Sarepta Therapeutics to Present Results from its Gene Therapy and RNA Platforms at the 2021 Annual MDA Clinical and Scientific Conference

3/15/21

-- Ten abstracts, including four podium presentations, reflect Sarepta's ongoing commitment to advancing genetic medicine for rare neuromuscular disease and facilitating greater understanding of these devastating conditions --

CAMBRIDGE, Mass., March 15, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, will present at the Muscular Dystrophy Association (MDA) Annual Clinical and Scientific Conference, which will take place virtually March 15-18, 2021. Among the research that will be presented:

- New, two- and one-year data including expression data from muscle biopsies taken two years post-treatment, from Study 9003-101 of SRP-9003, an investigational gene therapy for the treatment of limb-girdle muscular dystrophy (LGMD) type 2E/R4;
- Data from Part 1 of Study 9001-102, an ongoing clinical trial of SRP-9001, Sarepta's investigational gene therapy for Duchenne muscular dystrophy and pre-clinical approaches to the challenge of pre-existing antibodies; and,
- An analysis of time to loss of ambulation in patients taking eteplirsen, a phosphorodiamidate morpholino oligomer (PMO) for the treatment of Duchenne, compared to standard of care.

All posters are available on-demand throughout the Congress beginning on Monday, March 15, 2021 at 6:00 a.m. ET. Podium presentations will take place on Thursday, March 18, 2021. The full MDA 2021 Virtual Congress program is available here: https://mdaconference.org.

Podium Presentations:

Title	Program	Date, Time
Micro-dystrophin Gene Therapy Delivery and Therapeutic Plasma Exchange in Non-Human Primates	SRP-9001	Thurs., March 18, 2021 10:30-10:45 AM ET
A Randomized, Double-Blind, Placebo-Controlled, Gene-Delivery Clinical Trial of rAAVrh74.MHCK7.micro-dystrophin for Duchenne Muscular Dystrophy	SRP-9001	Thurs., March 18, 2021 3:30-3:45 PM ET
Safety, β-sarcoglycan Expression, and Functional Outcomes from Systemic Gene Transfer of rAAVrh74.MHCK7.SGCB in Limb Girdle Muscular Dystrophy Type 2E/R4	SRP-9003	Thurs., March 18, 2021 4:30-4:45 PM ET
Delay in Duchenne Muscular Dystrophy Progression with Eteplirsen: Longer Time to Loss of Ambulation Versus Standard of Care	Eteplirsen	Thurs., March 18, 2021 5:45-6:00 PM ET

Poster Presentations:

Poster #	Title
6	Biological Efficacy of the Peptide-Conjugated Phosphorodiamidate Morpholino Oligomer SRP-5051 in Preclinical Models of Duchenne Muscular Dystrophy
54	Casimersen Treatment in Eligible Patients with Duchenne Muscular Dystrophy: Safety, Tolerability, and Pharmacokinetics Over 144 Weeks of Treatment
90	Patterns of Clinical Progression Among Patients with Autosomal Recessive Limb-Girdle Muscular Dystrophy (LGMDR): A Systematic Review
92	Progression to Loss of Ambulation (LOA) Among Patients with Autosomal Recessive Limb-Girdle Muscular Dystrophy (LGMDR): A Systematic Review
104	Health-related quality of life (HRQoL) Associated with Duchenne Muscular Dystrophy (DMD): A Study Using the Health Utilities Index Mark 3 (HUI3)
113	NorthStar Ambulatory Assessment (NSAA) and Health Utilities Index (HUI) Scores are Weakly Correlated Among Boys with Duchenne Muscular Dystrophy (DMD)

Presentations will be archived on the events and presentations page in the Investor Relations section of www.sarepta.com for one year following their presentation at MDA.

About Sarepta Therapeutics
At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare

disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy 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.

Source: Sarepta Therapeutics, Inc.

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