CRITICAL PATH INSTITUTE

Workshop on Clinical Outcome Assessments (COAs) in Cancer Clinical Trials

April 26, 2016

Sheraton Silver Spring Hotel
8777 Georgia Avenue
Silver Spring, MD 20910

The Workshop on Clinical Outcome Assessments in Cancer Clinical Trials, was co-sponsored by the FDA's Office of Hematology and Oncology Products and the Critical Path Institute's Patient-Reported Outcome (PRO) Consortium. The workshop aimed to provide a forum for collaborative and productive multidisciplinary discussions to advance our understanding of the complex regulatory, healthcare policy, and scientific issues surrounding the use of PRO measures in cancer clinical trials.

In the workshop, a broad array of international stakeholders involved in oncology drug development, regulation, utilization, and reimbursement provided perspectives on the current approaches to PRO assessment in oncology trials, explored the utility of information derived from existing PRO measures, and identified potential ways to improve alignment and strategic use of PRO measures to support oncology drug development and better inform treatment decisions.

Note: Degrees and affiliations are included the first time the presenters and panelists appear in the agenda

Workshop Agenda

7:30-8:15am	**Registration and Continental Breakfast – Cypress Ballroom**
8:15-8:30 am	**Welcome and Opening Remarks** *Paul G. Kluetz, MD* — Associate Director of Clinical Science, Office of Hematology and Oncology Products (OHOP), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA) *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute

8:30-10:00 am	**Session 1: Reviewing the Patient-Reported Outcome (PRO) Data Needs of Stakeholders: What Questions are We Asking?** **Chair:** *Stephen Coons* **Presentations:** • **US Regulator's Perspective:** *Paul Kluetz* • **European Regulator's Perspective:** *Daniel O'Connor, MB, ChB, PhD, MFPM* – Expert Medical Assessor, Medicines and Healthcare products Regulatory Agency (MHRA) • **European Health Technology Assessment Perspective:** *Keith Tolley, MPhil* – Tolley Health Economics Ltd. • **An Industry Perspective** – "Caught in the Middle": *Joseph O'Connell, MD* – Vice President and Global Therapeutic Lead, Hematology Oncology, InVentivHealth/Clinical **Panel Discussion:** ***Reviewing the Needs of all Stakeholders: Where are They Similar and Where do They Differ?*** **Additional Panelists:** • *Mary Lou Smith, MPA, MBA, JD* – Co-Founder, Research Advocacy Network • *Naomi Aronson, PhD* – Executive Director, Clinical Evaluation, Innovation, and Policy, Blue Cross Blue Shield Association • *Chiun-Fang Chiou, PhD* – Senior Director, Patient Reported Outcomes, Janssen **Q & A**
10:00–10:30 am	**Break**

10:30 am – Noon	**Session 2: Using Multiple Instruments to Create a Comprehensive PRO Assessment Strategy in Cancer Trials** **Chair:** *Stephen Coons* **Presentations:** • **Considerations when Integrating Multiple Instruments:** *Paul Kluetz* • **A Patient's Perspective on PRO Assessment:** *Patty Spears* – Research Advocate, Cancer Information and Support Network • **An Industry Example of Instrument Modification:** *Alicyn Campbell, MPH* – Global Head, Patient-Centered Outcomes Research for Oncology, Genentech • **Considerations in Addressing Respondent Burden:** *Charles S. Cleeland, PhD* – McCullough Professor of Cancer Research and Founding Chair, Department of Symptom Research, MD Anderson Cancer Center, University of Texas *Sandra A. Mitchell, PhD, CRNP* – Research Scientist and Program Director, Outcomes Research Branch, National Cancer Institute (NCI) *Andrew Bottomley, PhD* – Assistant Director, Head of Quality of Life Department, European Organisation for Research and Treatment of Cancer (EORTC) *David Cella, PhD* – Chair, Department of Medical Social Sciences, Northwestern University Feinberg School of Medicine **Panel Discussion:** ***Leveraging Existing and Emerging Tools to Optimize PRO Assessment Strategies in Cancer Trials*** **Additional Panelists:** • *Elektra Papadopoulos, MD, MPH* – Acting Associate Director, Clinical Outcome Assessments (COA) Staff, OND, CDER, FDA • *Jeff A. Sloan, PhD* – Professor of Biostatistics and Oncology, Mayo Clinic **Q & A**
Noon – 1:00 pm	**Lunch – Elm I, Elm II and Magnolia Ballroom**

1:00–2:30 pm	**Session 3: Existing Options for Assessing Patient-Reported Physical Function (PF)** **Chair:** *Paul Kluetz* **Presentations:** • **Importance of Physical Function in Cancer Patient Assessment:** *Ethan Basch, MD, MSc* – Director, Cancer Outcomes Research Program, University of North Carolina • **Introduction to Patient-Reported PF Measures:** *Selena R. Daniels, PharmD, MS* – Reviewer and Acting Team Lead, COA Staff, OND, CDER, FDA • **Assessing Patient-Reported Physical Function Using EORTC QLQ-C30:** *Mogens Groenvold, MD, PhD, DMSc* – Professor of Palliative Care and Quality of Life Assessment, University of Copenhagen and Bispebjerg Hospital • **The Patient-Reported Outcome Measurement Information System (PROMIS®) PF Item Bank and Efforts to Create a Common Physical Function Metric:** *David Cella* **Panel Discussion:** ***Assessing Physical Function in Cancer Clinical Trials*** **Additional Panelists:** • *Wen-Hung Chen, PhD* – Reviewer, Study Endpoints, Study Endpoints and Labeling Development, OND, CDER, FDA • *Daniel O'Connor* • *Ashley Wilder Smith, PhD, MPH* – Chief, Outcomes Research Branch, NCI **Q & A**
2:30–3:00 pm	**Break**

3:00–4:30 pm	**Session 4: Physical Function (PF) Data in Cancer Trials: Data Collection, Analysis, and Interpretation** **Chair:** *Laura Lee Johnson, PhD* – Associate Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences (OTS), CDER, FDA **Presentations:** • **An FDA Example of PF Analysis:** *Marian Strazzeri, MS* – Mathematical Statistician, Office of Biostatistics, CDER, FDA • **An EORTC QLQ-C3O Example of PF Analysis, Presentation, and Interpretation:** *Andrew Bottomley* • **A PROMIS® Example of PF Analysis, Presentation, and Interpretation:** *Roxanne E. Jensen, PhD* – Assistant Professor of Oncology, Cancer Prevention and Control Program, Lombardi Comprehensive Cancer Center, Georgetown University • **An Industry Perspective on PF Measures as Endpoints:** *Katarina Halling, MSc* –Global Head Patient Reported Outcomes, AstraZeneca **Panel Discussion:** ***Considerations Regarding Assessment, Analysis, and Presentation of Patient-Reported PF in Cancer Trials*** **Additional Panelists:** • *Ashley Wilder Smith* • *Jeff Sloan* • *Rajeshwari (Raji) Sridhara, PhD* – Division Director, Division of Biometrics V, Office of Biometrics, FDA • *Paul Kluetz* **Q & A**
4:30 – 5:00 pm	**Wrap Up and Adjourn** Paul Kluetz and Stephen Coons