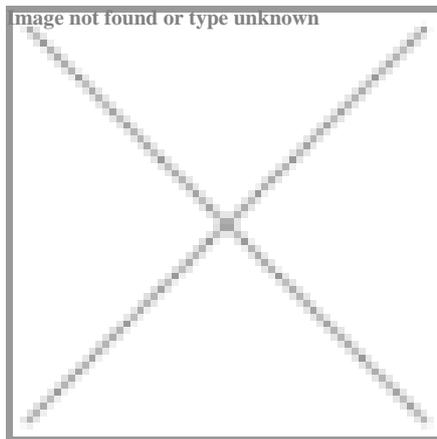
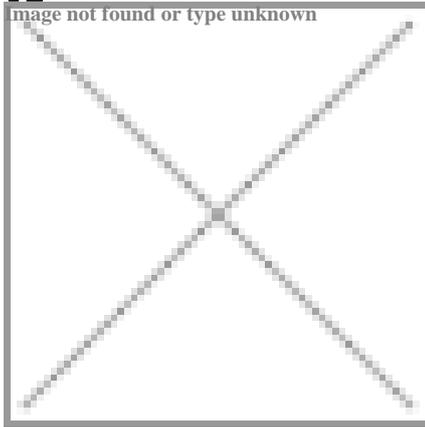


C-Path and FARA Launch Friedreich's Ataxia Integrated Clinical Database to Advance the Development of Treatments for FA



TUCSON, Ariz. and DOWNINGTOWN, Penn., February 27, 2019 — Critical Path Institute's (C-Path) Data Collaboration Center (DCC) and the Friedreich's Ataxia Research Alliance (FARA) today announced the launch of the Friedreich's Ataxia Integrated Clinical Database (FAICD). The new platform will enable collaborative research and data sharing to support the understanding of natural history, potential biomarkers and clinical endpoints, and promote research into novel clinical trial design in Friedreich's ataxia (FA). By making this data available to researchers, the organizations hope to enable the development of tools that will help design and interpret efficient clinical trials — leading to effective treatments for FA as soon as possible.

“FA is a rare, progressive and fatal disease that affects multiple organ systems, and those living with the disease are in urgent need of effective treatments,” explained FARA’s Executive Director Jennifer Farmer. “FAICD addresses this need by providing a platform to share data and making it available to qualified researchers to expedite the drug development process. We believe this resource will inform and give future clinical trials of potential therapies the best chance of success.”

FAICD contains data contributed by collaborating companies that have carried out clinical trials in FA, as well as natural history and clinical outcome measure data from the Friedreich's Ataxia Clinical Outcomes Measures Study (FACOMS) collected by the Collaborative Clinical Research Network in FA and funded by FARA.

All data contributed to this project is de-identified, mapped to standards defined by the Clinical Data Interchange Standards Consortium (CDISC) and curated by C-Path's DCC before inclusion in FAICD.

“C-Path has a long history of expertise in data standards development, curation, and oversight of multiple data sharing initiatives,” said Richard Liwski, Director of the DCC and C-Path's Chief Technology Officer. “We are proud to be part of the effort to coordinate collaborative contributions from data owners and

integrate that data into a single database for this rare, degenerative and life-shortening neuromuscular disorder.”

Researchers interested in contributing data to FAICD and/or utilizing the database to advance FA-related research initiatives can learn more at <https://cpathdev3.lotosnile.com/programs/dcc/projects/friedreichs-ataxia/>.

About Friedreich’s Ataxia:

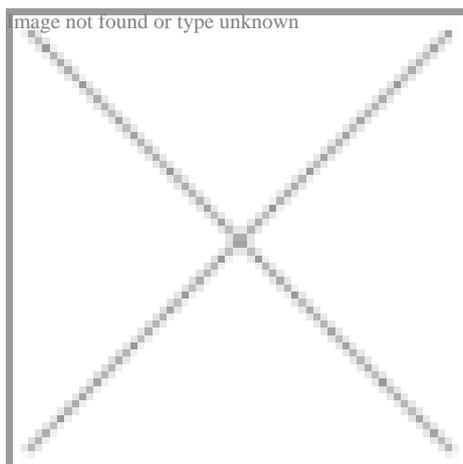
Friedreich’s ataxia (FA) is a debilitating, life-shortening, degenerative neuromuscular disorder. About one in 50,000 people in the United States has FA, with most having onset of symptoms between the ages of 5 and 18. Adult, or late-onset, FA is less common (less than 25 percent of diagnosed individuals), and can occur anytime during adulthood.

The signs and symptoms of Friedreich’s ataxia are:

- loss of coordination (ataxia) in the arms and legs
- fatigue, energy deprivation and muscle loss
- vision impairment, hearing loss, and slurred speech
- aggressive scoliosis (curvature of the spine)
- diabetes mellitus (insulin-dependent, in most cases)
- serious heart conditions, including hypertrophic cardiomyopathy and arrhythmias

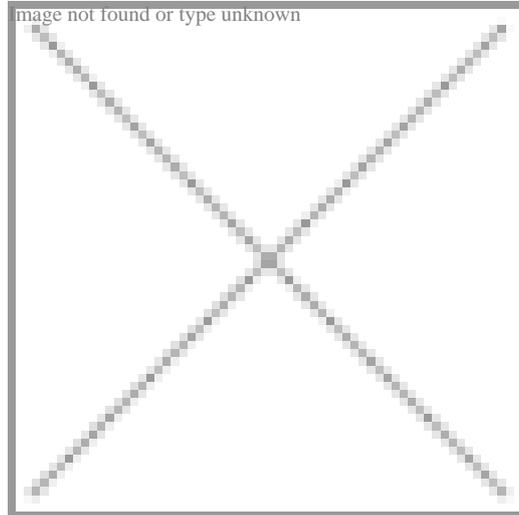
The progressive loss of coordination and muscle strength leads to motor incapacitation and the full-time use of a wheelchair. Most young people diagnosed with FA require mobility aids such as a cane, walker or wheelchair by their teens or early 20s. There is presently no treatment for FA.

About the organizations:



C-Path (Critical Path Institute) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path’s mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader

in forming collaborations, C-Path has established numerous global consortia that currently include over 1,500 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and dozens of pharmaceutical and biotech companies. C-Path is headquartered in Tucson, Arizona, with additional staff in multiple remote locations. For more information, visit www.c-path.org.



The **Friedreich's Ataxia Research Alliance (FARA)** is a national, public, 501(c)(3), nonprofit, tax-exempt organization dedicated to the pursuit of scientific research leading to treatments and a cure for Friedreich's ataxia. FARA's mission is to marshal and focus the resources and relationships needed to cure FA by raising funds for research, promoting public awareness, and aligning scientists, patients, clinicians, government agencies, pharmaceutical companies, and other organizations dedicated to curing FA and related diseases. For more information, visit www.curefa.org.

Contacts:

Kissy Black
Critical Path Institute
615.298.1144
kissyblack@lotosnile.com

Felecia DeRosa
FARA
484.879.6160
felicia.derosa@curefa.org