Sarepta Therapeutics Announces Positive Vote from U.S. FDA Advisory Committee Meeting for SRP-9001 Gene Therapy to Treat Duchenne Muscular Dystrophy

5/12/23

– ***Advisory committee voted 8-6 in support of accelerated approval of SRP-9001***

– ***Regulatory action date is May 29, 2023***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 12, 2023-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC) voted 8 to 6 in support of accelerated approval of SRP-9001 (delandistrogene moxeparvovec) for the treatment of ambulatory patients with Duchenne muscular dystrophy with a confirmed mutation in the DMD gene.

"Today's advisory committee outcome is extremely important to the patient community, who are in urgent need of new therapies," said Doug Ingram, president and chief executive officer, Sarepta. "With the May 29 action date our top priority, we will work collaboratively with the FDA to complete the review of our BLA for SRP 9001. We extend our sincere appreciation to the families, clinicians, FDA presenters and committee members who participated in today's panel and to all those who provided input and comments both in the written record and in the open public hearing."

SRP-9001 is intended to treat the underlying cause of Duchenne, which is characterized by mutations in the *dystrophin* gene that results in the lack of dystrophin protein. In the absence of dystrophin, which is required to strengthen and protect muscles, muscles become weakened and damaged. SRP-9001 is intended to deliver a gene that codes for a shortened, functional form of dystrophin to muscle cells. The committee's positive vote is based on the evaluation of the totality of evidence including the SRP-9001 product design as well as biological and empirical data. SRP-9001 is supported by non-clinical evidence in addition to efficacy and safety data from studies 101, 102 and 103 as well as an integrated analysis across these three clinical studies comparing functional results to a propensity-score-weighted external control (EC).

The CTGTAC's vote, while not binding, will be considered by the FDA when making its decision regarding the potential accelerated approval of SRP-9001. The Biologics License Application (BLA) for SRP-9001 is currently under priority review by the FDA with a regulatory action date of May 29, 2023.

About SRP-9001 (delandistrogene moxeparvovec)

SRP-9001 (delandistrogene moxeparvovec) is an investigational gene transfer therapy designed to address the underlying cause of DMD through the targeted production of functional components of dystrophin in muscle tissue. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval. In December 2019, Roche partnered with Sarepta to combine Roche's global reach, commercial presence and regulatory expertise with Sarepta's gene therapy candidate for Duchenne to accelerate access to SRP-9001 for patients outside the United States.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, fatal neuromuscular genetic disease that occurs in approximately one in every 3,500-5,000 newborn males worldwide. DMD is caused by a change or mutation in the gene that encodes instructions for dystrophin. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As the disease progresses, muscle weakness in the lower limbs spreads to the arms and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living such as using the restroom, bathing and feeding. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and patients usually succumb to the disease in their twenties.

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; SRP-9001's potential for accelerated approval; the Company's plans to continue working with the FDA as they complete their review of the SRP-9001 BLA; the potentially transformative benefits of SRP-9001; and that the FDA is not bound by the advisory committee recommendation but takes its advice in consideration when reviewing applications.

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 the BLA for SRP-9001 by the application PDUFA date or at all; we may not be able to comply with all FDA requests, including with respect to our SRP-9001 BLA, 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, as well as the development of our product candidates and our financial and contractual obligations; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; our data for SRP-9001 may not be sufficient for obtaining regulatory 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 with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; 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 the ongoing COVID-19 pandemic; 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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