Sarepta Therapeutics Shares New Protein Expression and Safety Results from ENDEAVOR in Participants 2 Years Old at Time of Treatment

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- ***Treatment with ELEVIDYS for Duchenne muscular dystrophy resulted in mean protein expression of 93.87% as measured by western blot in study participants (n=6)***

- ***Safety profile consistent with prior studies of ELEVIDYS and real-world experience***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 16, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported new results from Study 9001-103. Also known as ENDEAVOR, Study 9001-103 is a multi-cohort study of ELEVIDYS (delandistrogene moxeparvovec-rokl) for the treatment of Duchenne muscular dystrophy.

Treatment with ELEVIDYS in the ENDEAVOR participants in cohort 6 who were 2 years old at the time of treatment (n=6), demonstrated mean expression of 93.87% of normal, as measured by western blot, and 79.9 percent dystrophin positive fibers (PDPF), as measured by immunofluorescence. The results were seen in biopsies taken 12 weeks after treatment. Safety in cohort 6 was consistent with clinical and real-world experience with ELEVIDYS. The most common adverse events were nausea and vomiting. Elevated liver enzymes were seen in two patients and resolved with steroid administration.

“The strength of the biomarker results that we are seeing in younger patients is extremely encouraging and we have a meeting with U.S. FDA next month to discuss expanding the ELEVIDYS label to include younger patients,” said Louise Rodino-Klapac, Ph.D., chief scientific officer and head of research and development, Sarepta Therapeutics. “In addition to positive expression results, the safety profile in these patients is consistent with what we’ve seen in prior studies and in patients who have been prescribed treatment.”

Sarepta had previously shared expression and safety from Cohort 4 of ENDEAVOR, in which participants were 3 years old at the time of treatment. In biopsies taken 12 weeks after treatment with ELEVIDYS, participants in Cohort 4 (n=7) demonstrated mean protein levels of 99.64 percent, as measured by western blot. More than 25 patients under the age of 4 have now been treated in our clinical studies.

About ENDEAVOR (Study 9001-103)
Study SRP-9001-103, also known as ENDEAVOR, is an open-label, Phase 1b study assessing the expression and safety of ELEVIDYS (delandistrogene moxeparvovec) in multiple cohorts of male patients with Duchenne. The study has enrolled 55 participants across 7 cohorts, and has dosed participants aged 4-7 at time of treatment, older ambulant and non-ambulant individuals, and individuals younger than age 4.

The primary endpoint in ENDEAVOR is the change from baseline in the quantity of ELEVIDYS micro-dystrophin protein expression measured by western blot at 12 weeks. Secondary outcome measures include change from baseline in micro-dystrophin expression measured by percent dystrophin positive fibers at 12 weeks. Exploratory endpoints include the change in vector genome copies per nucleus, NSAA and certain timed functional tests. Including the initial 12-week period, patients are followed for a total of five years.

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, the potential benefits and risks of ELEVIDYS, and expected plans and milestones, including the potential to expand the label 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, 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 with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; 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; 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; 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.

References:

1. U.S. Centers for Disease Control: https://www.cdc.gov/cytomegalovirus/about/index.html

View source version on businesswire.com: https://www.businesswire.com/news/home/20250516650167/en/

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