# RepliGen

FOR IMMEDIATE RELEASE

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# Repligen Receives Additional Research Grants to Support Development of HDAC Inhibitors for Friedreich's Ataxia and Huntington's Disease

WALTHAM, MA – August 2, 2010 – Repligen Corporation (NASDAQ: RGEN) today announced that the Company has received three additional grants to support its research program to develop HDAC inhibitors for neurodegenerative diseases. Repligen has received \$436,000 in research funding to support the ongoing development of histone deacetylase (HDAC) inhibitors for Friedreich's ataxia. The funding is comprised of \$218,000 from the Friedreich's Ataxia Research Alliance (FARA) and \$218,000 from Go Friedreich's Ataxia Research (GoFAR). The grants will support the synthesis and characterization of additional HDAC inhibitors in support of the development of a potential therapy for Friedreich's ataxia. Our continued collaboration with FARA and GoFAR provides access to a global network of scientific thought leaders and patients and partially funds the development of our Friedreich's ataxia program.

In addition, The National Institute of Neurological Disorders and Stroke (NINDS) has awarded a grant in the amount of \$6.05 million over four years to The Scripps Research Institute and Repligen for the development of a novel HDAC inhibitor for Huntington's disease. Repligen is part of a collaborative network receiving the grant and has the potential to receive \$2.9 million based on successful completion of various milestones during the four year program. Dr. Joel M. Gottesfeld from The Scripps Research Institute is the principal investigator on the grant which is based on the demonstration of efficacy of a prototype HDAC inhibitor in an animal model of Huntington's disease conducted at Scripps. The goals of the grant include the identification, characterization, optimization and preclinical GLP toxicology and safety testing of a novel HDAC inhibitor for Huntington's disease.

"We are very pleased by the support of FARA, GoFAR and NINDS for our research efforts," stated Walter C. Herlihy, President and Chief Executive Officer of Repligen Corporation. "Friedreich's ataxia and Huntington's disease represent areas of high unmet medical need for which HDAC inhibitors have the potential to have a significant impact on patients' lives."

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## **About Friedreich's ataxia**

Friedreich's ataxia is an inherited neurodegenerative disease caused by a single gene defect that results in inadequate production of the protein frataxin resulting symptoms that typically emerge between the ages of five and fifteen and often progress to severe disability, incapacitation or loss of life in early adulthood. These symptoms include loss of strength and coordination in the arms and legs leading to wheelchair use, vision impairment, hearing loss and slurred speech, aggressive scoliosis (curvature of the spine), diabetes and a serious heart condition. Preclinical studies in animal models and patients' cells have shown that RG2833 crosses the blood brain barrier, activates the defective frataxin gene and increases production of the protein frataxin. These results indicate that RG2833 may increase frataxin production and arrest disease progression in patients with Friedreich's ataxia. We have filed an IND for RG2833, and we hope to initiate a Phase 1 normal volunteer study this year. There are approximately 15,000 patients worldwide with Friedreich's ataxia with no effective treatment for their disease.

# About Huntington's disease

Huntington's disease is a progressive neurodegenerative genetic disorder, which affects muscle coordination and leads to cognitive decline and dementia. The disease is caused by a defective HTT gene, which provides instructions for making a protein called huntingtin. Although the function of this protein is unknown, it appears to play an important role in nerve cells in the brain. HD typically begins in mid-life, between the ages of 30 and 50. Symptoms of the disease include depression, forgetfulness, impaired judgment and motor skill impairment. There are approximately 35,000 patients with Huntington's disease in the U.S. and there is currently no effective treatment.

## **About The Friedreich's Ataxia Research Alliance (FARA)**

The Friedreich's Ataxia Research Alliance (FARA) is a national, public, non-profit, tax-exempt organization dedicated to the pursuit of scientific research leading to treatments and a cure for Friedreich's ataxia. FARA's mission is to slow, stop, and reverse the damage caused by this disorder. For more information, go to www.curefa.org.

# About GoFAR

GoFAR - Friedreich's Ataxia Research is an Italian, non-profit organization dedicated to facilitating and promoting through fund-raising, the scientific research for the treatment of Friedreich's ataxia. For more information, go to <a href="https://www.fagofar.org">www.fagofar.org</a>.

## About The National Institute of Neurological Disorders and Stroke

The National Institute of Neurological Disorders and Stroke (NINDS) conducts and supports research on brain and nervous system disorders. Created by the U.S. Congress in 1950, NINDS is one of the more than two dozen research institutes and centers that comprise the National Institutes of Health (NIH). The NIH, located in Bethesda, Maryland, is an agency of the Public Health Service within the U.S. Department of Health and Human Services. NINDS has occupied a central position in the world of neuroscience for more than 50 years. For more information go to <a href="https://www.ninds.nih.gov">www.ninds.nih.gov</a>.

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# **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 large-scale protein manufacturing and 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 current or future financial performance and position, management's strategy, plans and objectives for regulatory approval, litigation, intellectual property, product development, plans and objectives for regulatory approval, litigation, intellectual property, product development, manufacturing plans and performance such as the anticipated growth in the monoclonal antibody market and our other target markets and projected growth in product sales, 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 current and future collaborative relationships, the market acceptance of our products, our ability to compete 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, the success of our clinical trials, our ability to develop and commercialize products, our ability to obtain required regulatory approvals, 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 hier and retain skilled persomel, our volat