CRITICAL PATH INSTITUTE

Seventh Annual Patient-Reported Outcome Consortium Workshop

Partners in Progress: Sharing the Vision, Shaping the Future

April 27 – 28, 2016

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

On April 27-28, 2016 the SEVENTH ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP was held in Silver Spring, Maryland. The overall Workshop objectives were to:

• Provide updates on FDA's Clinical Outcome Assessment (COA) Qualification Program and COA Compendium
• Discuss progress made within the PRO Consortium, with particular focus on the development of the Symptoms of Major Depressive Disorder Scale (SMDDS) by the Depression Working Group
• Discuss ways of generating evidence to support the content validity of performance outcome (PerfO) measures used to assess efficacy endpoints in clinical trials
• Provide examples of the development and implementation of COA tools in pediatric clinical trials
• Describe NCI's Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE) and some of the methodological considerations for its implementation in trials
• Build on last year's workshop presentation titled Interpreting Change in Scores on COA Endpoint Measures by discussing emerging approaches to estimating meaningful change on COA (particularly PRO) measures

The following Workshop Agenda provides an overview of the day-and-a-half-long meeting as well as links to the slide sets and posters presented.

Request Session Recordings

Workshop Agenda – Day 1
April 27, 2016

7:30 – 8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**

	Day 1 Morning Moderator: *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, Clinical Outcome Assessments (COA) Qualification Program, COA Staff, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
8:30 – 8:50 am	**Welcome and Patient-Reported Outcome Consortium Update***Stephen Joel Coons, PhD* – Executive Director, Patient-Reported (PRO) Outcome Consortium, Critical Path Institute
8:50 – 10:20 am	**Session 1: Update from FDA Regarding the Clinical Outcome Assessment Qualification Program and the COA Compendium****Moderator**: *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA) **Presenters**: *Elektra Papadopoulos, MD, MPH* – Acting Associate Director, COA Staff, OND, CDER, FDA *Virginia Kwitkowski, MS, ACNP-BC* – Associate Director for Labeling, Division of Hematology Products, Office of Hematology and Oncology Products (OHOP), OND, CDER, FDA **Q & A**
10:20 – 10:45 am	**Break – 25 min**
10:45 – 12:15 pm	**Session 2: Assessing the Content Validity of Performance Outcome (PerfO) Measures****Moderator**: *Elizabeth (Nicki) Bush, MHS* – Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company **Presenters**: *Rachel Ballinger, PhD* – Lead Outcomes Researcher, Clinical Outcome Assessment, ICON Clinical Research *J. Scott Andrews, PharmD* – Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company *Richard S.E. Keefe, PhD* – Professor of Psychiatry, Psychology, and Neuroscience, Duke University Medical Center and CEO, NeuroCog Trials, Inc. **Panelists**: *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA *Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute *Billy Dunn, MD* – Director, Division of Neurology Products, OND, CDER, FDA **Q & A**
12:15 – 1:15 pm	**Lunch – Elm I, Elm II and Magnolia Ballroom**

	Day 1 Afternoon Moderator: *Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
1:15 – 2:45 pm	**Session 3: Addressing Key Challenges in Developing, Testing and Implementing Clinical Outcome Assessments in Pediatric Trials** **Moderator**: *Linda Abetz-Webb* – Paediatric PRO Expert, CEO/Senior Research Director, Patient-Centered Outcome Assessments, Ltd. (P-COA) **Presenters**: *Linda Lowes, PT, PhD* – Clinical Therapies Research Director, Nationwide Children's Hospital *Rob Arbuckle* – Vice President and UK Managing Director, Patient-Centered Outcomes, Adelphi Values *Valdo Arnera, MD* – Scientific Advisor and General Manager ERT Geneva, ERT **Panelists**: *Laura Lee Johnson, PhD* – Associate Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences (OTS), CDER, FDA *Andrew E. Mulberg, MD, FAAP, CPI* – Division Deputy Director, Gastroenterology and Inborn Errors Products, OND, CDER, FDA *Josephine Norquist, MS* – Patient-Reported Outcome (PRO) & Study Endpoint Group Lead, Center for Observational and Real-world Evidence (CORE), Merck Sharp & Dohme, Corp. *Anna Rydén, PhD* – Director, Patient Reported Outcomes, AstraZeneca **Q & A**
2:45 – 3:10 pm	**Break – 25 min**

3:10 – 4:40 pm	**Session 4: NCI's Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE): Selecting Items and Assessment Frequency for Cancer Trials** **Moderator**: *Sandra A. Mitchell, PhD, CRNP* – Research Scientist and Program Director, Outcomes Research Branch, National Cancer Institute (NCI) **Presenters**: *Paul G. Kluetz, MD* – Associate Director of Clinical Science, OHOP, OND, CDER, FDA *Lori Minasian, MD, FACP* – Deputy Director, Division of Cancer Prevention, NCI *Ethan Basch, MD, MSc* – Director, Cancer Outcomes Research Program, University of North Carolina *Katarina Halling, MSc* – Global Head Patient Reported Outcomes, AstraZeneca and Industry Co-Director, PRO Consortium **Panelist**: *Selena R. Daniels, PharmD, MS* – Reviewer and Acting Team Lead, COA Staff, OND, CDER, FDA **Q & A**
4:40 – 4:55 pm	**A Review of FDA PRO Labeling (2011 – 2015)** *Ari Gnanasakthy, MSc, MBA* – Head, Patient Reported Outcomes, RTI Health Solutions
4:55 – 5:00 pm	**Day 1 Closing Remarks** **Adjourn**
5:30 – 7:00 pm	**Reception and Poster Session – Magnolia Ballroom**

Workshop Agenda – Day 2
April 28, 2016

7:30 – 8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**
	Day 2 Moderator: *Katarina Halling, MSc* – Global Head Patient Reported Outcomes, AstraZeneca and Industry Co-Director, Patient-Reported Outcome Consortium

8:30 – 10:00 am	**Session 5: Developing the Symptoms of Major Depressive Disorder Scale (SMDDS): From Patient Input to Final Instrument** **Moderator**: *Elizabeth (Nicki) Bush, MHS* – Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company **Presenters**: *Elizabeth (Nicki) Bush, MHS* – Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company *Kelly P. McCarrier, PhD, MPH* – Senior Research Scientist, Health Research Associates, Inc. *Donald Bushnell, MA* – Associate Director, Health Research Associates, Inc. *Valdo Arnera, MD* – Scientific Advisor and General Manager ERT Geneva, ERT **Panelists**: *Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute *Tiffany R. Farchione, MD* – Deputy Director, Division of Psychiatry Products (DPP), CDER, FDA **Q & A**
10:00 – 10:25 am	**Break – 25 min**
10:25 – 11:55 am	**Session 6: Expanding Our Understanding of Meaningful Change from a Patient Perspective** **Moderator**: *Cheryl D. Coon, PhD* – Principal, Outcometrix **Presenters**: *Mona Martin, RN, MPA* – Executive Director, Health Research Associates, Inc. *Allison Martin Nguyen, MS* – Senior Principal Scientist, Patient-Reported Outcomes & Study Endpoints, Merck Research Laboratories, Merck & Co., Inc. *Katarina Halling, MSc* – Global Head Patient Reported Outcomes, AstraZeneca and Industry Co-Director, PRO Consortium *Karon F. Cook, PhD* – Research Professor, Department of Medical Social Sciences, Feinberg School of Medicine, Northwestern University **Panelists**: *Wen-Hung Chen, PhD* – Reviewer, COA Staff, OND, CDER, FDA *Tara Symonds, PhD* – Strategic Lead, Clinical Outcomes Assessments, Clinical Outcomes Solutions *Kathleen (Kathy) Wywrich, PhD* – Vice President, Outcomes Research, Evidera **Q & A**
11:55 – 12:15 pm	**Closing Remarks** **Adjourn**