



Sarepta Therapeutics Agrees to Sale of Priority Review Voucher for \$125M

2/21/17

-- Sale of PRV Provides a Significant Infusion of Non-Dilutive Capital --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 21, 2017-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage developer of innovative RNA-targeted therapeutics, today announced it has entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV). Sarepta received the PRV when EXONDYS 51™ was approved by the U.S. Food and Drug Administration (FDA) for the treatment of patients with Duchenne muscular dystrophy amenable to exon 51 skipping.

The voucher was awarded by the FDA under a provision that encourages development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. With the passage of the 21st Century Cures Act, this PRV program has been extended through September 30, 2020.

As part of the agreement, Sarepta will receive an upfront payment of \$125M upon the closing of the transaction, which is subject to customary closing conditions and is expected to occur following expiration of the applicable U.S. antitrust clearance requirements. Credit Suisse served as Sarepta's advisor on this transaction and conducted an extensive sales process, which included outreach to multiple pharmaceutical and biotech companies.

"Our mission at Sarepta Therapeutics is to treat more boys with Duchenne muscular dystrophy," said Edward Kaye, Sarepta's chief executive officer. "The sale of the PRV provides an important source of non-dilutive capital to support the rapid advancement of our follow on exon skipping candidates and next generation RNA targeted antisense platform."

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 DMD drug candidates. For more information, please visit us at www.sarepta.com.

About the Rare Pediatric Disease Priority Review Voucher Program

The program is intended to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. A PRV may be issued to the sponsor of a rare pediatric disease product application and would entitle the holder to priority review of a single New Drug Application or Biologics License Application, which reduces the target review time and could lead to an expedited approval. The sponsor receives the PRV upon approval of the rare pediatric disease product application and it can be sold without limitation, subject to applicable FDA requirements for filing and use.

Forward-Looking Statements

This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995 (the "Act") and the protection of the Act's Safe Harbor for forward-looking statements. Examples of such statements include, but are not limited to, statements relating to Sarepta's commercialization and development activities, including the potential for success and timing for the progression of Sarepta's drug candidates; current regulatory requirements for approval of the purchase transaction and the viability of the priority review voucher sold; and Sarepta's capital needs. Such statements are based on management's current expectations, but actual results may differ materially due to various risks and uncertainties, including, whether regulatory requirements may change that affect the timing for payment and risk of payment under the royalty agreement with the pharmaceutical company purchaser. For further information regarding these and other risks related to Sarepta's business, investors should consult Sarepta's most recent Quarterly Report on Form 10-Q filing with the Securities and Exchange Commission. Forward-looking statements are not guarantees of future performance, and Sarepta's actual results of operations, financial condition and liquidity, and the development of the industry in which it operates, may differ materially from the forward-looking statements contained in this press release. Sarepta assumes no obligation to update its forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

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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