Sarepta Therapeutics Applauds Addition of Duchenne Muscular Dystrophy to the U.S. Recommended Uniform Screening Panel (RUSP)

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- U.S. Federal recommendation for Duchenne on newborn screening panel signals benefits of early detection

- Early diagnosis in Duchenne is now more essential than ever, as available treatments can help slow disease progression and preserve mobility

CAMBRIDGE, Mass.-- Dec. 17, 2025 -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, applauds the addition of Duchenne muscular dystrophy (Duchenne) to the U.S. Recommended Uniform Screening Panel (RUSP). The inclusion of Duchenne on the RUSP represents a pivotal advancement for the Duchenne community, encouraging broader newborn screening at the state level and empowering more families with early diagnosis and timely information to pursue earlier care, including access to available therapies and clinical trials.

"The average age of Duchenne diagnosis, 5 years, has remained unchanged for nearly 30 years. By age 5, Duchenne patients have already experienced significant muscle degeneration, and muscle lost cannot be regained. As HHS noted, 'approved gene therapies have been shown to dramatically improve outcomes in managing the disease.' Early diagnosis and treatment are critical to help children keep their abilities longer," said Diane Berry, Ph.D., executive vice president and chief global policy & advocacy officer, Sarepta. "We are grateful to Parent Project Muscular Dystrophy and the Muscular Dystrophy Association for their years-long commitment to drive newborn screening data collection through pilots, and submitting the comprehensive RUSP nomination package, and thankful for the leadership of families who have advocated across the country.

Dr. Berry continued, "With the approval of the first exon-skipping therapies and the only FDA-approved gene therapy, the treatment landscape for Duchenne has been transformed, with more treatments on the horizon. We commend the Department of Health and Human Services for recognizing the urgent need for early identification and taking action to change the trajectory of this disease. Our work is far from done—Sarepta will continue to support community-led efforts to accelerate implementation nationwide, ensuring families can make informed decisions.

Each state administers their own newborn screening programs, and states look to federal recommendations – the RUSP – to inform what conditions they add to their panels. Adding a condition to the RUSP signals the importance of early detection and may accelerate adoption across states. Fourteen states have laws requiring that they align their screening panels with conditions on the RUSP and so they may now begin the process of reviewing Duchenne for inclusion on their state panel. Ten states have already passed legislation or approved the addition of Duchenne to their newborn screening panels.

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**.

Forward-Looking Statements

This statement contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements relating to our future operations, ELEVIDYS, planned clinical trials and the timing of such trials, the potential benefits of an enhanced immunosuppression regimen, and ongoing interactions with FDA related to ELEVIDYS.

Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials are positive, 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 our products; 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; we may not be able to comply with all FDA requests in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; and those risks identified under the heading "Risk Factors" in our 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 herein. 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.

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.

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