Welcome

FOURTH ANNUAL
PATIENT-REPORTED OUTCOME (PRO) CONSORTIUM WORKSHOP

April 24- 25, 2013 ■ Silver Spring, MD

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Acknowledgments

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Welcome and PRO Consortium Update

Stephen Joel Coons, PhD
Executive Director, PRO Consortium

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Workshop Packet Contents

- Welcome Letter
- Workshop Agenda
- PRO Consortium Fact Sheet
- PRO Consortium Mission Statement and Objectives
- Speaker and Panelists Biographical Sketches
- Pre-Registrant List
- Workshop Feedback Form
- Press Release Regarding New C-Path CEO

PRO Consortium

Formed in late 2008 by the Critical Path Institute in cooperation with the FDA and the pharmaceutical industry

- **Membership**
  - 25 members (pharmaceutical firms) in 2013
- **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 – 11 member firms

Cognition – 9 member firms

Depression – 9 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

Participation by Member Firms

Of the 25 member firms…

- Three are in four working groups
- Five are in three working groups
- Seven are in two working groups
- Six are in one working group
- Four are not participating in any current working groups

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 5:30 pm – 7:30 pm this evening.

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.

Asthma Working Group

Co-Chairs: Linda Nelsen (Merck Sharp & Dohme Corp) and Richard H. Stanford (GlaxoSmithKline LLC)

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 Amy Duhig (AbbVie)

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

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

Role in endpoint hierarchy: Primary or co-primary endpoint to establish treatment benefit

Depression Working Group

Co-Chairs: Steven I. Blum (Forest Research Institute) and Nicholas Greco IV (AbbVie)

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 Karen Lasch (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 (UCB Pharma)

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

"Toward Consensus Development: Qualifying Endpoint Measures for Rheumatoid Arthritis Clinical Trials"

Workshop held on August 28, 2012, in Silver Spring, MD

The PRO Consortium was uniquely positioned to initiate, organize, and convene a diverse group of key stakeholders for a face-to-face consensus development workshop.

Along with RA WG members and C-Path personnel, participants included RA patients and representatives from the FDA, American College of Rheumatology, Outcome Measures in Rheumatology, European League Against Rheumatism, and the National Institute of Arthritis and Musculoskeletal and Skin Diseases.

Outcome: WG to focus on FDA qualification of a measure to support a secondary endpoint of RA-related fatigue to document treatment benefit.

Path to FDA Qualification (aka Working Group Stages)

Letter of Intent

Scoping Stage

Vendor Selection Stage

Content Validity Stage

- Step I: Qualitative Research
- Step II: Quantitative Research

Psychometric Analysis Stage

Submission and Review of Qualification Dossier

Qualification

Position on Path to Qualification

Scoping Stage

- 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/PROPresentations.cfm

Publications

http://c-path.org/PROPublications.cfm

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)**
- **Abhilasha Ramasamy – Forest Labs (Co-Chair)**
- Rich Barron – Amgen
- Steven Blum – Forest Labs
- Laurie Burke – FDA
- Nick Greco – AbbVie
- Indira Hills – FDA
- Dianne (Dee) Kennedy – FDA
- Josephine Norquist – Merck
- Elektra Papadopoulos – FDA
- Liz Piault-Louis – Roche/Genentech
- Diana Rofail – Roche
- Juliana Setyawan – Shire
- Ashley Slagle – FDA
- Yun Su – Bristol-Myers Squibb
- Yasuhiro Torigoe – Roche/Genentech
- Jessica Voqui – FDA

Thank You!

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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
- Alex Mutebi, MSc – Graduate Research Associate

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 team to the PRO Consortium include:

- Laurie B. Burke, RPh, MPH – Associate Director, Office of New Drugs
- Ashley F. Slagle, PhD, MS – Oak Ridge Institute for Science and Education (ORISE) Fellow

Thank You!

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