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Advances in ataxia research focus of symposium

Friedreich's Ataxia Research Alliance and USF to host Aug. 26 event

Tampa, FL (August 3, 2010) – Building on last year's momentum, scientists, clinicians and patients will gather Aug. 26 at the University of South Florida to discuss research progress that may lead to therapies for Friedreich's ataxia and other ataxias.

The scientific symposium "Cultivating a Cure" will be held from **6 to 8 p.m. on Thursday, Aug. 26**, in the USF Marshall Student Center Ballroom, 4103 Cedar Circle, Tampa, FL 33620. The symposium, **free and open to the public**, is hosted by the Friedreich's Ataxia Research Alliance (FARA) and the USF Ataxia Research Center (ARC). At the pre-symposium poster session, from 5 to 6 p.m., attendees will have the opportunity to learn about new research on Friedreich's ataxia and talk directly to investigators about the findings.

Friedreich's ataxia is a rare, debilitating neuromuscular disorder. Symptoms, typically emerging between ages 5 and 15, often progress to severe disability and include the following: loss of coordination and muscle weakness that leads to wheelchair use, energy deprivation and fatigue, vision impairment, hearing loss, slurred speech, aggressive scoliosis, diabetes, and life-shortening cardiac disease. There is not yet an approved treatment or a cure.

In recent years, fundamental advances have been made in understanding the cell biology of neural degeneration and in developing cell and animal models that will allow improved testing of drug candidates for Friedreich's ataxia.

"More clinical trials for Friedreich's ataxia are moving into the pipeline, and we're hopeful that some of these promising drugs, or combinations of drugs, will end up being the first approved treatment for Friedreich's," said Theresa Zesiewicz, MD, professor of neurology and director of the USF Ataxia Research Center, who will speak about her clinical experience with the drug varenicline and ataxia. "USF is grateful for FARA's commitment towards finding a cure for this disease."

Other speakers will include Joel Gottesfeld, PhD, a professor in The Scripts Research Institute Department of Molecular Biology, and Guy Miller, MD, PhD, CEO of Edison Pharmaceuticals, Inc. Compounds developed by Gottesfeld's team – histone deacetylase (HDAC) inhibitors -- reversed in cell culture the genetic defect that prevents adequate production of the protein frataxin in those with

Friedreich's ataxia. Edison Pharmaceuticals develops drugs for rare inherited mitochondrial diseases, including Friedreich's.

Ron Bartek, president and co-founder of FARA, and Jennifer Farmer, FARA executive director, will address progress nationwide in the research, care and management of Friedreich's ataxia. Stephen Klasko, MD, MBA, CEO for USF Health and dean of the College of Medicine, will moderate a panel discussion on patients' perspectives of living with ataxias, with input from Clifton Gooch, MD, chair of neurology at USF Health. .

"This is the second year we've participated in this interactive scientific symposium with USF, and we've already outgrown our first location," said FARA's Farmer. "Our partnership continues to grow with a new biomarkers study about to begin and USF's tireless dedication to research and education that will provide the highest level of clinical care for patients."

A portion of funds raised from last year's FARA Energy Ball is supporting a $100,000 study at the USF Health to test gait and balance biomarkers that may serve as more sensitive measures for detecting the progression of Friedreich's ataxia. Researchers from the USF Ataxia Research Center and the School of Physical Therapy & Rehabilitation Sciences will follow adults and adolescents diagnosed with Friedreich's ataxia and a control group without Friedreich's or other conditions causing gait or balance disturbances. "Such biomarkers, if validated, would be helpful in assessing the effectiveness of investigational treatments, rather than relying on clinical rating scales, which tend to be more subjective and less sensitive," Dr. Zesiewicz said.

USF is one of 11 sites included in FARA's Collaborative Clinical Research Network, an international network of centers that share data and resources to advance treatments and clinical research for people with Friedreich's ataxia.

For more information about the upcoming symposium, please call (813) 974-5909.

- About USF Health –

USF Health (www.health.usf.edu) 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 $380.4 million in research grants and contracts last year, the University of South Florida is one of the nation's top 63 public research universities and one of only 25 public research universities nationwide with very high research activity that is designated as community-engaged by the Carnegie Foundation for the Advancement of Teaching.

- About The Friedreich's Ataxia Research Alliance (FARA) –

The Friedreich's Ataxia Research Alliance is a non-profit organization dedicated to the pursuit of scientific research leading to treatments and a cure for Friedreich's ataxia. FARA also serves as a catalyst between the public and scientific community to create worldwide exchanges of information that drive medical advances. For more information, go to www.curefa.org.