



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.