The Food and Drug Administration (FDA) has embarked on an effort to include “meaningful” patient experience data in its decisions on new drug approvals and post-marketing surveillance. The idea is to supplement clinical trial data with input from patients that would be collected and submitted by drug companies, caregivers, research teams, and disease-focused groups. The FDA will be publishing four guidance documents that will discuss various aspects of which data should be collected, how it should be collected, how it should be submitted, and various other topics.

The agency’s initiative stems from a provision in the 21st Century Cures Act1 and from commitments the agency made as part of the sixth iteration of the Prescription Drug User Fee Act, which Congress approved as part of the FDA Reauthorization Act in 2017.2 The idea behind the initiative is that patients affected by an illness can—and should—be involved in the FDA’s decisions about the benefits and risk of any drug meant to help them.

Patient views are already collected in other countries by drug regulatory agencies. The European Union has been working on patient experience involvement since early 2016 with a project called Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (PREFER), which is run under the aegis of the Innovative Medicines Initiative. Major pharmaceutical companies support that effort with funding. PREFER published its latest call for proposals in March 2018. When the research projects are picked, they will be funded with a budget of 82 million euros.3

According to an FDA spokeswoman, the agency plans to spend approximately $2.3 million on patient-focused drug development (PFDD). From 2012 to 2017, the FDA conducted 24 disease-specific PFDD meetings to more systematically obtain the patient perspective on specific diseases and their treatments. According to the agency, these meetings provided key stakeholders, including the FDA, patient advocates, researchers, drug developers, health care providers, and others, an opportunity to hear the patient’s voice.4 In addition, the FDA encourages patient organizations to identify and organize patient-focused externally led PFDD meetings to generate public input on disease areas not yet examined.5

The FDA has been collecting some patient data based on guidance documents it has issued previously, including the Patient Reported Outcomes (PRO) guidance and the Qualification Process for Drug Development Tools. That has led drug industry officials such as Carol Haley, Director of U. S. Regulatory Policy at Pfizer, Inc., to wonder how these various patient input programs will dovetail. “It is important that the agency state how the new guidance will complement or be distinct from existing guidance,” Haley wrote in Pfizer’s comments to the FDA. “It will also be important for the agency to ensure that the new guidance documents are consistent and in alignment with existing guidance.”

The FDA plans to issue four separate guidance documents—issued consecutively not simultaneously—implementing the new patient experience data program. Haley wrote that Pfizer is concerned that developing the documents in a step-wise approach may lead to discrepancies and inconsistencies across the documents. “In addition, this approach may lead to uncertainty as to how a sponsor should proceed with the studies once the first guidance is issued and before the other three are available, given that it may take up to two years to complete a given patient-experience study,” she stated.

Patient experience data can include information about the experiences, perspectives, needs, and priorities of patients related to: 1) the symptoms of their condition and its natural history; 2) the impact of the condition on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issue as defined by patients.

That definition, found in the Cures Act, is very broad. It is close to the definition of PRO data, which has caused some confusion. It is not clear how the FDA will use the data in its regulatory process. The discussion draft makes this very general statement: “The patient experience data is used to help inform clinical trial design, trial endpoint selection, and regulatory reviews, including benefit-risk assessments.”

Robyn Carson, Head of Patient-Centered Outcomes Research at Allergan, suggested that the FDA be explicit about its intended use of this data and answer questions about whether it will inform the agency’s review of trial protocols, assessment of endpoint development and selection, calculations of risk–benefit tradeoffs, or considerations of labeling language. “For this effort to be successful in encouraging an increase in patient-focused drug development, it is critical for the FDA to better describe how they intend to utilize information submitted as ‘patient experience data’ so that stakeholders can determine the appropriate research questions and study design for the specific context of use,” she stated.

The FDA has its work cut out writing guidance documents on patient experience data. But this seems to be one area

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where drug companies, patient advocacy groups, research entities, and others all have a similar goal. The lack of pushback from interest groups—which isn’t always the case when the FDA tries to stand up for a new program—will go a long way toward allowing the agency to tap new sources of important data, thereby improving the drug approval process.

REFERENCES