Public Workshop

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

December 13, 2024

10:00 a.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

10:05 a.m. Opening Remarks

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

10:10 a.m. Overview of Patient Experience Data

During this session, FDA staff will provide an overview of different types of patient experience data before discussing the role of this data throughout the drug development process. Presenters will then talk about how FDA documents inclusion of patient experience data in their reviews and provide some examples of how patient experience data has informed reviews of marketing applications.

Presentations

- Robyn Bent- Director, Patient-Focused Drug Development Program, Office of the Center Director, Center for Drug Evaluation and Research, US Food and Drug Administration
- Teresa Buracchio- Director, Office of Neuroscience, Center for Drug Evaluation and Research, US Food and Drug Administration
- Megha Kaushal- Branch Chief, Benign Hematology Branch, Office of Clinical Evaluation, Center for Biologics Evaluation and Research, US Food and Drug Administration

10:45 a.m. Submissions of Patient Experience Data

This session will include a panel discussion where FDA staff and Industry representatives will talk about submitting patient experience data to FDA as part of a marketing application. The discussion will center around what information should be included in the Reviewers Guide and experiences related to the submission of patient experience data.

Panel Discussion

Moderator

- Pujita Vaidya- Director, Regulatory Science and Policy, Sanofi

Panelists

- Elizabeth (Nicki) Bush- Global Head, Endpoint Strategy and COA Measurement, OPEN Health Group
- Lesley Maloney- Global Regulatory Policy Lead, Genentech
- Monica Morell- Senior Statistician, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration
- David Reasner- Division Director, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration
- Rosa Sherafat-Kazemzadeh- Branch Chief, General Medicine Branch 2, Office of Clinical Evaluation, Center for Biologics Evaluation and Research, US Food and Drug Administration

- Aliza Thompson- Acting Division Director, Division of Cardiology and Nephrology, Center for Drug Evaluation and Research, US Food and Drug Administration

Audience Q+A

12:00 p.m. Lunch Break

12:45 p.m. Delphi Methods- Challenges and Opportunities

This session will explore the challenges and opportunities of using Delphi methods including how to keep the Delphi process patient centric. Participants will present case studies followed by a panel discussion.

Presentations

- Ebony Dashiell-Aje- Executive Director, Head, Patient Centered Outcomes Science, BioMarin Pharmaceutical Inc.
- Holly Peay- Senior Research Scientist, RTI International

Panel Discussion

Moderator

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

Panelists

- Rob Arbuckle- Managing Director, Patient-Centered Outcomes, Adelphi Values
- Selena Daniels- Deputy Division Director, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration
- Eleanor Perfetto- Consultant and Caregiver
- Weimeng Wang- Staff Fellow, Division of Biometrics III, Office of Biostatistics, Center for Drug Evaluation and Research, US Food and Drug Administration

Audience Q+A

2:00 p.m. Qualitative/Embedded Interviews

This session will explore the challenges and opportunities of qualitative and embedded interviews. Participants will present case studies followed by a panel discussion.

Presentations

- Anna-Karin Berger- Director, Patient Centered Outcomes, Lundbeck
- Dana DiBenedetti- Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions
- Hilary Wilson- Director, US Medicine, Boehringer Ingelheim

Panel Discussion

Moderator

- Sarah Stothers- Clinical Analyst, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration

Panelists

- Emily Freilich- Division Director, Division of Neurology I, Office of Neuroscience, Center for Drug Evaluation and Research, US Food and Drug Administration
- Onyeka Illoh- Team Lead, Division of Clinical Outcome Assessment, Center for Drug Evaluation and Research, US Food and Drug Administration
- Lola Rahib- Senior Scientific Consultant, Translational Research, Mission: Cure

Audience Q+A

3:15 p.m. Break

3:30 p.m. Two Hot Topics: When to Consider Age-Normed Scores and Repurposing COAs for New Uses

In this session panelists will discuss:

- The use of age-normed scores in clinical trials
- Challenges related to using scales that were developed for use in clinical care then repurposed for use in clinical trials

Presentations

- Cheryl Coon- Vice President, Clinical Outcome Assessment Program, Critical Path Institute
- Marian Strazzeri- Mathematical Statistician, Division of Biometrics III, Office of Biostatistics, 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:

- Robyn Carson- Vice President, Patient-Centered Outcomes Research & HEOR Aesthetics, AbbVie
- Rikki Mangrum- Director, Patient Centered Research, Vector Psychometric Group
- Bryce Reeve- Professor of Population Health Sciences and Pediatrics, Director, Center for Health Measurement, Duke University School of Medicine

Audience Q+A

4:55 p.m. Closing Remarks

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