Clinical Research Resources

FARA-funded research has facilitated the discovery and development of research resources such as animal models, cell models, antibodies, biorepositories etc. We are grateful to the discovery scientists who have worked hard to bring us these important assets and continue to give to the community by sharing their results, knowledge, expertise and resources. It is our goal to promote collaboration throughout the research community by communicating with the discovery, translational and clinical scientists and facilitating their access to such resources. For more information visit: http://www.curefa.org/researchresources.

Collaborative Clinical Research Network

Clinical Network: FARA and clinical researchers established the Collaborative Clinical Research Network in Friedreich's Ataxia (CCRN), which is a growing international network of a dozen clinical research centers that work together to understand natural history, test and validate endpoints and biomarkers and advance treatments and clinical care for individuals with FA. Locations include Children's Hospital of Philadelphia, University of California Los Angeles, Emory University, University of Iowa, Ohio State University, University of Florida, University of Colorado, University of Rochester and University of South Florida in the USA along with sites in Australia, Canada, and Brazil.
The CCRN has been in place for over 15 years All recent FA clinical trials have been conducted through the CCRN.

Contact: Jen.Farmer@curefa.org

Natural History Data

The CCRN has collected natural history data on over 1000 FA patients. The natural history for more than 500 patients goes back more than 5 years and has more than 250 patients with baseline visits before 18 years of age. This data is available on request for data mining to answer questions on clinical protocol design, end points, biomarkers etc. It is possible to use this natural history data to expand the interpretation of data collected in clinical trials. FARA is working with the Critical Path Institute to integrate natural history data with data from the placebo arm of past clinical trials in order to expand the database and provide insights into the placebo effect. This database will be formatted using CDISC standards, so will be suitable for regulatory uses. In 2017/2018 the CCRN received a special grant from the FDA to further study the natural history and outcome measures in young children with FA.

Contact: Jen.Farmer@curefa.org

Patient Registry

FARA created and maintains the largest worldwide registry of FA patients. The registry currently has more than 36000 registered patients. This registry has been valuable in recruiting for clinical

trials and understanding the prevalence and geographic distribution of FA patients worldwide. FARA has successfully recruited patients for 11 trials, representing all three phases of clinical trials, and can do so quickly – we filled an adult phase 2 trial of 60 patients in only 3 hours and a pediatric trial of 30 patients in under 2 hours. Most FA trials recruit within only a few weeks rather than the months or years sometimes seen in other diseases. This registry is currently moving to a new platform which will expand the research capacity of the registry and allow for improved engagement with international community. The FA patient community is well informed, educated, and motivated.
Contact: Susan.walther@curefa.org

Patient Preference Data

In 2017 FARA held a Patient Focused Drug Development Meeting focused on identifying patient experience with FA and preferences for treatment. Data collected from that meeting and information relating to patient preferences in drug development is in the Voice of the Patient report at http://www.curefa.org/patient-focused-drug-development. Raw data may be made available for specified research questions.
Contact: Jane.Larkindale@curefa.org

Common Data Elements:

The National Institute of Neurological Disorders and Stroke (NINDS), at the National Institutes of Health (NIH), has developed Friedreich's ataxia Common Data Elements (CDEs) for use in clinical research. The CDE Project aims to develop content standards, both generic and disease-specific, that enable clinical investigators to systematically collect, analyze, and share data across the research community. NINDS assembled an external working group of experts, the Friedreich's ataxia CDE Working Group, to develop "Version 1.0" of the Friedreich's ataxia CDEs and they are ready for use in Friedreich's ataxia clinical research community. The Friedreich's ataxia CDE Working Group has focused on isolating elements that will be useful across multiple Friedreich's ataxia clinical studies. The NIH is currently working with the community to develop a "Version 2.0" of these elements, which are available at https://www.commondataelements.ninds.nih.gov/FA.aspx#tab=Data_Standards.

For more information can be found here:

- Lynch D, Pandolfo M, Schulz J, Perlman S, Delatycki M, Payne RM, Shaddy R, Fishbeck K, Farmer J, Kantor P, Raman S, Hunegs L, Odenkirchen J, Miller K, and Kaufmann P. (2013) Common data elements for clinical research in Friedreich's ataxia. *Mov. Disord.* **28**: 190-5.