Sarepta Provides Progress Update for SRP-1003, its Investigational siRNA treatment for Myotonic Dystrophy Type 1

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 24, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today shared progress in the Phase 1/2 multiple ascending dose (MAD) clinical study of SRP-1003 (formerly ARO-DM1), an investigational small interfering RNA (siRNA) therapeutic for the treatment of type 1 myotonic dystrophy (DM1).

Cohorts 1 (1.5 mg/kg) and 2 (3 mg/kg) of the study are complete, and cohort 3 (4.5 mg/kg) is fully enrolled and ongoing. Following a positive, pre-specified drug safety committee review, the study advances with additional drug escalating cohorts. Having achieved the pre-specified patient enrollment target, a second milestone payment of USD$200 million will be made to Arrowhead Pharmaceuticals within 60 days.

Patients are currently being dosed in cohort 4 (6 mg/kg) with plans to initiate dosing in the final cohort, cohort 5 (12 mg/kg), in early 2026.

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

- Facioscapulohumeral muscular dystrophy (FSHD)
- Myotonic dystrophy type 1 (DM1)
- Spinocerebellar ataxia type 2 (SCA2)
- Idiopathic Pulmonary Fibrosis (IPF)
- Huntington's disease (HD)

Sarepta is also pursuing preclinical programs for Spinocerebellar ataxia type 1 (SCA1) and Spinocerebellar ataxia type 3 (SCA3) and has an exclusive collaboration with Arrowhead Pharmaceuticals to develop therapies for skeletal muscle diseases, with plans to pursue up to six discovery targets in muscle or central nervous system disorders.

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 (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 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 priorities and research and development programs and technologies, including the siRNA platform and its potential benefits; and our payment of the milestone pursuant to the collaboration agreement with Arrowhead.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; 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 or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; 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 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 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.

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