



Friedreich's ataxia (FA) is a debilitating, degenerative neuro-muscular disorder. About 1 in 50,000 people in the United States have FA. It is considered a rare disease, based on the fact that it affects fewer than 200,000 people in the US. Onset of symptoms is usually between the ages of 5 and 15. However, some patients are diagnosed with late onset FA.

Symptoms can include:

- 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)
- a serious heart condition (enlarged heart-hypertrophic cardiomyopathy)

Each of these symptoms occur at varying degrees in individual FA patients

What is FARA

The Friedreich's Ataxia Research Alliance (FARA) is a 501c3 non-profit that drives medical and research advances for Friedreich's ataxia. FARA was founded in 1998 by a group of parents, patients, and scientists wanting to make a difference. FARA has grown to be a leading, recognized force in FA research.

Rapidly Advancing Research

FARA advances FA research by providing competitive grants to leading scientists worldwide to identify scientific and medical discoveries and translate them into treatments. Proof of progress and success is that clinical trials for new therapies are in process. We have reached the clinical era in research.

FARA assembles the necessary resources through grassroots fundraising, individual and corporate donors, foundations and by partnering with government, pharmaceutical companies, and other advocacy organizations around the world. Together, we will find a cure!

You Can Help

FARA's research program would not be successful without the support of grassroots fundraising, individual and corporate donors and foundations. As you consider ways to get involved, please consider an investment in finding a cure.

- local fundraisers
- individual and corporate donations
- corporate matching programs
- memorial gifts

Visit <http://www.curefa.org/help.html>

If you do not have access to the internet, please call your physician or contact the FARA office at 484-875-3105.

HELP US ADVANCE THE RESEARCH TO CURE FRIEDREICH'S ATAXIA

**Acting alone,
there is very little
we can accomplish,
Acting together,
there is little we
will not accomplish.**

Ron Bartek

FARA Founder and President

Navigating the terrain of a new diagnosis can be overwhelming. This brochure will help you find the basic and important resources that guide patients, families, and physicians through this new world.

These resources will help you find your way to a caring community that is committed to supporting one another and the advancement of research to cure Friedreich's ataxia.

Making a Difference

After your diagnosis, you may want to investigate more about FA and what steps you can take to become more involved in FA patient care and research. Following are some key steps you can take:

- Learn more about Friedreich's ataxia and FARA. Join the mailing list for e-updates.
www.cureFA.org
- Sign up on the FA Patient Registry. If you or your child has been diagnosed with FA, you will be notified through the registry about important research, including clinical trials, in which you may be eligible to participate.
www.curefa.org/registry
- Make an appointment at one of the Collaborative Clinical Research Network in FA sites listed in this brochure. These sites collect information on the progression of FA in each patient and provide excellent clinical care. This information is critical to the success of clinical trials, which take place at these sites.
www.curefa.org/network.html
- FA parents can seek support and advice from an established online parent's group.
www.faparents.org
- Get to know other FA families through fundraising events. A complete list of all events is listed on the FARA website.
www.curefa.org/events

The FA Patient Registry

Treatments are on the horizon. Get involved now! To ensure we reach the finish line, clinical trials are required to monitor and measure success. The FARA Patient Registry is our most important tool for helping researchers reach the patient community and recruit patients for clinical trials. The difference between a successful trial and those that are not, is usually sufficient patient participants.

Please register today! www.cureFA.org/registry



Dr. David Lynch, (middle) with Jeff and Debbie Golanec, FA Parents, not only dedicates much of his professional time to FA research, he also participates in Ride Ataxia, a FARA fundraiser held around the country.

The CCRN in FA

The Collaborative Clinical Research Network in Friedreich's Ataxia (CCRN in FA) was established to expedite the movement of research from the laboratory to patients and to provide FA patients with access to the most current clinical care available.

Each CCRN in FA site is a place where FA patients can go to participate in research studies and to receive clinical care. These specialty centers demonstrate the highest levels of experience and dedication to providing medical care for patients, while also delivering ongoing clinical research studies on FA.

Each CCRN in FA center is a launching pad for clinical trials with highly-trained staff and ready knowledge of patients who may be available for new drug trials. The investigators and coordinators at each of these sites are very familiar with medical issues related to FA and can provide clinical services, as well.

CCRN in FA Locations

To contact a CCRN in FA site nearest to you visit www.curefa.org/network.html for contact information or call FARA at 484-875-3015.

Children's Hospital of Philadelphia, Philadelphia, PA
Principal Investigator - Dr. David Lynch

University of California Los Angeles, Los Angeles, CA
Principal Investigator - Dr. Susan Perlman

University of Chicago, Chicago, IL
Principal Investigator, Dr. Christopher Gomez

Emory University, Atlanta, GA
Principal Investigator, Dr. George Wilmot

University of Minnesota, Minneapolis, MN
Principal Investigator - Dr. Khalaf Bushara

University of Iowa, Iowa City, IA
Principal Investigator - Dr. Kathy Matthews

University of Rochester, Rochester, NY
Principal Investigator - Dr. Bernard Ravina

University of Florida, Gainesville, FL
Principal Investigators, Drs. Tetsuo Ashizawa and Sub Subramony

University of South Florida, Tampa, FL
Principal Investigator, Dr. Teresa Zesiewicz

The Bruce Lefroy Centre for Genetic Health Research, Murdoch Children's Research Institute, Melbourne, Australia
Principal Investigator Dr. Martin Delatycki

Please make an appointment today!
www.cureFA.org/network.html