

What is Friedreich's Ataxia (FA)?

N. (*frē-dricks ah-tax-ē-ah*)

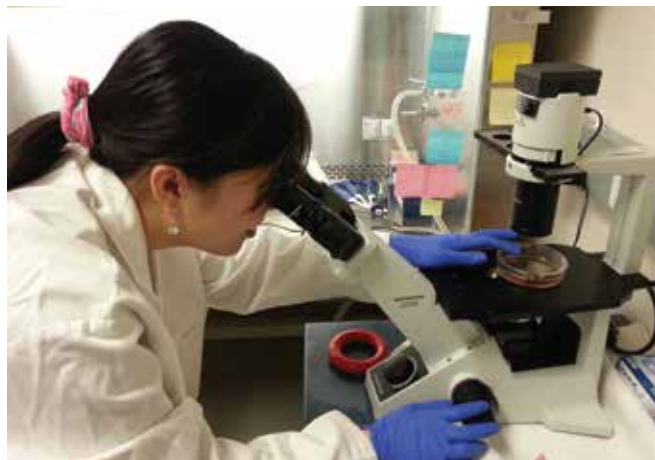
Friedreich's ataxia (FA) is a debilitating multi-system disease affecting 5,000 people in the US and 15,000 worldwide. 1 in 100 people are carriers of the FA gene. What begins as difficulty with balance and coordination progresses over a short period of time to a life altering loss of mobility, energy, speech and hearing. FA also presents serious risk of diabetes and life shortening cardiac disease.

As of today, there is no treatment or cure.

Symptoms of Friedreich's ataxia include :

- Loss of coordination in arms and legs
- Energy deprivation and muscle loss
- Severe scoliosis (spinal curvature)
- Diabetes
- Impaired vision, hearing and speech
- Cardiomyopathy and arrhythmia

What is FARA?



The Friedreich's Ataxia Research Alliance (FARA)

FARA is a national, public, 501(c)(3), non-profit, tax-exempt organization dedicated to curing Friedreich's ataxia (FA) through research.

Mission:

- to marshal and focus the resources and relationships needed to cure FA.

Strategy:

- to grow the scientific community and industry relationships
- to fund basic and translational research to advance our knowledge of FA
- to promote drug discovery and develop tools that advance drug development
- to sustain and strengthen the Clinical Research Infrastructure
- to address FA regulatory issues; advocate with the FDA, NIH, EMA
- to grow and strengthen FA patient engagement and education

Advancing Treatments

FARA works to advance progress, and has put significant effort into developing the infrastructure needed to accelerate therapeutic programs. FARA maintains a **Patient Registry** that includes more than 2,200 FA patients. This allows for clinical trials to recruit participants quickly – and recent FA clinical trials have recruited in just days. FARA funds and leads the **Collaborative Clinical Research Network**, or CCRN, with sites in the USA, Canada, Australia, and Brazil. The network has been in place over 10 years, and has lead critical clinical research studies in FA, including natural history studies, outcome measures, and clinical trials. The CCRN has an outstanding reputation as a partner. The **Natural History Study** has over 800 patients enrolled, evaluated annually, and over 100 patients have data for over 10 years. The CCRN is deeply involved in biomarker studies and end point development; and has developed and validated the **Friedreich's Ataxia Rating Scale** that is used as an endpoint in clinical trials. FARA has also invested in **Tools Needed for Research**, including mouse models, biorepositories, gene expression datasets and cell lines.



Advancing Research into Treatments for Friedreich's Ataxia

CUREFA.ORG