UF and the Advocacy Group GoFAR Announce Gene Therapy program for Friedreich's Ataxia

Gainesville, FL, Sept.13, 2016 — Researchers at the University of Florida have received a grant for the development of a gene therapy program aimed at treating Friedreich's Ataxia, a progressive, debilitating and life-shortening neuromuscular disorder from the Italian patient advocacy group GoFAR.

GoFAR awarded the grant, worth nearly $750,000, to Manuela Corti, PT, PhD, of the University of Florida's Powell Gene Therapy Center. The award will fund an18-month comprehensive gene therapy program, including both pre-clinical and planned human studies that address the underlying cause of Friedreich's Ataxia (FA).

FA is a genetic disease caused by defects in the frataxin gene. Symptoms of FA usually begin in late childhood and include progressive loss of neuromuscular function and physical coordination. The disorder, which affects about 1 in 50,000 people, can also cause heart disease, diabetes and premature death.

The gene therapy project aims to treat both the cardiac and neurological aspects of the disease by delivering a functional frataxin gene to the heart and nervous system via an adeno-associated virus vector. The project will employ several novel mechanisms to accomplish its objectives.

Corti, together with UF Powell Gene Therapy Center director Barry Byrne, MD, PhD, will initiate the program this month."The project is an important milestone in the development of a treatment strategy that could dramatically improve the quality of life for patients," Corti said.

Filomena D'Agostino, President of the Comitato RUDI Onlus also known as GoFAR, said "GoFAR is pleased to award this grant to Drs. Corti and Byrne for the first comprehensive gene therapy program in FA. GoFAR looks forward to continued support to this team and working with others that strive to advance gene therapy treatments for FA into human studies. We hope that the innovative strategy adopted by GoFAR in financially supporting a public institution with significant experience in gene therapy treatments for rare diseases, will increase significantly the opportunity for FA patients to participate in developing an effective treatment."

"At the University of Florida Clinical and Translational Science Institute (CTSI), we are very excited to participate in cutting edge clinical development such as the collaboration with GoFAR", said David R. Nelson, M.D., Assistant Vice President for

Research and Director of the CTSI. “Academic and foundation collaboration is the cornerstone of developing innovative therapies in rare disease.”

About UF: The UF Powell Gene Therapy Center is a world leader in gene therapy, having successfully conducted trials in inherited retinal disorders, Pompe disease and related neuromuscular conditions. Researchers at UF pioneered the use of adeno-associated virus vectors, which are safe variants of viruses used to deliver correct copies of genes where they are needed.

About GoFAR: The Comitato RUDI Onlus is a voluntary non-profit association registered in Italy (n.7603044400-1, Torino), devoted to facilitate and promote scientific research dedicated to finding a cure for Friedreich's ataxia. For more information about GoFAR, visit www.fagofar.org
Contact: Filomena D’Agostino (Mina), filomena.dagostino@fagofar.org

For media inquiries in the U.S., please call Mindy Cameron at 317-853-6294 or email: mindycameron@ufl.edu

For media inquiries in EU, please contact Annaluisa Ponchia at +39 339 562 3766 or email: aponchia@fagofar.org