Fourth Annual Patient-Reported Outcome (PRO) Consortium Workshop

April 24 – 25, 2013

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 24-25, 2013 the **FOURTH ANNUAL PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP** was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Provide updates on the ongoing PRO instrument qualification activities within the PRO Consortium's working groups
- Describe the status of the FDA's drug development tool (e.g., PRO instrument) qualification program
- Discuss the use of a mixed methods approach (i.e., qualitative and quantitative research) to ensure content validity during the PRO instrument development process
- Explore the implications of the reauthorized Prescription Drug User Fee Act (PDUFA V) on the role of patients in the drug development process
- Discuss the critical need for well-defined and reliable clinical outcome assessment tools for pediatric clinical trials
- 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

Request Session Recordings

Workshop Agenda – Day 1
April 24, 2013

7:30-8:30 am	**Registration and Continental Breakfast Cypress Room**
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)
8:50-8:55 am	**Morning Moderator:** *Ari Gnanasakthy, MSc, MBA* — Co-Director, PRO Consortium and Head, Patient Reported Outcomes, Novartis Pharmaceuticals Corporation
8:55-9:30 am	**Workshop Kickoff and FDA Update on DDT Qualification Program** *ShaAvhrée Buckman, MD, PhD, FAAP* — Director, Office of Translational Sciences, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
9:35-10:25 am	**Session 1 – Patient Participation in Drug Development** **Patient Representation at FDA** *Richard Klein* — Director, Patient Liaison Program, Office of Health and Constituent Affairs, FDA
	FDA's Patient-Focused Drug Development Initiative *Theresa Mullin, PhD* — Associate Director, Office of Planning and Informatics, CDER, FDA
10:25-10:45 am	**Break – 20 min**
10:45-11:10am	**Session 2 – Target Product Profile (TPP) Process** *Michele L. Sharp, PharmD* — Senior Director, Global Regulatory Affairs – US Advertising, Promotion and Policy, Eli Lilly and Company

11:15-12:45 pm	**Panel Discussion 1 – Mixed Methods in Assuring Content Validity** *Mixed Methods – FDA Perspective: Incorporating Mixed Methods to Enhance Content Validity in Drug-Development Tools* **Moderator**: *Ashley F. Slagle, PhD, MS* — ORISE Fellow, Study Endpoints and Labeling Development (SEALD), Office of New Drugs (OND), CDER, FDA **Presenter/Panelist**: *James P. Stansbury, PhD, MPH* — Consumer Safety Officer, SEALD, OND, CDER, FDA **Panelists**: *Laurie B. Burke, RPh, MPH* — Associate Director, OND, SEALD, CDER, FDA; *Lisa Kammerman, PhD* — Master Reviewer, Office of Biostatistics, CDER, FDA; *Scott Komo, DrPH* — Senior Statistical Reviewer, Office of Biostatistics, CDER, FDA; *Päivi Miskala, MSPH, PhD* — Study Endpoints Reviewer/Senior Clinical Analyst, SEALD, OND, CDER, FDA *Mixed Methods – Industry and Academic Experience* **Moderator**: *Josephine M. Norquist, MS* — Patient-Reported Outcomes Specialist, Department of Epidemiology, Merck Sharp & Dohme Corporation **Presenters and Panelists**: *Joseph C. Cappelleri, PhD, MPH* — Senior Director, Biostatistics, Pfizer Inc.; *Ron D. Hays, PhD* – Professor, Department of Medicine, David Geffen School of Medicine, UCLA
12:45-1:45 pm	**Lunch Magnolia Room**
1:45-1:55 pm	**Afternoon Moderator**: *Abhilasha Ramasamy, BPharm, MSc, MS* — Senior Manager, Health Economics and Outcomes Research, Forest Research Institute

1:55-2:55 pm	**Panel Discussion 2 – Selection of Appropriate Recall Period** **Moderator**: *Steven I. Blum, MBA* — Director of Health Economics, Forest Research Institute **Presenter**: *Sheri E. Fehnel, PhD* — Vice President of Patient-Reported Outcomes, RTI-Health Solutions **Panelists**: *Robyn T. Carson, MPH* — Associate Director, Health Economics and Outcomes Research, Forest Research Institute; *Nicholas Greco IV, MS, BCETS, CATSM* — Clinical Research Manager – Psychometrics and Assessment, AbbVie, Inc.; *William Lenderking, PhD* — Senior Research Leader, United BioSource Corporation; *Linda Nelsen, MHS* — Senior Principal Scientist, Epidemiology, Merck Sharp & Dohme Corporation
2:55-3:15 pm	**Break – 20 min**

3:15-4:45 pm	**Panel Discussion 3 – Challenges in the Implementation of ePROs in Clinical Trials** **Moderator**: *Risa Hayes, PhD* — Lead Scientist for Patient-Reported Outcomes, Eli Lilly and Company **Presenters**: *Stephen Joel Coons, PhD* — C-Path*;* *Sonya L. Eremenco, MA* — Principal, ePRO Scientific Solutions, Bracket, a subsidiary of United BioSource Corporation; *Cindy Howry*, MS— Principal, ePRO Solutions, Bracket, a subsidiary of United BioSource Corporation **Panelists**: *Heather Blaudow* — Associate Consultant Research, Global Health Outcomes, Eli Lilly and Company*;* *Tim Davis* — CEO and Co-Founder, Exco InTouch; *Sarah Fleming, MPH* — Analyst, Patient Reported Outcomes, Janssen Global Services; *Alison Greene, MPH* — Senior Scientist, Patient Reported Outcomes, Genentech, a member of the Roche Group; *Cindy Howry, MS — Bracket*; *Wilhelm Muehlhausen*, DVM — Vice President, eCOA and Innovation, ICON and Vice-Director, ePRO Consortium; *Paul O'Donohoe* — Manager of Health Outcomes, CRF Health; *Jean Paty, PhD* — Chief Scientist and Regulatory Advisor, Outcomes, ERT Inc.; *Sheila Rocchio, MBA* — Vice President of Marketing & Product Management, PHT Corporation; *Eric Ross* — Associate Product Manager, Patient Management & ePRO, Almac Clinical Technologies; *Tara Symonds, PhD* — Senior Director, Global Head PRO Center of Excellence, Pfizer Ltd.
4:45-5:00 pm	**Day 1 Closing Remarks** Day 2 Preview
5:30-7:30 pm	**Reception and Working Group Update Poster Session – Magnolia Room** **Asthma** **Cognition** **Depression** **Functional Dyspepsia** **Irritable Bowel Syndrome (IBS)** **Non-Small Cell Lung Cancer (NSCLC)** **Rheumatoid Arthritis**

Workshop Agenda – Day 2
April 25, 2013

7:30-8:30 am	**Registration and Continental Breakfast Cypress Room**
8:30-8:40 am	**Day 2 Moderator**: *Risa Hayes, PhD* — Lilly
8:40-10:10 am	**Panel Discussion 4 – Decision-making to Include PRO Endpoints in Oncology Trials** **Moderator**: *Ari Gnanasakthy, MSc, MBA* — Novartis **Presenters/Panelists**: *Tom Simon* — FDA Patient Representative; *Virginia Kwitkowski, MS, RN, ACNP-BC* — Lead Clinical Analyst, Clinical Team Leader, Division of Hematology Products, CDER, FDA; *Patrick Marquis, MD* — Independent Consultant; *Margaret Rothman, PhD* — Senior Director, PRO Group, Janssen Pharmaceutical Companies of Johnson and Johnson **Panelists**: *Ethan Basch, MD, MSc* — Director, Cancer Outcomes Research Program, University of North Carolina at Chapel Hill; *Laurie B. Burke, RPh, MPH — FDA;* *Alicyn Campbell, MPH* — Global Head, Patient Reported Outcomes – Oncology, Genentech, a member of the Roche Group and co-chair of NSCLC WG;
10:10-10:30 am	**Break – 20 min**

10:30-11:50 am	**Panel Discussion 5 – Considerations for the Implementation of Clinical Outcome Assessments in Pediatric Drug Development Programs** **Moderator/Presenter**: *Melissa S. Tassinari, PhD, DABT* — Senior Clinical Analyst, Pediatric and Maternal Health Staff, OND, CDER, FDA **Presenters/Panelists**: *Omar Khwaja, MD, PhD* — Translational Medicine Leader, Neurosciences, F. Hoffmann-LaRoche AG*;* *Tiina Urv, PhD* — Health Scientist Administrator, Intellectual and Developmental Disabilities Branch, National Institute of Child Health & Human Development **Panelists**: *Nicholas Kozauer, MD* — Acting Clinical Team Leader, Division of Neurology Products (DNP), CDER, FDA; *Ranjit Mani, MD* — Medical Reviewer, DNP, CDER, FDA*;* *Elektra Papadopoulos, MD, MPH* — Endpoint Reviewer, SEALD, OND, CDER, FDA; *Diana Rofail, PhD, CPsychol* — Global Head of Patient-Reported Outcomes, CNS & Metabolism, Product Development, Biometrics, EpiPRO, Roche Products, Ltd.; *Juliana Setyawan, PharmD, MS* — Director in Global Health Economics and Outcomes Research/Epidemiology, Shire Development, LLC;
11:50-12:00 pm	**Closing Remarks**
12:00 Noon	**Adjourn**