

Public Stakeholder Meeting on Prescription Drug User Fee Act (PDUFA) Reauthorization

October 30, 2020

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Outline for this meeting

- Welcome and Roll Call
- Presentation Topics:
 - Patient Focused Drug Development
 - Model-Informed Drug Development
 - Complex Innovative Designs for Clinical Trials
 - Other Areas of Regulatory Science: Advancing Translational Models & Tools
- Topics for upcoming meetings

Patient Focused Drug Development (PFDD)

October 30, 2020

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Enhancing the Incorporation of the Patient's Voice in Drug Development and Decision-Making

PDUFA VI Commitment articulates this overarching goal:

“To facilitate the advancement and use of systematic approaches to collect and utilize robust and meaningful patient and caregiver input that can more consistently inform drug development and, as appropriate, regulatory decision making”

PFDD: Enhancing Quality of Submitted Patient Experience Data For Regulatory Decision Making

Background

- Patient experience data can provide a direct source of evidence regarding drug benefits and risks. It can inform the choice of clinical trial endpoints to ensure that the endpoints being measured are endpoints that matter to patients, and it can be used to capture information on patient preferences and the potential acceptability of tradeoffs between treatment benefit and risk outcomes.
- **Soundness** of sponsor methods and approaches to developing and using patient experience data and their appropriateness to individual applications under consideration are **critical to their regulatory acceptability**.

PFDD Sustainability

What was committed to in PDUFA VI?

- ✓ **Strengthen staff capacity** to facilitate development and use of patient-focused methods to inform drug development and regulatory decisions. This staff, composed primarily of clinical, statistical, psychometric, and health outcomes research expertise, will be integrated into review teams as core members of the team during drug development and application review
- ✓ **Develop a series of guidance documents** to focus on approaches and methods to bridge from initial PFDD-type meetings to fit-for-purpose tools to collect meaningful patient and caregiver input for use in regulatory decision making.
- ✓ **Create and maintain repository** of publicly available tools on FDA's website as a resource for stakeholders, including COA compendium, patient-focused drug development meeting resources, and ongoing efforts on patient-focused drug development.
- **As appropriate, revise existing MAPPs and SOPPs** to include suggested approaches for incorporating an increased patient focus in other on-going or planned FDA public meetings. In addition, as appropriate, develop and implement staff training
- ✓ **By end of FY 2019, conduct a public workshop** with primary purpose of gathering ideas and experiences of patients and caregivers and recommendations on approaches and best practices to enhance patient engagement in clinical trials, with published report on proceedings and recommendations from the meeting.

PFDD: What is current status of commitments related to methodological guidances and public workshops?



<https://www.ctti-clinicaltrials.org/briefing-room/meetings/enhancing-incorporation-patient-perspectives-clinical-trials>

https://www.ctti-clinicaltrials.org/sites/www.ctti-clinicaltrials.org/files/meeting_summary_-_enhancing_incorporation_of_patient_perspectives_-_final.pdf

Create and maintain a publicly available repository of tools and resources


External Resources or Information Related to Patients' Experience

This webpage is intended to facilitate public discussion of patient-focused drug development and evaluation. This webpage provides links to certain publicly available external reports and resources relating to patient experience data. The patient community, patient advocates, researchers, drug developers, and federal agencies may find these materials useful.

Please note that although FDA reviews the materials at these links before posting them to ensure that the materials are within the scope of the webpage, FDA does not assess their scientific merit or compliance with regulatory requirements. Our decision to post links to these materials does not reflect an endorsement of their authors, sponsors, or content.

For more information regarding what types of resources may be included on this webpage, how to submit a publicly available website link to FDA, and other general questions, please review our [Frequently Asked Questions](#). We request that links include a [cover page](#) or similar opening statement as part of their report or resource to provide information about the authors, funding, and related information. For specific questions related to a report or resource, FDA recommends reaching out to the point of contact listed on this cover page.

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Externally-led PFDD Meeting Reports or Other Stakeholder Meeting Reports

Proposed Draft Guidance Relating to Patient Experience Data

Natural History Studies or other Disease-specific Background on Condition and Discussion of Unmet Medical Need

Externally-led PFDD Meeting Reports or Other Stakeholder Meeting Reports

To help expand the benefits of FDA's [Patient-Focused Drug Development \(PFDD\) initiative](#), FDA [welcomes patient organizations](#) to identify and organize patient-focused collaborations to generate public input on other disease areas. Submitted links to summary meeting reports from these [externally-led PFDD](#) meetings may be found here. FDA also welcomes submission of links to meeting reports from other stakeholder meetings collecting patient perspectives on disease burden and treatment burden.

• [Amyloidosis](#)

In November 2015, the Amyloidosis Research Consortium hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with systemic amyloidosis and their loved ones on the impact of amyloidosis on their daily lives, and their perspectives on approaches to treating amyloidosis.

• [Complement 3 Glomerulopathy \(C3G\)](#)

In August 2017, the National Kidney Foundation hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with C3G and their loved ones on the impact of C3G on their daily lives, and their perspectives on approaches to treating C3G.

• [Friedreich's Ataxia](#)

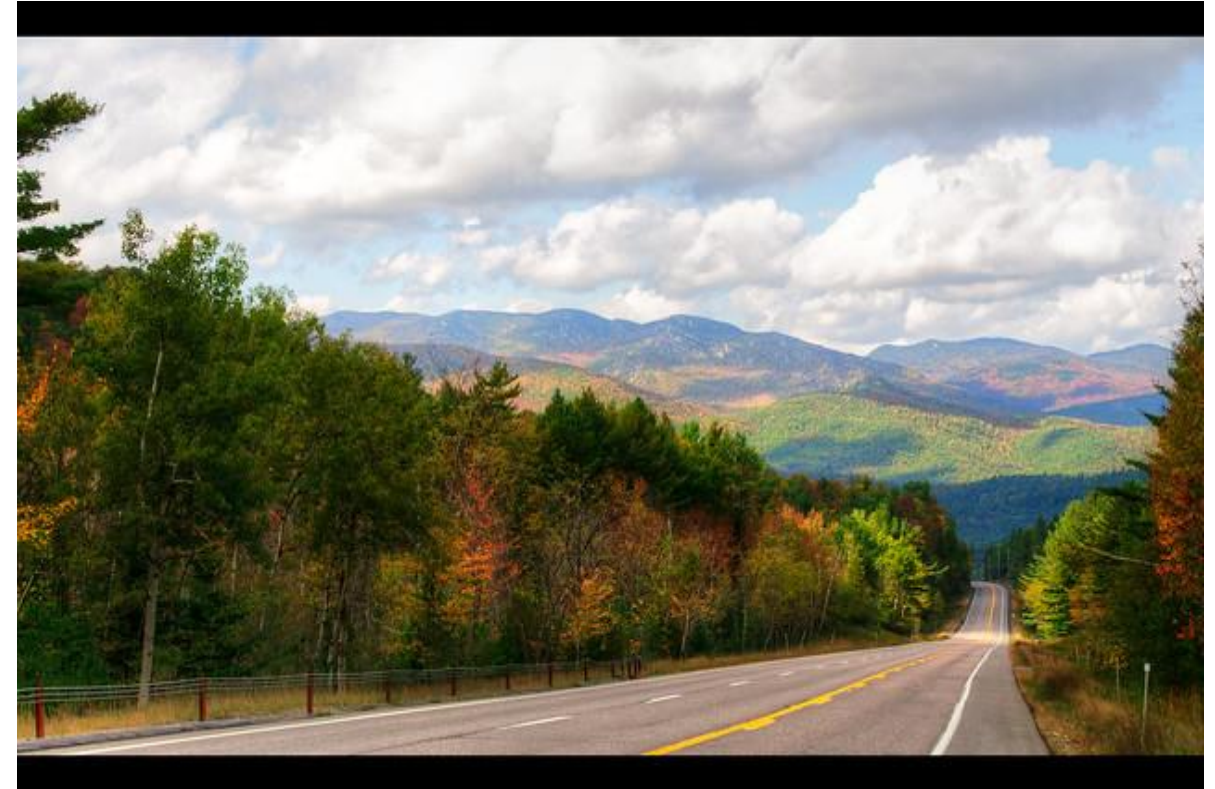
In June 2017, the Friedreich's Ataxia Research Alliance hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with Friedreich's Ataxia and their loved ones on the impact of Friedreich's Ataxia on their daily lives, and their perspectives on approaches to treating Friedreich's Ataxia.

• [Hyperhidrosis](#)

In November 2017, the International Hyperhidrosis Society hosted an externally-led Patient-Focused Drug Development meeting to hear directly from individuals living with hyperhidrosis and their loved ones on the impact of hyperhidrosis on their daily lives, and their perspectives on approaches to treating hyperhidrosis.

Sustaining integration of the patient's perspective –making it a standard practice

1. Ensure confidence in reliability and accuracy of patient experience data for regulatory decision making
2. Promote rapid consistent adoption
3. Increase predictability for sponsors
4. Sustained incorporation of patient's experience in drug development and decision making—make it standard practice



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<https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development>