

PUBLIC WORKSHOP ON PATIENT-FOCUSED DRUG DEVELOPMENT:  
DEVELOPING AND SUBMITTING PROPOSED DRAFT GUIDANCE  
RELATING TO PATIENT EXPERIENCE DATA

**BACKGROUND DOCUMENT**

On March 19, 2018, the U.S. Food and Drug Administration (FDA or Agency) will hold a public workshop to obtain stakeholder input to inform the Agency's development of a guidance on how a person seeking to develop and submit proposed draft guidance relating to patient experience data for consideration by the FDA may submit such a proposed draft guidance to the Agency. FDA is developing this guidance to support patient-focused drug development and implement requirements under Section 3002(c)(5) of the [21<sup>st</sup> Century Cures Act](#).

This background document is intended to provide some additional information to support stakeholder discussion at the workshop.

**Background on 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. As experts in what it is like to live with their condition, patients are uniquely positioned to help inform the clinical context and provide invaluable overarching insights to assist in framing the assessment of benefits and risks of medical products. FDA's initial PFDD work focused on convening meetings with patients living with a particular disease to hear about what it is like to live with their condition and their experiences with available treatments. The information shared in these meetings have provided tremendous insights. To build on these efforts FDA is undertaking development of a series of guidelines to describe fit-for-purpose methods and approaches to better enable sponsors and the community to advance from powerful narratives of patient experience as obtained in these initial PFDD meetings, to collection of data that can even further inform drug development and regulatory decision making.

To address this important need and opportunity, the 21<sup>st</sup> Century Cures Act, Section 3002(c) also directs FDA to develop guidance to address key topics to inform and enable patient focused drug development. The topics to be addressed include methodological approaches to collection of patient experience data that are relevant and objective and ensure that such data are accurate and representative of the intended patient population, and methodological approaches to identification of what matters most to patients regarding burden of their disease, burden of treatment, and benefits and risks of new therapies. The topics also include approaches to identifying and developing methods to measure impacts to patients that will help facilitate collection of patient experience data in clinical trials, and methods to develop and analyze patient-reported and other clinical outcome assessments for the purposes of regulatory decision making.

## Scope and Objectives of Public Workshop

The 21<sup>st</sup> Century Cures Act, under 3002(c)(5), directs FDA to issue guidance on how a person seeking to develop and submit a proposed draft guidance relating to patient experience data for consideration by FDA may submit such proposed draft guidance to the Agency.

External stakeholders, including patients, caregivers and groups with particular knowledge of or access to the patient community, may be well-positioned to collect patient experience data. These stakeholders may be able to develop work products related to patient experience that would provide helpful data and information to support patient-focused drug development in a specified disease area. In some cases, this work product could be well-suited for submission as a proposed draft guidance.

The March 19 public workshop will give FDA the opportunity to hear from a broad range of stakeholders, including patients, patient advocates, academic and medical researchers, expert practitioners, medical product developers and other interested persons. The workshop will be organized into two sessions each beginning with a moderated panel discussion.

### Session I: Opportunities for Patient Stakeholders – FDA Perspective

**Objective:** Identify areas where patient experience data might be particularly helpful to inform medical product development and regulatory decision making. This session will focus on providing an FDA panel’s perspective on the types of information on the patient experience to collect and measure throughout medical product lifecycle and the varying formats for effective sharing of the collected patient experience data.

Panelists will also reflect on what they see as the range of opportunities for patient stakeholders, highlighting potential areas where patient stakeholders may be particularly well-positioned to inform medical product development and regulatory decision making.

### Session II: Opportunities for Patient Stakeholders – Stakeholder Perspective

**Objective:** FDA seeks input from patient stakeholders on how best to communicate FDA’s current thinking on submitting proposed draft guidance relating to patient experience data.

This session will provide perspectives from a panel of patient stakeholders on what questions would be most helpful for FDA to address in its forthcoming draft guidance on how to develop and submit a proposed draft guidance relating to patient experience data. A set of proposed questions that FDA may address in the forthcoming draft guidance will be presented to prompt stakeholder feedback and suggestions from both the panel and other workshop participants in the audience.