Second Annual Patient-Reported Outcome Consortium Workshop

March 15, 2011

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

SPONSORED BY:
Critical Path Institute
Food and Drug Administration (FDA)

The Patient-Reported Outcome 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 in clinical trials designed to evaluate the safety and efficacy of medical products.

On March 15, 2011 the **SECOND ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP** was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Provide updates on PRO instrument development activities within the Consortium's working groups
- Discuss important lessons learned during the past year
- Describe the FDA's draft guidance on the qualification process for drug development tools (e.g., PRO instruments)
- Examine important methodological issues surrounding PRO instrument development and use 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.

Workshop Agenda
March 15, 2011

7:30-8:30 am	**Registration and Continental Breakfast**

Morning Session	**Moderator:** Stephen Joel Coons, PhD – Executive Director, PRO Consortium, Critical Path Institute
8:30-8:45 am	**Welcome and PRO Consortium Update** *Stephen Joel Coons, PhD*
8:45-9:55 am	**PRO Consortium Working Group Updates** **Irritable Bowel Syndrome:** Mollie Baird, MPH – Associate Director, Clinical Operations, Ironwood Pharmaceuticals **Cognition:** Christopher Leibman – Sr. Director: Health Economics/Market Access, Janssen Alzheimer Immunotherapy R&D, LLC **Asthma:** Linda Nelsen, MHS – Associate Director, Epidemiology, Merck, Sharp & Dohme Corp **Depression:** Nicholas Greco IV, M.S., BCETS, CATSM – Clinical Research Manager – Psychometrics and Assessment, Global Pharmaceutical Research & Development, Abbott Laboratories **Non-Small Cell Lung Cancer:** Ben Gutierrez, PhD – Director, Global Health Economic & Outcomes Research, Bristol-Myers Squibb **Functional Dyspepsia:** Robyn T. Carson, MPH – Assistant Director, HEOR, Forest Research Institute **Rheumatoid Arthritis:** Enkeleida Nikai, MSc Psych, M.B. – Senior Health Outcomes Manager, Global Market Access, UCB Pharma S.A.
9:55-10:15 am	**Break**
10:15-10:45 am	**Outcomes Targeted for Labeling – What Works and What Doesn't?** Laurie Beth Burke, RPh, MPH – Director, Study Endpoints and Labeling Development, Office of New Drugs, CDER, FDA and Marc Walton, MD – Associate Director for Translational Medicine, Office of Translational Sciences, CDER, FDA
10:45-11:00 am	**Lessons Learned – A Perspective from the Pharmaceutical Industry** Risa Hayes, PhD – Co-Director, PRO Consortium; Research Advisor, Global Health Outcomes, Eli Lilly and Company
11:00-11:20 am	**FDA/NIH Interagency Collaboration Involving PRO Measurement** Wendy R. Sanhai, PhD – Senior Scientific Advisor, Office of Chief Scientist, Office of the Commissioner, FDA
11:20-11:40 am	**Drug Development Tools Qualification Guidance** ShaAvhrée Buckman, MD, PhD, FAAP – Director, Office of Translational Sciences, CDER, FDA

11:40-11:55 am	**Proposed ePRO Consortium** J. Jason Lundy, PhD – Assistant Director, PRO Consortium, Critical Path Institute
11:55 am-Noon	**Morning Session Wrap-up**
Noon-1:00 pm	**Lunch** (buffet lunch in Chesapeake Ballroom)
Afternoon Session	**Moderator:** Risa Hayes, PhD
1:00-2:00 pm	**Panel Discussion 1** **To Combine or Not Combine: Individual Symptom Scores Versus Symptom Summary Scores** **Moderator:** Margaret L. Rothman, PhD – Senior Director, Worldwide Market Access, Patient-Reported Outcomes, Johnson & Johnson Pharmaceutical Services, LLC **Panelists:** Charles S. Cleeland, PhD – McCullough Professor of Cancer Research and Chair, Department of Symptom Research, MD Anderson Cancer Center; Donald L. Patrick, PhD, MSPH – Professor and Director, Seattle Quality of Life Group, University of Washington; Ruyi He, MD – Medical Team Leader, Div. of Gastroenterology Products, CDER, FDA; Rima Izem, PhD – Mathematical Statistician, Office of Biostatistics/Division of Biometrics 4, CDER, FDA
2:00-3:00 pm	**Panel Discussion 2** **Identifying Optimal Recall Period for Measuring Specific Concepts** **Moderator:** Josephine M. Norquist, MS – Patient-Reported Outcomes (PROs) Specialist, Dept. of Epidemiology, Merck Sharpe & Dohme, Corp. **Panelists:** Arthur A. Stone, PhD – Distinguished Professor and Vice-Chair, Dept. of Psychiatry & Behavioral Science, Stony Brook University; Dennis A. Revicki, PhD – Senior Vice President, Health Outcomes Research, United BioSource Corporation; Elektra Papadopoulos, MD, MPH – Endpoint Reviewer, Study Endpoints & Labeling Team, Office of New Drugs, CDER, FDA; Joseph G. Toerner, MD, MPH – Associate Director for Medical Affairs, Office of Antimicrobial Products, CDER, FDA
3:00-3:20 pm	**Break**

3:20-4:20 pm	**Panel Discussion 3** **Enhancing Interpretation of Patient-reported Outcomes:** Responder Analysis, Cumulative Distributions, and Regulatory Insights **Moderator:** David S. Reasner, PhD – Senior Vice President, Data Science – North America, Sunovion Pharmaceuticals Inc. **Panelists:** Kathleen W. Wyrwich – Senior Research Leader, United BioSource Corporation (UBC); Joseph C. Cappelleri, PhD, MPH – Senior Director, Biostatistics, Pfizer Inc.; Lisa A. Kammerman, PhD – Master Reviewer, Office of Biostatistics, CDER, FDA
4:20-4:50 pm	Open Panel Discussion ShaAvhrée Buckman, MD, PhD, FAAP Laurie Beth Burke, RPh, MPH Stephen Joel Coons, PhD Risa Hayes, PhD
4:50-5:00 pm	**Closing Remarks** Risa Hayes, PhD
5:00 pm	**Adjourn**

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