based on efficacy and safety results from clinical studies, including longer-term functional outcomes from EMBARK global phase 3 study***
- ***Company is eligible to receive up to $103.5M in near-term regulatory and commercial milestone payments***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 13, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the Japanese Ministry of Health, Labour, and Welfare (MHLW) has approved ELEVIDYS (delandistrogene moxeparvovec) for the treatment of Duchenne muscular dystrophy (DMD) under the conditional and time-limited approval pathway in Japan. ELEVIDYS is approved for individuals ages 3- to less than 8-years-old, who do not have any deletions in exon 8 and/or exon 9 in the DMD gene, and who are negative for anti-AAVrh74 antibodies. This is the first global approval to include individuals younger than 4 years of age.

The approval is based on the efficacy and safety data for ELEVIDYS, which includes muscle health and longer-term functional results from the ELEVIDYS clinical programs, including the two-year data from the global Phase 3 EMBARK clinical trial (Study SRP-9001-301). These longer-term functional outcomes showed that individuals treated with ELEVIDYS had significantly better outcomes in multiple motor function measures, compared to a well-matched external control group. No new safety signals were observed in the EMBARK study over the two-year duration. These findings from Part 2 of EMBARK were presented at the 2025 Muscular Dystrophy Association meeting. In addition, one-year data from part 1 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.

“For nearly a decade, Sarepta has been a leader in advancing the treatment of Duchenne muscular dystrophy through innovative treatment options. With the approval of ELEVIDYS in Japan, children who are living with this rare, fatal disease, one marked by progressive muscle deterioration and weakness, now have an additional treatment option,” said Louise Rodino-Klapac, Sarepta’s head of research and development and chief scientific officer. “Sarepta is committed to supporting our partners so we can bring this treatment to additional people with Duchenne around the world who urgently need a therapy that potentially addresses the root cause of disease.”

The conditional and time-limited approval pathway in Japan provides for marketing authorization in Japan for up to seven years for innovative medicines to treat serious conditions. To be eligible for the pathway, certain additional criteria must also be met, including early clinical trial results that have demonstrated significant efficacy and safety.

As part of a collaboration agreement signed in 2019, Sarepta is working with Roche to transform the future for the Duchenne community, 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 via its alliance with Roche.

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 in U.S. 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 in the U.S. 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).

U.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 receipt of financial milestones, our future operations, research and development programs, the potential timing of 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; 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; the possible impact of regulatory decisions by, and any halts imposed by, 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.

Source: Sarepta Therapeutics, Inc.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250513283552/en/

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.