What Is Patient-Focused Drug Development?

Patient-focused drug development (PFDD) incorporates the patient’s voice in the development and the U.S. Food and Drug Administration’s (FDA) evaluation of new medicines.

During clinical trials, researchers study whether new medicines are safe and effective for patients and whether the medicine’s benefits outweigh the risks. The FDA considers the benefit-risk assessment when making a decision on whether or not to approve a medicine for patient use.

As important stakeholders in the drug development process, patients, family members and caregivers can provide unique and valuable perspectives on their disease and available treatment options. These perspectives can inform evaluation of a medicine’s benefits and risks and provide the context for FDA’s regulatory decision making.

How is the Patient Input Being Used in Drug Development?

The FDA has been working to incorporate patient perspectives into its regulatory review process since the Prescription Drug User Fee Act (PDUFA) was reauthorized in 2012 (PDUFA V). In recent years, FDA has made progress on its goal to obtain the patient perspective on certain disease areas and incorporate patient input in its drug review process. The FDA has committed to hold 24 disease area-specific PFDD meetings with individual patients and patient groups.

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How is the Patient Input Being Used in Drug Development? (continued)

Patient perspectives on their conditions have already been used to help inform FDA decision making. In FDA’s 2015 approval of a new melanoma therapy, patient input on the cosmetic and psychological advantages of seeing melanoma lesions shrink was instrumental in the FDA’s advisory committee’s decision on an overall favorable benefit-risk profile of the therapy for patients, and was a key factor in the Agency’s decision.

Advancing science-based approaches to collect robust and meaningful patient and caregiver input can more consistently inform drug development and regulatory decision making. For example, since 2014, PhRMA has collaborated with the Genetic Alliance to explore the use of a technology-enabled, crowd-sourcing approach to gather insights into patients’ experiences with a disease or condition.

THE BENEFITS OF PATIENT PERSPECTIVES IN THE DRUG DEVELOPMENT AND REVIEW PROCESS

- Researchers collect patient perspective data on disease measures and treatment outcomes, and integrate these findings throughout the drug development process.
- FDA considers patient perspective during regulatory review.
- Approvals of new medicines reflect information that is meaningful to patients, their families and health care providers.

How Can Patient-Focused Drug Development Continue to Improve Drug Development?

As scientific advances lead to a better understanding of specific diseases or conditions, knowing which elements of the disease or of a potential treatment are most important to patients is increasingly important to researchers. Continuing to develop a science-based and systematic approach to gathering patient input robust enough to support FDA’s regulatory decision making is necessary to realize the goal of a patient-centered approach to drug development and regulatory review.

It is important to continue building upon the FDA’s PFDD efforts to encourage the development and use of patient experience data in the drug development and review process.

Future efforts that clarify how FDA will collect and translate patient perspective data into tangible outcomes will help advance the science of patient input, enhancing drug discovery and development to better focus on patient needs.