Sarepta Therapeutics Submits Efficacy Supplement to Expand the ELEVIDYS Label to include Duchenne Muscular Dystrophy Patients without Restriction to Age or Ambulatory Status

12/22/23

- ***Application includes request for Priority Review from the US FDA***
- ***Sarepta has also submitted the EMBARK postmarketing requirement to the FDA seeking conversion of the ELEVIDYS accelerated approval to traditional approval***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 22, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced submission of an efficacy supplement to the Biologics License Application (BLA) for ELEVIDYS (delandistrogene moxeparvovec-rokl) to expand its labeled indication as follows "[ELEVIDYS is indicated for] *the treatment of Duchenne muscular dystrophy (DMD) patients with a confirmed mutation in the DMD gene."*

The efficacy supplement is supported by results from EMBARK (Study SRP-9001-301), a global, randomized, double-blind, placebo-controlled, Phase 3 clinical study in patients with Duchenne between the ages of 4 through 7 years and data from ENDEAVOR (Study SRP-9001-103), an open label clinical study in patients with Duchenne, that is enrolling patients ages 2 years and older. The supplement was submitted to the U.S. Food and Drug Administration (FDA) with a request for Priority Review. An efficacy supplement is a submitted request for a proposed change to an approved product's labeling, including adding or modifying an indication previously filed with the FDA.

Sarepta has also completed the EMBARK postmarketing requirement (PMR) and submitted the PMR to FDA requesting conversion from accelerated approval to traditional approval.

"Consistent with our patient commitment, Sarepta moved rapidly to submit an efficacy supplement to the biologics license application for ELEVIDYS seeking label expansion to remove age and ambulation restrictions from the approved indication," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "Understanding that Duchenne patients are in a race against time, we appreciate that the Agency also moved quickly to engage and provide us with guidance on the process for reviewing our BLA supplement."

As part of a collaboration agreement signed in 2019, Sarepta Therapeutics 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. Together, the companies are implementing a comprehensive joint clinical development plan to maximize the chances of broad approval and access so that ELEVIDYS can reach as many individuals with Duchenne as rapidly as possible.

Patients and physicians can access more information about ELEVIDYS at www.SareptAssist.com or by calling 1-888-727-3782.

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 dystrophin gene that result in the lack of dystrophin protein – through the delivery of a shortened transgene that codes for the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle. ELEVIDYS is a one-time infusion indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene and is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin in skeletal muscle observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. ELEVIDYS has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

In addition to EMBARK, which serves as the postmarketing confirmatory study, ELEVIDYS has been evaluated in three clinical studies: SRP-9001-101, SRP-9001-102 and SRP-9001-103. Accelerated approval was primarily based on data from SRP-9001-102 and SRP-9001-103.

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:

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS. 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.
- 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.

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 (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, 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 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, business plans, priorities, research and development programs; the potential for our efficacy supplement to expand the ELEVIDYS label to include Duchenne patients without restriction to age or ambulatory status; the potential conversion from accelerated approval to traditional approval for ELEVIDYS; and our collaboration with Roche.

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: the FDA may not approve a supplement to expand the approved label for ELEVIDYS; 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 data may not be sufficient for obtaining regulatory approval; we are subject to uncertainty related to reimbursement policies; 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 with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; continued approval may be contingent upon verification of a clinical benefit in confirmatory trials; the commencement and completion of our clinical trials and announcement of results may be delayed or

prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; 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 of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; 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, many 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, 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 Annual Report on Form 10-K for the year ended December 31, 2022, and 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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