THIRD ANNUAL CLINICAL OUTCOME ASSESSMENTS IN CANCER CLINICAL TRIALS (COA-CCT) WORKSHOP

Measurement, Analysis and Communication of Core Patient Symptoms and Function in Cancer Clinical Trials

Conference Hashtag: #OCEOutcomes

FDA White Oak Campus- Great Room B & C
10903 New Hampshire Avenue ■ Silver Spring, MD 20993

June 22, 2018

Co-sponsored by

FDA U.S. FOOD & DRUG ADMINISTRATION
ONCOLOGY CENTER OF EXCELLENCE

ASCO®

Document last modified June 18, 2018

FDA-ASCO 2018 COA-CCT WORKSHOP

AGENDA – CONTINUED

7:30 - 8:15 am	**Registration – Great Room Reception Area**
8:15 - 8:30 am	**Welcome and Opening Remarks** *Paul Kluetz, MD* - Associate Director for Clinical Science, Oncology Center of Excellence (OCE), U.S. Food and Drug Administration (FDA) *Heidi Klepin, MD, MS* - Chair, Cancer Research Committee, American Society of Clinical Oncology (ASCO)
8:30 - 10:00 am	**Session 1: WHAT to Measure: PRO Core Concepts in Cancer Trials** **Chair:** *Belinda King-Kallimanis, PhD* - Social Scientist, Office of Hematology and Oncology Products (OHOP), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA **Presentations:** ▪ ***Fit for Purpose Instruments*****:** *Selena Daniels, PharmD, MS* - Team Lead, Clinical Outcome Assessment Staff (COA Staff), OND, CDER, FDA ▪ ***Illustrating the Issue*****:** *Lynn Howie, MD* - PRO Lead, Division of Oncology Products 1 (DOP1), OHOP, OND, CDER, FDA ▪ ***Proposed Core PRO Assessment Strategy*****:** *Paul Kluetz, MD* - OCE, FDA **Panel Discussion:** **Additional Panelists:** ▪ *Andrea Ferris* - Patient Advocacy, LUNGevity ▪ *Beate Wieseler, Dr. rer. nat.* - Head, Department of Drug Assessment, Institute for Quality and Efficiency in Health Care (IQWiG), Germany ▪ *Ethan Basch, MD, MSc* - Director, Cancer Outcomes Research Program, University of North Carolina ▪ *Daniel O'Connor, MB, ChB, PhD, MFPM* - Expert Medical Assessor, Medicines and Healthcare Products Regulatory Agency (MHRA), England **Q & A**

FDA-ASCO 2018 COA-CCT WORKSHOP

AGENDA – CONTINUED

10:00 - 10:30 am	**Break**
10:30 – 12 pm	**Session 2: HOW to Measure: Case Based Examples of PRO Strategies** **Chair:** *Paul Kluetz, MD* - Associate Director for Clinical Science, OCE, FDA **<u>Presentations: Each speaker will build an assessment strategy using existing measurement systems and libraries to assess the core outcomes discussed in session 1.</u>** ▪ *Roxanne Jensen, PhD* - Clinical Research Scientist, Clinical Monitoring Research Program, Division of Cancer Control and Population Sciences, National Cancer Institute, National Institutes of Health ▪ *Aaron Kaat, PhD* - Research Assistant Professor, Feinberg School of Medicine, Northwestern University ▪ *Mogens Groenvold, MD, PhD, DSc* - Professor, Department of Public Health, Section for Health Services Research, University of Copenhagen; and The Research Unit, Department of Palliative Medicine, Bispebjerg University Hospital, Denmark **<u>Panel Discussion:</u>** **Additional Panelists:** ▪ *Robert (Bob) Harrison* - Prostate Cancer Patient - tentative ▪ *Alicyn Campbell* - Global Head of Patient-Centered Outcomes Research for Oncology, Product Development - Biometrics, Genentech, a Member of the Roche Group ▪ *Selena Daniels, PharmD, MS* - COA Staff, OND, CDER, FDA **Q & A**
12:00 - 1:00 pm	**Lunch**

FDA-ASCO 2018 COA-CCT WORKSHOP

AGENDA – CONTINUED

1:00 - 2:30 pm	**Session 3: Efforts to Standardize PRO Analysis Methods** **Chair:** *Laura Lee Johnson, PhD* - Director (Acting), Division of Biometrics III (DB III), Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA **Presentations:** ▪ **SISAQOL- An International Collaboration:** *Andrew Bottomley, PhD* - Head, Quality of Life Department, Assistant Director, EORTC, Belgium ▪ **A Regulatory Perspective on PRO Research Objectives:** *Kathy Soltys, MD*- Acting Director, Marketed Pharmaceuticals and Medical Devices Bureau, Marketed Health Products Directorate, Health Products and Food Branch, Health Canada ▪ **Missing Data - Common Issues, Proposed Solutions, and Future Work:** *Amylou Dueck, PhD* - Head, Section of Biostatistics, Division of Health Sciences Research, Mayo Clinic, Arizona ▪ **Statistical Methods - Common Challenges and Potential Solutions:** *Rajeshwari (Raji) Sridhara, PhD* - Division Director, Division of Biometrics V (DBV), OB, OTS, CDER, FDA **Panel Discussion:** **Additional Panelists:** ▪ *Nancy Roach* - Patient Advocate - tentative ▪ *Beate Wieseler, Dr. rer. nat.* – Head, Department of Drug Assessment, Institute for Quality and Efficiency in Health Care (IQWiG), Germany ▪ *Aaron Kaat, PhD* - Research Assistant Professor, Feinberg School of Medicine, Northwestern University ▪ *Li Li, PhD* - Senior Research Scientist, Statistics – Real World Analytics – Oncology, Eli Lilly and Company **Q & A**

2:30 - 3:00 pm	**Break**
3:00 - 4:30 pm	**Session 4: Using PRO Data in Regulatory Review: Current FDA Approaches to Analyze Descriptive Patient Experience Data** **Chair:** *Jason Schroeder, PhD* - Associate Director, DBV, OB, OTS, CDER, FDA **Presentations:** ▪ **Clarifying Disposition and Completion:** *Lynn Howie, MD* - PRO Lead, DOP1, OHOP, OND, CDER, FDA ▪ **Current FDA Approach:** *Mallorie Fiero, PhD* - Mathematical Statistician, DBV, OB, OTS, CDER, FDA ▪ **PRO Data Overview- Presenting the Big Picture:** *Karon Cook, PhD* - Research Professor of Medical Social Sciences, Feinberg School of Medicine, Northwestern University **Panel Discussion:** **Additional Panelists:** ▪ *Jane Perlmutter, PhD, MBA* - Patient Representative ▪ *Michael Brundage, MD, MSc, FRCPC* - Director, Division of Cancer Care and Epidemiology, Cancer Research Institute, Queen's University, Canada ▪ *Josephine Norquist* - Distinguished Scientist, Patient-Centered Endpoints and Strategy Group Lead, Center for Observational and Real-World Evidence, Merck **Q & A**
4:30 - 5:00 pm	**Wrap Up** *Paul Kluetz* and *Heidi Klepin*
5:00 pm	**Adjourn**

Copies of the Workshop's slide presentations will be available after the workshop.