Long-term functional data from Sarepta Therapeutics' Most Advanced Gene Therapy Programs to be Presented at Upcoming Annual Congress of the World Muscle Society

9/14/20

-- Webcast conference call to be held on Monday, Sept. 28, 2020 at 8:30 a.m. Eastern Time --

-- Additional poster presentations at WMS will highlight data from Sarepta's RNA and gene therapy programs --

CAMBRIDGE, Mass., Sept. 14, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that new data from its most advanced gene therapy programs will be presented at the WMS25 Virtual Congress, the 25th International Annual Congress of the World Muscle Society, being held Sept. 28 – Oct. 2.

Sarepta will host a webcast and conference call on Monday, Sept. 28, 2020 at 8:30 a.m. ET, to discuss the results, which include two-year functional data from Study 101 of SRP-9001 for Duchenne muscular dystrophy and 18-month functional results from Cohort 1 in the study of SRP-9003 for Limb-girdle muscular dystrophy Type 2E.

This will be webcast live under the investor relations section of Sarepta's website at https://investorrelations.sarepta.com/events-presentations and will be archived there following the call for one year. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 6793650. Please specify to the operator that you would like to join the "Long-term Functional Data from Sarepta's Gene Therapy Programs" call.

In total, Sarepta will present 16 abstracts at this year's meeting. All posters will be available on-demand throughout the Congress beginning on Monday, Sept. 28 at 7:00 a.m. EST. The full WMS25 Virtual Congress program is available here: https://www.wms2020.com/programme/.

Gene Therapy:

TITLE	PROGRAM	POSTER #
Treatment of Aged Mice and Long-term Durability of AAV-Mediated Gene Therapy in Two Mouse Models of Limb Girdle Muscular Dystrophy	SRP-9003, SRP-9004	P.137
Expression-Functional Correlation and Validation of a Surrogate Marker for DAPC Restoration in LGMD2E Mouse Model	SRP-9003	P.139
Systemic Gene Transfer with rAAVrh74.MHCK7.SGCB Increased β-sarcoglycan Expression in Patients with Limb Girdle Muscular Dystrophy Type 2E	SRP-9003	P.140
Evaluation of the Lipid-Binding and Stability Properties of Recombinant Dystrophin Spectrin-Like Repeat Constructs	SRP-9001	P.206
Systemic Gene Transfer with rAAVrh74.MHCK7.micro-dystrophin in Patients with Duchenne Muscular Dystrophy	SRP-9001	P.280
Systemic Dose-Finding Study with AAV-Mediated γ-Sarcoglycan Gene Therapy for Treatment of Muscle Deficits in LGMD2C Mice	SRP-9005	P.138

RNA Platform:

TITLE	PROGRAM	POSTER #
Long-term Safety and Efficacy of Golodirsen in Male Patients with Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping	Golodirsen	P.283
Casimersen Treatment in Eligible Patients with Duchenne Muscular Dystrophy: Safety, Tolerability, and Pharmacokinetics Over 144 Weeks of Treatment	Casimersen	P.288
Open-Label Evaluation of Eteplirsen in Patients With Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping: PROMOVI Trial	Eteplirsen	P.289
Delay in Duchenne Muscular Dystrophy Progression with Eteplirsen: Attenuation of Pulmonary Decline and Projected Freedom from Continuous Ventilation	Eteplirsen	P.290
Real-world Evidence of Eteplirsen Treatment Effects on Duchenne Muscular Dystrophy Related Health Outcomes Using Claims Data in the United States	Eteplirsen	P.291

Natural history and other presentations:

TITLE	POSTER #
Development of Cardiomyopathy, Respiratory Insufficiency and Loss of Ambulation in Becker Muscular Dystrophy: A Systematic Literature Review	P.41
Disease Attributes Most Important from a Societal Perspective: A Case Study Involving Duchenne Muscular Dystrophy	P.56
Identification of Disease Progression Stages in Patients with Duchenne Muscular Dystrophy Using Administrative Claims Data in the United States	P.119
The Age at Loss of Ambulation Among Patients with Limb-Girdle Muscular Dystrophy (LGMD) Subtype 2: A Systematic Review	P.142

Rasch Analysis of the Pediatric Quality of Life Inventory 4.0 Generic Core Scales Administered to Patients with Duchenne Muscular Dystrophy	P.345

Presentations will be archived under the events and presentations section of the Sarepta Therapeutics website at www.sarepta.com for one year following their presentation at WMS25.

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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