ASGCT 2019
Post-Approval Commercialization Workshop
Role of the Patient
April 28, 2019

Role of the Patient

Ron Bartek
Co-Founder/President
Friedreich's Ataxia Research Alliance (FARA)

FARA Friedreich's Ataxia Research Alliance

FARA Background

- FARA Co-Founder & President - 1997, son Keith, then 11, diagnosed with Friedreich's Ataxia (FA). Learned:
  - FA is rare, hereditary, life-shortening, neuro-muscular disease relentlessly degenerative, causing loss of vision, hearing, speech, strength & coordination in all four limbs, scoliosis, increased chance of diabetes, heart failure.
  - 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).
- But, one piece of good news—the FA gene had been identified one year earlier. Set out to:
  - Grow the field to Identify and fill the research gaps
  - Collaborate and cooperate rather than confront and compete
- Assembled full field 6 times ('99-'17- 7th in Nov) –from 80 scientists, 0 pharma, 1 other PAG to >400 scientists, ~3 dozen pharmas, 10 PAGs.
- World's largest funder of FA research.

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

FARA Friedreich's Ataxia Research Alliance

Role of FA Patient & FARA in Therapy Development

- Well before PFDD & many of the fantastic opportunities Celia has described, we began building the tools patients would need in playing their important role, taking advantage of those opportunities:
  - -- Communications, trust, hope then confidence;
  - -- Research grant program to characterize disease, mechanisms of potential therapeutics, etc.;
  - -- Contact Registry, Natural History Database, Clinical Network.
- Pursued collaborative relationships among academic investigators, government agencies, industry partners, other PAGs.
- Industry partners began inviting us to their FDA meetings (e.g. pre-INDs).
- We began working closely with NIH, serving on NIH National Advisory Councils; conducting international scientific workshops w/NIH support/participation, supporting applicants for NIH grants, etc.
- Began working closely with FDA, participating in public meetings including those first aimed at PFDD & resulting in PFDD provisions in PDUFA V (FDASIA), inviting FDA to participate in our scientific conferences.
- Provided patient reps for the FDA Patient Representative Program

NIH

FDA

FARA Friedreich's Ataxia Research Alliance

Role of FA Patient & FARA in Therapy Development

- Testified before congressional appropriators and advocated regularly in congressional offices seeking robust budgets & good policy for NIH & FDA, including 21st Cures, PDUFAs.
- Member of & serve on boards of NORD, Alliance for Stronger FDA, ARM.
- Invited to present at number of FDA symposia regarding FDA-Patient group collaboration.
- Industry partners continue inviting us to their FDA meetings.
- Have submitted public comments on FDA draft guidances, in close coordination with industry & advocacy partners.
- Requested & conducted meetings with FDA review divisions.
- Conducted our Externally Led Patient-Focused Drug Development Meeting w/ large FDA participation, Voice of the Patient Report, and tremendous benefits we continue to enjoy.
- Supported successful academic application for FDA/OOPD grant to support natural history study with pediatric patients.
- Privileged to serve on FDA Patient Engagement Collaborative.
- Post Commercialization – Looking Forward to it & the Patient Role.
- Summary – FDA, NIH Hear the Patient Voice – On our side.

NIH

FDA

FARA Friedreich's Ataxia Research Alliance