



## PRESS RELEASE

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#### **New study to examine whether smoking cessation drug Varenicline may benefit adults with Friedreich's ataxia**

May 26, 2009- A drug approved for smoking cessation may hold promise for people suffering from Friedreich's Ataxia (FA), an inherited disease that causes progressive damage to the neuromuscular system.

A new clinical study, sponsored by the Friedreich's Ataxia Research Alliance (FARA), will investigate whether varenicline (Chantix®) improves neurological symptoms, such as balance, coordination, and sensory perception, all of which are significantly impaired in patients with FA. Chantix® is approved by the U.S. Food and Drug Administration to help cigarette smokers stop smoking. Currently, there is no effective treatment for FA. The double blind, randomized, placebo-controlled pilot study will be 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.

Dr. Zesiewicz noticed that the uncoordinated movements (ataxia) and balance problems of a patient with fragile X tremor /ataxia syndrome improved greatly after he started varenicline in an attempt to quit smoking. The symptoms worsened when the medication was discontinued. Dr. Zesiewicz found similar results when treating patients with other types of ataxia, and several of her case reports were published last year in medical journals.

"Our preliminary findings with varenicline in ataxia patients provided information that warrants further formal clinical research. Varenicline possibly has a novel mechanism of action in the central nervous system that we were previously unaware of," said Dr. Zesiewicz, who developed a protocol for the clinical trial and applied to FARA for funding. "We are eternally grateful to FARA for their support and enthusiasm as we further investigate these findings, and to the Pfizer corporation as well."

The pilot study aims to determine whether the findings Dr. Zesiewicz observed in a few ataxia patients can be replicated in a larger group of adults diagnosed with FA.

Chantix®, a Pfizer drug, acts at sites in the brain affected by nicotine. "We do not completely understand how Chantix® may be working to improve symptoms of ataxia, however it is clearly different from the other agents under development for FA," Dr. Lynch said. "If Chantix does prove useful, it would offer a complementary approach for treating

FA.”

The researchers do not recommend that those with FA or other types of ataxia begin off-label use of Chantix®, which requires a prescription. Clinical trials are needed to determine the effectiveness, proper dose and potential side effects of Chantix® in this population, they say.

“This is the first clinical trial that FARA has supported through a research grant. We are grateful to our talented team of investigators who have moved quickly to bring this discovery to FA patients in an organized and rigorous study and to the Pfizer corporation for providing the drug for this study,” said FARA Executive Director Jennifer Farmer. “Clinical studies are needed to determine the risks and benefits of a drug. In such cases where the drug has been approved for another indication, all too often proper trials do not occur and patients are taking risks with a drug without understanding the real potential benefit.”

For more information on this study – “Double-Blind, Randomized, Placebo-Controlled Pilot Study of Varenicline in the Treatment of Friedreich’s Ataxia” -- go to <http://www.CureFA.org/registry> and select Clinical Trials or visit <http://www.clinicaltrials.gov> and search for “Friedreich’s ataxia.”

#### **About FA**

FA is a degenerative, neuromuscular disease that gradually robs patients of their ability to walk, compromises speech, hearing, and vision, and often comes with complications of serious diabetes and heart disease. Although rare, FA is the most prevalent inherited ataxia, affecting about one in every 50,000 people in the United States.

#### **About FARA**

The Friedreich's Ataxia Research Alliance's (FARA) mission is to marshal and focus the resources and relationships needed to cure FA by raising funds for research, promoting public awareness, and aligning scientists, patients, clinicians, government agencies, pharmaceutical companies and other organizations dedicated to curing FA and related diseases. <http://www.CureFA.org>

#### **About USF Health**

USF Health is dedicated to creating a model of health care based on understanding the full spectrum of health. It includes the University of South Florida’s colleges of medicine, nursing, and public health; the schools of biomedical sciences as well as physical therapy & rehabilitation sciences; and the USF Physicians Group. With more than \$360 million in research grants and contracts last year, USF is one of the nation’s top 63 public research universities and one of 39 community-engaged, four-year public universities designated by the Carnegie Foundation for the Advancement of Teaching. <http://www.health.usf.edu>

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