Third Annual Patient-Reported Outcome Consortium Workshop

April 4, 2012

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

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Critical Path Institute
Food and Drug Administration (FDA)

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 4, 2012 the **THIRD ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP** was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Discuss the methodological advantages of a mixed methods (qualitative and quantitative) approach to ensuring content validity during the PRO instrument development process
- Discuss the crucial need for well-defined and reliable clinical outcome assessment tools for pediatric clinical trials
- Discuss regulatory issues surrounding PRO assessment using electronic data collection technologies
- Provide updates on the ongoing PRO instrument development activities within the Consortium's working groups
- Describe the status of the FDA's drug development tools (e.g., PRO instruments) qualification program
- Examine challenges and best practices in the implementation of electronic PRO data collection (ePRO) in clinical trials
- Discuss the selection of the appropriate recall period for PRO endpoint measures
- Explore the role of PRO endpoints in oncology 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.

Workshop Agenda – Day 1
April 4, 2012

7:30-8:30 am	**Registration and Continental Breakfast**
Morning Session	**Moderator:** Nicholas Greco IV, MS, BCETS, CATSM — Clinical Research Manager — Psychometrics and Assessment, Abbott Laboratories
8:30-8:45 am	**Welcome and PRO Consortium Update** *Stephen Joel Coons, PhD* — Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
8:45-10:05 am	**Panel Discussion 1** Perspectives on Decision-making in the Early Stages of Instrument Development **Moderator:** Laurie Beth Burke, RPh, MPH — Director, Study Endpoints and Labeling Development (SEALD), Office of New Drugs, Immediate Office (ONDIO), Center for Drug Evaluation and Research (CDER), FDA**Panelists:** Richard S. Levy, MD — Executive Vice President, Chief Drug Development and Medical Officer, Incyte Corp Debra Silberg MD, PhD — Senior Director Clinical Medicine, Shire Vibeke Strand, MD, FACP, FACR — Clinical Professor, Adjunct, Division of Immunology and Rheumatology, Stanford University Josephine Norquist, MS — Patient-Reported Outcome Specialist, Merck Sharp & Dohme, Corporation **FDA Response:** Marc K. Walton, MD, PhD — Associate Director for Translational Medicine, Office of Translational Sciences (OTS), CDER, FDA Laurie Beth Burke, RPh, MPH
10:05-10:25 am	**Break**
10:25-11:25 am	**Panel Discussion 2** Mixed Methods Approach to Assuring Content Validity **Moderator:** J. Jason Lundy, PhD — Assistant Director, PRO Consortium, C-Path **Panelists:** Jeremy Hobart, PhD, FRCP — Professor of Clinical Neurology and Health Measurement, Peninsula College of Medicine and DentistryJoseph C. Cappelleri, PhD, MPH — Senior Director, Biostatistics, Pfizer Inc. Ron D. Hays, PhD — Professor, Department of Medicine, David Geffen School of Medicine, UCLA **FDA Response:** James P. Stansbury, PhD, MPH — Consumer Safety Officer, SEALD, ONDIO, CDER, FDA

11:30-11:50 am	Update on FDA's Drug Development Tools (DDT) Qualification Program ShaAvhrée Buckman, MD, PhD, FAAP — Director, OTS, CDER, FDA
11:50-Noon	**Morning Session Wrap-up**
Noon-1:00 pm	**Lunch – Grant and Lincoln Rooms**
Afternoon Session	**Moderator:** Richard L. Barron, MS — Director, Global Health Economics, Amgen
1:00-2:00 pm	**Panel Discussion 3** Electronic Capture of Patient-Reported Outcome (ePRO) Data in Clinical Trials: Regulatory Considerations **Moderator:** Jay D. Pearson, PhD — Senior Director, Epidemiology, Merck Research Laboratories **Panelists:** Barbara Marino, PhD, RN — Senior Scientist, Director of Outcomes and Study Design, PHT CorporationDavid S. Reasner, PhD — Vice President, Data Science – North America — Sunovion Pharmaceuticals J. Jason Lundy, PhD — C-Path **FDA Response:** Sean Y. Kassim, PhD — Pharmacologist, Office of Compliance, CDER, FDA
2:05-3:20 pm	**Panel Discussion 4** Selection and Development of Clinical Outcome Assessments (COAs) for Use in Pediatric Clinical Trials **Moderator:** Melissa S. Tassinari, PhD, DABT — Senior Clinical Analyst, Pediatric and Maternal Health Staff, OND, CDER, FDA **Panelists:** Paul Wang, MD — Vice President, Clinical Development, Seaside Therapeutics, Inc.Linda Abetz-Webb, MA — Senior Director (Vice President), PRO Practice Lead-Europe, Adelphi Values Donald Patrick, PhD, MSPH — Professor, University of Washington Diana Rofail, PhD, MBPSs — Principal Patient-Reported Outcomes, CNS, Roche Products Ltd. **FDA Response:** Jessica J. Lee, MD — Medical Officer, Division of Gastroenterology and Inborn Error Products, CDER, FDA Elektra Papadopoulos, MD — Medical Officer, SEALD, CDER, FDA

3:20-3:40 pm	**Break**
3:40-3:50 pm	**Brief Update on PRO Consortium Working Groups** Asthma Cognition Depression Functional Dyspepsia Irritable Bowel Syndrome (IBS) Non-Small Cell Lung Cancer (NSCLC) Rheumatoid Arthritis
3:50-4:55 pm	**Panel Discussion 5** Lessons Learned: Challenges and Wins **Moderator:** Clarice (Risa) Hayes, PhD — Co-Director, Patient-Reported Outcome (PRO) Consortium, Research Advisor, Eli Lilly and Company **Panelists:** Asthma WG Linda Nelsen, MHS — Associate Director, Epidemiology, Merck Sharpe & Dohme, CorporationDepression WG Steven I. Blum, MBA — Director of Health Economics, Forest Research Institute Functional Dyspepsia WG Robyn T. Carson, MPH — Associate Director, Health Economics & Outcomes Research, Forest Research Institute IBS WG Mollie J. Baird, MPH — Associate Director, Patient Reported Outcomes Research and Development, Ironwood Pharmaceuticals NSCLC WG Rajiv Mallick, PhD — Director, Health Economics and Outcomes Research (HEOR), Daiichi Sankyo **FDA Response:** Laurie Beth Burke, RPh, MPH Marc K. Walton, MD, PhD
4:55-5:00 pm	**Closing Remarks & Adjourn**