Sarepta Therapeutics Announces Advisory Committee Meeting will be Held for SRP-9001

3/16/23

– ***Advisory committee meeting to be held in advance of target action date***

– ***Company will hold conference call today at 4:30 p.m. Eastern time***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 16, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that at its late cycle meeting for the SRP-9001 (delandistrogene moxeparvovec) biologics license application (BLA), the U.S. Food and Drug Administration's Office of Therapeutics (OTP) has determined that an advisory committee meeting will be held for SRP-9001 in advance of the May 29, 2023 regulatory action date. SRP-9001 is Sarepta's investigational gene therapy for the treatment of Duchenne muscular dystrophy.

"FDA's decision to hold a public advisory committee meeting on the SRP 9001 BLA is a change from the communicated position at the midcycle meeting. FDA leadership has noted publicly that FDA is interested in exploring the use of surrogate endpoints, biomarkers, and innovative approaches like accelerated approval to advance cell and gene therapies, particularly for rare, life-ending degenerative diseases. It is our understanding that as one of the first gene therapy BLAs founded on a surrogate endpoint, the advisory committee will primarily relate to the totality of evidence supporting the conclusion that the SRP 9001 dystrophin is reasonably likely to predict clinical benefit, the standard for accelerated approval," said Doug Ingram, president and chief executive officer, Sarepta. "While we are disappointed that we must communicate a change in decision after our prior statement on the topic, we are not disappointed with the decision to hold an advisory committee. We had been preparing for an advisory committee meeting from the filing of the BLA in the fall of 2022. We will be well prepared, and look forward to presenting the wealth of evidence supporting the transformative potential of SRP-9001. We would like to thank CBER for moving expeditiously to schedule the advisory committee in advance of our May 29, 2023, regulatory action date, once the change in decision was made."

Conference call details

At 4:30 p.m. ET on March 16, 2023, Sarepta will host a conference call and webcast to discuss this update.

The event will be webcast live under the investor relations section of Sarepta's website at **https://investorrelations.sarepta.com/events-presentations** and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using **this online form**. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

About SRP-9001 (delandistrogene moxeparvovec)

SRP-9001 (delandistrogene moxeparvovec) is an investigational gene transfer therapy intended to deliver SRP-9001 to muscle tissue for the targeted production of functional components of dystrophin. 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 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 potentially transformative benefits of SRP-9001; our understanding from the FDA that the advisory committee will primarily relate to the totality of evidence supporting the conclusion that the SRP 9001 dystrophin is reasonably likely to predict clinical benefit; and expected timelines, plans and milestones, including the regulatory action date and our understanding that the advisory committee will be held prior to the regulatory action date.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. 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 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 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 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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