Sarepta Therapeutics Presents New Data from its Gene Therapy and RNA Platforms at World Muscle Society 2022

10/7/22

CAMBRIDGE, Mass., Oct. 07, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, will share new data from across its genetic medicine portfolio at the 27th International Hybrid Annual Congress of the World Muscle Society 2022 Congress (WMS 2022), taking place Oct. 11-15, 2022, in Halifax, Nova Scotia, Canada.

Data includes a late-breaking real-world evidence presentation on eteplirsen in treated patients with Duchenne amenable to exon 51 skipping, in addition to research from the Company's gene therapy platform including preclinical data supporting the functionality of the SRP-9001 (delandistrogene moxeparvovec) Sarepta's investigational gene therapy for the treatment of Duchenne.

"As a leader in genetic medicine, the body of data generated from our science is a reflection of our commitment to our mission to advance science in the interest of patients. Our scientific presence at World Muscle demonstrates our commitment and helps further our collective understanding of these conditions to advance new treatments for Duchenne and other rare neuromuscular diseases," said Louise Rodino-Klapac, Ph.D., executive vice president and chief scientific officer, Sarepta Therapeutics.

The full WMS 2022 program is available at https://www.wms2022.com/page/programme.

RNA Platform Presentations

Poster #	Title	Program	Date, Time
P.132	Casimersen in Patients with Duchenne Muscular Dystrophy Amenable to Exon 45 Skipping: Interim Results from the Phase 3 ESSENCE Trial	Casimersen	Oct. 14, 2022 1:30-3:00 PM EDT (2:30-4:00 PM ADT
LSP.31	Interim Analysis of EVOLVE: A Long-term Observational Study Evaluating Eteplirsen, Golodirsen, or Casimersen in Routine Clinical Practice	PMO	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)

Gene Therapy Presentations (does not reflect encore presentations)

Poster #	Title	Program	Date, Time
LSP.35	Analysis of adeno-associated virus vector shedding following treatment with delandistrogene moxeparvovec, an investigational gene therapy for Duchenne muscular dystrophy	SRP-9001	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)
VP.82	PK/PD modelling to inform clinical development of an adeno-associated virus gene transfer therapy for Duchenne muscular dystrophy	SRP-9001	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)
P.197	Evaluating pharmacology and efficacy of delandistrogene moxeparvovec in DMD^{mdx} rats	SRP-9001	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)

Health Economic Outcomes Research (HEOR)

Poster #	Title	Date, Time
P.70	Rasch Analysis of the PROMIS Parent Proxy Item Banks Administered to Caregivers of Patients with Duchenne Muscular Dystrophy	Oct. 12, 2022 3:00-4:30 PM EDT (4:00-5:30 PM ADT)
LSP.22	Estimating Health State Utilities in Duchenne Muscular Dystrophy (DMD) using the EQ5D and Health Utilities Index (HUI)	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)
LSP.30	Survival in Eteplirsen-Treated vs DMD Natural History Controls: An Indirect Treatment Comparison Using Real-World Data	Oct. 14, 2022 4:00-5:00 PM EDT (5:00-6:00 PM ADT)

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 related to our research and development programs, technologies, scientific approaches and product candidates and our mission to advance science and new treatments for Duchenne and other rare neuromuscular diseases in the interest of patients.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its products to U.S. and ex-U.S. markets for various reasons 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 Sarepta's product candidates; 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.

Source: Sarepta Therapeutics, Inc.

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