FARA Best Practices for Engagement with the FDA

FARA Background

- In 1997, Keith Andrus, then 11, was diagnosed with Friedreich's Ataxia (FA).

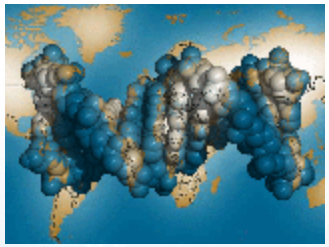
- Found plenty of bad news:

  - Affects vision, hearing, speech, scoliosis, loss of strength & coordination leading to wheelchair, diabetes, life-shortening heart condition.

  - No treatment, no clinical trials, very little research; no organization devoted solely to research of FA.

  - Isolated patients (~ 5,000 U.S., ~ 15,000 world), no hope or help.

Byron, Keith and Stuart Andrus;
photo courtesy of Raychel Bartek



FA and FARA – Getting Started

- Established FARA to focus on research
- Initial goals:

  - Grow the field to Identify and begin to fill the research gaps

  - Collaborate and cooperate rather than confront and compete
- Assembled the community – patients, families, scientists, companies, NIH, FDA etc.

FDA

NIH

"If you are interested in FA research and we are not collaborating with you, it means we have not found you, but we will."

FARA Friedreich's Ataxia Research Alliance

FARA – Focus on Partnership

When we see a problem, we assemble the people we need to resolve that problem. About 8 years ago we put together a meeting on clinical endpoints and invited FDA to participate.

They came, we discussed the issue, and it was the beginning of a partnership

FARA Friedreich's Ataxia Research Alliance

What works for us?

- We work under the assumption of partnership – what can we offer FDA, and what can they help us with?

- We are an organization focused on research – many of our interactions are based around determining what data the FDA needs to make regulatory decisions, and how we can get those data to them.

- We recognize that FDA is busy, and try to look to them for advice/consultation after we have done significant ground work and have specific questions that we need answered.

FARA – Focus on Partnership

Recent FARA/FDA meetings include:

- Meeting on mouse models for FA – with the office of orphan product development (OOPD)– January 2016
- Introduction to FA and resources available to aid understanding of FA Natural History for CBER – Jan 2017
- FA Patient Focused Drug Development Meeting – June 2017 [with partner organizations]

FARA also attends FDA meetings with any drug company that invites us, to offer the perspective of the patient.

Allowing the voice of the FA patient to be heard in drug development

- Welcome and introductory comments from FARA, MDA, NAF and the FDA
- Two patient/caregiver panels:
  - ✓ How FA symptoms affect your life
  - ✓ How you manage symptoms and current & future approaches to treatment
- Polling Questions & Moderated Discussion with the audience after each panel
- FDA Closing comments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