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Patient Voice and Its Role In Bringing Meaningfulness into Selection of Clinical Outcome Measures

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Introductions & Roadmap

- ***Patroula Smpokou, MD*** **– FDA Perspective**
  - Deputy Director (acting), Division of Rare Diseases & Medical Genetics, Office of Rare Diseases, Pediatrics, Urologic & Reproductive Medicine, OND, CDER, FDA

- ***James Valentine, MHS, JD*** **– Patient Perspective**
  - Associate, Hyman, Phelps & McNamara, PC

- ***Alison Skrinar, PhD*** **– Industry Perspective**
  - Vice President, Clinical Outcomes Research and Evaluation, Ultragenyx Pharmaceutical, Inc.

- ***Q&A***

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Overview

- Patients are uniquely positioned to inform drug development, and they are playing an increasing role in these efforts.

- This session discusses on one area where this true – in understanding clinical meaningfulness and selection and development of clinical outcome measures.

- Examples:
  - Patient-focused drug development meetings
  - Listening sessions
  - Interviews (e.g., structure interviews) & surveys

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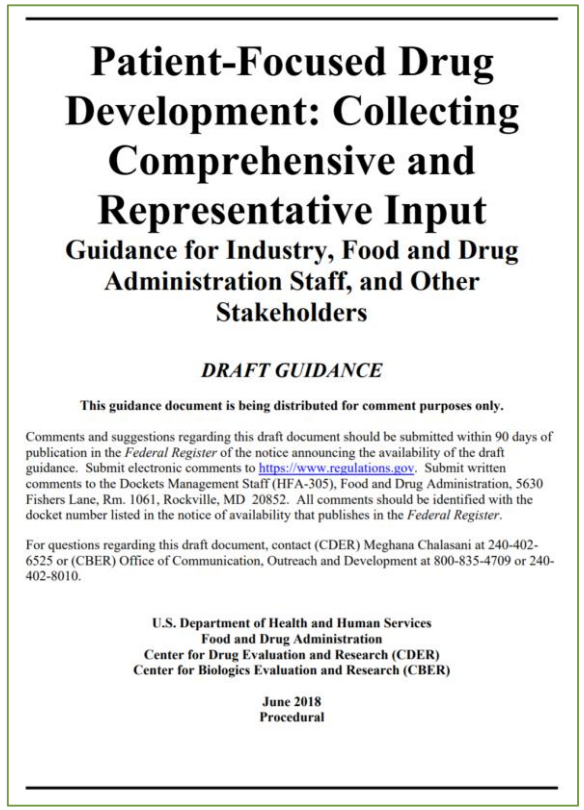
Evidentiary Standards

- "Substantial evidence" FDCA § 505(d)
- "Adequate and well-controlled clinical trials" FDCA § 505(d)
- Methods of assessment are "well-defined and reliable" (21 CFR 314.126)
- Compliance with FDA regulatory requirements for recordkeeping, maintenance, and access (21 CFR Part 11)

Patient-Focused Drug Development Guidances



Patient-Focused Drug Development: Collecting Comprehensive and Representative Input

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

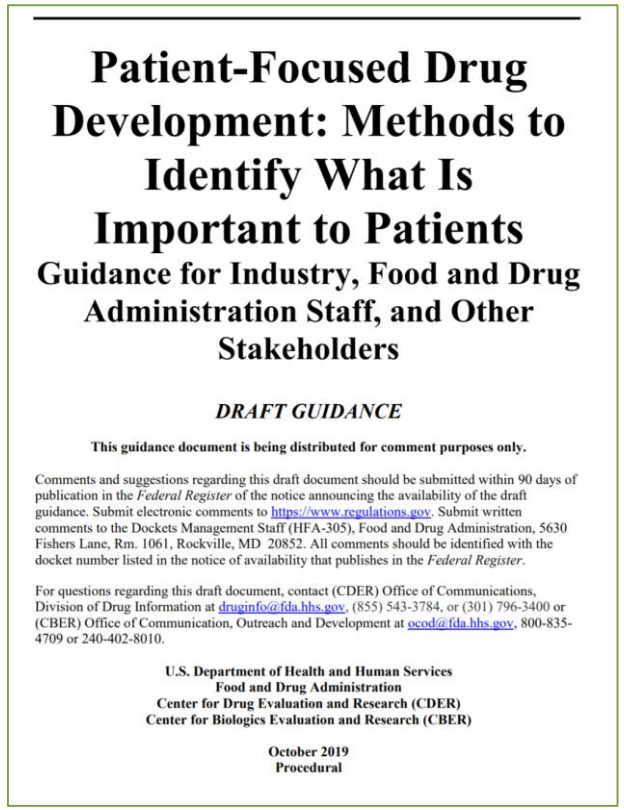
Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Meghana Chalasani at 240-402-6525 or (CBER) Office of Communication, Outreach and Development at 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

June 2018
Procedural

Final Guidance Issued Today (June 16, 2020)



Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

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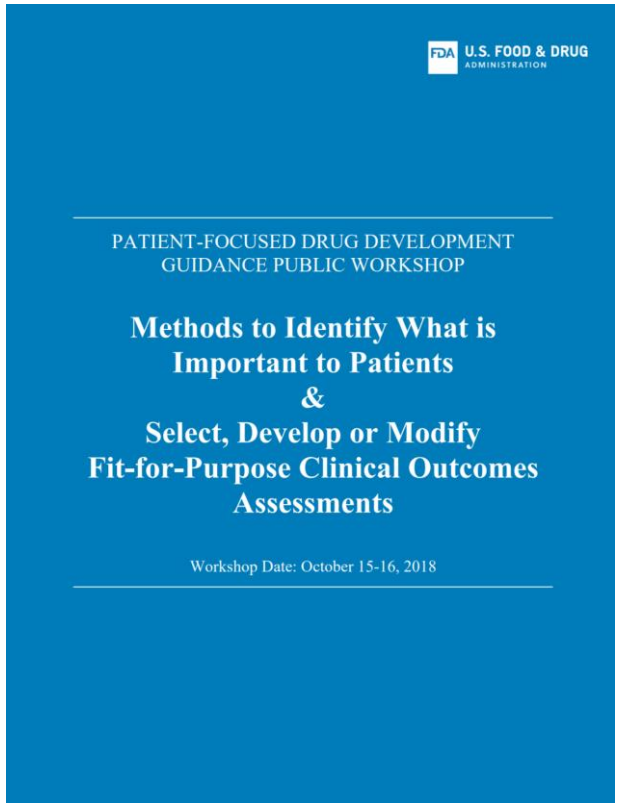
U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

October 2019
Procedural

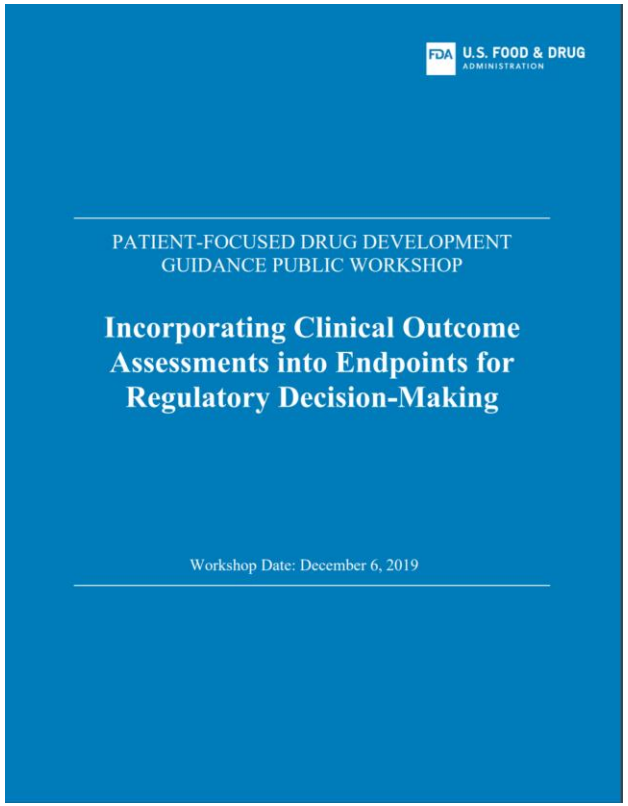


FDA U.S. FOOD & DRUG ADMINISTRATION

PATIENT-FOCUSED DRUG DEVELOPMENT
GUIDANCE PUBLIC WORKSHOP

Methods to Identify What is Important to Patients
&
Select, Develop or Modify Fit-for-Purpose Clinical Outcomes Assessments

Workshop Date: October 15-16, 2018



FDA U.S. FOOD & DRUG ADMINISTRATION

PATIENT-FOCUSED DRUG DEVELOPMENT
GUIDANCE PUBLIC WORKSHOP

Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision-Making

Workshop Date: December 6, 2019

Patient-Focused Drug Development Guidances

- **(#1) Collecting comprehensive patient community input on burden of disease and current therapy**
  - How to engage with patients to collect meaningful patient input?
  - What methodological considerations to address?

- **(#2) Development of holistic set of impacts (e.g., burden of disease and burden of treatment) most important to patients**
  - How to develop a set of impacts of the disease and treatment?
  - How to identify impacts that are most important to patients?

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Patient-Focused Drug Development Guidances

- **(#3) Identifying and developing good measures for the identified set of impacts that can then be used in clinical studies**
  - How to best measure impacts (e.g., endpoints, frequency) in a meaningful way?
  - How to identify measure(s) that matter most to patients?

- **(#4) Incorporating measures (COAs) into endpoints considered significantly robust for regulatory decision making**
  - E.g., technologies to support collection through analysis of the data

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Assessing Treatment Benefit

- **Treatment Benefit**
  - Treatment benefit is demonstrated by evidence that the treatment has a positive impact on how a person (i) survives, or (ii) feels or functions

- **Types of Outcome Assessments**
  - Survival
  - **Clinical outcome assessments (COAs)**
  - Surrogate endpoints
    - Biomarker
      - Validated surrogate (e.g., blood pressure lowering) – Regular approval
      - Unvalidated surrogate (e.g., 6-minute walk test) – Accelerated approval

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Clinical Outcome Assessment

- **COA is any assessment that may be influenced by human choices, judgement, or motivation and may support either direct or indirect evidence of treatment benefit.**

- **<u>4 main types of COA measures:</u>**
  - Patient-reported outcome (PRO)
  - Clinician-reported outcome (ClinRO)
  - Observer-reported outcome (ObsRO)
  - Performance outcome (PerfO)

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Requirements for COAs

- **Measures are "well-defined and reliable"**
  - Empiric evidence demonstrates that the score quantifies the concept of interest in the targeted context of use

(1) Measuring the right thing (concept of interest)

(2) In the right way in defined population (targeted context of use)

(3) Score that quantifies the "thing" does so accurately & reliably

- **<u>Goal:</u>**
  - The effects seen in the outcome assessment can be interpreted as a clear treatment benefit

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Patient-Focused Drug Development Efforts

21st Century Cures and PDUFA VI

FDA-led PFDD Meetings

COA Grant Program

Externally-led PFDD Meetings

Other Resources

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Example of FDA Patient-Focused Drug Development Resources

https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical

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Thank You!

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Questions?

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