

## 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

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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 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, multistakeholder 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
	<b>Moderator:</b> <i>Ashley F. Slagle, MS, PhD</i> – 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, FDACatherine Acquadro, MD – Scientific Advisor, Mapi Research TrustDonald 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, FDATara 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

	<b>Day 1 Afternoon Moderator:</b> <i>Risa Hayes, PhD</i> – 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
	<b>Presenters and/or Panelists:</b> <i>Enkeleida Nikaï, MSc, MB</i> – Senior Research Scientist, Global Patient Outcomes and Real World Evidence, Eli Lilly and Company <i>April N. Naegeli, DrPH, MPH</i> – 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 a 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 Ground 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

2.55.5.15	Session 5 – Developing Pediatric COA Measurement Strategy: A
3:55-5:15 pm	Case Study in Asthma
	<b>Moderator</b> : Elisabeth Piault-Louis, PharmD, MA – Senior PRO
	Scientist, Genentec0h
	<b>Presenters and/or Panelists:</b> <i>Linda Nelsen, MHS</i> – Director, Patient Reported Outcomes, GlaxoSmithKline <i>Louis Matza, PhD</i> – 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
	Reception and Poster Session – Magnolia BallroomAsthma
6:00-7:30 pm	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	<b>Day 2 Moderator</b> : <i>Ari Gnanasakthy, MSc, MBA</i> – Co-Director, PRO Consortium Patient Reported Outcomes, Novartis Pharmaceuticals Corporation

0.40.0.40	Session 6 – C-Path Collaborative Efforts to Address Specific ePRO Data Colle
8:40-9:40 am	<u>Challenges</u> <u>Moderator: Linda S. Deal, MS – Head of Patient-Reported Outcomes, Gastrointes Business Unit Lead, Shire</u>
	<b>Presenters and/or Panelists:</b> <i>Paul O'Donohoe, BSc</i> – Director of Health Outcome Health <i>Sarah Fleming, MPH</i> – Manager, Patient Reported Outcomes, Janssen Glob
	Alexandra I. Barsdorf, PhD – Associate Director, PRO Center, Pfizer, Inc.
	<i>Ari Gnanasakthy, MSc, MBA</i> – Co-Director, PRO Consortium and Head, Patient Routcomes, 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	Session 7 – Sponsor Infrastructure, Resources and Roles/Positions Needed to S
10:10-11:10 am	Successful Execution of ePRO/eCOA Strategies  Moderator: Sue Vallow, RPh, MBA, MA – Senior Director, Patient Reported Outo GlaxoSmithKline
	<b>Presenters and/or Panelists:</b> Sue Vallow, RPh, MBA, MA – Senior Director, Patier Outcomes, GlaxoSmithKlineKathryn 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 Business Shire
	Jason Eger - Vice-President, Project Management, ERT
	Sarah Fleming, MPH – Manager, Patient Reported Outcomes, Janssen Global Serv
	Q & A
	, the state of the

	Session 8 – ePRO: Bring Your Own Device (BYOD) – Application in Clinical
11:15-12:15 pm	Moderator: Jason Lundy, PhD – Director, ePRO Consortium; Associate Director
	Consortium, C-Path
	<b>Presenters and/or Panelists:</b> <i>Hannah O'Gorman</i> – ePRO Specialist, Exco InToucl <i>Symonds</i> , <i>PhD</i> – 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