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FDA publishes first patient-focused drug development guidance

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As experts in what it is like to live with a health condition, patients and caregivers are uniquely positioned to inform the therapeutic context for drug development and evaluation. This perspective is particularly valuable when considering the impact of acute and chronic diseases on pediatric patients and their caregivers.

The Food and Drug Administration (FDA) launched a patient-focused drug development initiative to facilitate systematic approaches to collect and use meaningful patient and caregiver input to better inform medical product development and regulatory decision-making.

In June, it published the first of four documents. Titled *Patient-Focused Drug Development: Collecting Comprehensive and Representative Input*, the draft guidance answers the questions "Whom do you get input from and why?" and "How do you collect the information?" Sampling methods that could be used when planning to collect patient input are discussed along with a general overview of the relationship between potential research questions and methods when deciding on the sources for data.

The next guidance document will discuss methods for eliciting information from the individuals identified in the initial guidance and gathering information about issues important to patients. The third document will define the concepts to include in the development of clinical outcome assessment (COA) study instruments, and the final guidance will address topics related to COA endpoint development and interpretation.

In May 2017, the FDA issued a *Plan for Issuance of Patient-Focused Drug Development Guidance*, with a roadmap for public workshops and guidance development over the next five years.

The guidance series implements new requirements under 21st Century Cures Title III Section 3002 and new commitments the FDA made under the 2017 reauthorization of the Prescription Drug User Fee Act under Title I of FDA Reauthorization Act of 2017. These commitments were based on patient and other stakeholder input obtained from 24 disease-specific meetings held from 2013-'17. Eighteen of the meetings addressed diseases that included pediatric manifestations such as autism, sickle cell disease and inborn errors of metabolism.

Resource

- Information and documents related to the patient-focused drug development guidance series, including public workshops, a glossary of terms and hypothetical case examples