PRESS RELEASE

Repligen Corporation Announced BioMarin Pharmaceutical Acquired Assets in HDAC Inhibitor Program

January 21, 2014- Downingtown, PA- Today, Repligen Corporation announced that BioMarin Pharmaceutical has acquired the assets in the company's Histone DeACetylase (HDAC) inhibitor program. Repligen has been advancing a therapeutic research and development program in Friedreich's Ataxia (FA) in which specific HDAC inhibitors have been designed to increase transcription of the frataxin gene so as to ameliorate the primary defect in FA - reduced expression of the frataxin protein. In early 2013, Repligen completed a Phase1 clinical trial of its initial HDAC inhibitor candidate, RG2833, and demonstrated that orally dosed HDAC inhibitors can increase frataxin mRNA (gene expression) in FA patients. However, to increase both safety and effectiveness, Repligen has developed a number of follow-on HDAC inhibitor product candidates with improved properties.

Jennifer Farmer, Executive Director of FARA, says, "Over the past six years, FARA and several of our funding partners, particularly the Muscular Dystrophy Association and GoFAR, have been supporting the early drug discovery, translational and clinical research that have progressed this program to this promising milestone. We are encouraged that BioMarin, a company with a strong commitment and success in developing treatments for rare diseases, will be taking this program forward. FARA is eager to work with BioMarin to bring an optimal follow-on HDAC inhibitor candidate to clinical trials in the FA community."

For more information on the BioMarin acquisition please see Repligen and BioMarin Press Releases:
Repligen Press Release BioMarin Press Release

About FA
Friedreich's ataxia is a rare, degenerative, life-shortening neuro-muscular disorder that affects children and adults, and involves the loss of strength and coordination usually leading to wheelchair use; diminished vision, hearing and speech; scoliosis (curvature of the spine); increased risk of diabetes; and a life-threatening heart condition. There are no FDA-approved treatments.

About FARA
The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. www.CureFA.org

Contact:
Jennifer Farmer
Executive Director, Friedreich's Ataxia Research Alliance
(484) 879 6160
info@curefa.org