Sarepta Therapeutics Announces Approval of Clinical Trial Application for SRP-1005, Its Investigational Treatment for Huntington's Disease

2/4/26

– ***The first-in-human clinical study of SRP-1005, known as INSIGHTT, is expected to begin in the second quarter of 2026***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 4, 2026-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that Medsafe, the New Zealand Medicines and Medical Devices Safety Authority, has granted approval for its clinical trial application (CTA) for Study SRP-1005-101, also known as INSIGHTT. Sarepta expects to initiate this first-in-human clinical trial of SRP-1005 (formerly ARO-HTT) in the second quarter of 2026. SRP-1005 is an investigational small interfering RNA (siRNA) therapeutic for the treatment of Huntington's Disease.

INSIGHTT is a Phase 1, multi-center, dose escalation study that will evaluate the safety and tolerability of subcutaneous dosing of SRP-1005 in approximately 24 participants. SRP-1005 leverages an advanced TfR1 (transferrin receptor protein 1) approach that uses monovalent fragment antigen binding (fAb) designed for efficient delivery to the central nervous system. Subcutaneous administration is intended to avoid saturating the transferrin receptor and achieve constant and robust penetration across the blood brain barrier. Preclinical data with SRP-1005 have demonstrated the potential for significant protein knockdown in key deep brain regions, including the putamen and caudate, as well as the temporal and frontal cortexes.

About Huntington's Disease
Huntington's Disease (HD) is a rare and ultimately fatal inherited neurodegenerative disorder which is passed down from generation to generation within affected families. HD is caused by a mutation in the gene for a protein called huntingtin, or HTT, which leads to progressive deterioration of nerve cells in the brain, affecting cognition, movement, and behavior. In the U.S. about 40,000 people are living with symptomatic HD, and an additional 200,000 individuals carry the genetic mutation and are at risk of developing symptoms. Symptoms typically appear between ages 30 and 50 and worsen over time, with every child of an affected parent having a 50% chance of inheriting the condition. There is currently no known cure or approved disease-modifying treatments that address the underlying cause.

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 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 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, technologies and research and development programs; the potential benefits of SRP-1005; and expected plans and milestones, including our expectation to initiate SRP-1005 in the second quarter of 2026.

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 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; if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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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