Sarepta Announces First Clinical Data from siRNA Pipeline Targeting FSHD1 and DM1

3/25/26

- ***In early clinical results, Sarepta's αvβ6 integrin-targeted siRNA approach achieves high muscle concentrations without dose limiting toxicity for FSHD1 and DM1***
- ***Company to host investor call on March 25, 2026, at 8:30 a.m. Eastern time***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 25, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today shared the first clinical results from two of its siRNA programs for neuromuscular diseases.

Early results from Phase 1/2 ascending dose studies of SRP-1001 for facioscapulohumeral muscular dystrophy type 1 (FSHD1) and SRP-1003 for myotonic dystrophy type 1 (DM1) demonstrated dose- dependent muscle exposure, early biomarker effects, and favorable tolerability, reinforcing scientific confidence in the potential for differentiated benefits of the αvβ6 integrin-targeted delivery platform. In addition, the Company has generated proof-of-concept data which found that after a single dose, both SRP-1001 and SRP-1003 support reduction, or knockdown, of the target protein or mRNA. In both studies, the majority of adverse events were mild to moderate and were not dose dependent.

For rare, genetic diseases, such as FSHD and DM1, which are caused by overexpression of mutant proteins or toxic mRNA, RNA-targeted therapies hold significant promise but have been limited by rapid degradation of drug before it reaches the intended cells. SRP-1001 and SRP-1003 are each designed with an optimized siRNA chemistry and a proprietary, αvβ6 integrin-targeted ligand, intended to enable the siRNA to enter the cell and penetrate muscle tissue. Through this targeted delivery approach, these investigational treatments aim to overcome some of the delivery and safety challenges of other approaches.

"We are pleased that these early clinical results showed high levels of siRNA delivery to muscle, with no saturation of muscle siRNA uptake or dose-limiting safety signals to date. We believe this supports the differentiated potential of this siRNA platform and strengthens our belief that this approach could meaningfully change the treatment landscape for patients with FSHD and DM1," said Louise Rodino-Klapac, Ph.D., President, Research & Development and Technical Operations. "These preliminary clinical data show consistent dose-dependent increases in plasma and muscle drug exposures across clinical and nonclinical studies and suggest that the αvβ6 integrin-targeting ligand mediates robust siRNA muscle delivery, which we hypothesize will ultimately enable higher dosing and translate into clinical efficacy for patients with FSHD1 and DM1."

Investor Webcast Information
The Company will host an investor call on March 25, 2026, at 8:30 a.m. Eastern time to discuss the data in greater detail. The event will be webcast live under the investor relations section of Sarepta's website at **https://investorrelations.sarepta.com/events-presentations** and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using **this online form**. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

About FSHD and SRP-1001
Facioscapulohumeral muscular dystrophy (FSHD) is a rare genetic disease that causes progressive weakness of skeletal muscles. The disease typically affects muscles of the face, shoulder girdle, upper limbs, abdomen, pelvis, and legs, although the pattern and severity of muscle involvement vary widely among individuals. FSHD is caused by abnormal activation of the *DUX4* gene on chromosome 4, leading to production of the DUX4 protein, which is toxic to muscle cells and drives muscle degeneration. There is currently no cure for FSHD, and no disease modifying treatments are available. Approximately 16,000 individuals in the United States are diagnosed with FSHD.

SRP-1001 is an investigational siRNA treatment designed to reduce, or knock down, the production of DUX4 protein in skeletal muscle in patients living with FSHD1. Study 1001-101 is combined Phase 1/2, single ascending dose (SAD)/multiple ascending dose (MAD), randomized, placebo-controlled trial in participants with FSHD1 aged 16 through 70.

About DM1 and SRP-1003
Myotonic dystrophy type 1 (DM1) is the most common form of adult-onset muscular dystrophy and is caused by a repeat expansion in the *DMPK* gene. DM1 is a progressive, multisystem disorder that affects skeletal and smooth muscle, as well as the heart, respiratory system, eye, endocrine, gastrointestinal, and central nervous systems. The disease is commonly associated with muscle weakness, daytime sleepiness, breathing difficulties, and cardiac conduction abnormalities. There is currently no cure for DM1, and no disease modifying treatments are available. Approximately 40,000 individuals in the United States are diagnosed with DM1.

SRP-1003 is an investigational siRNA treatment for DM1 and is designed to target and knockdown, or silence, the *DMPK* mRNA in target cells. Study SRP-1003-101 is a first-in-human, Phase 1/2, SAD/MAD, randomized, placebo-controlled clinical trial being conducted in individuals with DM1 aged 18 to 65.

About Sarepta's siRNA Platform
Sarepta's next-generation siRNA platform is focused on chronically administered therapies for neurodegenerative and pulmonary diseases and includes the following investigational treatments:

- SRP-1001 for Facioscapulohumeral muscular dystrophy (FSHD)
- SRP-1002 for Idiopathic Pulmonary Fibrosis (IPF)
- SRP-1003 for Myotonic dystrophy type 1 (DM1)

- SRP-1004 for Spinocerebellar ataxia type 2 (SCA2)
- SRP-1005 for Huntington's disease (HD)

Sarepta is advancing these programs, as well as preclinical programs for Spinocerebellar ataxia type 1 (SCA1) and Spinocerebellar ataxia type 3 (SCA3) and additional discovery targets in muscle or central nervous system disorders, under an exclusive license with Arrowhead Pharmaceuticals.

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 a leadership position in Duchenne muscular dystrophy (Duchenne) and are building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases. For more information, please visit **www.sarepta.com** or follow us on **LinkedIn**, **X**, **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 presentation contains forward-looking statements. Any statements contained in this presentation 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 priorities, technologies and research and development programs; early and limited results from our Phase 1/2 clinical trials of SRP-1001 and SRP-1003; and the potential benefits of SRP-1001, and SRP-1003, and Arrowhead Pharmaceuticals' TRiM™ platform.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results; differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials; interim, initial, "topline" and preliminary data from our clinical trials that we announce or publish from time to time may differ materially from final data as more patient data become available, including data and analyses of additional cohorts; pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and the different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results; even if we believe data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval, including that FDA or foreign regulatory authorities could interpret these data in different ways from us; we rely on third parties, including in some cases our strategic partners, to conduct some aspects of our early-stage research and pre-clinical and clinical development, and accordingly, the inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development, including delayed timelines; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; we face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products before our candidates; our product candidates may cause undesirable side effects, result in new safety signals or have other properties that could delay or prevent regulatory approval of product candidates; we may not be able to execute on our business plans, including meeting expected or planned regulatory milestones and timelines, clinical development plans, and bringing products to markets for various reasons including possible limitations of 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 our product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K 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 presentation. 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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