

# 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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**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	<b>Registration and Continental Breakfast</b>
Morning Session	<b>Moderator:</b> Nicholas Greco IV, MS, BCETS, CATSM — Clinical Research Manager — Psychometrics and Assessment, Abbott Laboratories
8:30-8:45 am	<b><u>Welcome and PRO Consortium Update</u></b> <i>Stephen Joel Coons, PhD</i> — Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
8:45-10:05 am	<b><u>Panel Discussion 1</u></b> <b><u>Perspectives on Decision-making in the Early Stages of Instrument Development</u></b> <b>Moderator:</b> 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  <b>Panelists:</b> 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  <b>FDA Response:</b> 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	<b>Break</b>

10:25-11:25 am	<p><b><u>Panel Discussion 2</u></b>  <u><a href="#">Mixed Methods Approach to Assuring Content Validity</a></u>  <b>Moderator:</b>  J. Jason Lundy, PhD — Assistant Director, PRO Consortium, C-Path</p> <p><b>Panelists:</b>  Jeremy Hobart, PhD, FRCP — Professor of Clinical Neurology and Health Measurement, Peninsula College of Medicine and Dentistry</p> <p>Joseph C. Cappelleri, PhD, MPH — Senior Director, Biostatistics, Pfizer Inc.</p> <p>Ron D. Hays, PhD — Professor, Department of Medicine, David Geffen School of Medicine, UCLA</p> <p><b>FDA Response:</b>  James P. Stansbury, PhD, MPH — Consumer Safety Officer, SEALD, ONDIO, CDER, FDA</p>
11:30-11:50 am	<p><u><a href="#">Update on FDA’s Drug Development Tools (DDT) Qualification Program</a></u>  ShaAvhrée Buckman, MD, PhD, FAAP — Director, OTS, CDER, FDA</p>
11:50-Noon	<p><b>Morning Session Wrap-up</b></p>
Noon-1:00 pm	<p><b>Lunch – Grant and Lincoln Rooms</b></p>
<b>Afternoon Session</b>	<p><b>Moderator:</b>  Richard L. Barron, MS — Director, Global Health Economics, Amgen</p>
1:00-2:00 pm	<p><b><u>Panel Discussion 3</u></b>  <u><a href="#">Electronic Capture of Patient-Reported Outcome (ePRO) Data in Clinical Trials: Regulatory Considerations</a></u>  <b>Moderator:</b>  Jay D. Pearson, PhD — Senior Director, Epidemiology, Merck Research Laboratories</p> <p><b>Panelists:</b>  Barbara Marino, PhD, RN — Senior Scientist, Director of Outcomes and Study Design, PHT Corporation</p> <p>David S. Reasner, PhD — Vice President, Data Science – North America — Sunovion Pharmaceuticals</p> <p>J. Jason Lundy, PhD — C-Path</p> <p><b>FDA Response:</b>  Sean Y. Kassim, PhD — Pharmacologist, Office of Compliance, CDER, FDA</p>

<p>2:05-3:20 pm</p>	<p><b><u>Panel Discussion 4</u></b>  <u><a href="#">Selection and Development of Clinical Outcome Assessments (COAs) for Use in Pediatric Clinical Trials</a></u>  <b>Moderator:</b>  Melissa S. Tassinari, PhD, DABT — Senior Clinical Analyst, Pediatric and Maternal Health Staff, OND, CDER, FDA</p> <p><b>Panelists:</b>  Paul Wang, MD — Vice President, Clinical Development, Seaside Therapeutics, Inc.</p> <p>Linda Abetz-Webb, MA — Senior Director (Vice President), PRO Practice Lead-Europe, Adelphi Values</p> <p>Donald Patrick, PhD, MSPH — Professor, University of Washington</p> <p>Diana Rofail, PhD, MBPSs — Principal Patient-Reported Outcomes, CNS, Roche Products Ltd.</p> <p><b>FDA Response:</b>  Jessica J. Lee, MD — Medical Officer, Division of Gastroenterology and Inborn Error Products, CDER, FDA</p> <p>Elektra Papadopoulos, MD — Medical Officer, SEALD, CDER, FDA</p>
<p>3:20-3:40 pm</p>	<p><b>Break</b></p>
<p>3:40-3:50 pm</p>	<p><b><u>Brief Update on PRO Consortium Working Groups</u></b></p> <p><u><a href="#">Asthma</a></u>  <u><a href="#">Cognition</a></u>  <u><a href="#">Depression</a></u>  <u><a href="#">Functional Dyspepsia</a></u>  <u><a href="#">Irritable Bowel Syndrome (IBS)</a></u>  <u><a href="#">Non-Small Cell Lung Cancer (NSCLC)</a></u>  <u><a href="#">Rheumatoid Arthritis</a></u></p>

<p>3:50-4:55 pm</p>	<p><b>Panel Discussion 5</b>  <a href="#">Lessons Learned: Challenges and Wins</a>  <b>Moderator:</b>  Clarice (Risa) Hayes, PhD — Co-Director, Patient-Reported Outcome (PRO) Consortium, Research Advisor, Eli Lilly and Company</p> <p><b>Panelists:</b>  Asthma WG Linda Nelsen, MHS — Associate Director, Epidemiology, Merck Sharpe &amp; Dohme, Corporation</p> <p>Depression WG Steven I. Blum, MBA — Director of Health Economics, Forest Research Institute</p> <p>Functional Dyspepsia WG Robyn T. Carson, MPH — Associate Director, Health Economics &amp; Outcomes Research, Forest Research Institute</p> <p>IBS WG Mollie J. Baird, MPH — Associate Director, Patient Reported Outcomes Research and Development, Ironwood Pharmaceuticals</p> <p>NSCLC WG Rajiv Mallick, PhD — Director, Health Economics and Outcomes Research (HEOR), Daiichi Sankyo</p> <p><b>FDA Response:</b>  Laurie Beth Burke, RPh, MPH</p> <p>Marc K. Walton, MD, PhD</p>
<p>4:55-5:00 pm</p>	<p><b>Closing Remarks &amp; Adjourn</b></p>