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Agenda for Externally Led Patient-Focused Drug Development Meeting Friedreich Ataxia

- 7:30 a.m. – 8:30a.m. Registration
- 8:30 a.m. – 8:50a.m. Opening Remarks & Overview of FA - *Jennifer Farmer, Executive Director, FARA & Ronald Bartek, Founding President and Co-founder, FARA*
- 8:50 a.m. – 9:10 a.m. FDA Welcoming Remarks - *Wilson Bryan, MD, Director of Office of Tissues and Advanced Therapies, CBER and Richard Moscicki, MD, Deputy Center Director for Science Operations, CDER*
- 9:10 a.m. – 9:20a.m. Meeting Overview - *James Valentine, JD, MHS, Associate, Hyman, Phelps and McNamara, P.C. Moderator*
- 9:20 a.m. – 9:30 a.m. Audience and Remote Polling: Demographic Information Collecting Patient Input
- 9:30 a.m. – 9:40 a.m. Video: “Our Life with FA - Always Changing”
- 9:40 a.m. – 10:05 a.m. Patient Panel 1: Living with Friedreich’s Ataxia – *Frankie Perazzola, Jack DeWitt, Kristin Morrow, RJ Mercure, Nelda Van Schoick*

Topic 1 Questions:

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life (Examples may include difficulty moving, issues with balance, issues with sight or hearing, lack of energy etc.)

2. How do your symptoms and their negative impacts affect your daily life?
3. Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition?
4. What worries you most about your condition, and the progression of your condition?

10:05 a.m. -10:45 a.m. Topic 1 facilitated audience discussion & audience and remote polling.

10:45 a.m.-11:00 a.m. Break

11:00 a.m. – 11:25 a.m. Patient Panel 2: Current & future approaches to treating Friedreich's Ataxia – *Patrick Ritschel, Nicholas Carbone, Emily Young, Alex Fielding, Mary Caruso*

Topic 2 Questions:

1. What are you currently doing to manage your symptoms? (examples might include scoliosis surgery, cardiac drugs, physical therapy, over the counter antioxidants, etc.)
2. How well do these treatments treat your symptoms?
3. How well do these treatments improve your ability to do specific activities that are important to you in your daily life?
4. What specific things would you look for in an ideal treatment for your condition, short of a complete cure?

11:25 a.m.- 12:05 a.m. Topic 2 facilitated audience discussion & audience and remote polling.

12:05 p.m. – 12:20 p.m. FDA Concluding Remarks - *Jonathan Goldsmith, MD, Associate Director for Rare Diseases, CDER*

12:20 p.m. – 12:30p.m. Closing Comments & Next Steps - *FARA*

Following the meeting, please join us:

12:30 p.m. - 1:30 p.m. Lunch

1:30 p.m. - 3:30 p.m. Research update for patients/caregivers