Sarepta Therapeutics Announces Addition of Kenneth Fischbeck, M.D. and Matthew Wood M.D., Ph.D. to the Company's Strategic and Scientific Advisory Board

CAMBRIDGE, Mass.—(GLOBE NEWSWIRE)—March 30, 2017— Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, today announced the addition of Kenneth Fischbeck, M.D., and Matthew Wood, M.D., Ph.D., to the Company's Strategic and Scientific Advisory Board. Dr. Fischbeck and Dr. Wood join Dr. Beverly Davidson and Dr. Louis Kunkel, who were appointed to the board at the time of its formation in 2015.

"We are honored and excited to have Dr. Fischbeck and Dr. Wood join our advisory board," said Edward Kaye, Sarepta's chief executive officer. "Following the approval of our exon 51 skipping drug, EXONDYS 51™ (eteplirsen) Injection, our board members helped in guiding our strategy to move additional exon skipping therapies into later stage clinical development, rapidly advance our PPMO platform into the clinic, explore complimentary therapies to further improve the lives of patients with DMD, and expand our partnerships and research collaborations. The addition of Dr. Fischbeck and Dr. Wood adds to our board's diversity of experience with Duchenne and neurological diseases and provides Sarepta with unique counsel for our research and development efforts."

Kenneth Fischbeck, M.D., received A.B. and A.M. degrees from Harvard University and a M.D. degree from Johns Hopkins. After a medical internship at Case Western Reserve University and a neurology residency at the University of California in San Francisco, he did postdoctoral research on muscular dystrophy at the University of Pennsylvania. In 1982, he joined the faculty in the Neurology Department at the University of Pennsylvania Medical School. In 1998, he joined the National Institute of Neurological Disorders and Stroke (NINDS) as Chief of the Neurogenetics Branch. He received the Cotzias Award from the American Academy of Neurology and the Jacoby Award from the American Neurological Association, and he was elected to the Institute of Medicine. His research group is identifying the causes and studying the mechanisms of hereditary neurological and neuromuscular diseases with the goal of developing effective treatment for these disorders.

Matthew Wood, M.D., Ph.D., is a Professor of Neuroscience and Deputy Head of the Medical Sciences Division at the University of Oxford and directs the Laboratory of RNA biology and Neuromuscular Disease. Dr. Wood is a leading pioneer in the field of oligonucleotide therapies and co-leads the International MDEX Consortium, a major international translational medicine collaboration to develop oligonucleotide treatments for DMD and related neuromuscular conditions, with Dr. Francesco Muntoni of University College London. Sarepta's EXONDYS 51 was the first oligonucleotide worked on by this Consortium. Dr. Wood is an executive member of the global alliance TREAT-NMD. Dr. Wood serves as a Director of several organizations, including the Oxford MDUK Centre for Translational Neuromuscular Science, the University of Oxford's technology transfer organization, Oxford University Innovation, and Evox Therapeutics, a company he founded based on his work with exosomes. He currently serves as the Deputy Head of the Medical Sciences Division of the University of Oxford, and holds a Director role on the Board of MedCity.

About EXONDYS 51™

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of EXONDYS 51 in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

Important Safety Information

- Adverse reactions in DMD patients (N=8) treated with 30 or 50 mg/kg/week of EXONDYS 51 with incidence of at least 25% more than placebo (N=4) (Study 1) were: balance disorder (38%), vomiting (38%) and contact dermatitis (25%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.
- In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

- There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

Please see the U.S. Full Prescribing Information for EXONDYS 51 (eteplirsen) at www.EXONDYS51.com.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit us at www.sarepta.com.

Forward-Looking Statements

This press release contains statements that are forward-looking within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. 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,” “may,” “intends,” “prepares,” “looks,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to the potential benefits of Sarepta’s Strategic and Scientific Advisory Board (SSAB), including in guiding Sarepta’s strategy to move follow on exons into later stage trials, rapidly advance PPMO candidates into clinical studies, improve the lives of patients by exploring complimentary therapies, and expand collaborations; Sarepta’s ability to execute its strategy; and the potential benefits of adding Dr. Fischbeck and Dr. Wood to Sarepta’s SSAB, including providing Sarepta with unique counsel for its research and development efforts.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control. 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: we may not be able to capitalize and benefit from having the SSAB or from each of its specific members; we may not be able to complete clinical trials required by the FDA for approval of our product candidates; the results of our ongoing research and development efforts and clinical trials for our product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit; we may not be able to execute on our business strategy and plans, including successfully moving more follow on exons into later stage trials, rapidly advancing PPMO candidates into clinical studies, improving the lives of patients by exploring complimentary therapies, and expand collaborations, bringing EXONDYS 51 to

markets outside the United States, bringing our other product candidates to market or be able to meet related expected or planned clinical development plans, regulatory milestones and timelines, for various reasons including possible limitations of Company 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 for the year ended December 31, 2016 and 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. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

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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