FARA Friedreich's Ataxia Research Alliance

Your Community News

Hello friends,

I hope this message finds you and your family well. Our team remains hard at work from their home offices. In between our monthly updates, we shared some of the ways in which FA research is continuing during this time. If you didn't get a chance to read that message, you can access it here.

As you will see below, there are a number of ways for you to be active in research and advocacy efforts from home. Screening for some clinical trials and studies is being done by phone and one clinical study survey can be completed online. There are calls to action for advocacy and of course, our ongoing request that all people with FA to sign up for the Global Patient Registry.

May is just around the corner, and we have several virtual activities planned in honor of FA Awareness month. Stay tuned for upcoming emails about these activities including- Lend Us Some Muscle, the Cure FA Soirée, rideATAXIA NorCal Virtual Challenge and weekly webinars with FA researchers. I am looking forward to an active May and meeting up with you online.

Sincerely,

Jen Farmer,
Chief Executive Officer

Open & Enrolling Studies

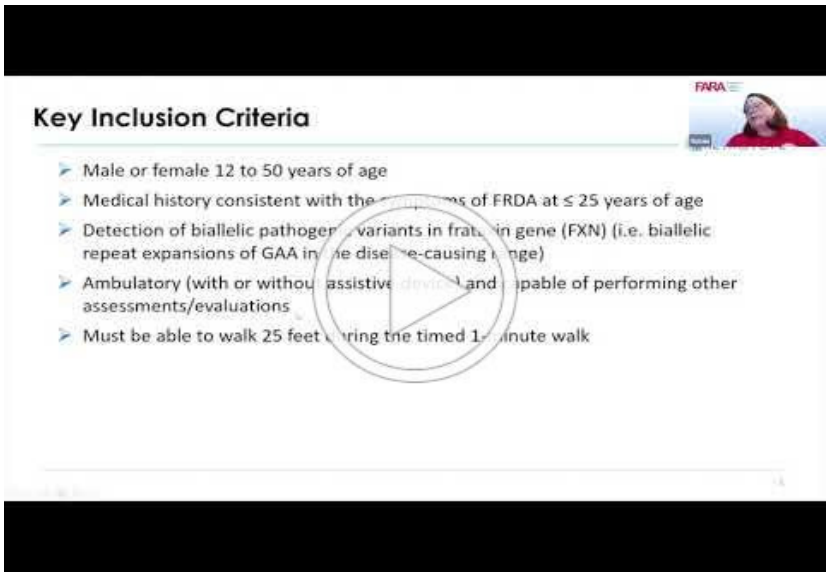
During this time of social-distancing, let's keep the momentum going for FA research. The studies below are recruiting by phone screening, until travel to clinical sites can resume.

IDEA Study
FARA is supporting the IDEA research study to test body-worn sensors to measure movement. The goal of the study is to evaluate progression and severity of ataxia. The study involves visits every 6 months over 2 years at a clinic located in Baltimore, Boston, Chicago, or Los Angeles. Participants must be 12-30 years of age and be able to walk 10-feet independently, without an assistive device. For additional eligibility criteria and clinics open for enrollment, please contact study coordinator Hannah Casey (hannahcasey@uchicago.edu) or (773) 702-4610.

Retrotope RT001-006 Phase 2/3 trial for protection against oxidative damage in the central nervous system

Retrotope is seeking 45 volunteers to participate in a clinical trial for the drug RT001, which may protect against lipid peroxidation, the process that is believed to cause disability in many neurodegenerative diseases, including Friedreich's ataxia. This study will involve five visits to a research clinic over a 12-month period. Sites are open at Long Beach CNS, UCLA, University of Iowa, and University of South Florida. Click here for the eligibility criteria and study schedule.

FARA's Director of Patient Engagement, Susan Walther recently conducted a Q& A with Retrotope's Vice President of Medical Affairs, Dr. Mark Midei, about their study drug. You can access that interview by clicking the image below.



The Friedreich's Ataxia Health Index Study

This study aims to identify the symptoms that have the greatest impact on quality-of-life for individuals with FA. The results of the study will help guide future research involving FA patients through the development of a patient-reported measure of health. The study involves completing a survey to include questions on demographics and symptoms of FA. Please see this recruitment flyer for more information and to access the survey links.

Anyone considering participating in a clinical trial should discuss the matter with his or her physician. FARA does not endorse or recommend any particular studies.

Newly Funded FARA Grants

Cardiac Imaging Biomarkers in Friedreich's Ataxia

Postdoctoral Research Award: Marcondes França Jr, MD, PhD and Thiago de Rezende, MSc – University of Campinas, Brazil

Cardiac studies are needed in Friedreich´s ataxia (FRDA) to provide relevant information on the natural history and pathophysiology of the disease, in order to identify useful and sensitive biomarkers for clinical trial and follow-up. Cardiac magnetic resonance imaging (cMRI) has emerged as a promising diagnostic technique. Click the Cardiac Research tab to read more.

Mechanisms of diabetes mellitus related to Friedreich's Ataxia

Postdoctoral Fellowship: Jaclyn Tamaroff, MD – Children's Hospital of Philadelphia, USA

Friedreich's Ataxia (FA) related diabetes affects 5% to 40% of individuals with FA. In children FA-related diabetes presents differently than in adults, but studies assessing how the body uses sugar (glucose) have not yet been done in children. Dr. Tamaroff plans to enroll twenty children, ages 7 to 17 years, who have FA but are not known to have diabetes in order to better understand how their bodies process glucose. Click the Outcome Measures & Biomarkers tab to read more.

Meet the Researcher

We're excited to introduce our new monthly "Meet the Researcher" interview series brought to you by the Ambassador Program. Each month, we will introduce you to a member of FARA's Scientific Advisory Board. The first interview in our series is with FARA's Associate Director of Research- Elisabetta (Liz) Soragni.

"All FA patients I have met throughout the years have been an inspiration and the biggest motivation for our research. Sometimes we lab rats need to be reminded why we are doing what we are doing and that our jobs have a beautiful purpose."

Click here to read Liz's interview and to access the other Meet the Researcher interviews on the Ambassador blog.

Cure FA Soirée

The Gehr family invites you to the **VIRTUAL 3rd Annual Cure FA Soirée** on Saturday, May 16, 2020, from 7:00-9:00 pm (Central).

An evening filled with music, art, and stories of courage in the face of a shortening and debilitating genetic disease with no cure…yet.

Click here for more information.

Working Together, Even When We are Apart!

Ask Congress to Expand Paid Leave During COVID-19 to Cover the Rare Disease Community

NORD is asking for our help in asking Congress to expand paid leave during COVID-19 to cover the rare disease community. They will generate letters to your US Senators and US Representative based on the address you provide. There is a spot where you can include personal information (explain FA, how that impacts your life, how has COVID-19 changed that, etc). Elected officials are counting on us to educate them as to what measures would help constituents. It will only take a few minutes and sharing your story could make the difference in someone voting yes to paid leave for the rare disease community.
Click here to learn more.

We hope you and your family are staying safe during this time!

So far Congress has passed three COVID-19 relief packages and is considering a fourth. Last week, NORD joined with 156 other patient organizations calling on Congress to expand eligibility for the paid family and medical leave program to include high risk individuals and working members of their households in the next COVID-19 relief package.

Currently, the law provides paid sick leave in limited circumstances for certain employees during the COVID-19 pandemic, but this doesn't go far enough. People living with rare diseases could be more vulnerable to severe complications from COVID-19. Yet, they and their working family members may not be eligible for leave if they decide to stay home to avoid exposure of COVID-19. Family members of loved ones with rare diseases shouldn't be forced to choose between losing their job or putting their loved ones at risk.

We need YOU to join our efforts by advocating for this important expansion of leave in the next COVID-19 relief package.

Ask Congress to Support Scientific and Medical Researchers

Join Research!America in asking Congress to support scientific and medical researchers! Their policy alert will also generate the letter to your Congressional member once you enter your home address. This funding would be very helpful to all who support FA research! Click here to sign up.

NORD Launches Financial Assistance Program

NORD also launched a new financial assistance program to support critical, non-medical needs in the rare disease community impacted by COVID-19. Through the new program, NORD will provide financial assistance to eligible patients, covering up to $1,000 annually. It provides funds for essential expenses including, but not limited to unexpected utility expenses; cellular or internet service; emergency repairs to car, home or major appliances; and rent or mortgage payment assistance. Interested rare disease patients and families can reach out to NORD to find out if they meet eligibility requirements. For more information on the NORD COVID-19 Critical Relief Program and to apply, please contact NORD by telephone at 203.242.0497, via email at COVID19assistance@rarediseases.org, or visit rarediseases.org.

Research!America FA Fact Sheet

One One of our advocacy partners, Research!America, runs a program where they create one pagers on various diseases/agencies/locations. FARA has been working with them to create a fact sheet on FA. This document can help you explain FA and the value of research when visiting with your elected officials (when we can do that again!). Click here to view the Friedreich's Ataxia Research!America fact sheet.

Sign Up for the Patient Registry Today!

Friedreich's Ataxia Global Patient Registry	The goals of the FA Global Patient Registry (FAGPR) are to collect information on all FA patients in one registry, to develop the registry into a powerful resource for research, and to engage the FA community in studies aimed at advancing our knowledge of FA and the treatments being developed.
This registry is only for people diagnosed with Friedreich's ataxia. Parents of minors diagnosed with FA can register an account on behalf of their children. Be sure you are included in the new registry. Click the button below to enroll and to engage with resources related to living with FA.	

Register Today!