



FOR IMMEDIATE RELEASE

SAREPTA ENTERS INTO PARTNERSHIP WITH FLAGSHIP BIOSCIENCES TO DIGITALLY AUTOMATE THE MEASUREMENT OF DYSTROPHIN, A KEY THERAPEUTIC EFFICACY MARKER FOR MUSCULAR DYSTROPHY

- *New technology designed to speed dystrophin measurement while ensuring consistency*
- *Automation of dystrophin measurement will aid development of Sarepta's Duchenne muscular dystrophy candidates*

CAMBRIDGE, Mass., Aug 21, 2014 -- Sarepta Therapeutics, Inc., a developer of innovative RNA-based therapeutics, and Flagship Biosciences LLC, a leading tissue-based companion diagnostics firm, today announced a multi-year, multi-product partnership for the development of automated quantitative endpoint measurements in muscular dystrophy to support the advancement of Sarepta's Duchenne muscular dystrophy (DMD) drug pipeline, including its lead candidate, eteplirsen.

DMD is caused by the absence of functional dystrophin in affected patients' muscle tissue. Dystrophin protein level is a fundamental biomarker used to assess therapies that aim to produce and restore the expression of dystrophin, such as exon-skipping therapies like eteplirsen. In order to optimally and efficiently evaluate therapeutic efficacy in patients, the next generation of protocols are being developed to digitally automate and standardize dystrophin measurement in tissue biopsies to speed the process while ensuring consistency. The establishment of these new standardized methods for automated quantitation is being enabled through the proprietary image analysis platform and digital pathology capabilities developed by Flagship.

This newly established collaboration with Flagship demonstrates Sarepta's commitment to enhancing the objective measurement of dystrophin in tissue samples for its growing pipeline of RNA-based therapeutics to treat DMD. The agreement between the companies also further strengthens Flagship Biosciences' leadership role in diagnostic image analysis and digital pathology to support and optimize targeted drug development.

Dave Young, Flagship's Chief Pathologist, commented, "Flagship Biosciences has developed tools and expertise in quantitative pathology, image analysis, and tissue-based assays that are well-suited for use in a regulated environment. It's exciting to work with a partner like Sarepta to design and implement an integrated fit-for-purpose assay and automated quantitative interpretation approach that accelerates the development of drugs for unmet needs such as eteplirsen for the treatment of DMD."

“Sarepta is fully committed to quickly and thoughtfully developing effective therapies to treat Duchenne muscular dystrophy,” said Ed Kaye, M.D., Chief Medical Officer of Sarepta. “As our RNA-based therapies advance in Duchenne and other disease areas, Flagship’s quantitative tissue-based diagnostics development expertise will play a key role in accelerating our clinical success. The sophistication and rigor of Flagship’s image analysis capabilities are exceptional. As we embark upon several clinical studies within multiple centers both in the US and Europe, our ability to automate the dystrophin quantification process while insuring speed, accuracy and consistency is important to our ongoing and future clinical development efforts.”

Steve Potts, Ph.D., Chief Executive Officer of Flagship Biosciences, further added, “Sarepta’s novel approaches to therapeutic RNA targeting such as exon-skipping must be matched with equally ground-breaking approaches in digital diagnostic laboratory measurements. It is a pleasure to see the discipline and commitment by both Sarepta and Flagship to meet the demand for automating the precise evaluation and standardization of endpoints used in these clinical trials.”

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’s Proprietary Exon-Skipping Platform Technology

Eteplirsen is Sarepta's lead drug candidate and is designed to address the underlying cause of DMD by enabling the production of a functional internally deleted 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 an internally deleted dystrophin protein is intended to stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD. Sarepta is also developing other PMO-based exon-skipping drug candidates intended to treat additional genetic subpopulations of patients with DMD by restoring dystrophin production. Sarepta’s exon-skipping technology has the potential to treat a large majority of the DMD population.

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 and follow-on drug candidates, 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.

About Flagship Biosciences

Flagship Biosciences delivers quantitative pathology services for drug and medical device development. Flagship specializes in the development of [tissue-based diagnostic assays](#) which support pharmacodynamic, surrogate efficacy, or patient selection approaches. Flagship Biosciences services are currently utilized by over 100 pharmaceutical and biotech firms for both diagnostic and therapeutic programs. Flagship Biosciences is a privately held company located in Westminster, Colorado, and serves a global base of clients. For more information, please visit us at www.flagshipbio.com.

Forward Looking Statements

Except for historical information, the matters discussed in this news release may be considered "forward-looking" statements within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. Such statements include declarations regarding the intent, belief or current expectations of the Company and its management, including those related to cash flow, gross margins, revenues, and expenses are dependent on a number of factors outside of the control of the company including, inter alia, the markets for the Company's products and services, costs of goods and services, other expenses, government regulations, litigations, and general business conditions. See Risk Factors in the Company's Form 10-K for the fiscal year ended July 31, 2013. Investors are cautioned that any such forward-looking statements are not guarantees of future performance and involve a number of risks and uncertainties that could materially affect actual results. The Company disclaims any obligations to update any forward-looking statement as a result of developments occurring after the date of this press release.

Sarepta Media Contact:

Tony Plohoros, (908) 591-2839
tplohoros@6degreespr.com