CRITICAL PATH INSTITUTE

Second Annual Workshop On Clinical Outcome Assessments In Cancer Clinical Trials

Assessing Tolerability of Cancer Treatments: Optimizing the Role of Patient-Reported Data

April 25, 2017

Hyatt Regency Bethesda
1 Bethesda Metro Center
Bethesda, MD 20814

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Critical Path Institute
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Workshop Agenda

7:30-8:15 am	**Registration and Continental Breakfast – Regency I and II**	
8:15-8:30 am	**Welcome and Opening Remarks** *Paul G. Kluetz, MD* — Acting Associate Director of Patient Outcomes, Oncology Center of Excellence (OCE), U.S. Food and Drug Administration (FDA) *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)	

8:30-10:00 am	**Session 1: Exploring the Concepts of Safety and Tolerability – Incorporating the Patient Voice** **Chair:** *Bindu Kanapuru, MD* – Medical Officer, Division of Hematology Products, Office of Hematology and Oncology Products (OHOP), FDA **Presentations:** • ***The Patient Perspective:*** *James (Randy) Hillard, MD* – Professor of Psychiatry, Michigan State University • ***Safety vs Tolerability – A Clinician's Perspective:*** *Crystal Denlinger, MD, FACP* – Associate Professor, Department of Hematology/Oncology; Chief, Gastrointestinal Medical Oncology; Director, Survivorship Program; Deputy Director, Phase 1 Program, Fox Chase Cancer Center • ***Safety vs Tolerability – The Canadian Regulatory Perspective:*** *Katherine Soltys, MD* – Acting Director, Bureau of Medical Sciences, Therapeutic Products Directorate, Health Products and Food Branch, Health Canada • ***Patient Advocate and Survivor:*** *Karen E. Arscott, DO, MSc* – Associate Professor of Medicine-Patient Advocate and Survivor, Geisinger Commonwealth School of Medicine **Panel Discussion:** **Additional Panelists:** • *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, Medicines and Healthcare products Regulatory Agency (MHRA) • *Eric Rubin, MD* – Vice President and Therapeutic Area Head, Merck & Co., Inc. • *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA • *Selena R. Daniels, PharmD, MS* – Team Leader, Clinical Outcome Assessments Staff (COA Staff), OND, CDER, FDA **Q & A**	
10:00–10:30 am	**Break**	

10:30 am – Noon	**Session 2: Assessment of Safety and Tolerability – Emerging Patient-Reported Methods** **Chair:** *Steven Lemery, MD, MHS* – Lead Medical Officer (Team Leader), OHOP, FDA **Presentations:** • ***Adverse Event Reporting – CTCAE and PRO-CTCAE:*** *Lori Minasian, MD, FACP* – Deputy Director, Division of Cancer Prevention, National Cancer Institute (NCI), National Institutes of Health (NIH) • ***Overview of the PRO-CTCAE Industry Working Group – Objectives, Goals, Activities:*** *Sheetal Patel, PhD* – Outcomes Research Scientist – Oncology, Genentech, a member of the Roche Group; Co-Chair, PRO-CTCAE Industry WG • ***Experience of Implementing PRO-CTCAE in Clinical Trials:*** *Anna Rydén, PhD* – Director, Patient Science, AstraZeneca • ***Toxicity over Time (ToxT) – Longitudinal Adverse Event Analysis in Cancer Clinical Trials:*** *Gita Thanarajasingam, MD* – Senior Associate Consultant, Division of Hematology, Mayo Clinic; Assistant Professor of Medicine, Mayo Clinic College of Medicine **Panel Discussion:** **Additional Panelists:** • *Christopher R. Blackburn* – Cancer Patient and Senior Corporate Development Manager, GZA GeoEnvironmental (unable to attend) • *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, MHRA • *Rajeshwari (Raji) Sridhara, PhD* – Division Director, Division of Biometrics V, Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA **Q & A**	
Noon – 1:00 pm	**Lunch – Cabinet, Judiciary, Congressional and Old Georgetown Rooms**	

1:00–2:30 pm	**Session 3: Analysis and Display of PRO-Based Tolerability Data – Metrics and Paths Forward** **Chair:** *Laura Lee Johnson, PhD* – Deputy Director, Division of Biometrics III, OB, OTS, CDER, FDA **Presentations:** • ***Regulatory Considerations in Europe for Trials Collecting PRO Data on Tolerability:*** *Yolanda Barbachano, PhD* – Senior Statistical Assessor, Licensing Division, MHRA • ***Defining the Population, Missing Data vs Completion Rate and Presentation Methods:*** *Mallorie H. Fiero, PhD* – Mathematical Statistician, Division of Biometrics V, OB, OTS, CDER, FDA • ***Analysis Strategies for PRO CTCAE:*** *Diane Fairclough, DrPH* – Professor, Biostatistics, Colorado School of Public Health **Panel Discussion:** **Additional Panelists:** • *Corneel Coens, MSc* – Lead Statistician, QoL Department, EORTC • *Joseph Cappelleri, PhD, MPH, MS* – Senior Director of Biostatistics, Pfizer, Inc. • *Sandra A. Mitchell, PhD, CRNP* – Research Scientist and Program Director, Outcomes Research Branch, NCI, NIH • *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA **Q & A**	
2:30–3:00 pm	**Break**	

3:00–4:30 pm	**Session 4: From Individual Symptoms to Overall Side Effect Burden** **Chair:** *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA **Presentations:** • ***Concise Measurement of Cancer Treatment Side Effect Burden and its Relationship to Outcomes:*** *David Cella, PhD* – Professor and Chair, Department of Medical Social Sciences, Feinberg School of Medicine, Northwestern University • ***Symptoms and Functional Interference During Cancer Treatment:*** *Charles S. Cleeland, PhD* – McCullough Professor of Cancer Research, University of Texas MD Anderson Cancer Center • ***How EORTC Considers Overall Side Effect Burden and Strengths/Limitations of Summating Individual Symptoms into An Overall Side Effect Score:*** *Galina Velikova, BMBS(MD), PhD, FRCP* – Professor, University of Leeds **Panel Discussion:** **Additional Panelists:** • *Mary Lou Smith, MPA, MBA, JD* – Co-Founder, Research Advocacy Network • *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, MHRA • *Ethan Basch, MD, MSc* – Director, Cancer Outcomes Research Program, University of North Carolina • *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA **Q & A**	
4:30 – 5:00 pm	**Wrap Up** Paul Kluetz and Stephen Coons	
5:00 pm	**Adjourn**	