

Patient-Focused Drug Development: Learning from Patients to Develop Better Treatments



As patients become more empowered in their health care and decision making, regulatory authorities and pharmaceutical companies are placing greater emphasis on the contribution that patients and caregivers bring to the drug development process. Patients are the experts on their own health and health conditions. Engaging with patients and patient representatives, understanding their experience with a disease and incorporating the patient voice has great potential to benefit drug development overall.

In 2012, as part of the reauthorization of the Prescription Drug User Fee Act (PDUFA V), the U.S. Food and Drug Administration (FDA) established a program to help ensure patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into the development and review processes. This was formalized as Patient-Focused Drug Development, or PFDD, a novel approach to gathering input from patients who are willing to share their personal experience of living with a disease or condition.

At the center of this initiative are Patient-Focused Drug Development Meetings – meetings led by the FDA and focused around one specific disease or condition. PFDD meetings provide an important opportunity for the FDA and pharmaceutical companies to hear directly from patients, patient advocates, caregivers and health care professionals about the symptoms that matter most, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments.

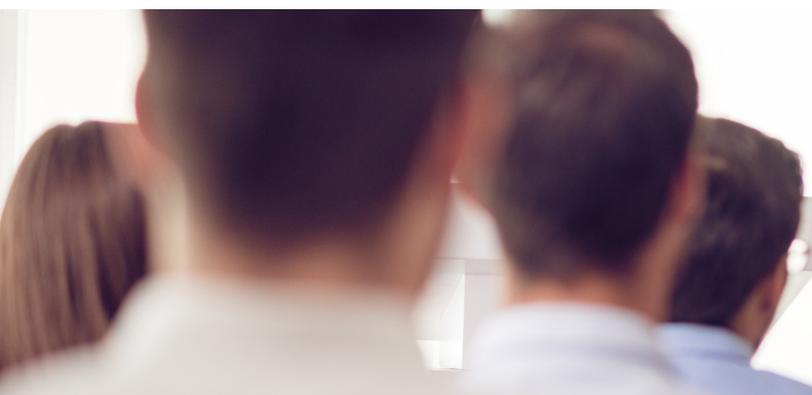
A detailed summary of the patient input generated from each PFDD meeting is summarized into a Voice of the Patient report and made available publicly, including those for meetings focused on rare diseases such as sickle cell disease and hemophilia A/B. These

reports express to the FDA and the pharmaceutical industry the improvements that patients hope new treatments could make in their daily lives.

To date, the FDA has led more than 25 condition-specific meetings, inviting patients, caregivers and patient representatives to share their experiences with a goal of using the input to improve drug development and evaluation.

To expand the benefits of the PFDD meetings, the FDA established a formal process to welcome patient organizations to form patient-focused collaborations and host their own PFDD meetings. As with the FDA-led meetings, patient organizations summarize their findings into a Voice of the Patient report to provide to the FDA. These externally-led PFDD meetings have successfully amplified the voices of countless patients, including those with rare diseases such as Barth syndrome, Friedreich's Ataxia and Charcot-Marie-Tooth disease.

Opportunities to expedite and enhance the process of discovery, development and delivery for disease treatments continues to be prioritized at the federal level. In 2016 Congress passed the 21st Century Cures Act (Cures Act). The Cures Act is designed to help accelerate medical product development and



bring new innovations and advances to patients who need them faster and more efficiently. Recognizing the significance of including the patient experience in regulatory decisions, the Cures Act expands on the concept of Patient-Focused Drug Development by establishing a framework for its application, guidance and evaluation within the FDA.

As the innovators who develop tomorrow's medicines, pharmaceutical companies not only collaborate with the FDA but look internally for opportunities to incorporate the patient perspective into the research and development process. Many companies have escalated their efforts to engage with patients and patient advocacy groups at regular points in the development lifecycle by asking questions and listening to patients' opinions about potential new ideas, processes and services. While each pharmaceutical company has its own unique approach to engaging with patient communities, some examples might include hosting patient advisory board meetings, bringing patients to meet with researchers or surveying patients and caregivers.

Understanding the community's needs and preferences can help shape, for example, locations for clinical trial sites, content for patient education materials and other experiences that are relevant to the patients who will eventually receive the treatments.

Today's patients are better informed than ever. They are knowledgeable, engaged and willing to share their experiences to advance medical research. The rise of the empowered patient coupled with the FDA's emphasis on Patient-Focused Drug Development will continue to create a greater understanding among all stakeholders. When regulatory agencies and pharmaceutical companies prioritize efforts to understand patients as individuals with unique needs, preferences and behaviors, the resulting outcomes can lead to faster, better drug development and treatments that improve lives.

Resources

Learn more about Patient-Focused Drug Development and opportunities to provide input at future PFDD meetings.

FDA Email Alerts

Get regular email updates on the FDA's Patient-Focused Drug Development programs. Subscribe here: <https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development#subscribe>

PFDD Meeting Tracker

FasterCures Patient-Focused Drug Development Meeting Tracker provides a list of FDA-hosted and externally-led PFDD meetings, recordings and links to Voice of the Patient reports.

[FasterCures.org/programs/patients-count/pfdd/](https://www.fastercures.org/programs/patients-count/pfdd/)

PFDD Meetings for Rare Diseases

Search the National Organization for Rare Disorders (NORD) Event Calendar for upcoming externally-led Patient-Focused Drug Development meetings for rare diseases. <https://rarediseases.org/events/>

