Sarepta Therapeutics Announces Update on Regulatory Review of SRP-9001

5/24/23

New regulatory action date is June 22, 2023

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 24, 2023-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, today provided the following update on the Biologics License Application (BLA) for SRP-9001 (delandistrogene moxeparvovec), which is currently under review for the treatment of ambulant individuals with Duchenne muscular dystrophy (DMD) who have a confirmed mutation of the *DMD* gene.

- Following discussions with FDA, the Agency has indicated that, subject to the completion of the BLA review, it is working toward potentially granting an accelerated approval for SRP-9001, initially for use in Duchenne patients ages 4-5 years old.
- EMBARK, the global, randomized, double-blind, placebo-controlled Phase 3 trial of SRP-9001, is the proposed confirmatory study. The Agency has informed Sarepta that, in addition to confirming the results of the initial BLA approval, if the trial meets its objectives the Agency intends to entertain a non-age-restricted expansion of the SRP-9001 label based upon the review of the EMBARK data. EMBARK is fully enrolled, with top-line results expected in the fourth quarter of 2023.
- The FDA has also informed Sarepta that it requires modest additional time to complete the review, including final label negotiations and postmarketing commitment discussions, and that it anticipates that the review will be complete by June 22, 2023.

Sarepta will remain in a quiet period for the duration of the BLA review.

About SRP-9001 (delandistrogene moxeparvovec)
SRP-9001 (delandistrogene moxeparvovec) is an investigational gene transfer therapy designed to address the underlying cause of Duchenne 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 Sarepta Therapeutics
Sarepta is on an urgent mission: To 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; a potential accelerated approval for SRP-9001 for 4-5 year olds; the potential for an expansion of the SRP-9001 label upon completion and FDA review of EMBARK; the potentially transformative benefits of SRP-9001; and plans and milestones, including top-line results for EMBARK in the fourth quarter of 2023 and the anticipated regulatory action date of June 22, 2023.

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 June 22, 2023, 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 to secure in a timely manner 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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