

FA Patient Focused Drug Development Meeting– Your opportunity to tell the FDA what is important to you in finding a treatment for Friedreich’s Ataxia

What is a Patient Focused Drug Development Meeting?

In 2015 the Food and Drug Administration (FDA) began a new initiative to expand the way it looks at therapies, specifically seeking input from patients. The agency wanted to hear directly from the community about patients’ needs, rather than assuming that internal FDA scientists understood every disease and patient population. Thus, it launched the Patient-Focused Drug Development (PFDD) Initiative, a series of public meetings led by the FDA and designed to systematically gather input from patients regarding their experience of living with specific diseases, the preferred impact of potential treatments, and the benefit/risk analysis when considering a new targeted medication.. The FDA scheduled the initial 20 meetings, many of which have taken place and are considered very successful. In order to reduce their administrative burden however, the FDA, has now moved to a new model where such meetings are led by patients and patient groups, with the FDA attending and participating. FA has been selected to be one of the disease areas to host such a meeting in 2017.

Why should you be involved in the Patient Focused Drug Development Meeting for FA?

The Patient Focused Drug Development Meeting is your opportunity to tell the FDA and drug developers what matters to you about living with FA, including symptoms that are most troublesome, and your vision for potential meaningful therapies . The information that we capture at the meeting will be published and submitted to the FDA’s Division of Neurology Products, Office of New Drugs, for inclusion in the framework used to evaluate future FA therapies. The FDA will use that information as it evaluates the potential benefits of a therapy to the FA patient population, relative to any potential risks. This is your opportunity to describe your view of a successful therapy, and how it would improve your quality of life. The report will summarize the input from patients across the country, so we encourage everyone to get involved, whether through in-person attendance at the meeting, on-line attendance and voting or through participation in surveys we send out to gather data prior to the meeting.

How does a Patient Focused Drug Development Work?

PFDD meetings are all designed with input and feedback from the FDA, and follow

a proscribed format, which typically includes patient panels focused on very specific questions from the FDA, and additional live feedback from meeting attendees on those questions and others the FDA may ask during the meeting. The FA PFDD meeting will run from 8am until to 12:30pm on June 2, 2017 at the College Park Marriott and Conference Center in Bethesda, MD.

During the meeting, invited panels of speakers selected to represent the diversity of the disease will briefly speak on each of the topics. We will include video comments from a few patients who have progressed in their disease to a level that they cannot attend, so as to represent all stages of disease. Members of the live audience will then have the opportunity to comment on the same topics, and everyone in the live and on-line audiences will be able to vote on answers to each question and see the results in real time. After the meeting, the in-person and online answers to the panel questions, along with data collected prior to the meeting, will be summarized as a "Voice of the Patient" report, which will be submitted to FDA.

How you can get involved:

- We encourage any interested patients, families or other caregivers to come to the meeting in Washington DC on June 2nd.
- If you cannot attend in-person, join the online streaming of the meeting and vote on the specific panel questions, as well as provide demographic information that will be useful for our report to the FDA. We will let you know how to do this closer to the meeting time.
- We will be seeking input from the community on the wording of the questions and to discuss your presentations prior to the meeting. Please keep an eye out for surveys and communications from us as to how you can contribute your thoughts.

How does this relate to past efforts?

FARA, National Ataxia Foundation and Muscular Dystrophy Association have all worked closely with various divisions of the FDA over time to help them to understand the disease and the needs of the community. We have had multiple meetings to discuss the disease in general; we've educated them on the tools and data we have available to help them to interpret new therapies; and we have accompanied drug developers to FDA meetings as they propose new therapies to move into the clinic. However, this meeting is the first opportunity for a group

of patients, families and other caregivers to speak directly to the FDA, and share their experiences in their own words. It is also the first time a systematic report on the patient experience will be completed and shared with the FDA. We need FA community participation to make sure our collective community voice makes a positive impact.

Why does the FDA Matter to you?

The Food and Drug Administration (FDA) is the government agency that regulates all new therapies (drugs and medical devices) as they move through development to commercially available medications. FDA scientists decide if a potential new drug is safe enough to be tested in humans, if it can continue development after early trials, and ultimately if there is enough evidence that it is effective so it can be approved for use in the U.S. Similar organizations regulate drugs in other parts of the world, but FDA decisions often influence approvals in other regions, and regulatory agencies often work together to study potential drugs, especially for rare diseases.