FDA's Patient-Focused Drug Development Program

Advancing the Science of Patient Input

Pujita Vaidya, MPH
Senior Advisor, Patient-Focused Drug Development Program
Office of the Center Director
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

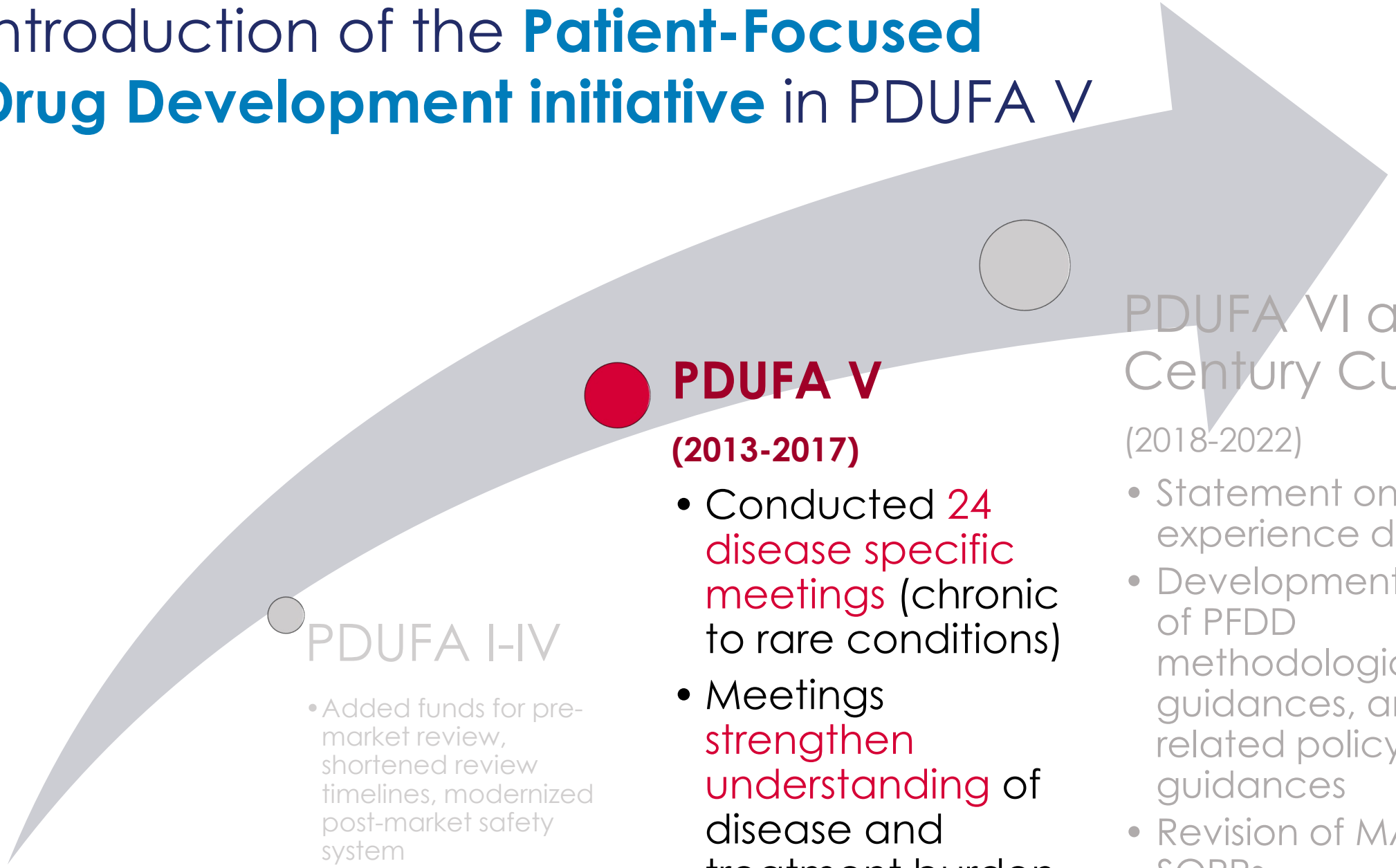
#PFDD

The views and opinions expressed in this presentation are those of the individual presenter and should not be attributed to or considered binding on the U.S. Food and Drug Administration (FDA).

FDA Benefit-Risk Assessment: Qualitative approach that is grounded in quantification of various data elements

Dimension	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition **Current Treatment Options**	**Sets the context for the weighing of benefits and risks:** • How serious is this indicated condition, and why? • How well is the patient population's medical need being met by currently available therapies?	
Benefit	**Characterize and assess the evidence of benefit:** • How meaningful is the benefit, and for whom? • How compelling is the expected benefit in the post-market setting?	
Risk and Risk Management	**Characterize and assess the safety concerns:** • How serious are the safety signals identified in the submitted data? • What potential risks could emerge in the post-market setting? **Assess what risk management (e.g., labeling, REMS) may be necessary to address the identified safety concerns**	
Benefit-Risk Summary and Assessment		

Introduction of the **Patient-Focused Drug Development initiative** in PDUFA V



PDUFA I-IV

- Added funds for pre-market review, shortened review timelines, modernized post-market safety system

PDUFA V

(2013-2017)

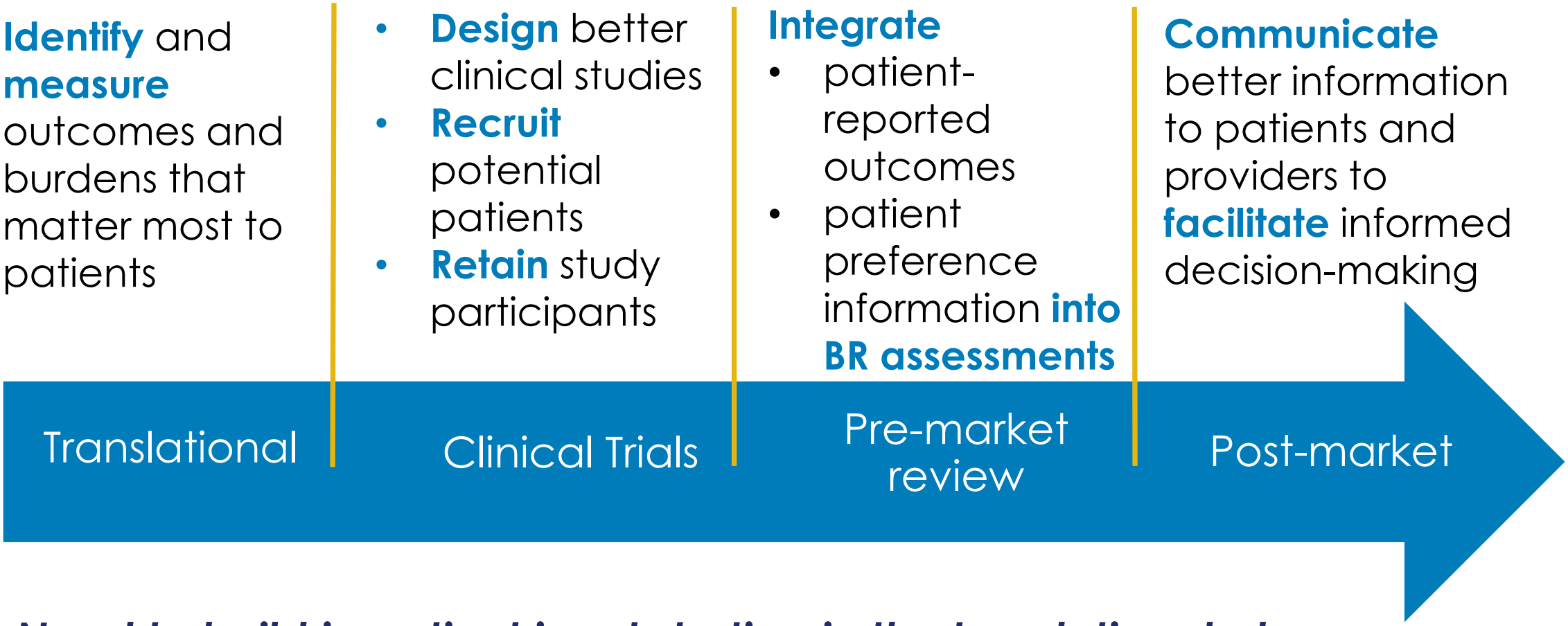
- Conducted 24 disease specific meetings (chronic to rare conditions)
- Meetings strengthen understanding of disease and treatment burden

PDUFA VI and 21st Century Cures Act

(2018-2022)

- Statement on patient experience data
- Development of series of PFDD methodological guidances, and other related policy guidances
- Revision of MAPPS and SOPPs

Integrating patient input into medical product development and decision making



Need to build in patient input starting in the translational phase

Patients are **uniquely positioned to inform** understanding the **therapeutic context**, which is an important aspect of **B-R assessment**

- Which **symptoms have the most significant impact** on your daily life?... On your ability to do specific activities?
- How well does your **current treatment regimen treat the most significant symptoms** of your disease?
- What specific things would you look for in an **ideal treatment** for your condition?
- What factors do you take into account when making **decisions about using treatments**? …. Deciding whether to participate in a clinical trial?

Each meeting results in a **Voice of the Patient report** that faithfully captures patient input

Positive feedback from participants

The patients truly appreciate … that you contributed to our cause and plea for help. **We feel heard** and we have hope for the future… (CFS/ME)

I was very inspired by the event and **left wanting to do more** for lung cancer, survivors and of course FDA…

… a tremendously insightful meeting.
(sickle cell disease, industry participant)

I was part of the webcast … **I could relate** to almost all the symptoms, many much more severe than I suffer.
(fibromyalgia)

…. By listening to ME/CFS patients first, and listening fully as demonstrated in the Voices report, **FDA sent our community a powerful message**: we hear you, we know you are seriously ill, and we want to help.

[We] felt **a validation and a peace** that is often missing from our daily struggles.
(fibromyalgia)

Emerging PFDD Learnings

- **Patients** with chronic serious disease **are experts** on what it's like to live with their condition
- Patients **"chief complaints" may not be factored explicitly into drug development** plans, including measures of drug benefit planned in trials
- Patients want to be as **active as possible** in the work to develop and evaluate new treatments

Externally-led PFDD: The Opportunity

- Patient organizations identify and organize **patient-focused collaborations** to generate public input on specific disease areas

- Meetings provide an important opportunity to **hear directly from patients**, patient advocates, and caregivers about the symptoms that matter most to them, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments.

PFDD was intended to elicit broader patient input for a disease to better inform clinical context of BR assessment, but patient stakeholders were asking:

What's next?

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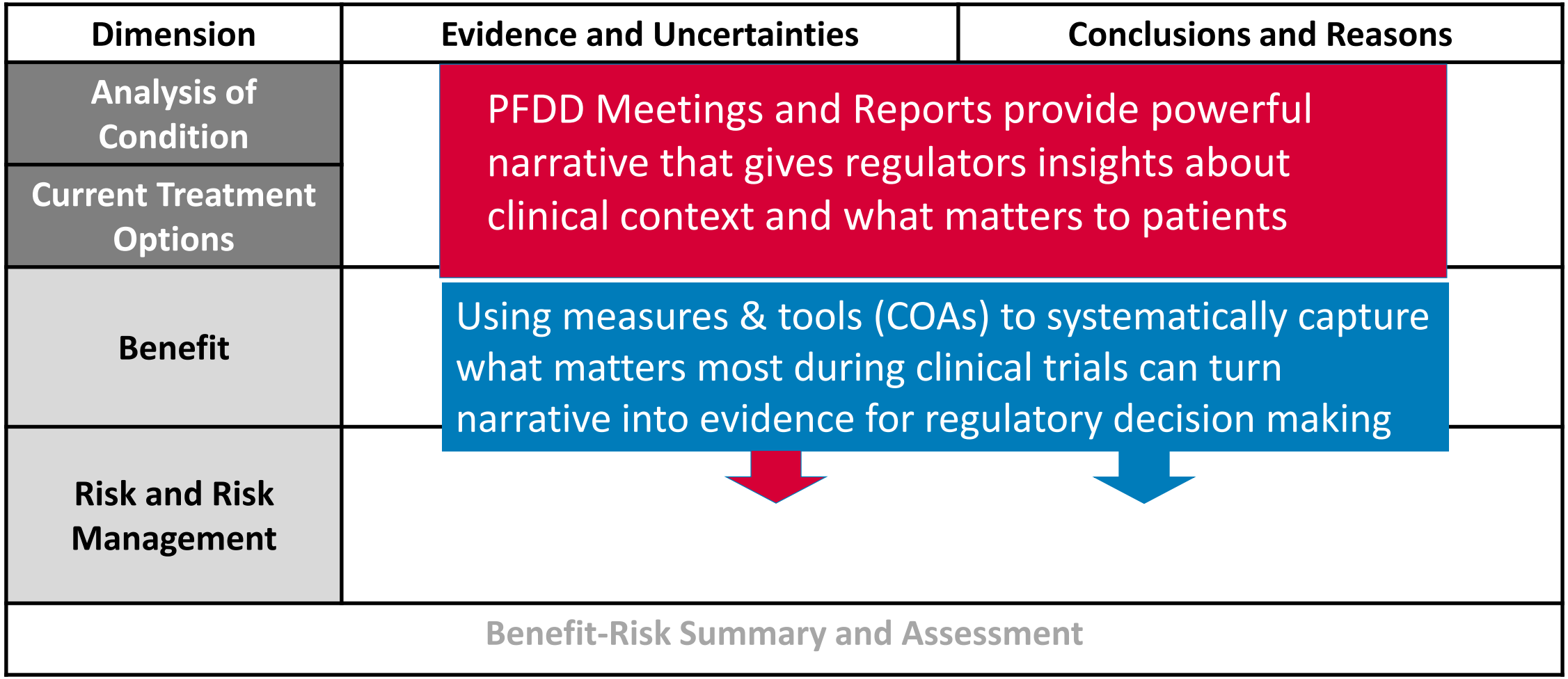
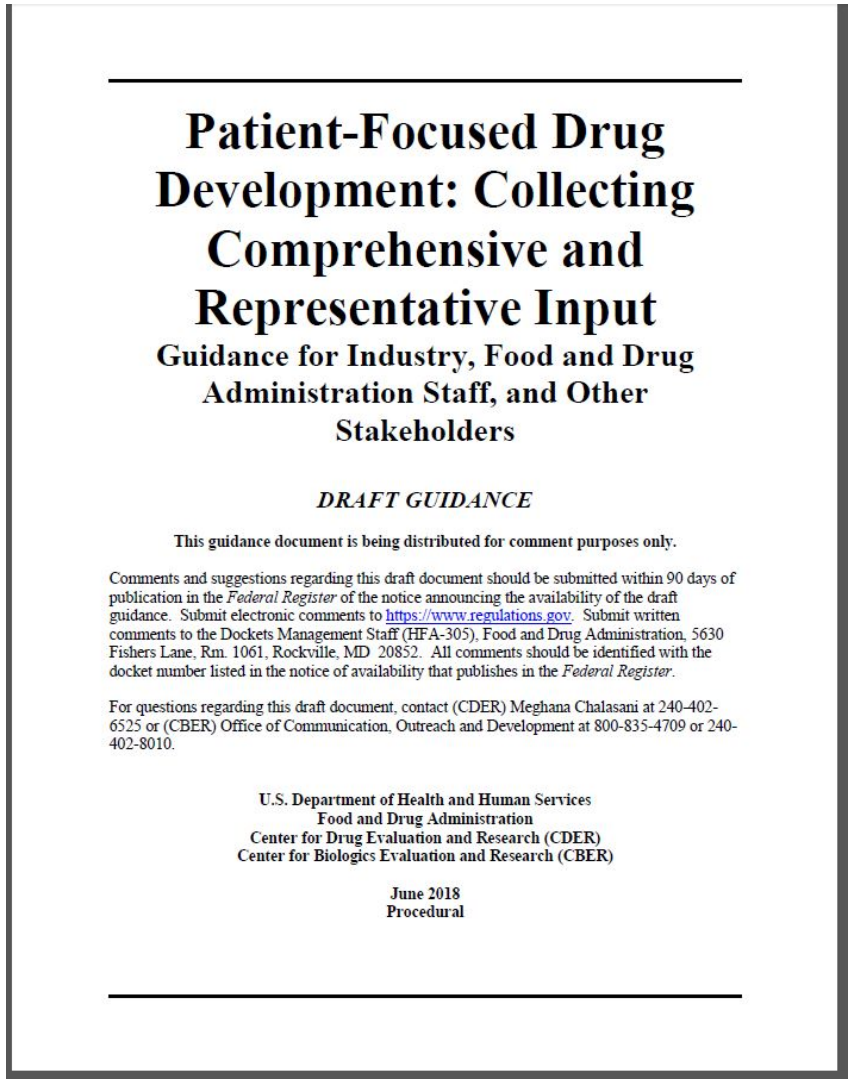
Series of Methodological Guidances to enable stakeholders to go beyond powerful narrative and collect data that can serve as study endpoints and be used as a basis for marketing decisions



Topics Addressed in Series of Four Patient-Focused Methodological 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)

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Procedural

1

- Whom do you get input from, and why?
- How do you collect the information?

2

- What do you ask, and why?
- How do you ask non-leading questions that are well-understood by a wide range of patients and others?

3

- How do you decide what to measure in a clinical trial and select or develop fit-for-purpose clinical outcome assessments (COAs) ?

4

- Once you have a COA measurement tool and a way to collect data using it, what is an appropriate clinical trial endpoint?

21st Century Cures
Patient Experience Data Table **in Review Documents**

				Section where discussed, if applicable
☐	**The patient experience data that was submitted as part of the application include:**			
	☐	Clinical outcome assessment (COA) data, such as		
✓		☐	Patient reported outcome (PRO)	
		☐	Observer reported outcome (ObsRO)	
		☐	Clinician reported outcome (ClinRO)	
		☐	Performance outcome (PerfO)	
	☐	Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel, etc.)		
	☐	Patient-focused drug development or other stakeholder meeting summary reports		
	☐	Observational survey studies designed to capture patient experience data		
	☐	Natural history studies		
	☐	Patient preference studies (e.g., submitted studies or scientific publications)		
	☐	Other: (Please specify)		
☐	**Patient experience data that were not submitted in the application, but were considered in this review:**			
		☐	Input informed from participation in meetings with patient stakeholders	
		☐	Patient-focused drug development or other stakeholder meeting summary reports	
		☐	Observational survey studies designed to capture patient experience data	
		☐	Other: (Please specify)	
☐	**Patient experience data was not submitted as part of this application.**			

Summary of FDA efforts

21st Century Cures and PDUFA VI

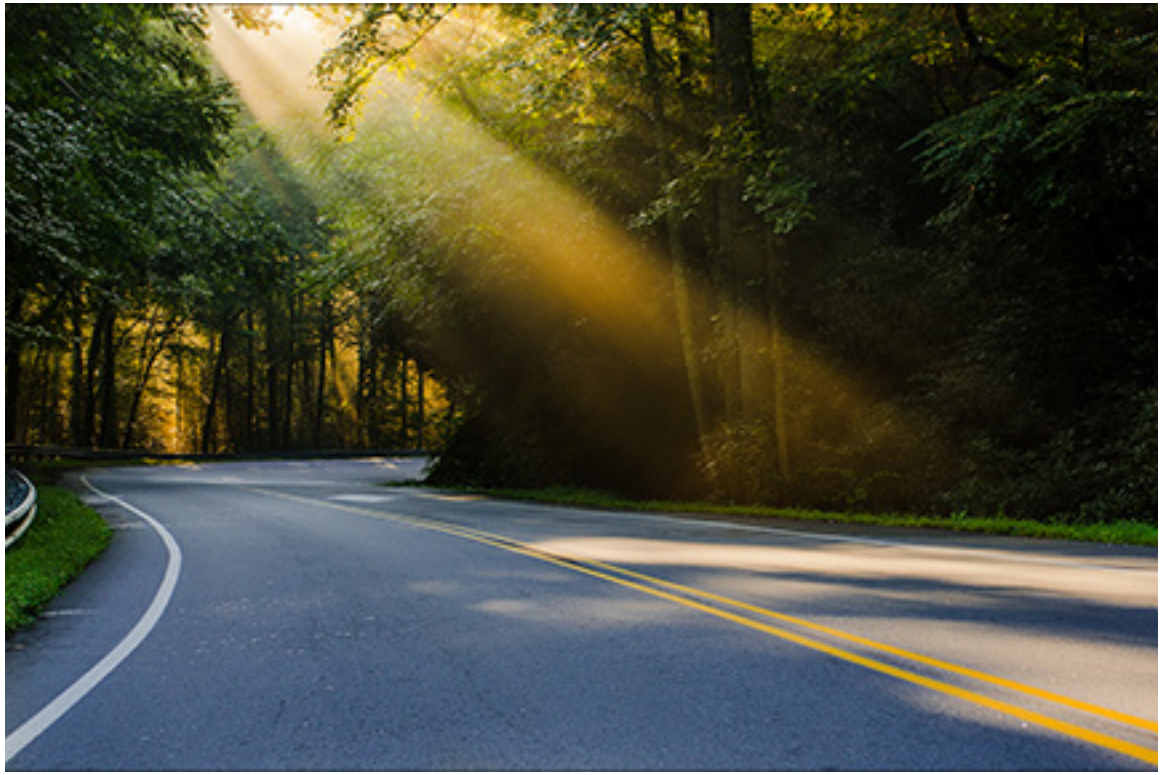
- 21st CC Plan for Issuance of Guidance
- Statement of Patient Experience
- PFDD Glossary
- Guidances (methodological and policy) and Public Workshops
- Revising MAPPs and SOPPs
- Repository

Other FDA Efforts

- FDA-led PFDD meetings
- Externally-led PFDD meetings
- CDER External Resources website
- CDER Standard Core COAs Grant Program
- CBER Science of Patient Input Initiative
- CDRH Patient Preference Initiative
- Increasing patient-focus in other existing FDA endeavors

Incorporating Patient Input as **Standard Practice**

- Ensure confidence in reliability and accuracy of PED for regulatory decision making
- Reduce regulatory uncertainty for sponsor
- Promote rapid consistent adoption
- Sustained incorporation of patient's experience in drug development and decision making—make it standard practice
  - FDA is soliciting grant applications to support the **development of a publicly available standard core set (s) of COAs and their related endpoints** for specific disease indications
  - Minimum list of impacts that matter most to patients and are likely to demonstrate change



CDER's Patient-Focused Drug Development Homepage

Contact FDA's Patient-Focused Drug Development Program Staff at:

patientfocused@fda.hhs.gov

Thank you!