Sarepta Therapeutics Initiates Screening in EMERGENE, a Phase 3 Clinical Study of SRP-9003 for the Treatment of Limb-Girdle Muscular Dystrophy Type 2E/R4

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 16, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that screening is underway in Study SRP-9003-301. Also known as EMERGENE, Study 9003-301 is a Phase 3, multi-national, open-label study of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E/R4), or beta sarcoglycanopathy. EMERGENE will enroll 15 participants (ambulatory and non-ambulatory), aged 4 and older, and uses commercially representative process SRP-9003 material.

“We are pleased to share our continued progress in advancing SRP-9003, our investigational gene therapy candidate for LGMD2E, a rare form of LGMD with no treatments beyond symptom management. Early results from the SRP-9003 clinical development program demonstrated significant protein expression at both 12-weeks and two years after treatment as well as functional benefits including slowing progression of this disease, improving mobility, and enhancing the quality of life for individuals living with LGMD2E,” said Louise Rodino-Klapac, Ph.D., executive vice president, chief scientific officer and head of research and development, Sarepta Therapeutics. “In addition to its importance for the LGMD2E community, EMERGENE will inform the clinical development of other programs for LGMD in Sarepta’s pipeline while serving as a pathfinder for viable regulatory pathways to support the development of gene therapies to treat ultra rare diseases.”

A webinar for the limb-girdle community is planned, details will be shared at a future date.

About SRP-9003 (bidridistrogene xeboparvovec)

SRP-9003 (bidridistrogene xeboparvovec) is an investigational gene therapy that uses the AAVrh74 vector, which is designed to be systemically and robustly delivered to skeletal, diaphragm and cardiac muscle, making it an ideal candidate to treat neuromuscular diseases. SRP-9003 is intended to deliver a full-length beta-sarcoglycan transgene and uses the MHCK7 promoter, chosen for its ability to robustly express in the heart, which is critically important for patients with limb-girdle muscular dystrophy Type 2E (LGMD2E), also known as beta-sarcoglycanopathy and LGMDR4, many of whom die from pulmonary or cardiac complications.

About Study SRP-9003-301 (EMERGENE)

EMERGENE, Study 9003-301 is a Phase 3, multinational, open-label study of SRP-9003 for the treatment of LGMD2E in 15 ambulatory and non-ambulatory participants, ages 4 and older. The EMERGENE design incorporates a six-month natural history lead-in. The primary endpoint is expression of beta-sarcoglycan 60 days after dosing. Other endpoints include functional measures through month 60 and safety.

About Limb-girdle Muscular Dystrophy

Limb-girdle muscular dystrophies are genetic diseases that cause progressive, debilitating weakness and wasting that begins in muscles around the hips and shoulders before progressing to muscles in the arms and legs. Sarepta’s six LGMD gene therapy programs in development include LGMD2E/R4, LGMD2D, LGMD2C, LGMD2B, LGMD2L and LGMD2A, which together represent more than 70 percent of known LGMD cases.

Patients with LGMD2E (beta-sarcoglycanopathy) begin showing neuromuscular symptoms such as difficulty running, jumping and climbing stairs before age 10. The disease, which is an autosomal recessive subtype of LGMD, progresses to loss of ambulation in the teen years and often leads to early mortality. There are currently no disease modifying treatments for LGMD2E.

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, technologies and scientific approaches, business plans, priorities, research and development programs; and the potential benefits of SRP-9003.

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: our data 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; 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; 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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