Sarepta Therapeutics Executes Licensing Agreement for Gene Therapy Program from Nationwide Children's Hospital to Treat Limb-Girdle Muscular Dystrophy Type 2A

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– ***Limb-girdle muscular dystrophy type 2A is the most common form of LGMD, accounting for a third of LGMD diagnoses***

– ***Sarepta's unrivaled portfolio of investigational gene therapies for LGMD offers the potential to address six LGMD subtypes, which together represent more than 70% of all known LGMDs***

CAMBRIDGE, Mass., Aug. 04, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that upon completion of a number of preclinical and safety studies, it had executed an exclusive license agreement for an investigational gene therapy candidate, calpain 3 (CAPN-3), to treat Limb-girdle muscular dystrophy type 2A (LGMD2A), developed by the Abigail Wexner Research Institute at Nationwide Children's Hospital (Nationwide Children's).

LGMDs represent a group of distinct genetic neuromuscular diseases with a generally common set of symptoms, including progressive, debilitating weakness and wasting that begins in muscles around the hips and shoulders before progressing to muscles in the arms and legs. Many LGMD sub-types are significantly life-limiting and often life-ending diseases. Also known as calpainopathy, LGMD2A is caused by mutations in the CAPN-3 gene and is the most common type of LGMD, accounting for almost a third of cases.

"Treatment plans for LGMD2A are currently limited to physical therapy, assistive devices and surgery for complications. We're excited about the opportunity to transform patient care for this significantly life-limiting disease by advancing the CAPN-3 program following extensive pre-clinical work by the team at Nationwide Children's. Preclinical research conducted to date has provided early proof of concept for CAPN-3 in LGMD2A and supports further advancement," said Louise Rodino-Klapac, Sarepta's executive vice president and chief scientific officer. "We intend to build off the knowledge we have gained from our lead investigational gene transfer programs for Duchenne muscular dystrophy and LGMD2E, as the CAPN-3 program also uses the AAVrh74 vector to address another well-characterized genetic disease. Sarepta's commitment and research investment in LGMD is unparalleled and we continue to work towards advancing all of our LGMD programs as quickly as possible."

Like SRP-9001, Sarepta's lead investigational gene transfer therapy for Duchenne muscular dystrophy, and the Company's five other LGMD programs, the LGMD2A program uses the AAVrh74 vector, designed to systematically and robustly deliver treatment to skeletal muscle, including the diaphragm, making it an ideal candidate to treat muscle disease.

The preclinical work on the CAPN-3 program in LGMD2A has been led by Zarife Sahenk, M.D., Ph.D., attending neurologist at Nationwide Children's, Director of Clinical and Experimental Neuromuscular Pathology at The Research Institute at Nationwide Children's and Professor of Pediatrics, Pathology and Neurology at The Ohio State University College of Medicine.

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, LGMD2D, LGMD2C, LGMD2B, LGMD2L and LGMD2A, which together represent more than 70 percent of known LGMD cases.

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 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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding the potential benefits of the licensing agreement; the design of the AAVrh74 vector to systematically and robustly deliver treatment to skeletal muscle, including the diaphragm, making it an ideal candidate to treat muscle disease; the potential of our portfolio of investigational gene therapies for LGMD to address six LGMD subtypes, which together represent more than 70% of all known LGMDs; and our plan to continue to advance all of our LGMD programs as quickly as possible.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: the expected benefits and opportunities related to the licensing agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development. In particular, activities under the license may not result in any viable treatments suitable for commercialization due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreement; success in preclinical trials does not ensure that later clinical trials will be successful; Sarepta may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, due to a variety of reasons, many of which may be outside of Sarepta's 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 Sarepta's product candidates and the COVID-19 pandemic; even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected revenues from the sale of such products; if the actual number of patients living with LGMD2A is smaller than estimated, Sarepta's revenue and ability to achieve profitability may be adversely affected; 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, 2020, 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.

Source: Sarepta Therapeutics, Inc.

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