Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

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Collaboration Represents Significant Progress in Sarepta's Path to Develop Treatments for Broader DMD Population

Nov 26, 2012 (Marketwire via COMTEX) --Sarepta Therapeutics (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, announced today a collaboration for the development of an additional exon-skipping drug targeting exon 53, its fourth drug in development, in support of Sarepta's broad-based program for the treatment of Duchenne muscular dystrophy (DMD).Sarepta's collaboration is with University College London's (UCL) scientist, Professor Francesco Muntoni, MD, the Dubowitz Neuromuscular Centre, the Institute of Child Health and other scientists from the EU and US.The EU Health Innovation-1 2012 Collaborative research grant will support certain IND-enabling activities and clinical proof of concept studies for an exon 53-skipping therapeutic.Sarepta recently announced positive results from its extension study of its Phase IIb trial of eteplirsen, its exon 51-skipping therapeutic candidate for the treatment of DMD.Sarepta is also developing other PMO-based exon-skipping drug candidates for exons 45 and 50.

"The recent compelling clinical data on eteplirsen targeting exon 51, which started with our work on the Phase I study in the UK, provides a strong foundation for using Sarepta's technology against exon 53," said Francesco Muntoni, professor of pediatric neurology and head of the Dubowitz Neuromuscular Centre at the UCL Institute of Child Health, London. "We are pleased to be working with Sarepta to bring this exciting exon-skipping therapeutic approach to Europe and the broader DMD population."

This program will be based on Sarepta's advance proprietary RNA-based platform, Phosphorodiamidate Morpholino Oligomers (PMOs), which is a novel backbone chemistry that provides enhanced target tissue specificity, increased potency to allow for more efficient dosing, and greater uptake within a cell to further increase protein production. Targeting exon 53 with this technology will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping (deletion of exons 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, or 52) by restoring the cellular machinery's ability to produce a functional dystrophin protein.

"The initiation of this program, along with our other collaborations for exons 45 and 50, continues to advance Sarepta's strategy in pursuing exon-skipping therapeutics for all of the DMD patients who may benefit from this drug technology," said Chris Garabedian, President and CEO of Sarepta Therapeutics."Our goal of demonstrating that the success of eterplirsen can be reproduced across other exon-skipping targets is a critical step in being able to treat more boys and young men affected with this devastating disease."

About EU Health Innovation-1 2012

The aim of the EU investment in health research is to improve the health of European citizens, to address global health issues and to boost the competitiveness of European health-related industries. Each year the European Commission publishes calls for proposals to which researchers can apply for collaborative research grants.

The European Commission implements a thorough and transparent evaluation process to identify proposals worthy of funding. The process is firmly grounded in the principle of access for all and the codes of fairness, impartiality, confidentiality, efficiency and ethics. For more information please visit http://ec.europa.eu/research/health/index_en.html.

About Dubowitz Neuromuscular Centre and UCL Institute of Child Health

The Dubowitz Neuromuscular Centre (DNC) provides a multidisciplinary service as a leading clinical and research center specializing in neuromuscular disorders affecting individuals in the pediatric age.The DNC provides clinical assessment, diagnostic services, advice on treatment and rehabilitation, and is involved in clinical trials and in basic research focused on understanding the cause of neuromuscular diseases in childhood, and identifying novel therapeutic intervention.Excellence in this field is demonstrated not only by the National Commissioning Group (NCG) status achieved in 2000 to provide a national specialist diagnostic and assessment service for Congenital Muscular Dystrophies and Congenital Myopathies, but also by the designation as a muscle center by the Muscular Dystrophy Campaign.

The DNC is part of the UCL Institute of Child Health and Great Ormond Street NHS Trust and is a member of the recently created MRC Neuromuscular Translational Research Centre at UCL. In addition to the service provided for all pediatric neuromuscular disorders, the DNC is involved with clinical, molecular and cell biological research mostly focused on the genetic basis of congenital muscular dystrophies; on muscle stem cell and on experimental therapies for Duchenne muscular dystrophy. For more information please visit http://www.ucl.ac.uk/ich/research-ich/dubowitz.

About Duchenne Muscular Dystrophy and Eteplirsen

Duchenne muscular dystrophy (DMD) is an X-linked rare, degenerative neuromuscular disorder causing severe, progressive muscle loss and a 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 inborn errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness eventually spreads to the arms, neck and other areas. Eventually, this progresses to complete paralysis and increasing difficulty in breathing due to respiratory muscle dysfunction requiring ventilatory support, as well as cardiac muscle dysfunction leading to heart failure. The condition is terminal, and death usually occurs before the age of 30.

Eteplirsen is Sarepta's lead drug candidate that is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression. Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene enabling the repair of specific genetic mutations that affect approximately 13 percent of the total DMD population. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA, or mRNA. Promoting the synthesis of a truncated dystrophin protein is intended to improve, stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

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.

Forward-Looking Statements and Information

In order to provide Sarepta's investors with an understanding of its current results and future prospects, 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 development of eteplirsen and its efficacy, potency and utility in the treatment of DMD and the potential for the creation of novel dystrophin to lead to significant clinical benefit over a longer course of treatment.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: clinical trials may not demonstrate the safety and efficacy of eteplirsen and/or Sarepta's antisense-based technology platform; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; and any of Sarepta's drug candidates, including eteplirsen, may fail in development, may not receive required regulatory approvals, or be delayed to a point where they do not become commercially viable.

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. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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