# Repligen Announces Licensing Agreement with Pfizer for Spinal Muscular Atrophy Program

Thursday, January 03, 2013 - 08:00am

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Repligen Corporation (NASDAQ:RGEN) announced today that it has entered into an exclusive worldwide licensing agreement with Pfizer Inc. to advance Repligen's spinal muscular atrophy (SMA) program, originally in-licensed from Families of SMA (FSMA). The SMA program includes RG3039, a small molecule drug candidate in clinical development for SMA, as well as backup compounds and enabling technologies. Under the terms of the agreement, Repligen is entitled to receive up to $70 million from Pfizer, commencing with an upfront payment of $5 million and total potential future milestone payments of up to $65 million as well as royalties on any future sales of SMA compounds developed under the agreement. SMA is an orphan neurodegenerative genetic disease that presents early in life.

"This agreement is consistent with the strategic decision we announced in August 2012 to focus Repligen's internal efforts on the growth of our bioprocessing business, while seeking external partners for our therapeutic development programs," said Walter C. Herlihy, Ph.D., President and Chief Executive Officer of Repligen. "We believe this collaboration with Pfizer, a leading pharmaceutical company with specialized efforts in orphan and genetic diseases, has the potential to accelerate the development of therapies for SMA."

"There is a critical need to expedite potential treatment solutions for rare diseases such as spinal muscular atrophy, where patients have such limited options," said Jose Carlos Gutierrez-Ramos, Senior Vice President, Pfizer BioTherapeutics R&D. "This partnership will combine our expert capabilities in advancing molecules for genetic diseases with

Repligen’s leading SMA program.”

Under the terms of the agreement, Repligen is responsible for completing the first two cohorts of an active Phase 1 trial evaluating RG3039 in healthy volunteers, which it anticipates will occur during the first quarter of 2013. Repligen will also provide certain technology transfer services to Pfizer who will then assume full responsibility for the SMA program moving forward, including the conduct of any registration trials necessary for product approval. Repligen has previously received U.S. Orphan Drug and Fast Track designations for RG3039 for the treatment of SMA, as well as Orphan Medicinal Product designation in the EU.

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“This licensing deal demonstrates the innovative collaborations that Families of SMA has successfully implemented between non-profit, biotech and big pharma,” stated Jill Jarecki, Ph.D., Research Director for Families of SMA. “These partnerships are critical for the development of new treatments for an orphan disease such as SMA. We are extremely pleased to see Pfizer taking the lead on the development and commercialization of the SMA program, following Repligen’s development work and FSMA’s original investment.”

Families of SMA, a patient organization dedicated to funding research to advance therapies for SMA, funded and directed the preclinical development of RG3039 with an investment of more than $13 million. This was the first drug discovery program ever conducted specifically for SMA. Repligen’s research and clinical efforts, including the current Phase 1b trial, have been partially supported by a $1.4 million grant from the Muscular Dystrophy Association.

**About Spinal Muscular Atrophy**

Spinal muscular atrophy (SMA) is an autosomal recessive neuromuscular disease in which a defect in the SMN1 (survival motor neuron) gene results in low levels of the protein SMN and leads to progressive damage to motor neurons. It is the leading cause of infant mortality and the second most common inherited neuromuscular disease, with symptoms that typically emerge before the age of two. SMA is characterized by progressive muscle weakness leading to severe physical disability and often, early loss of life due to respiratory insufficiency.

**About Families of SMA**

Families of SMA is the world’s leader focused on funding SMA research to develop a treatment and cure for the disease. The successful results and progress that the organization has delivered, from basic research to drug discovery to clinical trials, provide real hope for families and patients impacted by the disease. The charity has invested over $55 million in research and has been involved in funding half of all the ongoing novel drug programs for SMA. Families of SMA is a nonprofit 501(c)3 organization, with 31 Chapters and 90,000 members and supporters throughout the United States. The organization’s work has produced major discoveries, including identification of the underlying cause and a back-up gene for the disease, which provides a clearly defined target for disease altering therapies. The organization is also dedicated to supporting SMA families through networking, information and services and to improving care for all SMA patients. For more information: www.curesma.org.

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**About the Muscular Dystrophy Association**

The Muscular Dystrophy Association (MDA) is the leading nonprofit health agency dedicated to finding treatments and cures for more than 40 neuromuscular diseases, including SMA, by funding worldwide research. MDA also funds comprehensive health care and support services, advocacy, information and education, and accessible summer camp for thousands of youngsters fighting progressive muscle diseases. To date, MDA has invested more than $41.6 million in SMA research, funding basic research and clinical trials of therapeutic strategies such as gene-based therapies, smallmolecule development, and stem cells. MDA, along with other SMA patient advocacy groups, has been working with policymakers to explore the potential of expanding newborn screening panels to include SMA. For more information, visit mda.org and follow MDA on Facebook (facebook.com/MDAnational) and Twitter (@MDAnews).

**Repligen Corporation**

Repligen Corporation is a life sciences company focused on the development, production and commercialization of high-value consumable products used in the process of manufacturing biological drugs. Our bioprocessing products are sold to major life sciences and biopharmaceutical companies worldwide. We are a leading manufacturer of Protein A, a critical reagent used during the production of monoclonal antibody therapeutics. We also supply several growth factor products used to increase cell culture productivity during fermentation. In addition, we have developed and market a series of

chromatography products used in the purification of biologics, and sell test kits to ensure final product quality. Aside from our core bioprocessing business, we have a portfolio of clinicalstage partnering assets, including a pancreatic imaging agent in Phase 3 development and two central nervous system orphan drug candidates. Repligen's corporate headquarters are located in Waltham, MA, USA; we have an additional manufacturing facility in Lund, Sweden. For more information, please visit our website at www.repligen.com.

*This press release contains forward-looking statements, which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Investors are cautioned that statements in this press release which are not strictly historical statements, including, without limitation, express or implied statements regarding the potential utility of RG3039 for the treatment of SMA, the clinical success of RG3039 and its further clinical development and our receipt of any future payments under the terms of our agreement with Pfizer, Pfizer's ability to terminate the license agreement for convenience, our strategic decision to focus on the growth of our bioprocessing business, the future demand for our bioprocessing, growth factor and chromatography products, plans and objectives for future operations, our ability to successfully negotiate and consummate partnering transactions for our clinical stage assets, specifically RG1068, RG3039 and RG2833, plans and objectives for product development and acquisitions , plans and objectives for regulatory approval, product development, our market share and product sales and other statements identified by words like "believe," "expect," "may," "will," "should," "seek," or "could" and similar expressions, constitute forward-looking statements. Such forward-looking statements are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated, including, without limitation, risks associated with: the success of our clinical trials, including our Phase 1b clinical trial of RG3039 in patients with SMA; our ability to successfully grow our bioprocessing business, including as a result of acquisition, commercialization or partnership opportunities; our ability to successfully negotiate and consummate development and commercialization partnerships for our portfolio of clinical-stage assets on acceptable terms, if at all; our ability to develop and commercialize products and the market acceptance of our products;*

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*reduced demand for our products that adversely impacts our future revenues, cash flows, results of operations and financial condition; the ability to obtain, and the timing and*

*receipt of, FDA approval for our NDA for RG3039; our ability to obtain other required regulatory approvals; the success of current and future collaborative or supply relationships, including our agreement with Pfizer; our ability to compete with larger, better financed bioprocessing, pharmaceutical and biotechnology companies; new approaches to the treatment of our targeted diseases; our compliance with all Food and Drug Administration and EMEA regulations; our ability to obtain, maintain and protect intellectual property rights for our products; the risk of litigation regarding our intellectual property rights; our limited sales capabilities; our volatile stock price; and other risks detailed in Repligen's Annual Report on Form 10-K on file with the Securities and Exchange Commission and the other reports that Repligen periodically files with the Securities and Exchange Commission. Actual results may differ materially from those Repligen contemplated by these forwardlooking statements. These forward looking statements reflect management's current views and Repligen does not undertake to update any of these forward-looking statements to reflect a change in its views or events or circumstances that occur after the date hereof except as required by law.*

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