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International advocacy group GoFAR funds UF Health research for Friedreich's ataxia gene therapy program

ATTENTION EDITOR: For media inquiries, call Emma Crowley at 352-294-8625 or email ecrowley@ufl.edu. Go to http://bit.ly/2qFfvUk for a UF Health video about how AAV gene therapy works.

GAINESVILLE, Fla. — University of Florida Health pediatrics researchers have received a $450,000 grant from the Italian patient advocacy group GoFAR to continue developing a gene therapy for Friedreich's ataxia, a life-shortening genetic disorder that damages the nervous system.

GoFAR in January awarded the grant to Manuela Corti, P.T., Ph.D., an assistant professor in the UF College of Medicine's department of pediatrics. In 2016, the group began collaborating with the UF Powell Gene Therapy Center through a nearly $750,000 grant to Corti to develop gene therapy for Friedreich's ataxia.

Friedreich's ataxia symptoms typically arise in late childhood and include progressive loss of coordination and neuromuscular function. The disorder, which affects about one in 50,000 people, can also cause heart disease and diabetes.

The gene therapy project aims to treat the disease's cardiac and neurological aspects by using a small, harmless adeno-associated virus to deliver a functional frataxin gene to the heart and nervous system. Reduced frataxin levels prevent cells from properly producing energy, leading to muscle weakness, poor muscle coordination, heart muscle disease, and difficulty with vision, hearing and speech.

The latest award will be used to complete preclinical studies and prepare data for submission to the U.S. Food and Drug Administration, the European Medicines Agency, and the Italian regulatory authority, Instituto Superiore della Sanità.

Corti, who is heading the study, said the research team is grateful for the support of GoFAR and the families who help with the program.

"Our efforts on behalf of the Friedreich's ataxia community will hopefully correct the disease's fundamental problem and have a meaningful impact on the quality of life for patients and their families," she said.

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The partnership with GoFAR allows researchers to continue toward their ultimate goal: a gene therapy treatment for Friedreich's ataxia, said Barry J. Byrne, M.D., Ph.D., the director of the UF Powell Gene Therapy Center and a pediatrics professor in the UF College of Medicine. .

Filomena D'Agostino, president of the Comitato RUDI Onlus, also known as GoFAR, said, "I'm happy to have contributed, decisively and in such a short time, GoFAR's funds to the UF Powell Gene Therapy Center and its efforts toward the first clinical implantation of AVV-mediated gene therapy for Friedreich's ataxia."

The goal of the gene therapy study will be to lessen the severity of Friedrich's ataxia symptoms. Trials are expected to begin later this year at the UF Clinical and Translational Science Institute.

"We are excited to support projects like this, where researchers, patients and families collaborate to make a difference in people's lives," said David R. Nelson, M.D., the director of the UF Clinical and Translational Science Institute.

About the researchers: Barry J. Byrne, M.D., Ph.D., is director of the UF Powell Center for Rare Disease Research and Therapy at the UF College of Medicine; a professor in the departments of pediatrics, and molecular genetics and microbiology; and the associate chair of the department of pediatrics. Manuela Corti, Ph.D., is an assistant professor in the department of pediatrics.

About GoFAR: The Comitato RUDI Onlus is a nonprofit association registered in Italy that aims to facilitate and promote scientific research to find a cure for Friedreich's ataxia. For more information about GoFAR, visit www.fagofar.org.