Welcome and PRO Consortium Update

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Executive Director, PRO Consortium

Fifth Annual Patient-Reported Outcome (PRO) Consortium Workshop
April 29 - 30, 2014 ■ Silver Spring, MD

Co-sponsored by

CRITICAL PATH INSTITUTE
FDA
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Workshop Packet Contents

- Welcome Letter
- Workshop Agenda
- PRO Consortium Fact Sheet
- C-Path Information Sheet
- Presenters and Panelists Biographical Sketches
- Pre-Registrant List
- Workshop Feedback Form
PRO Consortium

Formed in late 2008 by the Critical Path Institute (C-Path), in cooperation with the FDA and the pharmaceutical industry

- **Membership**
  - 26 members (pharmaceutical firms) in 2014
    - Welcome to Bayer Pharma AG

- **Non-Voting Participants**
  - Representatives of governmental agencies
  - Clinical consultants, patients, academic researchers, and CROs partnering in the development of the PRO instruments
PRO Consortium Working Groups

Asthma – 12 member firms
Cognition – 7 member firms (2 additional pending)
Depression – 8 member firms
Functional Dyspepsia – 3 member firms
Irritable Bowel Syndrome (IBS) – 3 member firms
Lung Cancer (NSCLC) – 6 member firms
Rheumatoid Arthritis – 5 member firms (1 additional pending)
Of the 26 member firms...

- Three are in four working groups
- Three are in three working groups
- Seven are in two working groups
- Nine are in one working group
- Four are not participating in any current working groups
Goal of Working Groups

To produce and/or compile the necessary evidence to enable new or existing PRO instruments to be “qualified” by the FDA for use in clinical trials where PRO endpoints can be used to support product labeling claims.
During breaks in today’s Workshop, please view the seven working group posters at the back of the room. They will also be on display during the reception in the Magnolia Room from 6:00 pm – 7:30 pm this evening.
Asthma Working Group

Co-Chairs: Linda Nelsen (GlaxoSmithKline LLC) and Michelle Mocarski (Forest Research Institute)

Target population: Adults and adolescents with a clinical diagnosis of mild to severe persistent asthma

Measurement concept: Daytime and nighttime asthma symptoms

Role in endpoint hierarchy: Co-primary or secondary endpoint to establish or support treatment benefit
Co-Chairs: Julie Chandler (Merck Sharp & Dohme Corp) and Elisabeth Piault-Louis (Genentech, Inc.)

Target population: Adults with a clinical diagnosis of mild cognitive impairment (MCI) due to Alzheimer’s disease

Measurement concepts: interpersonal functioning and performance of complex activities of daily living

Role in endpoint hierarchy: Co-primary endpoint to establish or treatment benefit
Depression Working Group

Co-Chairs: Nicki Bush (Eli Lilly and Company) and Lucy Abraham (Pfizer, Inc.)

Target population: Adults with a clinical diagnosis of major depressive disorder

Measurement concepts: Symptoms of major depressive disorder

Role in endpoint hierarchy: Primary endpoint to establish treatment benefit
Co-Chairs: Robyn Carson (Forest Research Institute) and Linda Deal (Shire Development Corp.)

Target population: Adults with a clinical diagnosis of functional dyspepsia

Measurement concepts: Symptoms of functional dyspepsia

Role in endpoint hierarchy: Primary endpoint to establish treatment benefit
Co-Chairs: Robyn Carson (Forest Research Institute) and Gianna Rigoni (Takeda Pharmaceuticals International)

Target population: Adults with a clinical diagnosis of IBS, including the three main subtypes: IBS-C, IBS-D, and mixed

Measurement concepts: Abdominal symptoms and bowel movement-related symptoms

Role in endpoint hierarchy: Primary endpoint to establish treatment benefit
NSCLC Working Group

Co-Chairs: Alicyn Campbell (Genentech, Inc.) and Astra Liepa (Eli Lilly and Company)

Target population: Adult patients with advanced NSCLC (stages III/IV and ECOG performance status of 0 - 2)

Measurement concepts: Pulmonary and non-pulmonary symptoms of NSCLC

Role in endpoint hierarchy: Secondary endpoint to support treatment benefit
Co-Chairs: April Naegeli (Eli Lilly and Company) and Enkeleida Nikai (Eli Lilly and Company)

Target population: Adults with a clinical diagnosis of mild to severe rheumatoid arthritis

Measurement concept: Rheumatoid arthritis-related fatigue

Role in endpoint hierarchy: Secondary endpoint supporting treatment benefit
Path to FDA Qualification (aka Working Group Stages)

- Letter of Intent
- **Initial Briefing Package** Scoping Stage
- Vendor Selection Stage
- Content Validity Stage
  - Step I: Qualitative Research
  - Step II: Quantitative Research
- Submit to FDA for qualification of the instrument for use in exploratory studies
- Psychometric Analysis Stage
- Submit to FDA for qualification of the instrument as an effectiveness endpoint to support claims
Position on Path to Qualification

Scoping Stage Initial Briefing Package development
  ▪ Rheumatoid Arthritis Working Group

Content Validity Stage – Qualitative Step
  ▪ Functional Dyspepsia Working Group
  ▪ NSCLC Working Group *
  ▪ Asthma Working Group *
  ▪ Cognition Working Group *
  ▪ Irritable Bowel Syndrome Working Group *

Content Validity Stage – Quantitative Step
  ▪ Depression Working Group *

* Have draft versions of PRO instruments
Presentations and Publications

Presentations
http://c-path.org/category/publications/pro-publications/

Publications
http://c-path.org/category/presentations/pro-presentations/
Questions are Encouraged

The workshop is being audio recorded.

Please step to one of the microphones or let us bring a microphone to you before you speak.
Thank You!

Workshop Planning Subcommittee

- Risa Hayes – Lilly (Co-Chair)
- Ashley Slagle – FDA (Co-Chair)
- Steven Blum – GlaxoSmithKline
- Linda Deal – Shire
- Kathryn Engstrom – Lilly
- Sarah Fleming – Janssen
- Ari Gnanasakthy - Novartis
- Indira Hills – FDA
- Dianne (Dee) Kennedy – FDA
- Linda Nelsen - GlaxoSmithKline
- Elektra Papadopoulos – FDA
- Liz Piault-Louis – Genentech
- Abhilasha Ramasamy – Forest Research Institute
- Diana Rofail – Roche
- Margaret Rothman - Janssen
- Juliana Setyawan – Shire
- Yun Su – Bristol-Myers Squibb
- Sue Vallow - GlaxoSmithKline
- Jessica Voqui – FDA
- Randall Winnette - Novartis
Thank You!

C-Path’s PRO Consortium Team

- J. Jason Lundy, PhD – Assistant Director
- Theresa ("T") Griffey, PMP – Senior Project Manager
- Karla Lehmann, PMP – Senior Project Manager
- Theresa Swentesky – Project Coordinator
The FDA’s Office of Translational Sciences (OTS) provides oversight for CDER Critical Path Activities on behalf of Dr. Janet Woodcock. Official OTS liaisons to C-Path include:

- Indira Hills – FDA/CDER Project Manager for Critical Path Institute
- Marc Walton, MD, PhD – Associate Director for Translational Medicine
- ShaAvhrée Buckman-Garner, MD, PhD, FAAP – Director, OTS

The primary representatives from the SEALD staff to the PRO Consortium include:

- Ashley F. Slagle, MS, PhD – COA Qualification Scientific Coordinator and Endpoint Reviewer
- Elektra Papadopoulos, MD, MPH – Team Leader, Study Endpoints and Labeling Development Team
...to the roughly 150 scientists and clinicians that represent our PRO Consortium member firms on our working groups, committees, and subcommittees