

PATIENT-FOCUSED DRUG DEVELOPMENT  
**Collecting Comprehensive and Representative Input**

December 18, 2017

- 9:00 a.m. Welcome**  
Pujita Vaidya, Acting Director, Decision Support and Analysis Team (DSAT),  
Office of Program and Strategic Analysis (OPSA), Office of Strategic Programs (OSP),  
Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
- 9:05 a.m. Opening Remarks**  
Theresa Mullin, Director, OSP, CDER, FDA
- 9:20 a.m. Patient-Focused Drug Development: Defining Key Terminology**  
Meghana Chalasani, DSAT, OPSA, OSP, CDER, FDA
- 9:30 a.m. Overview of FDA's Approach to PFDD Guidance 1**  
Elektra Papadopoulos, Associate Director Clinical Outcome Assessments (COA) Staff,  
Office of New Drugs (OND), CDER, FDA  
  
Laura Lee Johnson, Acting Director, Division of Biometrics III, Office of Biostatistics (OB),  
Office of Translational Sciences (OTS), CDER, FDA
- 10:00 a.m. Session I: Defining Research Objectives and Methodological Considerations for Designing Studies to Collect Patient Experience Data**  
**Objective:** Discuss general considerations for collecting patient experience data. Explore factors and approaches to ensure that the patient input to be collected is sufficiently representative of the range of clinically relevant diversity in the patient population.
- Moderator:** Michelle Campbell, COA Staff, OND, CDER, FDA
- FDA Presentation:** Ebony Dashiell-Aje, COA Staff, OND, CDER and Kunthel By, Division of Biometrics V, OB, OTS, CDER, FDA
- Moderated Panel Discussion:**
- Kunthel By, Division of Biometrics V, OB, OTS, CDER, FDA
  - Steve Cohen, Vice President, Division of Statistical and Data Sciences, RTI International
  - Ebony Dashiell-Aje, COA Staff, OND, CDER, FDA
  - Richard Gershon, Professor of Medical Social Sciences and Preventative Medicine, Northwestern University
  - Meena Khare, Senior Advisor for Statistical Programs, Division of Research and Methodology, National Center for Health Statistics, Centers for Disease Control and Prevention
  - Elisabeth (Liz) Piau-Louis, Associate Director Patient Centered Outcomes Research Oncology, Genentech, a member of the Roche Group
  - Suzanne Vernon, Research Director, Bateman Horne Center
- Questions to address:**
1. Are there any other factors to consider when defining research objectives and designing studies to collect patient experience data that should be included in the guidance?
  2. What are other factors and/or approaches to consider to ensure collection of representative input from the target population (patients with disease of interest)?
  3. In which situations is it more important to sample patients using a probability-based method? In which situations is it less important? What will be gained and what may be lost?

**Audience Question and Answer**

11:30 a.m. Lunch

12:30 p.m. **Session II: Methodological Considerations for Data Collection, Analysis and Operationalization**  
**Objective:** Explore methods to consider at an early stage in drug development to gain a thorough account of patients' experience and perspectives on their disease and available therapy. Discuss approaches to consider for collecting, analyzing, managing, and reporting the information.

**Moderator:** Scott Komo, Division of Biometrics III, OB, OTS, CDER

**FDA Presentation:** Selena Daniels, COA Staff, OND, CDER

**Moderated Panel Discussion:**

- Steve Cohen, Vice President, Division of Statistical and Data Sciences, RTI International
- Selena Daniels, COA Staff, OND, CDER, FDA
- Sheri Fehnel, Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions
- Gary Globe, Director, Global Health Economics, Amgen
- Isabelle Lousada, President and CEO, Amyloidosis Research Consortium
- Kai Ruggeri, Director, Global Research Analytics for Population Health (GRAPH), Columbia University

**Questions to address:**

1. Future guidances will discuss in more detail qualitative, quantitative, and mixed methods. Is more detail (or less) needed in this first guidance about which source (e.g., interviews, focus groups, consensus panel, etc.) to use to collect data? Is anything missing?
2. Similar question about operationalizing and standardizing data collection and data management. Is more detail (or less) needed in this first guidance? Is anything missing?
3. Are there any other factors to consider regard to selection of methods to collect and analyze patient experience data that should be included in the guidance?
4. Are there any other factors to consider regard to operationalization of the data collection process?

**Audience Question and Answer**

1:45 p.m. **Session III: Translating Best Practice into Real Practice - Developing Guiding Examples**  
**Objective:** Identify common challenges in collecting patient experience data and explore ways to avoid these challenges and maximize success. Discuss situations where things did not go as planned, and situations where things did go well. Information from this panel session will inform the development of case studies or vignettes to help support important aspects of the draft guidance.

**Moderators:** Sara Eggers, DSAT, OPSA, OSP, CDER, FDA and Megan Moncur, Office of Biostatistics and Epidemiology (OBE), Center for Biologics Evaluation and Research (CBER), FDA

**Moderated Panel Discussion:**

- Richard Gershon, Professor of Medical Social Sciences and Preventative Medicine, Northwestern University
- Telba Irony, Deputy Director, OBE, CBER, FDA
- Susan McCune, Director, Office of Pediatric Therapeutics, Office of the Commissioner, FDA
- April Naegeli, Research Scientist, Eli Lilly and Company
- Sally Okun, Vice President, Advocacy, Policy & Patient Safety, PatientsLikeMe
- Elizabeth Stuart, Associate Dean for Education; Professor, Department of Mental Health, Department of Biostatistics, Department of Health Policy and Management, Johns Hopkins Bloomberg School of Public Health

**Questions to address:**

1. What are your thoughts on the examples that are currently included in the discussion document?
2. What concepts from the discussion document would be helpful to illustrate through examples or case studies?
3. When collecting patient experience data, what are some common challenges seen in study design or implementation that might be useful to address through additional examples? How can that challenge result in data that are less suitable for regulatory purposes? What are practical ways to avoid the challenge?
4. Are there novel approaches or exemplars for collecting patient experience data that could be useful examples? How could someone replicate the effort?

**Audience Question and Answer**

2:45 p.m. **Break**

3:00 p.m. **Session IV: Identifying Key Themes and Next Steps**

**Objective:** Reflect on the day's discussion, specifically any themes that emerged throughout the day. Discuss key considerations that should guide FDA's completion of the first in the series of PFDD guidances.

**Moderator:** Sara Eggers, DSAT, OPSA, OSP, CDER, FDA

**Moderated Panel Discussion:**

- Conny Berlin, Global Head Quantitative Safety and Epidemiology, Novartis
- Sonya Eremenco, Associate Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)
- Kimberly McCleary, Acting Executive Director and Managing Director, FasterCures
- Theresa Mullin, Director, OSP, CDER, FDA
- Elektra Papadopoulos, Associate Director, COA Staff, OND, CDER, FDA
- Celia Witten, Deputy Director, CBER, FDA

**Questions to address:**

1. What are the three most important messages you have taken away from the workshop discussion that should guide FDA as we complete this draft guidance?
2. How can FDA strike the right balance to meet stakeholders' needs? How well do the discussion documents for today's meeting strike this balance? For example,
  - What is the right level of detail?
  - What is the right technical level?
3. Would the overall structure and format of the discussion documents for today's meeting be reasonable for the guidance? Do you have additional structure and format recommendations for FDA to consider when developing the draft guidance?
4. Considering that this is a first in a series of guidances that will be developed over time, how might FDA best facilitate stakeholders' understanding of the big picture and how all the pieces fit together?

**Audience Question and Answer**

4:00 p.m. **Open Public Comment**

**Moderator:** Meghana Chalasani, DSAT, OPSA, OSP, CDER, FDA

4:30 p.m. **Closing Remarks**

Laura Lee Johnson, Acting Director, Division of Biometrics III, OB, OTS, CDER, FDA