

# Sarepta Therapeutics and BioMarin Pharmaceutical Inc. Announce Execution of a Global Settlement and a License Agreement Resolving Exon Skipping Patent Litigation

**-- Agreement terms resolve global patent proceedings regarding Sarepta's sale of EXONDYS 51® (etep-lirsen) and future Duchenne muscular dystrophy (DMD) exon-skipping products --**

CAMBRIDGE, Mass. and SAN RAFAEL, Calif., July 18, 2017 -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a U.S. commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, and BioMarin Pharmaceutical Inc. (NASDAQ:BMRN), a leading biotechnology company in therapies for rare genetic diseases, announced today that Sarepta and BioMarin executed a license agreement that provides Sarepta Therapeutics with global exclusive rights to BioMarin's DMD patent estate for EXONDYS 51 and all future exon-skipping products. BioMarin retains the right to convert the license to a co-exclusive right in the event it decides to proceed with an exon-skipping therapy for DMD. In addition, Sarepta and BioMarin executed a settlement agreement, resolving the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. The effectiveness of the agreements is subject to closing conditions including execution of necessary approvals by Academisch Ziekenhuis Leiden (AZL) by July 24, 2017.

Under the terms of the license and settlement agreements, Sarepta will make a one-time payment of \$35 million to BioMarin and certain additional regulatory and commercial milestone payments for exons 51, 45, 53 and possibly on future exon-skipping products.

In addition, Sarepta will pay royalties to BioMarin as follows:

- Exon-skipping compounds 51, 45, and 53 and possibly on future exon-skipping products: Sarepta will pay BioMarin 5 percent of net sales through the end of 2023 in the United States; and
- Exon-skipping compounds 51, 45, and 53 and possibly on future exon-skipping products: Sarepta will pay BioMarin 8 percent of net sales through September 30, 2024 in the European Union and in other countries where certain BioMarin / AZL patents exist.

"Upon their effectiveness, these global license and settlement agreements provide Sarepta worldwide freedom to operate for EXONDYS 51 and our future exon-skipping products," said Douglas Ingram, Sarepta's President and Chief Executive Officer. "The resolution of these legal matters provides us with more certainty to fully focus our resources and energy on our crucial mission of developing innovative medicines to improve the lives of those impacted by DMD around the world."

"We are pleased to reach a global settlement and license agreement with Sarepta that fairly recognizes the important innovation by the Leiden University Medical Center and allows patients certainty that this issue will not create a barrier to access," said G. Eric Davis, BioMarin's Executive Vice President and General Counsel.

## 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 EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). 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  $\geq 30$  mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in  $\geq 10\%$  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.

## **About Sarepta Therapeutics**

Sarepta Therapeutics is a U.S. 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 [www.sarepta.com](http://www.sarepta.com).

## **About BioMarin Pharmaceutical Inc.**

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.biomin.com](http://www.biomin.com). Information on BioMarin's website is not incorporated by reference into this press release.

## **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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the license agreement providing Sarepta with global exclusive rights to BioMarin's DMD patent estate for EXONDYS 51 and all future exon-skipping products; the settlement agreement resolving the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD; the payments and royalties that Sarepta will be making as part of the settlement and license agreements; the settlement and license agreements providing for Sarepta's worldwide freedom to operate for EXONDYS 51 and Sarepta's future exon-skipping products; the settlement providing Sarepta with the certainty to fully focus its resources and energy on its crucial mission of developing innovative medicines to improve the lives of those impacted by DMD around the world; and the statement that the patent proceedings between the parties will not create for patients a barrier to access to the innovation by the Leiden University Medical Center.*

*These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the settlement and license agreements may not become effective if their conditions to effectiveness are not met within the required deadline; the parties may not be able to fulfill their commitments and obligations under the settlement and license agreements; any future claims of infringement by other third parties; the expected benefits and opportunities related to the settlement and license agreements between the parties may not be realized or may take longer to realize than expected due to challenges and uncertainties regarding the sales of EXONDYS 51 and the research and development of future exon-skipping products; Sarepta may experience significant fluctuations in sales of EXONDYS 51 from period to period and, ultimately, Sarepta may never generate sufficient revenues from EXONDYS 51 to reach or maintain profitability or sustain its anticipated levels of operations; Sarepta may never receive regulatory approval to its future exon-skipping products due to a variety of reasons including that the results of additional research may not be consistent with past results or may not be positive or may otherwise fail to meet regulatory approval requirements for the safety and efficacy of product candidates; and even if Sarepta obtains regulatory approvals, it may not achieve any significant revenues from the sale of such products; Sarepta may not have worldwide freedom to operate for EXONDYS 51 and Sarepta's future exon-skipping products due to future proceedings brought by other parties.*

*Any of the foregoing risks could adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2016 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.*

## **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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.

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<https://investors.biomin.com/2017-07-18-Sarepta-Therapeutics-and-BioMarin-Pharmaceutical-Inc-Announce-Execution-of-a-Global-Settlement-and-a-License-Agreement-Resolving-Exon-Skipping-Patent-Litigation>