Sarepta Therapeutics Announces USPTO Decision in Patent Interference Case with BioMarin Pharmaceutical

- Importance of USPTO decision not ascertainable until determinations are rendered in two remaining composition interferences and planned appeal -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--September 30, 2015--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the Patent Trial and Appeal Board (PTAB) of the United States Patent and Trademark Office (USPTO) has issued a decision in the patent interference proceeding (Interference No. 106,013) concerning methods for treating Duchenne muscular dystrophy with certain exon 51 skipping oligonucleotides claimed in BioMarin's pending U.S. Patent Application No. 14/198,992 and Sarepta's granted U.S. Patent No. 8,486,907. The PTAB has not yet issued a decision in the two pending composition of matter patent interference proceedings relating to (i) the exon 51 skipping oligonucleotides underlying the methods that are the subject of this decision (Interference No. 106,008) or (ii) the exon 53 skipping oligonucleotides (Interference No. 106,007).

The PTAB decision was based on a procedural matter and did not include a decision on the substantive motions of unpatentability at issue in the pending interferences for exon 51 and 53 oligonucleotides. Additionally, the decision did not address the patentability of BioMarin's method claims in pending U.S. Patent Application No. 14/198,992 in the course of ordering the cancellation of Sarepta's U.S. Patent No. 8,486,907.

Sarepta intends to appeal this decision to the U.S. Court of Appeals for the Federal Circuit. Until there are final decisions in the appeal to this decision, the remaining interference proceedings, and any other potential future legal proceedings, it is unclear what potential importance, if any, this USPTO decision may ultimately have.

The PTAB decision does not impact Sarepta's key patent protection for eteplirsen and SRP-4053. Sarepta's primary patent protection for eteplirsen and SRP-4053 (United States Patent Nos. 9,018,368 and 9,024,007, respectively) is not the subject of any pending interferences and these patents are presumed valid and enforceable. United States Patent Nos. 9,018,368 and 9,024,007 both expire in June 2025 not including any potential patent term extension or regulatory exclusivity that would extend this date.

"We await the decisions for the two remaining composition interferences. We maintain confidence in our legal position and believe that the BioMarin patent claims should not be entitled to grant prior to completion of the appeals process, which we look forward to pursuing and which typically takes twelve to eighteen months," said Edward Kaye, M.D., Sarepta's interim chief executive officer and chief medical officer. "In the meantime, our focus remains on our regulatory milestones for our New Drug Application for eteplirsen, including the upcoming advisory committee meeting and our PDUFA date of February 26, 2016, as well as on our continued development efforts towards advancing product candidates targeting Duchenne muscular dystrophy,"

About Pending Appeal of BioMarin European Patent

Sarepta previously opposed BioMarin's European Patent EP 1 619 249 B1 and both Sarepta and BioMarin appealed the decision of the European Patent Office Opposition Division which upheld the claims of the patent in an amended form. This European patent is not related to pending U.S. Patent Application No. 14/198,992 that is involved in this PTAB decision. A forthcoming decision from the European Patent Office Board of Appeals would be independent of this decision from the USPTO.

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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 life threatening diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of infectious diseases, such as drug-resistant bacteria and other rare human diseases. For more information, please visit us at www.sarepta.com.

Forward-Looking Statements

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 regarding Sarepta's plans to appeal the USPTO's decision on Interference No. 106,013, the ascertainability of the importance of this decision until final decisions are rendered in planned appeal, pending interferences or future legal proceedings, Sarepta's beliefs regarding the impact of the USPTO decision on Sarepta's key patents for eterplirsen and SRP-4053, the expected expiration dates for these key patents, the strength of the legal positions Sarepta has asserted or may assert this case, an appeal, the two remaining interferences and any future litigation and Sarepta's potential for success, and the potential timelines for the appeals process and any potential USPTO grant decision for Biomarin's patent applications, and Sarepta's focus on regulatory milestones for the eteplirsen NDA and continued development of product candidates targeting Duchenne muscular dystrophy.

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: the outcomes, and impact of such outcomes on Sarepta, of the pending interferences, any appeals or future legal proceedings is not presently determinable or estimable and the USPTO, other agencies or courts may decide against Sarepta including by making decisions that are inconsistent with Sarepta's beliefs relating to the potential timing of any grants by the USPTO of BioMarin's relevant patent applications, Sarepta's understanding that Sarepta's key patents for eteplirsen and SRP-4053 are not impacted by the USPTO's interference decisions and the expected expiration dates for such key patents; there may be delays in Sarepta's projected regulatory and development timelines relating to the eteplirsen NDA and plans for commercializing eteplirsen and developing Sarepta's other product candidates for various reasons including possible limitations of Sarepta's financial and other resources; Sarepta may not be able to successfully complete its planned commercialization of eteplirsen or continue developing its

product candidates as planned for a variety of reasons including due to regulatory, court or agency decisions, such as decisions by the USPTO with respect to patents that cover Sarepta's product candidates, scale-up of manufacturing may not be successful, clinical safety and efficacy data collected on product candidates may not be positive or consistent with past results and any or all of Sarepta's product candidates may fail in development or may not receive required regulatory approvals for commercialization (including potentially under an accelerated pathway); and those risks identified under the heading "Risk Factors" in Sarepta's 2014 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2015 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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