Sarepta Therapeutics Announces Pipeline Progress for Multiple Limb-Girdle Muscular Dystrophy Programs

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– ***U.S. FDA has confirmed that screening and dosing may proceed in Study SRP-9005-101 for LGMD2C/R5***

– ***Enrollment and dosing completed in Study SRP-9004-102 for LGMD2D/R3***

– ***Data expected for SRP-9003 for LGMD2E/R4 by mid-2025***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 15, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today shared updates from its clinical programs focused on limb-girdle muscular dystrophy (LGMD) subtypes 2C/R5, 2D/R3, and 2E/R4.

- **SRP-9005 for LGMD type 2C/R5:** Following input from the U.S. Food and Drug Administration (FDA), Office of Therapeutic Products (OTP), Sarepta is cleared to proceed with dosing in Study SRP-9005-101 (COMPASS) in the U.S. COMPASS is a first-in-human clinical study of SRP-9005, an investigational gene therapy for LGMD type 2C/R5, or gamma-sarcoglycanopathy.
- **SRP-9004 for LGMD type 2D/R3:** Enrollment and dosing is complete in Study SRP-9004-102 (DISCOVERY). DISCOVERY is a phase 1, proof-of-concept study evaluating safety and expression of the alpha-sarcoglycan protein after treatment with SRP-9004, an investigational gene therapy for the treatment of LGMD type 2D/R3, or alpha-sarcoglycanopathy.
- **SRP-9003 for LGMD type 2E/R4:** Enrollment and dosing is complete in Study SRP-9003-301 (EMERGENE). EMERGENE is a phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of LGMD type 2E/R4, or beta-sarcoglycanopathy. EMERGENE is a global study and the primary endpoint is the biomarker expression of beta-sarcoglycan protein. A pre-Biologics License Application (BLA) meeting has occurred and the OTP has confirmed eligibility for the accelerated approval pathway for the program. Sarepta remains on track to submit a BLA to the U.S. FDA in the second half of 2025.

“There are no disease-modifying treatments approved for patients with any subtype of limb-girdle muscular dystrophy, and the unmet medical need is significant. Following feedback from U.S. FDA, we are pleased to announce that screening can proceed in Sarepta’s first clinical study for a gene therapy for individuals with LGMD type 2C – the fourth LGMD program that Sarepta has advanced into the clinic,” said Louise Rodino-Klapac, Ph.D., executive vice president, chief scientific officer and head of research and development, Sarepta Therapeutics. “In addition to our progress with SRP-9004 and SRP-9005, we remain on track to share data in the first half of this year from the EMERGENE study with SRP-9003. Our confidence in the potential for gene therapy to bring meaningful treatments to patients with rare, genetic based diseases remains high and the rapid progress across our LGMD pipeline is encouraging.”

About Limb-Girdle Muscular Dystrophy

Limb-girdle muscular dystrophies (LGMD) 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. There are more than 30 distinct subtypes of LGMD, each with a unique genetic mutation and distinct symptoms, progression, and treatment approaches.

Sarepta’s leading LGMD pipeline currently has gene therapy programs in different stages of development for LGMD 2B/R2, LGMD 2E/R4, LGMD 2D/R3, LGMD 2C/R5, and LGMD 2A/R1 which together represent more than 70 percent of known LGMD cases. Sarepta is also the sponsor of JOURNEY, a natural history study evaluating disease progression for four LGMD subtypes: 2C/R5, 2D/R3, 2E/R4 and 2A/R1. JOURNEY is actively enrolling globally.

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 SRP-9004 (patidistrogene bexoparvovec)

SRP-9004 (patidistrogene bexoparvovec) is an investigational gene therapy for limb-girdle muscular dystrophy Type 2D (LGMD2D/R3). LGMD2D/R3 causes muscle weakness and primarily affects the muscles around the hips, shoulders, and thighs. SRP-9004 is engineered using 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. Intended to deliver a full-length alpha-sarcoglycan transgene, SRP-9004 uses the tMCK promoter, chosen for its ability to selectively express in skeletal muscle which is critically important for patients with LGMD2D/R3.

About SRP-9005

SRP-9005 is an investigational gene therapy for patients with limb-girdle muscular dystrophy Type 2C (LGMD2C/R5), also known as gamma-sarcoglycanopathy. SRP-9005 is intended to deliver a full-length gamma-sarcoglycan transgene using the AAVrh74 vector. SRP-9005 uses the MHCK7 promoter, chosen for its ability to robustly express in the heart, which is critically important for those with LGMD2C, many of whom die from pulmonary or cardiac complications.

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 (Duchenne) and limb-girdle muscular dystrophies (LGMDs) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases. For more information, please visit **www.sarepta.com** or follow us on **LinkedIn**, **X**, **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

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our future operations, business plans, market opportunities, priorities and research and development programs and technologies; the potential benefits of our technologies, scientific approaches and strategic partnerships; and expected milestones and plans, including beginning dosing in Study SRP-9005-101, sharing data from our EMERGENE study in the first half of the year, and our expectation to file a BLA for SRP-9003 in the second half of 2025.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's 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: 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 expected benefits and opportunities related to our agreements with strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations, challenges and uncertainties inherent in product research and development and manufacturing limitations; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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, some 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, and 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, 2024 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

References

1. Pozsgai ER, et al. Systemic AAV-Mediated b-Sarcoglycan Delivery Targeting Cardiac and Skeletal Muscle Ameliorates Histological and Functional Deficits in LGMD2E Mice. Mol. Ther. 2017 Apr 5;25(4):855-869.
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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