Sarepta Therapeutics Announces Initiation of VOYAGENE, a Clinical Study of SRP-9003 for the Treatment of Limb-Girdle Muscular Dystrophy Type 2E/R4

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 17, 2023-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the first patient has been dosed in Study SRP-9003-102. Also known as VOYAGENE, Study 9003-102 is a phase 1 study of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of limb-girdle muscular dystrophy Type 2E/R4 (LGMD2E). VOYAGENE is a U.S.-only study enrolling ambulant patients aged 18 years or older and non-ambulant patients, ages 4-50 years, using clinical process SRP-9003 material.

“Sarepta has previously disclosed positive expression and function data from our initial clinical study, Study SRP-9003-101, exploring multiple doses of SRP-9003 in a younger, ambulant population. The VOYAGENE study, which also utilizes clinical material, provides an opportunity to generate additional data in a broader population of patients while we finalize plans for a global, Phase 3 study using commercially representative process material that we intend to begin later this year,” said Louise Rodino-Klapac, Ph.D., executive vice president, chief scientific officer and head of research and development, Sarepta Therapeutics. “Sarepta is applying lessons from across our gene therapy portfolio to help advance the development of our investigational LGMD programs as efficiently as possible. Current treatments for LGMD are limited to symptom management and we remain fully committed to working with urgency to bring forward new treatments that can slow the progression of this disease, improve mobility and enhance the quality of life for individuals living with LGMD2E and other LGMD subtypes.”

More information on the VOYAGENE study is available at https://genesislgmd.com/study/voyagene.

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 peripheral neuromuscular diseases. SRP-9003 also 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 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/R3, LGMD2C/R5, LGMD2B/R2, LGMD2L/R12 and LGMD2A/R1, which together represent more than 70 percent of known LGMD cases.

Patients with LGMD2E 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 is currently no treatment or cure 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 statements relating to our potential to apply lessons from across our gene therapy portfolio to help advance the development of our investigational LGMD programs as efficiently as possible, our commitment to working with urgency to bring forward new treatments that can slow the progression of LGMD, improve mobility and enhance the quality of life for individuals living with LGMD 2E and other LGMD subtypes, and expected plans and milestones, including additional data from our VOYAGENE study and our plan to begin our potentially global Phase 3 study for SRP-9003 later this year.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: 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; that we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, due to a variety of 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, 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 COVID-19 pandemic; success in pre-clinical trials and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; 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; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2021, and most recent Quarterly Report on 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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