CRITICAL PATH INSTITUTE

Eighth Annual Patient-Reported Outcome Consortium Workshop

April 26 – 27, 2017

Hyatt Regency Bethesda,
1 Bethesda Metro Center,
Bethesda, MD 20814

On April 26-27, 2017 the EIGHTH ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP was held in Bethesda, Maryland. The overall Workshop objectives were to:

• Provide an update on FDA's Clinical Outcome Assessment (COA) Qualification Program;
• Describe progress within the PRO Consortium, with focused attention on the development of the *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire* (*NSCLC-SAQ*) by the NCSLC Working Group;
• Discuss perceived barriers to the adoption of electronic collection of COA-based endpoint data in clinical trials along with potential solutions;
• Describe existing measurement gaps and challenges associated with the collection of COA-based endpoint data in pediatric treatment trials and explore an innovative assessment approach for childhood asthma;
• Compare and contrast approaches to generating scores from PRO measures and discuss the Asthma Working Group's *Asthma Daily Symptom Diary* (*ADSD*) as an example of one approach; and
• Having started with the end in mind, describe the process of getting from clinical outcome assessment to clinical trial endpoint to medical product labeling to direct to consumer advertising.

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

Agenda – Day 1

7:30 – 8:30 am	**Registration and Continental Breakfast – Outside Regency I and II in Foyer**
	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 Outcome (PRO) Consortium, Critical Path Institute (C-Path)
8:50 – 10:20 am	**Session 1: Update from FDA Regarding the Clinical Outcome Assessment Qualification Program** **Moderator**: *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA **Presenters**: *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, Oncology Center of Excellence (OCE), FDA *Theresa M. Mullin, PhD* – Director of Office of Strategic Programs, CDER, FDA *Elektra Papadopoulos, MD, MPH* – Associate Director, COA Staff, OND, CDER, FDA **Q & A**
10:20 – 10:45 am	**Break – 25 min**
10:45 – 12:15 pm	**Session 2: Case Study: The *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)*Moderator**: *Kendra DeBusk, PhD* – Principal Outcomes Research Scientist, Genentech, Inc. **Presenters**: *Astra Liepa, BS, PharmD* – Principal Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company *Kelly McCarrier, PhD, MPH* – Senior Research Scientist, Health Research Associates, Inc. *Thomas Atkinson, PhD* – Assistant Attending Behavioral Scientist, Memorial Sloan Kettering Cancer Center *Donald Bushnell, MA* – Associate Director, Health Research Associates, Inc. **Panelists**: *Paul G. Kluetz, MD* – Acting Associate Director of Patient Outcomes, OCE, FDA *Stephen Joel Coons, PhD* – Executive Director, PRO Consortium, C-Path **Q & A**
12:15 – 1:15 pm	**Lunch – Old Georgetown, Congressional, Cabinet, and Judiciary Rooms**
	Day 1 Afternoon Moderator: *Sonya Eremenco, MA* – Associate Director, PRO Consortium, C-Path

1:15 – 2:45 pm	**Session 3: Barriers to Adoption of Electronic Collection of COA-based Endpoint Data in Clinical Trials** **Moderator**: *David S. Reasner, PhD* – Vice-President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals **Presenters**: *Bill Byrom, PhD* – Senior Director, Product Innovation, ICON Clinical Research *Alexandra I. Barsdorf, PhD* – Director, Rare Disease, Patient & Health Impact, Pfizer, Inc. *Kelly McQuarrie, BSN* – Director, PRO Team, Janssen Pharmaceuticals *Sue Vallow, RPh, MBA, MA* – Vice President, Patient eSolutions, MedAvante, Inc. *Marieke Manders, MSc* – GCDO Trial Leader, Immunology, Janssen Research & Development **Panelists**: *Serge Bodart, MS* – eCOA Subject Matter Expert, Biomedical Systems **Q & A**
2:45 – 3:10 pm	**Break – 25 min**
3:10 – 4:40 pm	**Session 4: Clinical Outcome Assessments in Pediatric Trials: Measurement Gaps, Challenges, and Potential Solutions** **Moderator**: *Sonya Eremenco, MA* – Associate Director, PRO Consortium, C-Path **Presenters**: *Mira Patel, MS* – Graduate Research Associate, PRO Consortium, C-Path *Sonya Eremenco, MA* – Associate Director, PRO Consortium, C-Path *Linda Nelsen, MHS* – Senior Director and Head, Patient Centered Outcomes, GlaxoSmithKline **Panelist**: *Linda Abetz-Webb* – Paediatric PRO Expert, CEO/Senior Research Director Patient-Centred Outcome Assessments, Ltd (P-COA) *Tonya Winders* – President and CEO, Allergy & Asthma Network *Susan McCune, MD* – Director, Office of Pediatric Therapeutics, Office of the Commissioner, FDA **Q & A**

4:40 – 4:55 pm	**COMPASS Update** *Stacie Hudgens, MA* – CEO and Strategic Lead, Quantitative Science, Clinical Outcomes Solutions
4:55 – 5:00 pm	**Day 1 Closing Remarks** **Adjourn**
5:30 – 7:00 pm	**Reception and Poster Session – Terrace** Asthma Working Group Cognition Working Group Depression Working Group Electronic Patient-Reported Outcome (ePRO) Consortium Functional Dyspepsia Working Group Irritable Bowel Syndrome (IBS) Working Group Multiple Sclerosis Working Group Myelofibrosis Working Group Non-Small Cell Lung Cancer (NSCLC) Working Group Pediatric Asthma Working Group Rheumatoid Arthritis Working Group

Agenda – Day 2

7:30 – 8:30 am	**Registration and Continental Breakfast – Outside Regency I and II in Foyer**
	Day 2 Moderator: *Elizabeth (Nicki) Bush, MHS* – Director, Patient Focused Outcomes Center of Expertise, Eli Lilly and Company and Industry Co-Director, PRO Consortium
8:30 – 10:00 am	**Session 5: What's the Score? Moving from Items to Scores – Methods, Considerations, and Case Examples** **Moderator**: *Steve Blum, MBA, MA* – Director, Patient-Reported Outcomes, GlaxoSmithKline **Presenters**:*Kathleen (Kathy) W. Wyrwich, PhD* – Senior Research Advisor, Eli Lilly and Company *Bryce B. Reeve, PhD* – Professor, University of North Carolina *Cheryl D. Coon, PhD* – Principal, Outcometrix **Panelists**: *Laura Lee Johnson, PhD* – Deputy Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA **Q & A**
10:00 – 10:25 am	**Break – 25 min**

10:25 – 11:55 am	**Session 6: From Clinical Outcome Assessment to Clinical Trial Endpoint to Medical Product Labeling** **Moderator**: *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA **Presenters**:*Ashley F. Slagle, MS, PhD* – Principal, Regulatory and Scientific Consulting, Aspen Consulting, LLC *David S. Reasner, PhD* – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals *Emily Edson Heredia, MPH* – Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company *Ari Gnanasakthy, MSc, MBA* – Principal Scientist, Patient-Centered Outcomes Assessments, RTI Health Solutions **Panelists**: *Wen-Hung Chen, PhD* – Reviewer COA Staff, OND, CDER, FDA *Ann Marie Trentacosti, MD* – Medical Lead, Labeling Development Team, CDER, FDA **Q & A**
11:55 – 12:15 pm	**Closing Remarks** **Adjourn**