



## What is FA

Friedreich's ataxia (FA) is a debilitating, degenerative, neuro-muscular disorder affecting 15,000 people worldwide. It is caused by an inherited genetic mutation that limits the production of a protein called frataxin. 1 in 100 people are carriers and don't even know it until it affects their family. Onset of symptoms can occur at varying degrees of severity from childhood to adulthood.

Symptoms 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)

## Breakthroughs and Milestones

- 1996 Disease-causing gene mutation identified
- 1998 FARA was founded
- 2001 The first animal models of FA were created
- 2006 Number of researchers working in FA doubled and FARA Patient Registry was launched
- 2007 International Collaborative Clinical Research Network for FA was established
- 2009-11 Number of new drug candidates and pharmaceutical companies interested in FA doubled
- 2012 Gene therapy shown to reverse FA cardiac disease in a mouse model
- 2014 FARA established Penn Medicine / CHOP Friedreich's Ataxia Center of Excellence; 3 new pharmaceutical companies formed to advance gene therapy in FA
- 2015 FARA launched a collaborative FA Biomarker initiative with industry and academic partners
- 2017 FARA co-hosted an Externally-led FA Patient Focused Drug Development Meeting to educate FDA Representatives and Pharma Partners about the patient experience



## Contact

### Friedreich's Ataxia Research Alliance (FARA)

533 W. Uwchlan Ave, Downingtown, PA 19335, USA

Email: [info@cureFA.org](mailto:info@cureFA.org)

Phone: (484) 879-6160

Fax: (484) 872-1402



[www.cureFA.org](http://www.cureFA.org)

# 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



[www.cureFA.org](http://www.cureFA.org)



## About FARA

The Friedrich's Ataxia Research Alliance (FARA) is a 501(c)(3) non-profit that drives medical and research advances for Friedrich's ataxia. FARA was founded in 1998 by a group of parents, patients, and scientists wanting to make a difference and has now 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 underway. We have reached the clinical era in research.

## Since 1998, FARA has

- Funded \$30 million in FA drug discovery, drug development, and clinical research
- Created a worldwide FA patient registry to link researchers to individuals with FA
- Forged an FA scientific community by aligning a few independent investigators early on and growing interest in FA to hundreds of scientists in academia and the pharmaceutical industry
- Educated the medical and patient communities regarding FA and research advances
- Raised awareness of FA so that those diagnosed are no longer isolated

## The FA Patient Registry

The FARA Patient Registry is the only worldwide registry of patients with Friedrich's ataxia, holding the demographic and clinical information on more than 2,000 people from over 65 countries. It is our most important tool for helping researchers reach the patient community and recruit individuals for clinical trials. The Patient Registry has successfully recruited clinical trials and research studies for pharmaceutical and academic partners.

To register, visit: [cureFA.org/patient-registry](http://cureFA.org/patient-registry)



## Grant Program

FARA supports research through funding competitive grants, promoting collaboration among scientists, and advocating for public-private partnerships that support drug discovery, drug development and clinical research.

For a complete list of funded research projects, visit: [cureFA.org/grant](http://cureFA.org/grant)

## Scientific Conference Program

FARA has organized and supported a number of scientific conferences to keep the field informed of research progress and build collaborations and synergistic connections between FA researchers. FARA's International Scientific Conference on Friedrich's Ataxia has grown with each iteration demonstrating the remarkable research advances into the underlying mechanisms of FA and increased interest within the scientific research community.

## The CCRN in FA

The Collaborative Clinical Research Network in Friedrich's Ataxia (CCRN in FA) is an international network of clinical research centers that work together to advance treatments and clinical care for individuals with FA. The network collaborates with pharmaceutical companies, government agencies and other research centers and the patient community to facilitate clinical research and trials needed to identify new therapies.

Each of the CCRN in FA sites is a place where FA patients can go to participate in research studies and to receive clinical care. At every site researchers are conducting natural history studies and clinical trials. The investigators and coordinators at each of these sites are very familiar with medical issues related to FA.

To find a CCRN in FA site, visit: [cureFA.org/network](http://cureFA.org/network)



## 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