Sarepta Therapeutics Appoints Edward Kaye, M.D., as Interim CEO; Company on Track with Clinical and Regulatory Plans for Investigational Duchenne Muscular Dystrophy Drugs

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– Christopher Garabedian Resigns as Chief Executive Officer and Member of the Board Effective Today –

– Company will host conference call at 8:00 a.m. EDT, April 1, 2015 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 31, 2015-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-targeted therapeutics, today announced the appointment of Edward Kaye, M.D., the company's Chief Medical Officer (CMO), as interim Chief Executive Officer (CEO) effective immediately. He replaces Christopher Garabedian, who resigned as President and Chief Executive Officer and as a member of the Board, also effective immediately. Dr. Kaye, who will continue in a dual capacity as CEO and CMO while the company conducts a search for a new full-time CEO, will focus his efforts on heading the regulatory and clinical process for the company's lead product candidate, eteplirsen, and follow on "exon" drug candidates for Duchenne Muscular Dystrophy (DMD).

Dr. Kaye has been Sarepta's CMO since June of 2011, during which time he has been responsible for the company's medical and clinical operations. He was previously Group Vice President of Clinical Development at Genzyme Corporation, one of the most successful biotechnology companies in the United States, from April 2007 to June 2011, where he supervised clinical development programs for rare diseases, including lysosomal storage diseases and genetic neurological disorders. Prior to this, Dr. Kaye held various roles at Genzyme Corporation since 2001, including Vice President of Medical Affairs for Lysosomal Storage Diseases, Vice President of Clinical Research and Interim Head of PGH Global Medical Affairs.

"We believe this change will facilitate the company's clinical and regulatory discussions and relationships with the goal of meeting its stated timelines for bringing a potentially disease-modifying treatment to patients with DMD as soon as possible," said John Hodgman, Sarepta's interim Chairman of the Board. "Dr. Kaye has a proven track record of leading teams that have brought some of the most successful rare-disease drugs to market, including Myozyme, Lumizyme and Fabrazyme. Further, he has excellent relationships with the clinical, regulatory and patient advocacy communities so critical to making this treatment a reality for this underserved patient population."

Dr. Kaye is a Neurological Consultant at the Children's Hospital of Boston and is on the editorial boards of a number of journals, including the ***Journal of Child Neurology***. He is also on the Medical/Scientific Advisory Boards of the United Leukodystrophy Foundation, Spinal Muscular Atrophy Foundation, CureCMD, CureDuchenne and the Prize4Life.

"We remain on track in collecting and analyzing the data requested by the FDA necessary for us to submit the eteplirsen New Drug Application in the middle of this year as planned," said Dr. Kaye. "I'm pleased to say that I have the full support of the entire executive team and the Board to optimally focus and prepare for this important milestone. I look forward to further strengthening our relationship with the FDA and other regulatory agencies that share our goal of doing what's in the best interest of DMD patients and their families. I could not be more excited to take full charge of our DMD clinical and regulatory efforts, as well as to lead the advancement of our broader pipeline of treatments for rare diseases."

About Eteplirsen for DMD

Eteplirsen is Sarepta's lead drug candidate and is designed to address the underlying cause of DMD by enabling the production of a functional internally deleted dystrophin protein. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable and promising safety and tolerability profile and restoration of dystrophin protein expression.

Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene, enabling the repair of specific genetic mutations that

affect approximately 13 percent of the total DMD population. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA, or mRNA. Promoting the synthesis of an internally deleted dystrophin protein is intended to stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD. Sarepta is also developing other PMO-based exon-skipping drug candidates intended to treat additional patients with DMD.

About Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy (DMD) is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 boys born worldwide. A devastating and incurable muscle-wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and death usually occurs before the age of 30.

Conference Call Details

The Company will be hosting a conference call at 8:00 a.m. EDT, April 1, 2015. The conference call may be accessed by dialing 800-708-4539 for domestic callers and 847-619-6396 for international callers. The passcode for the call is 39371595. Please specify to the operator that you would like to join the “Sarepta Management Update Call.” The conference call will be webcast live under the investor relations section of Sarepta’s website at www.sarepta.com and will be archived there following the call for 90 days. Please connect to Sarepta’s website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. An audio replay will be accessible through April 30, 2015, by calling 888-843-7419 or 630-652-3042 and entering access code 39371595#.

About Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of drug-resistant bacteria and infectious, rare and other human diseases. For more information, please visit us at www.sarepta.com.

Forward-Looking Statements

In order to provide Sarepta’s investors with an understanding of its current results and future prospects, this press release contains statements that are forward looking. 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 Sarepta’s succession plan, including the search for a new full-time CEO and the effect that the appointment of Dr. Kaye as interim CEO will have on the Company’s regulatory and clinical discussions and relationships, including its impact on the expected timeline for bringing our product candidates to market, as well as statements relating to Sarepta’s future operations, financial performance, business plans, priorities and development of product candidates, including: our plans to submit a New Drug Application (NDA) for eteplirsen by mid-year 2015; our continued efforts to collect and analyze the additional datasets requested by the U.S. Food and Drug Administration (FDA); the data necessary for an NDA submission; the enduring safety and tolerability of eteplirsen; Sarepta’s clinical and regulatory plans for its investigational drugs; and eteplirsen’s ability to stabilize or significantly slow the disease process, restore dystrophin protein expression, and prolong and improve the quality of life for patients with DMD.

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 on our executive team's relationship and expertise to meet our expected timelines for NDA submission, clinical development plans and bringing our product candidates to market; we may not be able to comply with all FDA requests, including with respect to our planned NDA submission, in a timely manner or at all; the FDA may determine that our NDA submission for eteplirsen is incomplete or does not qualify for filing, even if we provide additional supporting information and datasets requested; the additional information and data we collect for the eteplirsen NDA submission may not be consistent with prior data or results or may not support an eteplirsen NDA submission, filing or approval; we may not be able to complete clinical trials required by the FDA for approval of eteplirsen and the results of our ongoing and new clinical trials may not be positive or consistent with prior results and may not support the safety and efficacy of or an NDA submission, filing or approval of eteplirsen, our other product candidates and/or Sarepta's anti-sense based technology platform; there may be delays in our projected timelines relating to eteplirsen clinical studies, our planned NDA submission for eteplirsen, meetings and discussions with the FDA, initiating new clinical trials for our product candidates, or making a product commercially available for various reasons, including possible limitations of Company resources and regulatory or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; scale-up of manufacturing may not be successful and any or all of the Company's drug candidates may fail in development or may not receive required regulatory approvals for commercialization (including potentially under an accelerated pathway); we may need and may not be able to obtain additional funds to conduct our planned research and development efforts and execute our business plans; and those risks identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the year ended December 31, 2014, 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.

Source: Sarepta Therapeutics, Inc.

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