



Sarepta Therapeutics Announces Favorable USPTO Decisions in Exon 51 and Exon 53 Composition of Matter Patent Interference Cases against BioMarin Pharmaceutical

- Final refusal of BioMarin's interfering claims facilitates commercialization of EXONDYS 51™ (eteplirsen) in US
- Key patent protection for EXONDYS 51 and SRP-4053 remains valid

CAMBRIDGE, Mass.--(BUSINESS WIRE)—Sep. 20, 2016--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 two favorable decisions for Sarepta in the composition of matter patent interferences for exon 51 (Interference No. 106,008) and exon 53 (Interference No. 106,007). These decisions, subject to appeal, finally refused all of BioMarin's claims in the exon 51 and exon 53 composition of matter interferences that, if granted, could have formed a basis for a claim of infringement against eteplirsen and SRP-4053. The PTAB decision for exon 53 allows BioMarin to obtain a narrow composition of matter claim, however, SRP-4053 does not infringe this claim. The PTAB ended the exon 51 interference in our favor based on a statute of limitations bar.

Accordingly, the PTAB did not decide the patentability of the Sarepta exon 51 patents involved in the interference (U.S. Patent Nos. 7,807,816 and 7,960,541). Although the PTAB did decide to cancel the Sarepta exon 53 patent involved in the interference (U.S. Patent No. 8,455,636), that exon 53 decision does not negatively impact Sarepta's key composition of matter patent protection for EXONDYS 51 and SRP-4053 (U.S. Patent Nos. 9,018,368 and 9,024,007, respectively). Neither of these key patents are involved in any pending interferences and are presumed valid and enforceable. These patents expire in June 2025, not including any potential patent term extension or regulatory exclusivity that would extend this date.

BioMarin has appealed the exon 53 composition of matter interference (Interference No. 106,007) to the Court of Appeals for the Federal Circuit. The exon 51 composition of matter interference (Interference No. 106,008) decided today by the PTAB remains subject to appeal.

“We are pleased that today’s exon 51 PTAB decision provides further clarity concerning BioMarin’s interfering patent claims as we begin commercialization of EXONDYS 51” said Edward M. Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “Moreover, we are optimistic that our key patents for eteplirsen and SRP-4053 will provide sufficient protection for the duration of further development and commercialization.”

In addition, previously decided Interference No. 106,013 concerning exon 51 methods for treating Duchenne muscular dystrophy with exon 51 skipping oligonucleotides is currently on appeal at the Court of Appeals for the Federal Circuit. BioMarin’s method claims currently on appeal involve the exon 51 skipping oligonucleotides found unpatentable in today’s exon 51 PTAB decision.

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 neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. 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 the consequences of the interference decisions including, subject to appeal, the finally refused BioMarin claims in the exon 51 and exon 53 composition of matter interferences; the allowable claim that Biomarin could raise under the decisions and Sarepta's understanding that SRP-4053 does not infringe on this claim; Sarepta's understanding that the cancelled exon 53 patent involved in the interferences does not negatively impact our composition of matter patent protection for EXONDYS 51 or SRP-4053; Sarepta's understanding that the decisions do not negatively impact Sarepta's key composition of matter patent protection for eteplirsen and SRP-4053 and the presumed validity, enforceability and expected expiration dates for these patents; Sarepta's commercialization plans for EXONDYS 51; the interference decisions being subject to appeals; and Sarepta's optimism that its key patents for eteplirsen and SRP-4053 will provide sufficient protection for the duration of further development and commercialization.

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 interference decisions may be appealed or subject to other challenges the results of which are not presently predictable or determinable but which could have a material negative impact on Sarepta; the USPTO, other agencies or courts may make decisions against Sarepta that negatively impact the EXONDYS 51 commercialization such as a decision in the pending appeal of Interference No. 106,013 which could result in an infringement claim against Sarepta if the patents subject to the appeal ultimately grant; any decision that results in BioMarin's patent applications granting or that otherwise erode the protections offered by Sarepta's patent estate; Sarepta's understanding that its key patents for eteplirsen and SRP-4053 are not impacted by the USPTO's interference decisions may not be accurate; the interference decisions may not support Sarepta's commercialization of EXONDYS 51; actual expiration dates for Sarepta's key patents for eteplirsen and SRP-4053 may differ from the expected expiration dates; we may not be able to execute on our business plans including meeting our expected or planned regulatory milestones and timelines, clinical development plans and bringing our product candidates to market 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, the results of our clinical trials may not be positive or consistent with prior results and regulatory, court or agency decisions, including any FDA approval decision, may negatively impact or limit Sarepta's activities; and those risks identified under the heading "Risk Factors" in Sarepta's 2015 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 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.

Media and Investors:

Sarepta Therapeutics, Inc.

Ian Estepan, 617-274-4052

iestepan@sarepta.com

or

W2O Group

Brian Reid, 212-257-6725

breid@w2ogroup.com