FARA Research Priorities

The FARA grant program supports basic, translational and clinical research with the goal of advancing therapeutic development in FA. All proposed research must fall within FARA's Grant Program Priorities, which are as follows:

FARA Grant Program Priorities	
Advancing understanding of neuroscience/ systems	Understanding the neurodevelopmental and neurodegenerative changes of FA and their implications for pathogenesis and therapies. Special consideration will be given to proposals addressing non cell-autonomous mechanisms as contributors to the pathophysiology of FA and as in relation to the development of effective treatments.
Advancing understanding of cardiac disease in FA	Several areas of research are a high priority: • Characterizing the molecular mechanisms, genetic factors, or biochemical pathways that determine heart health in FA, with a special emphasis on understanding the metabolism of the FA heart • Determine if cardiac function has a significant role in the development of fatigue in FA • Comparisons of FA hearts to other genetic and non-genetic cardiomyopathies, with the goal of identifying disrupted pathways that could potentially respond to treatments that are effective in more common forms of cardiac disease • Determine and validate quantitative tools reporting on functional clinical endpoints for heart in FA, such as cardiopulmonary exercise testing. This may also include development of new tools or approaches to accommodate the exercise limitations of FA patients. • Identification of biomarkers that are predictive of cardiac outcome or can be used in risk stratification of disease • Identification of biomarkers that can be used as a pharmacodynamic response to treatment and/or to monitor outcomes • Developing therapies to reduce morbidity and mortality of the cardiac disease. This may include assessing currently approved therapeutic interventions as applied to FA
Advancing understanding of the molecular basis of FA	Understanding the normal function(s) of frataxin and the consequences of loss of frataxin, as well as the precise mechanisms of FXN gene silencing as they relate to the identification of novel therapeutic targets. Priority will be given to proposals that explore the metabolic basis of cell/tissue-type specific vulnerability, and the role of stress in regulating frataxin expression and function.
Advancing drug discovery	Developing effective therapies for FA. Priority will be given to genetic, epigenetic, and protein replacement approaches that increase frataxin levels. FARA will consider high feasibility therapeutic discovery projects focused on pathways likely to affect disease progression and key symptoms.
Facilitating the drug development process and translational research	FARA prioritizes funding of in vitro and in vivo preclinical studies aimed at facilitating the rapid translation to the clinic of promising therapeutic approaches. These include studies to evaluate mechanism of action and target engagement, drug efficacy, safety and toxicity profiles in animals, identification of lead candidates, exploration of drug delivery systems (with particular focus on non-viral gene and protein delivery). High priority will be given to the discovery and validation of clinical outcome assessments and biomarkers, such as development of methods to measure frataxin in affected tissues and identification of novel pharmacodynamic markers to evaluate the response to therapeutics in affected tissues, in early stage clinical trials.

Advancing clinical research	FARA supports clinical research that informs and creates resources necessary for drug and clinical development, furthers our understanding of the natural history and improves clinical outcomes for those living with FA. These include natural history studies, biomarker and functional outcome measure discovery and validation, and investigator-initiated clinical trials. Special consideration will be given to proposals addressing: • the clinical and metabolic characterization of fatigue in FA; • identification of early (including pre-symptomatic) quantifiable functional and clinical outcome measures for pediatric clinical trials; • identification of novel, clinically meaningful, functional endpoints with measurable changes detectable within one year, especially those based on the use of digital and at-home monitoring devices. Priority will be given to proposals that utilize or expand resources of the Collaborative Clinical Research Network in FA

Apply now!
Click here to access FARA's grant submission portal.

(https://webportalapp.com/sp/fara_grants)

Grant program updates and announcements

View special interest RFPs and other grant program updates and announcements **here** (/grant#p1).

Newly awarded grants

Gene & Stem Cell Therapy (/grant-awards#d2c)
Changfan Lin, PhD - Caltech
Engineering adeno-associated viral vectors to evade immune responses

Mechanism or Pathway of Disease (/grant-awards#d4c)
Sarah Robinson-Thiewes, PhD - St. Jude Children's Research Hospital
Illuminating how SynGRs liberate gene expression from heterochromatin

Anna Stepanova, PhD - Weill Cornell Medical College
Calcium communication among intracellular compartments in FA patient-derived cells

David Lynch, MD, PhD - Children's Hospital of Philadelphia
Understanding ketogenesis in FRDA: Pathophysiology, biomarkers and nutritional therapies

Outcome Measures & Biomarkers (/grant-awards#d7c)
Louise Corben, PhD - Murdoch Children's Research Institute
Measuring ataxia in children with Friedreich ataxia

Joseph Baur, PhD & Shana McCormack, MD - University of Pennsylvania and Children's Hospital of Philadelphia
Detection and enhancement of tissue NAD+ levels in Friedreich's Ataxia

Ankur Jain, PhD & Ricardo Mouro Pinto, PhD - Harvard Medical School and Massachusetts General Hospital
A New Ultrasensitive Single-Molecule Assay for Frataxin Measurement

Improving Clinical Outcomes (/grant-awards#d9c)
Manuela Corti, PhD & Tanja Taivassalo, PhD - University of Florida
Functional electrical stimulation (FES) cycling training to improve motor and cardiac functions in patients with Friedreich's Ataxia: a feasibility and efficacy study

Are you subscribed to our science newsletter, Friedreich's ataxia Community Research News?
If you are not a subscriber, please **click here** (https://visitor.r20.constantcontact.com/manage/optin?v=001LJMCW0I5jW0Cqv-E-)F0WiJfO2UVCrFlX3pczFqWCMtvcJTgOKGsG2zBAXes_TUoixshzEcZ5gFWBdcasJfRtGbVa2W7bdADbTJ0uKFv43HAjL7MDQFvcCBxCdwCjFF1weGL6dymgKBuOjBTYUT1a to sign-up!

SHARE

(https://www.facebook.com/CureFA/) (https://twitter.com/CureFA_org) (https://www.linkedin.com/company/fara)

(https://www.youtube.com/user/FARA1998)


FARA
www.cureFA.org