

## 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**

CO-SPONSORED BY:

[Critical Path Institute](#)

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### **Workshop Agenda**

7:30-8:15 am	<b>Registration and Continental Breakfast – Regency I and II</b>	
8:15-8:30 am	<b><u>Welcome and Opening Remarks</u></b> <i>Paul G. Kluetz, MD</i> — Acting Associate Director of Patient Outcomes, Oncology Center of Excellence (OCE), U.S. Food and Drug Administration (FDA) <i>Stephen Joel Coons, PhD</i> — 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 VoiceChair:**

*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**

<p>10:30 am – Noon</p>	<p><b>Session 2: <u>Assessment of Safety and Tolerability – Emerging Patient-Reported Methods</u></b><b>Chair:</b> <i>Steven Lemery, MD, MHS</i> – Lead Medical Officer (Team Leader), OHOP, FDA</p> <p><b><u>Presentations:</u></b></p> <ul style="list-style-type: none"> <li>• <b><i>Adverse Event Reporting – CTCAE and PRO-CTCAE:</i></b> <i>Lori Minasian, MD, FACP</i> – Deputy Director, Division of Cancer Prevention, National Cancer Institute (NCI), National Institutes of Health (NIH)</li> <li>• <b><i>Overview of the PRO-CTCAE Industry Working Group – Objectives, Goals, Activities:</i></b> <i>Sheetal Patel, PhD</i> – Outcomes Research Scientist – Oncology, Genentech, a member of the Roche Group; Co-Chair, PRO-CTCAE Industry WG</li> <li>• <b><i>Experience of Implementing PRO-CTCAE in Clinical Trials:</i></b> <i>Anna Rydén, PhD</i> – Director, Patient Science, AstraZeneca</li> <li>• <b><i>Toxicity over Time (ToxT) – Longitudinal Adverse Event Analysis in Cancer Clinical Trials:</i></b> <i>Gita Thanarajasingam, MD</i> – Senior Associate Consultant, Division of Hematology, Mayo Clinic; Assistant Professor of Medicine, Mayo Clinic College of Medicine</li> </ul> <p><b><u>Panel Discussion:</u></b></p> <p><b>Additional Panelists:</b></p> <ul style="list-style-type: none"> <li>• <i>Christopher R. Blackburn</i> – Cancer Patient and Senior Corporate Development Manager, GZA GeoEnvironmental (unable to attend)</li> <li>• <i>Daniel O’Connor, MB, ChB, PhD, MFPM</i> – Expert Medical Assessor, MHRA</li> <li>• <i>Rajeshwari (Raji) Sridhara, PhD</i> – Division Director, Division of Biometrics V, Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA</li> </ul> <p><b>Q &amp; A</b></p>	
<p>Noon – 1:00 pm</p>	<p><b>Lunch – Cabinet, Judiciary, Congressional and Old Georgetown Rooms</b></p>	

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**

<p>3:00–4:30 pm</p>	<p><b>Session 4: <u>From Individual Symptoms to Overall Side Effect Burden</u></b>  <b>Chair:</b> <i>Paul G. Kluetz, MD</i> – Acting Associate Director of Patient Outcomes, OCE, FDA</p> <p><b><u>Presentations:</u></b></p> <ul style="list-style-type: none"> <li>• <b><i>Concise Measurement of Cancer Treatment Side Effect Burden and its Relationship to Outcomes:</i></b>  <i>David Cella, PhD</i> – Professor and Chair, Department of Medical Social Sciences, Feinberg School of Medicine, Northwestern University</li> <li>• <b><i>Symptoms and Functional Interference During Cancer Treatment:</i></b> <i>Charles S. Cleeland, PhD</i> – McCullough Professor of Cancer Research, University of Texas MD Anderson Cancer Center</li> <li>• <b><i>How EORTC Considers Overall Side Effect Burden and Strengths/Limitations of Summating Individual Symptoms into An Overall Side Effect Score:</i></b>  <i>Galina Velikova, BMBS(MD), PhD, FRCP</i> – Professor, University of Leeds</li> </ul> <p><b><u>Panel Discussion:</u></b></p> <p><b>Additional Panelists:</b></p> <ul style="list-style-type: none"> <li>• <i>Mary Lou Smith, MPA, MBA, JD</i> – Co-Founder, Research Advocacy Network</li> <li>• <i>Daniel O’Connor, MB, ChB, PhD, MFPM</i> – Expert Medical Assessor, MHRA</li> <li>• <i>Ethan Basch, MD, MSc</i> – Director, Cancer Outcomes Research Program, University of North Carolina</li> <li>• <i>Michelle Campbell, PhD</i> – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA</li> </ul> <p><b>Q &amp; A</b></p>	
<p>4:30 – 5:00 pm</p>	<p><b><u>Wrap Up</u></b>  Paul Kluetz and Stephen Coons</p>	
<p>5:00 pm</p>	<p><b>Adjourn</b></p>	