PRESS RELEASE

FOR IMMEDIATE RELEASE

Partnership and Collaboration take promising therapy, RG2833, from discovery to first-in-human trial in Friedreich's Ataxia (FA).

March 15, 2012- Today, Repligen Corporation announced enrollment of the first patient in a Phase 1 clinical trial of RG2833 in adult patients with FA -- a debilitating, life-shortening, degenerative neuro-muscular disorder that affects children and adults and currently has no treatment. RG2833 is a class 1 Histone Deacetylase (HDAC) inhibitor specifically designed to increase frataxin protein production in FA patients. This therapeutic approach targets the genetic mutation that is the fundamental cause of the disease.

The potential for these HDAC inhibitors as a therapy for FA was first demonstrated by Dr. Joel Gottesfeld and his colleagues at The Scripps Research Institute in La Jolla, CA. Repligen has worked with Dr. Gottesfeld and other FA scientists around the world to optimize and develop these HDAC inhibitors and prepare them for human clinical trials. FARA is proud to have funded the Gottesfeld's lab's earlier work in which he showed that class 1 HDAC inhibitors could address the basic cause of FA by increasing transcription of the FA gene and production of frataxin protein in cells from FA patients and in FA animal models.

"All of us in the global FA community are deeply excited about the launch of this clinical trial," said FARA President Ron Bartek. "Because FA is caused by the shortage of frataxin protein, this HDAC inhibitor's ability to increase the FA gene's production of that protein shows the potential for it to become a profoundly therapeutic compound. We are grateful to the scientific teams that have created this opportunity, to Repligen for pushing this program through preclinical development and into human trials, to our patient advocate partners around the world, to the FA patients who are always eager to participate in the research and to the patient families and their supporters who make all this possible."

"Friedreich's ataxia disease biology provides evidence that a small increase in expression of the defective gene could potentially slow disease progression," said James R. Rusche, Ph.D., Senior Vice President, Research and Development at Repligen. "RG2833 is the first compound that targets activation of this defective gene. If our unique approach of using small molecules for protein replacement is successful, it has the potential to significantly improve outcomes for patients with FA. We are hopeful that the Phase 1 trial will elucidate the role for HDAC inhibition in FA, and inform future efficacy studies."

"This important milestone illustrates the importance and power of public-private partnership in moving therapeutic discoveries through the challenging preclinical development needed to reach clinical trials," said Jen Farmer, FARA's Executive Director. From the very early stages of the HDAC inhibitor program, FARA worked closely with the scientific team at The Scripps Research Institute, the other FA scientists around the world who tested the compounds in their cell and animal models, the Repligen Corporation, FA patients who readily donated their blood samples for such testing, and with the other patient advocacy organizations who have continued to join us in supporting this effort. FARA is especially grateful in that regard to its advocacy partners of GoFAR, the Muscular Dystrophy Association, and the National Ataxia Foundation.

For more information visit: www.repligen.com

About FA
The first symptoms of FA are loss of balance and coordination leading to wheelchair use 6-8 years after onset. Additional symptoms include life-shortening cardiomyopathy and arrhythmias, scoliosis, diabetes, and diminished vision, hearing and speech. There are currently no effective treatments. In >95% of individuals with FA there is a mutation in the first intron of the FA gene that decreases transcription of the gene and subsequent production of the frataxin protein.

About Repligen
Repligen Corporation is a leading supplier of critical biologic products used to manufacture biologic drugs. Repligen also applies its expertise in biologic product development to SecreFlo(TM), a synthetic hormone being developed as a novel imaging agent for the diagnosis of a variety of pancreatic diseases. In addition, the Company has two central nervous system (CNS) rare disease programs in Phase 1 clinical trials. 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

About FARA
FARA is a (501)(c)(3) non-profit organization that supports scientific medical research, a clinical network, patient registry, education and awareness. FARA serves as a catalyst among the patient, scientific, pharmaceutical and government communities to create worldwide exchanges of information that drive medical advances to bring treatments and a cure to individuals with FA. www.CureFA.org

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