Welcome and PRO Consortium Update

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FIFTH ANNUAL
PATIENT-REPORTED OUTCOME (PRO) CONSORTIUM WORKSHOP

April 29 - 30, 2014 ■ Silver Spring, MD

Co-sponsored by

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Acknowledgments

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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 Member Firms

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)

Participation by Member Firms

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.

Working Group Updates

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

Cognition Working Group

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

Functional Dyspepsia Working Group

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

IBS Working Group

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

Rheumatoid Arthritis Working Group

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!

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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

Thank You!

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

Thank You!

…to the roughly 150 scientists and clinicians that represent our PRO Consortium member firms on our working groups, committees, and subcommittees