Sarepta Therapeutics and University of Western Australia Announce Exclusive Worldwide Licensing Agreement for Exon-Skipping Program in Duchenne Muscular Dystrophy

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Deal Enables Development of New Exon-Skipping Drug Candidates in DMD

CAMBRIDGE, MA and PERTH, AUSTRALIA -- (Marketwired) -- 04/11/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT) and the University of Western Australia (UWA) today announced that they have entered into an exclusive, worldwide licensing agreement for intellectual property rights to support the development of exon-skipping drug candidates for the treatment of Duchenne muscular dystrophy (DMD). The agreement grants Sarepta rights to UWA's extensive patent portfolio in DMD and enables the Company to build out its exon-skipping pipeline with new candidates based on its proprietary phosphorodiamidate morpholino oligomer (PMO) technology to address the majority of patients with the disorder worldwide. The deal expands an agreement first signed in 2008, which supported the development of several exon-skipping drugs including eteplirsen, Sarepta's lead clinical candidate for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

"This agreement underscores our commitment to pursue treatments for all DMD patients who can benefit from our exon-skipping technology, even those with rare genetic mutations," said Chris Garabedian, President and Chief Executive Officer of Sarepta Therapeutics. "We believe this collaboration allows us to apply our unique morpholino chemistry with UWA's groundbreaking research on the dystrophin gene to develop potentially best-in-class drugs for this disease across the globe."

"We are very encouraged by the recent progress of Sarepta's lead clinical program eteplirsen, which we believe demonstrates the strength of the underlying platform technology as well as our partner's drug development expertise in DMD," said Andy Sierakowski, Ph.D., Director of the Office of Industry and Innovation at UWA. "This expanded collaboration with Sarepta enables us to translate our understanding of the dystrophin gene into additional potential exon-skipping therapeutics that address a majority of patients with DMD, representing a major contribution by the University of Western Australia to the field of genetic medicine."

DMD is a rare and severe genetic disorder that affects boys and young men. It is associated with errors in the gene for dystrophin, a protein that plays a key structural role in muscle fibers. Patients with DMD lack functional dystrophin, and regular activity causes progressive muscle damage leading to weakness, loss of ambulation, respiratory and cardiac dysfunction, and eventually premature death. There are no approved treatments.

Exon-skipping is an innovative disease-modifying treatment approach designed to skip an exon in the dystrophin gene, thereby enabling the repair of specific genetic mutations and the production of a functional, but shorter, form of dystrophin. Sarepta currently has four exon-skipping programs in DMD addressing patients with genotypes amenable to skipping of exons 51, 45, 50 and 53.

Under the terms of the agreement, UWA is eligible to receive up to $7.1 million in upfront and development milestone payments, as well as a low single-digit royalty on net sales of all approved medicines under the collaboration. Additional financial terms were not disclosed.

About 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 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 eventually 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.

About Sarepta Therapeutics

Sarepta Therapeutics is focused on developing first-in-class RNA-based therapeutics to improve and save the lives of people

affected by serious and life-threatening rare and infectious diseases. The Company's diverse pipeline includes its lead program eteplirsen, for Duchenne muscular dystrophy, as well as potential treatments for some of the world's most lethal infectious diseases. Sarepta aims to build a leading, independent biotech company dedicated to translating its RNA-based science into transformational therapeutics for patients who face significant unmet medical needs. For more information, please visit us at www.sareptatherapeutics.com.

About the University of Western Australia

The University of Western Australia is a leading Australian research-based institution that ranks internationally among the world's top 100 universities. UWA is a member of Australia's Group of Eight top research universities, and one of only two Australian members of the Worldwide Universities Network, a partnership of 18 research-led universities from Europe, Africa, the Americas and the Asia-Pacific. The University is also a foundation member of the Matariki Network of high-quality, research-intensive universities with a particular focus on student experience. The campus is based in Perth, capital city of Western Australia.

Forward Looking Statement

This press release contains forward-looking statements. These forward-looking statements generally can be identified by use of words such as "believes or belief," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," "advance" and similar expressions. These forward-looking statements include statements about the development of eteplirsen and its potential commercialization as well as statements regarding the potential of Sarepta's other pipeline candidates.

Each forward-looking statement contained in this press release is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: subsequent clinical trials may fail to demonstrate the safety and efficacy of eteplirsen or replicate results; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; any of Sarepta's drug candidates, including eteplirsen, may fail in development, may not receive required regulatory approvals, or may not become commercially viable due to delays or other reasons; our enhanced intellectual property rights may provide us neither the ability to exclude competitors nor freedom to commercialize our product candidates; and those identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the year ended December 31, 2012, and filed with the Securities and Exchange Commission.

Any of the foregoing risks could materially and 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 the Company's filings with the Securities and Exchange Commission. 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.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995: The statements that are not historical facts contained in this release are forward-looking statements that involve risks and uncertainties, including, but not limited to, the results of research and development efforts, the results of preclinical and clinical testing, the effect of regulation by the FDA and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, and other risks detailed in the company's Securities and Exchange Commission filings.

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