

# Repligen Receives First Milestone Payment From Pfizer Under Licensing Agreement for Spinal Muscular Atrophy Program

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WALTHAM, Mass., Sept. 4, 2013 (GLOBE NEWSWIRE) -- Repligen Corporation (Nasdaq:RGEN) announced today that it has received a \$1 million milestone payment from Pfizer, Inc. under the terms of the companies' exclusive worldwide licensing agreement (the "Agreement") for the development of compounds to treat spinal muscular atrophy (SMA). This first milestone payment was triggered by completion of specific program activities and coincides with the successful completion of all transition obligations by Repligen. Repligen announced the Agreement in January of this year, at which time it received an upfront payment of \$5 million. Repligen remains eligible to receive up to \$64 million in additional success-based milestone payments, as well as royalties on any future sales of compounds developed under the Agreement.

"Consistent with our strategic decision last August to focus on building Repligen's bioprocessing business while scaling back our investment in therapeutics, we out-licensed the SMA program and have successfully completed its transition to Pfizer," said Walter C. Herlihy, Ph.D., President and CEO of Repligen. "We believe the Agreement preserves the potential for the SMA program to deliver significant long-term upside for our shareholders."

Repligen originally in-licensed the SMA program from Families of SMA (FSMA), a patient organization dedicated to supporting research to advance therapies for SMA. FSMA funded and directed the preclinical development of the program's lead compound, RG3039, with an investment of more than \$13 million. This was the first drug discovery program ever conducted specifically for SMA. The Muscular Dystrophy Association also provided critical support to Repligen's research and clinical efforts, including the conduct of a Phase 1b trial.

About Spinal Muscular Atrophy

Spinal muscular atrophy 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 genetic 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 Repligen Corporation

Repligen Corporation (Nasdaq:RGEN) 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 to separate and purify monoclonal antibody therapeutics. We also supply several growth factor products used to increase cell culture productivity during the fermentation stage of drug manufacturing. In addition, we have developed and market our OPUS® line of pre-packed "plug-and-play" chromatography columns, and we provide test kits to ensure final product quality. Aside from our core bioprocessing business, we have a portfolio of clinical-stage partnering assets, including a pancreatic imaging agent in Phase 3 development and an orphan drug candidate in Phase 1 development. Repligen's corporate headquarters are located in Waltham, MA (USA) and our manufacturing facilities are located in Waltham, MA and Lund, Sweden.

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, constitute forward-looking statements, including, without limitation, express or implied statements regarding the potential utility of RG3039 for the treatment of SMA, the safety and tolerability profile of RG3039 as well as its ability to achieve specific levels of target enzyme inhibition in future clinical trials, the potential for the SMA program to deliver significant long-term upside for our shareholders, 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, our ability to meet future milestones under the agreement with Pfizer, Pfizer's ability to terminate the license 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, plans and objectives for product development and acquisitions, our market share and product sales and other statements identified by words like "believe," "expect," "may," "will," "should," "seek," "anticipate," or "could" and similar expressions. 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 of RG3039 in patients with SMA and in healthy volunteers, 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; reduced demand for our products that adversely impacts our future revenues, cash flows, results of operations and financial condition; the impact of the expiration of Bristol-Meyers Squibb royalty payments from U.S. sales of Orencia®; 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; our ability to successfully integrate Repligen Sweden AB, including achieving manufacturing efficiencies at Repligen Sweden AB; 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 forward-looking 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.

CONTACT: Sondra S. Newman  
Director Investor Relations  
Repligen Corporation  
(781) 419-1881  
snewman@repligen.com

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