# 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

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| 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)<br>*Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path) | |

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| 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<br><br>**Presentations:**<br><br>• ***The Patient Perspective:*** *James (Randy) Hillard, MD* – Professor of Psychiatry, Michigan State University<br>• ***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<br>• ***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<br>• ***Patient Advocate and Survivor:*** *Karen E. Arscott, DO, MSc* – Associate Professor of Medicine-Patient Advocate and Survivor, Geisinger Commonwealth School of Medicine<br><br>**Panel Discussion:**<br><br>**Additional Panelists:**<br><br>• *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, Medicines and Healthcare products Regulatory Agency (MHRA)<br>• *Eric Rubin, MD* – Vice President and Therapeutic Area Head, Merck & Co., Inc.<br>• *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA<br>• *Selena R. Daniels, PharmD, MS* – Team Leader, Clinical Outcome Assessments Staff (COA Staff), OND, CDER, FDA<br><br>**Q & A** | |
| 10:00–10:30 am | **Break** | |

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| 10:30 am – Noon | **Session 2: Assessment of Safety and Tolerability – Emerging Patient-Reported MethodsChair:** *Steven Lemery, MD, MHS* – Lead Medical Officer (Team Leader), OHOP, FDA<br><br>**Presentations:**<br><br>• ***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)<br>• ***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<br>• ***Experience of Implementing PRO-CTCAE in Clinical Trials:*** *Anna Rydén, PhD* – Director, Patient Science, AstraZeneca<br>• ***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<br><br>**Panel Discussion:**<br><br>**Additional Panelists:**<br><br>• *Christopher R. Blackburn* – Cancer Patient and Senior Corporate Development Manager, GZA GeoEnvironmental (unable to attend)<br>• *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, MHRA<br>• *Rajeshwari (Raji) Sridhara, PhD* – Division Director, Division of Biometrics V, Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA<br><br>**Q & A** | |
| Noon – 1:00 pm | **Lunch – Cabinet, Judiciary, Congressional and Old Georgetown Rooms** | |

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| 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<br><br>**Presentations:**<br><br>• ***Regulatory Considerations in Europe for Trials Collecting PRO Data on Tolerability:*** *Yolanda Barbachano, PhD* – Senior Statistical Assessor, Licensing Division, MHRA<br>• ***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<br>• ***Analysis Strategies for PRO CTCAE:*** *Diane Fairclough, DrPH* – Professor, Biostatistics, Colorado School of Public Health<br><br>**Panel Discussion:**<br><br>**Additional Panelists:**<br><br>• *Corneel Coens, MSc* – Lead Statistician, QoL Department, EORTC<br>• *Joseph Cappelleri, PhD, MPH, MS* – Senior Director of Biostatistics, Pfizer, Inc.<br>• *Sandra A. Mitchell, PhD, CRNP* – Research Scientist and Program Director, Outcomes Research Branch, NCI, NIH<br>• *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA<br><br>**Q & A** | |
| 2:30–3:00 pm | **Break** | |

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| 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<br><br>**Presentations:**<br><br>• ***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<br>• ***Symptoms and Functional Interference During Cancer Treatment:*** *Charles S. Cleeland, PhD* – McCullough Professor of Cancer Research, University of Texas MD Anderson Cancer Center<br>• ***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<br><br>**Panel Discussion:**<br><br>**Additional Panelists:**<br><br>• *Mary Lou Smith, MPA, MBA, JD* – Co-Founder, Research Advocacy Network<br>• *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, MHRA<br>• *Ethan Basch, MD, MSc* – Director, Cancer Outcomes Research Program, University of North Carolina<br>• *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA<br><br>**Q & A** | |
| 4:30 – 5:00 pm | **Wrap Up**<br>Paul Kluetz and Stephen Coons | |
| 5:00 pm | **Adjourn** | |