Fifth Annual Patient-Reported Outcome (PRO) Consortium Workshop

Honoring the Past, Navigating the Present, Charting the Future

April 29 – 30, 2014

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

CO-SPONSORED BY:
Critical Path Institute
U.S. Food and Drug Administration

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, 2014 the **FIFTH ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP** was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Provide updates on the PRO instrument development and qualification activities within the PRO Consortium's working groups and beyond
- Discuss lessons learned regarding the practical, scientific, and regulatory issues surrounding the PRO instrument development and qualification process within a collaborative, pre-competitive, multi-stakeholder context
- Discuss the development and implementation of well-defined and reliable clinical outcome assessment (COA) tools for pediatric clinical trials in asthma
- Discuss the technical, scientific, and regulatory issues impacting the planning and implementation of electronic data collection of PRO endpoints in clinical trials

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, 2014

7:30-8:30 am	**Registration and Continental Breakfast – Cypress Grand BallroomDay 1 Morning** **Moderator:** *Ashley F. Slagle, MS, PhD* – COA Qualification Scientific Coordinator and Endpoint Reviewer, Study Endpoints and Labeling (SEALD), 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 PRO Consortium Update** *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)
8:55-10:25 am	**Session 1: Advancing the Assessment of Outcomes Meaningful to Patients in Drug Development: A Brief History at the FDA and Beyond** **Moderator:** *Stephen Joel Coons, PhD* – Executive Director, PRO Consortium, C-Path **Presenters and Panelists:** *Robert Temple, MD* – Deputy Center Director for Clinical Science and Acting Deputy Director of the Office of Drug Evaluation I, CDER, FDA*Catherine Acquadro, MD* – Scientific Advisor, Mapi Research Trust*Donald L. Patrick, PhD, MSPH* – Professor and Director, Seattle Quality of Life Group, University of Washington *Andrew E. Mulberg, MD, FAAP, CPI* – Deputy Director, Division Gastroenterology and Inborn Error Products (DGIEP), OND, CDER, FDA*Tara Symonds, PhD* – PRO Lead, Pfizer, Ltd.*Laurie Beth Burke, RPh, MPH* – Founder, LORA Group, LLC **Q & A**
10:25-10:50 am	**Break – 25 min**
10:50-11:20 am	**FDA Update on DDT Qualification Program** *ShaAvhrée Buckman-Garner, MD, PhD, FAAP* – Director, Office of Translational Sciences, CDER, FDA
11:25-12:25 pm	**Session 2 – Update on Clinical Outcome Assessment Qualification Program** **Moderator:** *Ashley F. Slagle, MS, PhD* – COA Qualification Scientific Coordinator and Endpoint Reviewer, SEALD, OND, CDER, FDA **Presenters:** *Nancy Kline Leidy, PhD* – Senior Vice President, Scientific Affairs; Senior Research Leader, Evidera, Inc.**Q & A**
12:25-1:25 pm	**Lunch – Magnolia Ballroom**

	Day 1 Afternoon Moderator: *Risa Hayes, PhD* – Research Advisor, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company
1:30-2:30 pm	**Session 3 – Lessons Learned from PRO Consortium Along the Path to PRO Instrument Qualification: A Case Study** **Moderator**: *Risa Hayes, PhD* – Research Advisor, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company **Presenters and/or Panelists:** *Enkeleida Nikaï, MSc, MB* – Senior Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company*April N. Naegeli, DrPH, MPH* – Senior Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company *Clifton O. Bingham III, MD* – Associate Professor of Medicine, Divisions of Rheumatology and Allergy and Clinical Immunology at Johns Hopkins University; Director, Johns Hopkins Arthritis Center *Amye L. Leong, MBA* – President and CEO, Healthy Motivation; Director of Strategic Relations, Bone and Joint Decade, the Global Alliance for Musculoskeletal Health *Lee S. Simon, MD, FACP, FACR* – Principal, SDG LLC *Ashley F. Slagle, MS, PhD* – COA Qualification Scientific Coordinator and Endpoint Reviewer, SEALD, OND, CDER, FDA *Sarah Yim, MD* – Supervisory Associate Director, Division of Pulmonary, Allergy, and Rheumatology Products (DPARP), OND, CDER, FDA **Q & A**
2:35-3:35 pm	**Session 4 – Social/Digital Media: The Future of Qualitative Data Collection in the Context of Labeling** **Moderator**: *Margaret Rothman, PhD* – Senior Director, PRO Group, Janssen Pharmaceutical Companies of Johnson and Johnson **Presenters and/or Panelists:** *Trena M. Paulus, PhD* – Associate Professor and Coordinator of the Graduate Certificate in Qualitative Research Methods, Department of Educational Psychology and Counseling, University of Tennessee *Paul Wicks, PhD* – Vice President of Innovation, PatientsLikeMe *Elektra Papadopoulos, MD, MPH* – Team Leader, Study Endpoints Team, SEALD, OND, CDER, FDA **Q & A**
3:35-3:55 pm	**Break – 20 min**

3:55-5:15 pm	**Session 5 – Developing Pediatric COA Measurement Strategy: A Case Study in Asthma** **Moderator**: *Elisabeth Piault-Louis, PharmD, MA* – Senior PRO Scientist, Genentec0h **Presenters and/or Panelists:** *Linda Nelsen, MHS* – Director, Patient Reported Outcomes, GlaxoSmithKline*Louis Matza, PhD* – Senior Research Scientist, Evidera *Elektra Papadopoulos, MD, MPH* – Team Leader, Study Endpoints Team, SEALD, OND, CDER, FDA *Rob Arbuckle, MA, MSc* – Director, Endpoint Development & Outcomes Assessment, Adelphi Values *Laura Lee Johnson, PhD* – Statistician, National Center for Complementary and Alternative Medicine (NCCAM), National Institutes of Health (NIH) *Andrew E. Mulberg, MD, FAAP, CPI* – Deputy Director, DGIEP, OND, CDER, FDA *Hari Cheryl Sachs, MD* – Pediatric Team Leader, Pediatric and Maternal Health Staff, OND, CDER, FDA **Q & A**
5:15-5:30 pm	**Day 1 Closing Remarks, Day 2 Preview** **Adjourn**
6:00-7:30 pm	**Reception and Poster Session – Magnolia Ballroom**Asthma Cognition Depression Functional Dyspepsia Irritable Bowel Syndrome (IBS) Non-Small Cell Lung Cancer (NSCLC) Rheumatoid Arthritis

Workshop Agenda – Day 2
April 30, 2014

7:30-8:30 am	**Registration and Continental Breakfast – Cypress Grand Ballroom**
8:30-8:35 am	**Day 2 Moderator**: *Ari Gnanasakthy, MSc, MBA* – Co-Director, PRO Consortium Patient Reported Outcomes, Novartis Pharmaceuticals Corporation

8:40-9:40 am	**Session 6 – C-Path Collaborative Efforts to Address Specific ePRO Data Colle Challenges** **Moderator:** *Linda S. Deal, MS* – Head of Patient-Reported Outcomes, Gastrointes Business Unit Lead, Shire **Presenters and/or Panelists:** *Paul O'Donohoe, BSc* – Director of Health Outcome Health*Sarah Fleming, MPH* – Manager, Patient Reported Outcomes, Janssen Globa *Alexandra* I. *Barsdorf, PhD* – Associate Director, PRO Center, Pfizer, Inc. *Ari Gnanasakthy, MSc, MBA* – Co-Director, PRO Consortium and Head, Patient R Outcomes, Novartis Pharmaceuticals Corporation *Jonathan S. Helfgott, MS* – Associate Director for Risk Science (Acting), Office of Investigations, CDER, FDA *Cindy Howry, MS* – Vice Director, ePRO Consortium and Vice President, eCOA/el Product Strategy, Y-Prime **Q & A**
9:40-10:10 am	**Break – 25 min**
10:10-11:10 am	**Session 7 – Sponsor Infrastructure, Resources and Roles/Positions Needed to S Successful Execution of ePRO/eCOA Strategies** **Moderator:** *Sue Vallow, RPh, MBA, MA* – Senior Director, Patient Reported Outc GlaxoSmithKline **Presenters and/or Panelists:** *Sue Vallow, RPh, MBA, MA* – Senior Director, Patie Outcomes, GlaxoSmithKline*Kathryn Engstrom* – Data Scientist – Auto Immune, E Company *Jonathan S. Helfgott, MS* – Associate Director for Risk Science (Acting), Office of Investigations, CDER, FDA *Valdo Arnera, MD* – General Manager, PHT Corporation Europe *Linda S. Deal, MS* – Head of Patient-Reported Outcomes, Gastrointestinal Busines Shire *Jason Eger* – Vice-President, Project Management, ERT *Sarah Fleming, MPH* – Manager, Patient Reported Outcomes, Janssen Global Serv **Q & A**

11:15-12:15 pm	**Session 8 – ePRO: Bring Your Own Device (BYOD) – Application in Clinical** **Moderator**: *Jason Lundy, PhD* – Director, ePRO Consortium; Associate Director Consortium, C-Path **Presenters and/or Panelists:***Hannah O'Gorman* – ePRO Specialist, Exco InTouch *Symonds, PhD* – PRO Lead, Pfizer, Ltd. *Jonathan S. Helfgott, MS* – Associate Director for Risk Science (Acting), Office of Investigations, CDER, FDA *Willie Muehlhausen, DVM* – Vice President, Head of Innovation, ICON plc **Q & A**
12:15-12:30 pm	**Closing Remarks** **Adjourn**