U.S. FOOD & DRUG ADMINISTRATION

PATIENT-FOCUSED DRUG DEVELOPMENT

# Methods to Identify What is Important to Patients and Select, Develop or Modify Fit-for-Purpose Clinical Outcome Assessments

October 15-16, 2018

**OCTOBER 15TH**

9:00 – 9:05 am **Welcome**
Michelle Campbell, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)

9:05 – 9:25 am **Opening Remarks**
Theresa Mullin, Office of the Center Director (OCD), CDER, FDA

9:25 – 9:45 am **Overview and Goals of Patient-Focused Drug Development Guidance 2**
Ebony Dashiell-Aje, OND, CDER, FDA

9:45 – 11:00 am **Methods to Identify What is Important to Patients**

**Moderator:** Ebony Dashiell-Aje, OND, CDER, FDA
**Panelists:**

- Dagmar Amtmann, Research Professor, University of Washington
- Elizabeth (Nicki) Bush, Director and Head, Global Patient-Focused Outcomes Center of Expertise, Eli Lilly and Company
- Emily Freeman, Director, Health Economics and Outcomes Research - Patient-Centered Outcomes, AbbVie
- Nova Getz, Research Associate, Center for Information and Study on Clinical Research Participation
- Patty Spears, Patient Research Advocate, University of North Carolina Lineberger Comprehensive Cancer Center
- Diane Turner-Bowker, Director, Patient-Centered Outcomes, Adelphi Values

**Audience Question and Answer**

11:00 – 11:15 am **Break**

11:15 – 12:30 pm **Emerging Best Practices for Methods to Identify What is Important to Patients**

**Moderator:** Selena Daniels, OND, CDER, FDA
**Panelists:**

- Vanessa Arnedo, Director, Research Partnerships, The Michael J. Fox Foundation for Parkinson's Research
- Antonia Bennett, Faculty Director, Patient-Reported Outcomes Core, University of North Carolina
- Bill Byrom, Vice President of Product Strategy and Innovation, CRF Bracket
- Sonya Eremenco, Associate Director, Patient-Reported Outcome Consortium, Critical Path Institute
- David Reasner, Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals
- Barbara Stussman, Survey Statistician, National Center for Complementary and Integrative Health, National Institutes of Health
- Tara Symonds, Chief Science Officer, Strategic Lead, Clinical Outcome Assessments, Clinical Outcomes Solutions

**Audience Question and Answer**

12:30 – 1:30 pm **Lunch**

1:30 – 2:00 pm **Overview and Goals of Guidance 3**
Elektra Papadopoulos, OND, CDER, FDA

2:00 – 3:15 pm **FDA Cross-Center Panel Discussion: Clinical Outcome Assessment Use to Support Patient-Focused Outcome Measurement Throughout the Medical Product Lifecyle**

**Moderator:** Elektra Papadopoulos, OND, CDER, FDA
**Panelists:**

- Billy Dunn, OND, CDER, FDA
- Martin Ho, Office of Surveillance and Biometrics (OSB), CDRH, FDA
- Telba Irony, Office of Biostatistics and Epidemiology (OBE), CBER, FDA
- Laura Lee Johnson, Office of Translational Science (OTS), CDER, FDA
- Paul Kluetz, Oncology Center of Excellence, FDA
- Larissa Lapteva, Office of Tissues and Advanced Therapies (OTAT), CBER, FDA
- Theresa Mullin, OCD, CDER, FDA
- Michelle Tarver, OCD, CDRH, FDA

**Audience Question and Answer**

3:15 – 3:30 pm **Break**

3:30 – 4:45 pm **Roadmap to Clinical Outcome Assessment Selection and/or Development for Clinical Trials**

**Moderator:** Michelle Campbell, OND, CDER, FDA
**Panelists:**

- Robyn Carson, Head, Patient-Centered Outcomes Research, Allergan
- Alicyn Campbell, Global Head, Patient-Centered Outcomes Research for Oncology, Genentech
- Stephen Joel Coons, Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
- Phyllis Foxworth, Advocacy Vice President, Depression and Bipolar Support Alliance
- Nancy Kline Leidy, Senior Vice President, Scientific Affairs and Patient-Centered Outcomes Research, Evidera
- Kevin Weinfurt, Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine

**Audience Question and Answer**

4:45 – 5:00 pm **Closing Remarks**
Megan Moncur, OBE, CBER, FDA

PATIENT-FOCUSED DRUG DEVELOPMENT

# Methods to Identify What is Important to Patients and Select, Develop or Modify Fit-for-Purpose Clinical Outcome Assessments

October 15-16, 2018

**OCTOBER 16TH**

9:00 – 9:05 am **Welcome**
Meghana Chalasani, OCD, CDER, FDA

9:05 – 9:30 am **Opening Remarks**
Michelle Tarver, OCD, CDRH, FDA

9:30 – 10:45 am **Considerations for the Selection and Use of Clinical Outcome Assessments in Special Populations**

**Moderator:** Vasum Peiris, OCD, CDRH, FDA
**Panelists:**

- Rosángel Cruz, Director of Research and Clinical Affairs, Cure SMA
- Katie Donohue, OND, CDER, FDA
- Dionna Green, Office of Pediatric Therapeutics, Office of the Commissioner, FDA
- Larissa Lapteva, OTAT, CBER, FDA
- Linda Nelsen, Senior Director and Head, Patient-Centered Outcomes, GlaxoSmithKline
- Carole Tucker, Chair, Department of Physical Therapy, Temple University College of Public Health

**Audience Question and Answer**

10:45 – 11:00 am **Break**

11:00 – 12:15 pm **Methods for Determining and Interpreting Within-Patient Meaningful Score Changes in Clinical Outcome Assessments**

**Moderator:** Michelle Campbell, OND, CDER, FDA
**Panelists:**

- Adam Carle, Associate Professor of Pediatrics, Cincinnati Children's Hospital Medical Center
- Wen-Hung Chen, OND, CDER, FDA
- Cheryl Coon, Principal, Outcometrix
- Linda S. Deal, Senior Director and Head of Patient-Centered Outcomes Assessment, Pfizer Inc.
- Leah Howard, Chief Operating Officer, National Psoriasis Foundation
- Bryce Reeve, Professor and Director of Center for Health Measurement, Duke University School of Medicine
- R.J. Wirth, President and Managing Partner, Vector Psychometric Group

**Audience Question and Answer**

12:15 – 1:15 pm **Lunch**

1:15 – 2:30 pm **Emerging Technologies to Support Fit-for-Purpose Clinical Outcome Assessment**

**Moderator:** Sarrit Kovacs, OND, CDER, FDA
**Panelists:**

- Bill Byrom, Vice President of Product Strategy and Innovation, CRF Bracket
- Chad Gwaltney, President, Gwaltney Consulting
- Martin Ho, OSB, CDRH, FDA
- Megan Moreno, Principal Investigator of the Social Media and Adolescent Health Research Team and Vice Chair of Digital Health, Department of Pediatrics, University of Wisconsin-Madison
- Kushang Patel, Research Associate Professor of Anesthesiology and Pain Medicine, University of Washington
- David Reasner, Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals
- Suzanne Schrandt, Patient/Patient Advocate and Director of Patient Engagement, Arthritis Foundation
- Brennan Spiegel, Director of Health Services Research, Professor of Medicine and Public Health, Cedars-Sinai Health System

**Audience Question and Answer**

2:30 – 2:45 pm **Break**

2:45 – 4:00 pm **Identifying Key Themes and Next Steps**

**Moderator:** Meghana Chalasani, OCD, CDER, FDA
**Panelists:**

- Stephen Joel Coons, Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
- Cynthia Grossman, Director, Science of Patient Input, FasterCures
- Katarina Halling, Global Head Patient Reported Outcomes, AstraZeneca
- Laura Lee Johnson, OTS, CDER, FDA
- Nancy Kline Leidy, Senior Vice President, Scientific Affairs and Patient-Centered Outcomes Research, Evidera
- Elektra Papadopoulos, OND, CDER, FDA
- Ashley Slagle, Clinical Outcome Assessments Scientific and Regulatory Consultant, Aspen Consulting
- Kevin Weinfurt, Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine

**Audience Question and Answer**

4:00 – 4:45 pm **Open Public Comment**
Shanon Woodward, OCD, CDER, FDA

4:45 – 5:00 pm **Closing Remarks**
Elektra Papadopoulos, OND, CDER, FDA