

FOR IMMEDIATE RELEASE

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Repligen Receives FDA Approval to Initiate Phase 1 Clinical Trial of Potential Treatment for Spinal Muscular Atrophy

WALTHAM, MA – May 19, 2011 – Repligen Corporation (NASDAQ: RGEN) announced today that it has received approval from the Food and Drug Administration (FDA) to initiate a Phase 1 clinical trial of RG3039, a potential treatment for Spinal Muscular Atrophy (SMA). SMA is an inherited neurodegenerative 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, loss of muscle function and, in many patients, early death. This is a double-blind study to evaluate the pharmacokinetic and safety profile of escalating doses of RG3039 in up to 40 healthy volunteers. This will be the first clinical trial of a novel drug specifically designed to treat SMA and the first treatment approach which seeks to increase levels of the protein SMN.

“We are very pleased to have received approval to initiate human clinical trials with RG3039,” stated Walter C. Herlihy, President and Chief Executive Officer of Repligen Corporation. “RG3039 targets the core deficit of SMA and if this therapeutic approach is successful, it has the potential to arrest or slow disease progression and significantly improve patients’ lives. We’re also grateful to the Muscular Dystrophy Association for helping fund the upcoming clinical trial.”

Patients lacking a functional SMN1 gene survive only because humans carry a second gene called SMN2 which produces low levels of SMN protein. RG3039, an orally bioavailable compound, is an inhibitor of an RNA processing enzyme which targets SMN2 and has been shown to increase production of SMN protein in cells derived from patients. In addition, RG3039 has been shown to improve mobility and lifespan in preclinical animal models of SMA. RG3039 is a new chemical entity, which is the subject of worldwide composition of matter patent applications which, if allowed, will remain in force until 2028 prior to any patent term extensions. Repligen’s ongoing research efforts have been funded in part with grants from the Muscular Dystrophy Association.

About Spinal Muscular Atrophy and Families of Spinal Muscular Atrophy

Spinal Muscular Atrophy is the second most common inherited neuromuscular disease. Symptoms of SMA typically emerge before the age of two and often progress to severe physical disability or loss of life. The prevalence of SMA in the United States and Europe is approximately 20,000 patients and there is currently no treatment or cure for the disease. This program was licensed in 2009 from Families of Spinal Muscular Atrophy, a non-profit organization with 30 chapters throughout the United States and over 70,000 members and supporters. Families of SMA fully funded and directed the preclinical development work with an investment of more than \$13 million prior to licensing RG3039 to Repligen. This was the very first drug development program ever done for SMA.

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About the Muscular Dystrophy Association

[MDA](#) is the nonprofit health agency dedicated to curing muscular dystrophy, ALS, SMA and related diseases by funding worldwide research. The Association also provides comprehensive healthcare and support services, advocacy and education. In addition to funding more than 330 research teams worldwide, MDA maintains a national network of some 200 hospital-affiliated clinics; orchestrates hundreds of support groups for families affected by neuromuscular diseases; facilitates extraordinary local summer camp opportunities for thousands youngsters fighting progressive muscle diseases. Known globally for the MDA Labor Day Telethon, the Association is the first nonprofit to receive a Lifetime Achievement Award from the American Medical Association “for significant and lasting contributions to the health and welfare of humanity.”

About Repligen Corporation

Repligen Corporation is a biopharmaceutical company focused on building an integrated company by developing and marketing innovative drugs that deliver the benefits of protein therapies in the fields of neurology and gastroenterology. We have a core competency in the development and manufacturing of biologics products, which is the basis for our bioprocessing business and we have out-licensed certain biologics intellectual property, which provide ongoing sources of revenue. Repligen’s corporate headquarters are located at 41 Seyon Street, Building #1, Suite 100, Waltham, MA 02453. Additional information may be requested 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. The forward-looking statements in this release do not constitute guarantees of future performance. Investors are cautioned that statements in this press release which are not strictly historical statements, including, without limitation, statements regarding future financial performance and position, management’s strategy, plans and objectives for future operations, plans and objectives for product development, plans and objectives for present and future clinical trials and results of such trials, plans and objectives for regulatory approval, litigation, intellectual property protection, product development, manufacturing plans and performance, projected changes in the size of our markets, 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; our ability to develop and commercialize products; our ability to obtain required regulatory approvals; the success of current and future collaborative relationships; the market acceptance of our products; our ability to compete with larger, better financed pharmaceutical and biotechnology companies; new approaches to the treatment of our targeted diseases; our expectation of incurring continued losses; our uncertainty of product revenues and profits; our ability to generate future revenues; our ability to raise additional capital to continue our drug development programs; our compliance with all Food and Drug Administration 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 and manufacturing capabilities; our dependence on third-party manufacturers and value added resellers; our ability to hire and retain skilled personnel; 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.