



# PRESS RELEASE

**FOR IMMEDIATE RELEASE**

## **Important Clinical Trial Update - "Double-Blind, Randomized, Placebo-Controlled Pilot Study of Varenicline in the Treatment of Friedreich's Ataxia"**

Springfield, VA - April 12, 2010

In June 2009, the Friedreich's Ataxia Research Alliance (FARA) announced the launch of a clinical trial of varenicline (Chantix®) to investigate both safety and potential efficacy in improving neurological symptoms such as balance, coordination and sensory perception in a diverse group of adults diagnosed with Friedreich's ataxia. This pilot study was initiated based on case reports indicating that uncoordinated movements (ataxia) and balance problems in patients with various forms of ataxia had improved significantly when the individuals were taking Chantix. The double blind, randomized, placebo-controlled pilot study has been led by principal investigator Dr. Theresa Zesiewicz, Professor of Neurology at the University of South Florida College of Medicine, and co-investigator Dr. David Lynch, Associate Professor of Neurology and Pediatrics at Children's Hospital of Philadelphia. Chantix® is approved by the U.S. Food and Drug Administration to help cigarette smokers stop smoking.

The Data Safety Monitoring Board (DSMB) responsible for monitoring the progress and associated risk/benefits of the study convened April 7<sup>th</sup> 2010 to review the data collected to date. Following that review, the DSMB recommended that the study be stopped as a result of concerns regarding safety and intolerability and insufficient evidence of efficacy. Twenty six subjects had been enrolled into the trial. The primary concern among those who were withdrawn was a worsening of gait and imbalance. FARA, the study sponsor, and the study investigators have decided to act upon the DSMB recommendation. Therefore, no further subjects will be enrolled and all current subjects in both the double blind and open label periods are being instructed to discontinue the study drug. Of note, this DSMB recommendation and this decision by FARA and the investigators pertain solely to this study, which involved only subjects with Friedreich's ataxia.

At this time, the study investigators and subjects continue to be blinded regarding each subject's treatment assignment as data collection is ongoing and subjects are still being evaluated and scheduled for final study visits. Once all of the final visits have been completed and data collected, the study team will be able to analyze the data and share the complete results. We all anxiously await this full review and FARA will keep the entire Friedreich's ataxia community informed, as new information and analyses become available.

FARA would like to thank and recognize the study investigators, DSMB and, most especially, the patients who participated in the study. FARA remains committed to exploring this type of therapeutic approach for Friedreich's ataxia. As mentioned above, the study team still needs to analyze the study data and issue a complete report, which we are confident will provide important details and insights. In addition, the initial case reports on which this pilot study was based opened an exciting new avenue of research investigating neurotransmission and improvement of nervous system function in Friedreich's ataxia. We believe that further basic investigation of this

area will lead to new studies in the future that will provide hope for a variety of methods of improvement of nervous system function in Friedreich's ataxia.

**About Friedreich's Ataxia (FA)**

FA is an aggressive neurodegenerative disease that debilitates people of all ages, robbing them of their ability to walk, see, speak and hear and cutting short their life expectancies. Currently, there is no cure or treatment for FA. Although rare, FA is the most prevalent inherited ataxia, affecting about one in every 50,000 people in the United States.

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

FARA advances research for Friedreich's Ataxia (FA) and is dedicated to curing FA. Founded in 1998 by FA parents and patients, FARA is a nonprofit, 501c3, whose mission is directing and funding research through a remarkable level of collaboration among the patient, medical and scientific communities. FARA funded research is bringing promising treatments forward fueling confidence in the ability to slow, stop and reverse this disease for patients living with FA today. <http://www.CureFA.org>

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