

Public Workshop

Patient-Focused Drug Development: Workshop #2 to Discuss Methodologic and Other Challenges Related to Patient Experience Data

September 18-19, 2025

Day 1: September 18

12:30 p.m. Welcome

- Ethan Gabbour- Public Health Analyst, Patient-Focused Drug Development Program, Office of the Center Director, Center for Drug Evaluation and Research, US Food and Drug Administration

12:35 p.m. Opening Remarks

- Theresa Mullin- Associate Center Director for Strategic Initiatives, Office of the Center Director, Center for Drug Evaluation and Research, US Food and Drug Administration

12:40 p.m. Understanding the Intersection of Meaningful Aspect(s) of Health and Concept of Interest

This session will explore the role and relationship between the meaningful aspect(s) of health and the concept of interest. We will start with a brief presentation from FDA staff, then transition into a panel discussion of considerations for conceptualizing clinical benefit and risk.

Presentation

- Ji Li- Senior Staff Fellow, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration

Panel Discussion

Moderator

- Selena Daniels- Deputy Division Director, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Bill Byrom- Vice President of Product Intelligence and Positioning, and Principal eCOA Science, Signant Health
- Erin O'Brien- Psychometrician, Division of Patient-Centered Development, Center for Devices and Radiological Health
- Elektra Papadopoulos- Director, Patient Experience Data & Strategy in Immunology and Oncology, AbbVie
- Poornima Sharma- Physician, Malignant Hematology Branch, Office of Clinical Evaluation, Center for Biologics Evaluation and Research, US Food and Drug Administration

Audience Q+A

1:40 p.m. Understanding Patient Baseline Severity and How it May Impact a COA's Ability to Detect Change

This session will explore considerations and impacts of choosing Clinical Outcome Assessments that are sensitive enough to detect meaningful changes for the severity of disease of the enrolled patient population. We will start with a brief presentation from FDA, then transition into a panel discussion.

Presentation

- David Reasner- Division Director, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration

Panel Discussion

Moderator

- Michelle Campbell- Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Stacie Hudgens- Chief Scientific and Strategy Officer, Clinical Outcomes Solutions
- Marian Strazzeri- Mathematical Statistician, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration
- Andrew Trigg- Statistical Innovation Lead, Bayer
- Therri Usher- Senior Mathematical Statistician, Division of Biometrics IV, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Audience Q+A

2:40 p.m. Break

2:55 p.m. Challenges and Opportunities for Deriving and Applying Meaningful Score Regions

This session will explore the challenges and opportunities for deriving and applying meaningful score regions to help interpret clinical trial data. We will start with a presentation from FDA staff, then transition into a panel discussion where panelists will explore the rationale for using meaningful score regions.

Presentation

- Monica Morell- Senior Statistician, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Panel Discussion

Moderator

- Gregory Levin- Associate Director for Statistical Science and Policy, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Jennifer Clark- Lead Mathematical Statistician, Division of Biometrics II, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration
- Ebony Dashiell-Aje- Executive Director & Head, Patient Centered Outcomes Science, BioMarin Pharmaceutical Inc
- Rikki Mangrum- Distinguished Scientist, Vector Psychometric Group
- Lori McLeod- Vice President of Patient-Centered outcomes Assessment, RTI Health Solutions
- Jammbe Musoro- Senior Statistician, Statistics and Quality of Life Departments, European Organisation for Research and Treatment of Cancer

Audience Q+A

4:55 p.m. Day 1 Wrap-Up

- Robyn Bent- Director, Patient-Focused Drug Development Program, Center for Drug Evaluation and Research, US Food and Drug Administration

Day 2: September 19

12:30 p.m. Welcome

- Ethan Gabbour- Public Health Analyst, Patient-Focused Drug Development Program, Office of the Center Director, Center for Drug Evaluation and Research, US Food and Drug Administration

12:35 p.m. Information on a COA Instrument Reviewed by the FDA: Updates to, and Learnings from, the 2009 Outline of the PRO Dossier

This session was developed to address stakeholder requests for an updated version of the appendix in the 2009 Guidance for Industry- Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. FDA staff will share an updated overview of areas that should be addressed in COA documents provided to the FDA for review. Staff will also walk through a detailed evidence dossier template that stakeholders may find useful and that may facilitate the submission of evidence, as discussed in the PFDD Guidance Series, to FDA to support a Clinical Outcome Assessment.

We will then transition into a discussion where panelists will share their thoughts on these draft documents and will explore considerations for implementation.

Presentations

- Vishal Bhatnagar- Associate Director for Patient Outcomes, Oncology Center of Excellence, US Food and Drug Administration
- Michelle Campbell- Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, Center for Drug Evaluation and Research, US Food and Drug Administration
- Lili Garrard- Master Mathematical Statistician, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration
- Cynthia Grossman- Division Director, Division of Patient-Centered Development, Center for Devices and Radiological Health, US Food and Drug Administration
- Naomi Knoble- Associate Director, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration
- Weimeng Wang- Staff Fellow, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Panel Discussion

Moderator

- Robyn Bent- Director, Patient-Focused Drug Development Program, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Cheryl Coon- Vice President, Clinical Outcome Assessment Program, Critical Path Institute
- Magdalena Harrington- Senior Director, Value & Evidence Patient-Centered Outcomes Assessment Lead, Pfizer
- Ashley Slagle- Clinical Outcome Assessments Scientific and Regulatory Consultant, Aspen Consulting

Audience Q+A

2:35 p.m. Break

2:50 p.m. Clinical Outcome Assessment Data: Interaction of Data Standardization and Statistical Programming

This session will start with brief presentations of resources and tools, such as technical specification guidances, developed as part of different standardization efforts by FDA and NCI. The group will then move to a panel discussion where they will be joined by industry representatives to explore considerations when using and developing standardized resources.

Presentation & Panel Moderator

- Laura Lee Johnson- Division Director, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Additional Presentations

- Ki Chung- Statistician, Division of Analytics and Informatics, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration
- Amylou Dueck- Professor of Biostatistics, Vice Chair, Department of Quantitative Health Sciences, Mayo Clinic
- Flora Mulkey- Lead Mathematical Statistician, Division of Biometrics V, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Pandu Kulkarni- Chief Executive Officer of Aparito, Chief Analytics Officer of Research and Development, Senior Vice President of Statistics, Data, and Analytics, Eli Lilly and Company
- Michael Otterstatter- Senior Principal Biostatistician Emmes Group

3:50 p.m. Closing Remarks

- Robyn Bent- Director, Patient-Focused Drug Development Program, Center for Drug Evaluation and Research, US Food and Drug Administration