Sarepta Therapeutics Introduces Online Resource Center on Exon Skipping for the Duchenne Muscular Dystrophy Community

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CAMBRIDGE, MA -- (Marketwired) -- 10/02/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced a new online resource center, called Let's Skip Ahead, for families affected by Duchenne muscular dystrophy (DMD) and their healthcare providers. The new website, available at www.skipahead.com, includes information and educational resources intended to make the science of exon skipping simple, and provides an opportunity to sign up for updates about upcoming Sarepta clinical trials.

"Sarepta is deeply committed to pursuing exon-skipping treatments for patients with DMD who can potentially benefit from our technology, and this website will help families and physicians stay informed about our efforts," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "We recognize that many in the Duchenne community are seeking to understand exon skipping and the promise this innovative and complex technology may hold for them. Resources on the Let's Skip Ahead website include information tools that can support discussions between patients, their families and the physicians who care for them."

Let's Skip Ahead (www.skipahead.com) also offers an exon mapping tool that allows visitors to explore the potential link between specific genetic mutations in DMD and exon skipping. In addition, the website includes important information on genetic testing and the clinical trial process.

About Exon Skipping

Exon skipping is a potential therapeutic approach in DMD designed to produce functional dystrophin, a protein involved in muscle function that is lacking in patients with the disorder. Each potential therapy based on this technology is designed to skip a specific exon, and thereby correct for certain genetic mutations and restore the gene's ability to make a functional, though shorter, form of the dystrophin protein. Available data suggest that at least 65 percent of genetic mutations in DMD could potentially be addressed by exon skipping.

Eteplirsen is Sarepta's lead investigational exon-skipping drug candidate in clinical studies, and is designed to skip exon 51 in the dystrophin gene. Sarepta plans to initiate a confirmatory clinical trial of eteplirsen in DMD patients with amenable genotypes in the first quarter of 2014. Eteplirsen is not yet approved or licensed for use in any country.

In addition, Sarepta is conducting early-stage research, intended to support future clinical studies in patients, on drug candidates targeting exons 53, 50 and 45. Additional research to target other exons is also planned.

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 born 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 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.sarepta.com.

Forward-Looking Statements and Information

This press release includes forward-looking statements, including statements about the development and clinical status of Sarepta's product candidates and the potential benefit of such product candidates to Duchenne Muscular Dystrophy

patients. These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Any such risks can materially and adversely affect the business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties we face, you're encouraged to review Sarepta's official corporate documents filed with the Securities and Exchange Commission including the risks and uncertainties disclosed in Sarepta's latest report on Form 10-Q. We do not undertake any obligation to publicly update these forward-looking statements based on events or circumstances after the date hereof.

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