U.S. FDA Grants Platform Technology Designation to the Viral Vector Used in SRP-9003, Sarepta's Investigational Gene Therapy for the Treatment of Limb Girdle Muscular Dystrophy Type 2E/R4

6/4/25

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 4, 2025-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that the rAAVrh74 viral vector used in the investigational gene therapy SRP-9003 (bidridistrogene xeboparvovec) for the treatment of limb-girdle muscular dystrophy type 2E/R4, has been granted platform technology designation by the U.S. Food & Drug Administration.

"This is one of the first programs to receive platform technology designation and an important recognition by FDA of the reproducibility and adaptability of this technology across multiple therapeutic programs," said Louise Rodino-Klapac, Ph.D., chief scientific officer and head of research & development, Sarepta. "The designation underscores and reinforces the consistency of the data we have seen with this AAVrh74 in multiple clinical programs and is yet another example of Sarepta's continued commitment to accelerating the development of potentially transformative treatments for patients with rare genetic diseases like LGMD type 2E/R4."

In keeping with FDA's pursuit of science-driven efficiency, the platform technology designation program is a critical tool that will help support rare disease therapies by streamlining drug development, manufacturing, and review processes for drug product applications by incorporating data from designated technologies.

To be eligible for this designation, the technology must be a well-understood and reproducible technology – such as a nucleic acid sequence, molecular structure, mechanism of action, delivery method, vector, or a combination of any such technologies – and can support the development of multiple drugs or biologics. A platform is also eligible for consideration if it is incorporated in an approved drug and preliminary evidence demonstrates that the technology has the potential to be incorporated in or utilized by more than one drug without an adverse effect on quality, manufacturing, or safety, including using the same manufacturing process. Once granted, the designation enables sponsors to leverage prior data from the platform to support investigational new drug applications (INDs), new drug applications (NDAs) or biologic license applications (BLAs).

About SRP-9003 (bidridistrogene xeboparvovec)
SRP-9003 (bidridistrogene xeboparvovec) is an investigational gene therapy designed to be systemically and robustly delivered to skeletal, diaphragm and cardiac muscle, making it an ideal candidate to treat neuromuscular diseases. SRP-9003 is intended to deliver a full-length beta-sarcoglycan transgene and uses the MHCK7 promoter, chosen for its ability to robustly express in the heart which is critically important for patients with limb-girdle muscular dystrophy Type 2E (LGMD2E), also known as beta-sarcoglycanopathy and LGMDR4, many of whom die from pulmonary or cardiac complications.

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 limb-girdle muscular dystrophies (LGMDs) 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 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, research and development programs, product candidates, the potential benefits of AAVrh74 and the potential for our therapies to be transformative for patients living with rare diseases.

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; success in 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 with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; we may not be able to comply with all FDA requests 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; the possible impact of regulatory decisions by, and any halts imposed by, 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.

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