Sixth Annual Patient-Reported Outcome Consortium Workshop

Partners in Progress: Sharing the Vision, Shaping the Future

April 29 – 30, 2015

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

The Patient-Reported Outcome (PRO) Consortium is a public-private partnership established by the Critical Path Institute (C-Path) in cooperation with the U.S. Food and Drug Administration (FDA) and the medical products industry in 2008. The PRO Consortium brings together scientists from C-Path, industry, academia, and regulatory agencies in a pre-competitive environment for the purpose of developing, evaluating, and qualifying PRO instruments for use as primary or secondary endpoint measures in clinical trials designed to evaluate treatment benefit.

On April 29-30, 2015 the **SIXTH ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP** was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Discuss how all stakeholders can work together to improve patient-focused drug development
- Provide updates on the PRO instrument development and qualification activities within the PRO Consortium's working groups
- Describe the qualitative and quantitative research that led to development of the *Asthma Daily Symptom Diary* (ADSD) within the Asthma Working Group
- Discuss the development and implementation of well-defined and reliable clinical outcome assessment (COA) tools for pediatric clinical trials
- Discuss ways to optimize qualitative and quantitative research to more efficiently generate evidence supporting the content validity of new PRO instruments

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 29, 2015

7:30-8:30 am	**Registration and Continental Breakfast – Cypress BallroomDay 1 Morning** **Moderator:** *Ashley F. Slagle, MS, PhD* – Clinical Outcome Assessment (COA) Qualification Scientific Coordinator and Endpoint Reviewer, Study Endpoints and Labeling Development (SEALD), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
8:30-8:45 am	**Welcome and Patient-Reported Outcome Consortium Update** *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)
8:45-9:45 am	**Session 1: Stakeholder Collaboration to Improve Patient-Centered Drug Development** **Moderator**: *Ashley F. Slagle, MS, PhD* – COA Qualification Scientific Coordinator and Endpoint Reviewer, SEALD, OND, CDER, FDA **Presenters and Panelists:** *Janet W. Maynard, MD, MHS* – Clinical Team Leader, Division of Pulmonary, Allergy, and Rheumatology Products (DPARP), OND, CDER, FDA *Elektra Papadopoulos, MD, MPH* – Acting Associate Director, Study Endpoints Team, SEALD, OND, CDER, FDA *Katarina Halling, MSc* – Patient Reported Outcomes Group Director, AstraZeneca and Industry Co-Director, PRO Consortium *Cynthia A. Bens* – Vice President, Public Policy, Alliance for Aging Research
9:45-10:10 am	**Session 2: COA Qualification and Study Endpoints Update** **Presenter:** *Ashley F. Slagle, MS, PhD* – COA Qualification Scientific Coordinator and Endpoint Reviewer, SEALD, OND, CDER, FDA
10:10-10:30 am	**Break – 20 min**
10:30-11:15 am	**Session 3 – The Asthma Working Group: On the Path to Success** **Moderator:** *Josephine M. Norquist, MS* – Patient-Reported Outcomes Specialist, Merck Sharp & Dohme, Corp **Presenters and Panelists:** *Linda Nelsen, MHS* – Director, Patient Reported Outcomes, GlaxoSmithKline *Adam Gater, MSc* – Director, Endpoint Development and Outcomes Assessment, Adelphi Values*Elektra* *Papadopoulos, MD, MPH – Acting Associate Director, Study Endpoints Team, SEALD, OND, CDER, FDA* **Q & A**

11:15-12:15 pm	**Session 4: Patient-Centric Endpoints in Oncology** **Moderator:** *Katarina Halling, MSc* – Patient Reported Outcomes Group Director, AstraZeneca and Industry Co-Director, PRO Consortium **Presenters and Panelists:** *Cindy Geoghegan* – Patient Advocate and Principal, Patient and Partners LLC *Paul G. Kluetz, MD* – Acting Deputy Director, Office of Hematology and Oncology Products (OHOP), OND, CDER, FDA *Ethan Basch, MD, MSc* – Director, Cancer Outcomes Research Program, University of North Carolina at Chapel Hill **Q & A**
12:15 – 1:15 pm	**Lunch – Elm I, Elm II and Magnolia Ballroom**
	Day 1 Afternoon Moderator: *Katarina Halling, MSc* – Patient Reported Outcomes Group Director, AstraZeneca and Industry Co-Director, PRO Consortium
1:15 – 2:15 pm	**Session 5: Optimizing Qualitative and Quantitative Research: How to Make the Process More Efficient** **Moderator**: J. Jason Lundy, PhD – Principal, Outcometrix **Presenters and Panelists:** *J. Jason Lundy, PhD* – Principal, Outcometrix *Stacie Hudgens, MA (AbD)* – Strategic Lead, Quantitative Science, Clinical Outcomes Solutions *R.J. Wirth, PhD* – Managing Partner, Vector Psychometric Group, LLC *Wen-Hung Chen, PhD* – Reviewer, Study Endpoints, SEALD, OND, CDER, FDA **Q & A**

2:15 – 3:15 pm	**Session 6: Interpreting Change in Scores on COA Endpoint Measures** **Moderator**: *Cheryl D. Coon, PhD* – Director, Healthcare Analytics, Adelphi Values **Presenters and Panelists:** *Joseph C. Cappelleri, PhD, MPH, MS* – Senior Director of Biostatistics, Pfizer Inc.*Cheryl D. Coon, PhD* – Director, Healthcare Analytics, Adelphi Values *Scott Komo, DrPH* – Senior Statistical Reviewer, Office of Biostatistics, CDER, FDA *Laura Lee Johnson, PhD* – Associate Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA **Q & A**
3:15 – 3:45 pm	**Break – 30 min**
3:45 – 4:45 pm	**Session 7: Thinking with the End in Mind: From COA Instrument to Endpoint** **Moderator**: *Jean Paty, PhD* – Principal Advisory Services, Quintiles **Presenters and Panelists:** *Paul G. Kluetz, MD* – Acting Deputy Director, OHOP, OND, CDER, FDA *David S. Reasner, PhD* – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals *Laura Lee Johnson, PhD* – Associate Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA *Elisabeth (Liz) Piault-Louis, PharmD, MA* – Principal Outcomes Research Scientist, Oncology Genentech, a member of the Roche Group **Q & A**
4:45 – 5:15 pm	**Day 1 Closing Remarks, Day 2 Preview** **Adjourn**
5:30 – 7:00 pm	**Reception and Poster Session – Magnolia Ballroom** **Asthma** **Cognition** **Depression** **Functional Dyspepsia** **Irritable Bowel Syndrome (IBS)** **Non-Small Cell Lung Cancer (NSCLC)**

Workshop Agenda – Day 2
April 30, 2015

7:30-8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**
8:30 – 9:00 am	**Session 8: Results from "Literature Review to Determine Empirical Basis for Response Scale Selection in Patient-Reported Outcome Instrument Development"** **Presenter:** *Katharine S. Gries, PharmD, PhD* – Senior Research Associate, Evidera **Q & A**
9:00 – 10:00 am	**Session 9: Practical Considerations in Implementing a Pediatric COA Measurement Strategy: A Case Study in Functional Constipation** **Moderator**: *Sarrit Kovacs, PhD* – Study Endpoints Reviewer, SEALD, OND, CDER, FDA **Presenters and Panelists:** *Andrew E. Mulberg, MD, FAAP, CPI* – Deputy Director, Division Gastroenterology and Inborn Errors Products (DGIEP), OND, CDER, FDA *Diane Turner-Bowker, PhD* – Engagement Leader I, Quintiles *Gina Calarco (Smith), MPH, RN, CCRC* – Associate Director and Deputy Head of the Pediatric Center of Excellence, Quintiles *Jean Paty, PhD* – Principal Advisory Services, Quintiles **Q & A**
10:00 – 10:30am	**Break – 30 min**
10:30 – 11:30 am	**Session 10: ePRO Science and Innovation: BYOD Approaches and Equivalence Across Administration Modalities** **Moderator**: Susan Vallow, RPh, MBA, MA – Senior Director, Patient Focused Outcomes, GlaxoSmithKline **Presenters and Panelists:** *Willie Muehlhausen, DVM* – Vice President, eCOA and Innovation, ICON plc *Chad Gwaltney, PhD* – Chief Scientist and Regulatory Advisor, Endpoints, ERT *Virginia (Gini) Kwitkowski, MS, RN, ACNP-BC* – Clinical Team Leader and Associate Director for Labeling, Division of Hematology Products, OHOP, OND, CDER, FDA *Cindy Howry, MS* – Vice President, Product Strategy and Innovation, YPrime and Vice Director, ePRO Consortium *Sheila Rocchio, MBA* – Vice President, Marketing and Strategy, PHT **Q & A**

11:30 – 12:15 pm	**Session 11: The Relevance of Patient-Reported Endpoints to Payers and Regulators: Is there Common Ground?** **Moderator**: *Peter C. Trask, PhD, MPH* – Principal Scientist, Patient Centered Outcomes Research, Genentech **Presenters and Panelists:** *David S. Reasner, PhD* – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals *Vasudha Bal, MSc, MBA* – Director, Patient Reported Outcomes, Novartis Pharmaceuticals Corporation *Katarina Halling, MSc* – Patient Reported Outcomes Group Director, AstraZeneca and Industry Co-Director, PRO Consortium *Selena R. Daniels, PharmD, MS* – Study Endpoints Reviewer, SEALD, OND, CDER, FDA *Alan L. Shields, PhD* – Vice President, Endpoint Development and Outcomes Assessment, Adelphi Values *Robin S. Turpin, PhD* – Director and Head, HEOR, U.S. Medical and Scientific Affairs, Takeda Pharmaceuticals, USA **Q & A**
12:15-12:30 pm	**Closing Remarks**
12:30 pm	**Adjourn**

Copies of the Workshop's slide presentations will be available on the Critical Path Institute's website (http://www.c-path.org/PRO.cfm) after May 30, 2015.