



Friedreich's  
Ataxia  
Research  
Alliance



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## **Four National Organizations, Hundreds of Families Impacted, Join Together to Speak with FDA about Drug Development in Friedreich's Ataxia**

**HYATTSVILLE, Md. June 1, 2017**—The Friedreich's Ataxia Research Alliance (FARA), National Ataxia Foundation (NAF), Muscular Dystrophy Association (MDA) and Cure FA Foundation will host the first ever Friedreich's Ataxia (FA) Patient-Focused Drug Development (PFDD) meeting on Friday, June 2, to give patients, families and caregivers the opportunity to speak directly to the U.S. Food and Drug Administration (FDA) about the impact FA has on their daily lives, and their highly unmet need for effective treatments.

Friedreich's ataxia is a debilitating, life-shortening and degenerative neuromuscular disorder. Loss of balance and coordination is the most common presenting symptom typically beginning between the ages of 5 and 15 years, with progression of symptoms leading to loss of ambulation and independence of all activities of daily living. It affects 1 in 50,000 people in the U.S. and is a multi-system disease. Symptoms can also include, muscle loss, scoliosis, vision and hearing impairments, slurred speech, diabetes mellitus and serious heart conditions. Adult or late-onset FA accounts for less than one quarter of all cases.

At the upcoming FA PFDD meeting, panelists and audience members will be invited to share their unique insight into what it is like to live with FA, including details about the symptoms that are most troublesome, current strategies for managing those symptoms and their urgent desire for meaningful therapies. The information captured at the meeting will be published in a document called *The Voice of the Patient Report* and submitted to the FDA to help evaluate future FA therapies — including what will be the first therapy for FA.

"The four nonprofit groups that have worked together to organize this meeting have developed a collaborative partnership over time that has supported research toward development of treatments for this rare and devastating disease," said FARA Executive Director Jen Farmer. "As a product of that collaboration, over 200 people, including about 25 FDA employees, 30 representatives from industry and over 140 patients and family members have registered to attend this meeting, and patients are looking forward to the opportunity to talk about what is meaningful to them in terms of a potential treatment. Although no treatments or cures are available for this devastating disease, there are promising therapies now being developed."

#### **FA PFDD Meeting Details:**

- When: Friday, June 2, 8 a.m. to 12:30 p.m. ET
- Where: College Park Marriott & Conference Center (3501 University Blvd E, Hyattsville, MD 20783) and live webcast for those who cannot attend, <https://youtu.be/Va1D4SqrSfw>
- More information and registration: [curefa.org/conference](http://curefa.org/conference)

The PFDD initiative is part of the FDA's commitments under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V), which aims to understand the burden of disease from the patient perspective and the factors for treatment decisions. Initiated as a series of FDA-led meetings to learn about 20 specific disorders, the FDA allows external stakeholders to hold their own PFDD meetings to engage the FDA through a formal process.

#### **About FARA**

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.

#### **About NAF**

The National Ataxia Foundation (NAF) is a nonprofit organization dedicated to finding a cure for Ataxia. NAF funds research for the disease, serving all types of Ataxia. NAF provides the most comprehensive information about Ataxia and is guided by the world's most renowned Ataxia scientists. Visit [ataxia.org](http://ataxia.org) to learn how you can get involved in finding the cure.

**About MDA**

MDA is a national nonprofit organization working to free individuals — and the families who love them — from the harm of muscular dystrophy, ALS and related muscle-debilitating diseases that take away physical strength, independence and life. Learn how you can fund cures, find care or champion the cause at [mda.org](http://mda.org).

**About CureFA Foundation**

CureFA Foundation is a tax-exempt 501(c)(3) organization dedicated to scientific research, building scientific infrastructure, and increasing information availability to the community working towards a cure for FA.

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