



# PRESS RELEASE

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## **New Clinical Trial in Friedreich's Ataxia in Italy**

FARA, January 5, 2012 -- The Italian Health Ministry and the Ethics Committee of the San Luigi Hospital in Torino, Italy, have given investigators at the hospital approval to initiate a Phase I clinical trial of a new drug designed specifically to treat Friedreich's ataxia. After additional study site preparations, this Phase I trial will test the Repligen drug, known as RG2833, in patients with Friedreich's ataxia.

The investigators will aim to find out whether the drug is safe, and to learn more about its effects in Friedreich's ataxia patients. In particular, they will determine if RG2833 increases production of frataxin, a key protein that is diminished in people with Friedreich's ataxia. Because lower frataxin levels are the base cause of Friedreich's ataxia symptoms, it is hoped that, if RG2833 acts to increase frataxin production, it could be beneficial to Friedreich's ataxia patients.

RG2833 is a type of drug called a Histone Deacetylase (HDAC) inhibitor and is being developed by Repligen working with an international team of scientists. HDAC inhibitors may provide a way to increase frataxin protein production by "switching on" the frataxin gene, which is too often "switched off" in people with Friedreich's ataxia. RG2833 has been awarded orphan drug designation by the U.S. Food and Drug Administration and the EMA.

The development of HDAC inhibitors for Friedreich's ataxia has been supported by FARA and other patient advocacy groups including the Muscular Dystrophy Association and GoFAR. Repligen has indicated that when the final preparatory steps are complete and the first patient is enrolled in the Italian trial, more details will be made available on the status of this study and global plans for the drug's development.

### **About FA**

FA 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 currently no effective 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](http://www.CureFA.org)

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