Externally-led Patient-Focused Drug Development (EL-PFDD) High Level Overview

What is Patient-Focused Drug Development?

Patient-Focused Drug Development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.

FDA's Role in Medical Product Development and Evaluation

One of FDA's missions is to protect and promote public health by evaluating the safety and effectiveness of new drugs, biologics, and devices. **FDA does not develop drugs nor conduct clinical trials.** FDA does, however, play a constructive role in guiding, helping, or evaluating at some stages of the pre-clinical, translational, and clinical development work.

History

- The PFDD initiative started in 2012 as part of FDA's commitments under the Prescription Drug User Fee Act (PDUFA) V. After conducting FDA-led PFDD meetings, FDA recognized there are many more diseases/conditions that can be addressed beyond those that were planned and conducted by FDA.
- To help expand the benefits of FDA's PFDD initiative, in 2015, FDA announced the opportunity for externally-led (EL-PFDD) meetings. EL-PFDD meetings are planned and hosted by patient organizations, with the input of FDA staff, and use the process established by FDA-led PFDD meetings as a model.

Key Characteristics of PFDD Meetings

Both FDA-led and EL-PFDD meetings have key characteristics that set them apart from other public meetings. PFDD meetings target disease areas for which there is:

- an identified need for patient input
- a disease area that is chronic, symptomatic, or affects functioning and activities of daily living
- a disease area for which aspects of the disease are not formally captured in clinical trials
- a disease area for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives
- disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly).

PFDD meetings follow a town hall style discussion format. The majority of the meeting is dedicated to hearing from patients and caregivers about their perspectives on their condition. Participants are asked to share their perspectives during two panels followed by open discussion.

- The first panel focuses on the symptoms and daily impacts of the condition, while the second panel focuses on the current treatment approaches and what participants would look for in an ideal treatment. Panel two may also include a discussion of what patients consider when determining whether or not to participate in clinical trials, and a discussion of benefit-risk to better understand what tradeoffs patients may perceive as acceptable.
- Each topic starts with a panel of patients and caregivers who each speak for a few minutes at a time to share their experiences and help set the tone for the rest of the discussion. Following each panel, the discussion is open to all patients and caregivers in the audience. The goal is to hear a diverse range of perspectives from people living with the condition.

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Audience

The key participants in PFDD meetings are patients, patient representatives, patient advocates, caregivers, loved ones, and anyone with a lived experience with the disease or condition. While patients and caregivers share their perspectives, key stakeholders are in listening mode as part of the audience. These stakeholders may include:

- FDA and other regulatory/federal agencies
- Medical product developers
- Academic researchers
- Clinicians and healthcare professionals

It is important for all these stakeholders to hear patient input as it may inform drug development. Aside from providing initial remarks from the FDA perspective, **FDA staff participate as listeners in EL-PFDD meetings**.

Some host patient organizations choose to incorporate PFDD-style sessions as part of their annual conferences, scientific workshops, and other meetings. It is important to get patient input early in the drug development process.

Resources and Deliverables (Voice of the Patient (VOP) meeting summary report)

- While EL-PFDD meetings do take a significant amount of effort and planning, they do not need to be resource intensive. FDA's PFDD Staff is here to serve as a helpful resource to host patient organizations throughout the planning process. We recommend submitting a letter of intent (LOI) approximately one year before the anticipated meeting date to allow for adequate time for planning and preparation. For more details, please see the linked guidelines for developing an LOI on our EL-PFDD Meetings webpage.
- Following each meeting, a meeting summary report called the Voice of the Patient report is developed by the host patient organization to capture patient experience data and perspectives shared during the EL-PFDD meeting. Links to these reports can be found on the Condition-Specific Meeting Reports and Other Information Related to Patients' Experience webpage.
- Patient input from meetings and meeting summary reports can support FDA staff:
    - In conducting **benefit-risk assessments** for products under review, by informing the therapeutic context
    - **Advising drug sponsors** on their development programs
- Patient input can support drug development more broadly:
    - Identify **areas of unmet need** in the patient population
    - Identify or develop **tools** that assess benefit of potential therapies
    - Raise awareness and **increase engagement** within the patient community

PFDD Potential Outcomes

- EL-PFDD meetings are a great networking and community-building opportunity for the patient community and EL-PFDD meetings can help to channel patient engagement and create momentum in the patient community. Several host patient organizations have hosted follow-up meetings to build on this momentum and have synergized their efforts with other patient organizations in the space.
- EL-PFDD meetings may also serve to identify opportunities for further discussions with patients about their condition and may also generate new research questions.
- EL-PFDD meetings offer an opportunity to foster relationships between patient organizations and industry/medical product developers.

Questions? Contact PatientFocused@fda.hhs.gov and your PFDD point of contact.

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