Sarepta Therapeutics Completes Enrollment 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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– ***Sarepta anticipates filing for accelerated approval of SRP-9003 in 2025***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 18, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that enrollment and dosing is complete in EMERGENE (Study SRP-9003-301), a Phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec). SRP-9003 is an investigational gene therapy for the treatment of limb-girdle muscular dystrophy Type 2E/R4 (LGMD2E/R4), or beta-sarcoglycanopathy. EMERGENE is a global study, and the primary endpoint is the biomarker expression of beta-sarcoglycan protein, the absence of which is the sole cause of LGMD2E/R4.

"The completion of enrollment in the EMERGENE study marks a significant milestone to bring a potentially disease-modifying treatment to individuals living with LGMD2E, an ultra-rare form of LGMD with no treatments beyond symptom management. We are committed to securing approval of SRP-9003 as quickly as possible and are now closer to reaching that goal for patients and their families," said Louise Rodino-Klapac, Ph.D., executive vice president, chief scientific officer and head of research and development, Sarepta Therapeutics. "The design of EMERGENE is an important precedent that informs development plans for Sarepta's other LGMD pipeline programs, including our LGMD2D program which we just initiated and our LGMD2C program which we expect to initiate in the first quarter of 2025, and serves as a pathfinder for heterogenous neuromuscular gene therapies more broadly."

Data from EMERGENE are expected in the first half of 2025. Assuming a positive pre-Biologics License Application (BLA) meeting and supportive data from EMERGENE, Sarepta anticipates submitting a BLA to the U.S. Food and Drug Administration seeking accelerated approval for SRP-9003 in 2025.

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/R4 in ambulatory and non-ambulatory participants, ages 4 and older. EMERGENE's primary endpoint is expression of beta-sarcoglycan 60 days after dosing. Secondary outcomes and endpoints include functional measures through month 60 and safety.

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[1,2,3] 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 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.

Patients with LGMD Type 2E/R4 (beta-sarcoglycanopathy) typically 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, frequently progresses to loss of ambulation in the teen years and often leads to early mortality.

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 LinkedIn, X (formerly Twitter), 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, the potential benefits of SRP-9003, including SRP-9003 serving as a pathfinder for certain gene therapy more broadly, and expected plans and milestones, including data from EMERGENE in the first half of 2025 and potentially submitting a BLA in 2025, and initiating our LGMD2C program in the first quarter of 2025.

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, 2023, 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.

Source: Sarepta Therapeutics, Inc.

References:

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2. Mendell JR, et al. Assessment of Systemic Delivery of rAAVrh74.MHCK7.micro-dystrophin in Children With Duchenne Muscular Dystrophy: A Nonrandomized Controlled Trial. JAMA Neurol. 2020 Jun 15;77(9):1-10.
3. Salva MZ, et al. Design of tissue-specific regulatory cassettes for high-level rAAV-mediated expression in skeletal and cardiac muscle. Mol Ther. 2007;15(2):320-329.

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