Welcome

SIXTH ANNUAL

PATIENT-REPORTED OUTCOME CONSORTIUM WORKSHOP

April 29 - 30, 2015 ■ Silver Spring, MD

Welcome
and
PRO Consortium Update

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Executive Director, PRO Consortium

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CRITICAL PATH INSTITUTE
a decade of excellence

10 YEARS

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

Active Participation is Encouraged

Before you speak, please step to a micro-phone or let us bring a microphone to you

The workshop is being audio recorded

Please turn off cell phones or set to vibrate

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 2015
- **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 – 10 member firms

Cognition – 10 member firms

Depression – 9 member firms

Functional Dyspepsia – 3 member firms

Irritable Bowel Syndrome (IBS) – 3 member firms

Lung Cancer (NSCLC) – 7 member firms

Rheumatoid Arthritis – 5 member firms

Goal of Working Groups

To produce and/or compile the necessary evidence to enable new or existing clinical outcome assessment (COA) tools to be "qualified" by the FDA for use in clinical trials where COA endpoints can be used to support product labeling claims.

Position on Path to Qualification

Initial Briefing Package Development

- Rheumatoid Arthritis Working Group
- Cognition Working Group*

Content Validity Stage – Qualitative Step

- Functional Dyspepsia Working Group *

Content Validity Stage – Quantitative Step

- Depression Working Group*
- Asthma Working Group*
- IBS Working Group*
- NSCLC Working Group *

* Have preliminary versions of PRO instruments

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 Ballroom from 5:30 pm – 7:00 pm this evening.

Asthma Working Groups

Co-Chairs: Linda Nelsen (GlaxoSmithKline LLC) and Sarah Fleming (Johnson & Johnson)

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 Scott Andrews (Eli Lilly and Company)

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

Measurement concepts: instrumental 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 (Actavis) 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 (Actavis) and TBD

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 IIIB/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

Since Last Year's Workshop…

Funded a measurement project titled

"Literature Review to Determine Empirical Basis for Response Scale Selection in Patient-reported Outcome Measure Development"

Involved in numerous presentations at scientific and professional meetings

http://c-path.org/category/presentations/pro-presentations/

Since Last Year's Workshop…

Published Articles

- Hayes RP, Blum SI, Gordon MF, Piault E, Burke LB, Slagle AF, Coons SJ. The Patient-Reported Outcome (PRO) Consortium: lessons learned along the path to PRO instrument qualification. *Therapeutic Innovation & Regulatory Science* 2015;49:132-138.

- Basch E, Geoghegan C, Coons SJ, Gnanasakthy A, Slagle AF, Papadopoulos EJ, Kluetz PG. Patient-reported outcomes in cancer drug development and US regulatory review: perspectives from industry, FDA, and the patient. *JAMA Oncology*. Published online April 16, 2015 (jamaoncology.com)

Since Last Year's Workshop…

Outcomes Psychometric Summit: Consensus Panel

March 19-20, 2015 ■ Tucson

Co-sponsored by Clinical Outcomes Solutions, University of Arizona, and Critical Path Institute

Topics

- Methods for determining clinically meaningful change
- Quantitative assessment of cross-cultural differences
- Use of mixed methods in instrument development
- Collecting and analyzing quantitative data from cognitive interviews
- Context effects for items within multi-item scales
- Use of CAT for assessment of efficacy endpoints
- Developing and scoring rating scales that allow subjects to choose their "worst" or "most bothersome" symptom(s)

In the Coming Year…

Special section of *Therapeutic Innovation & Regulatory Science* on "Advances in Clinical Outcome Assessments" edited by Joseph C. Cappelleri, PhD, MPH to be published in fall 2015

Two papers resulting from PRO Consortium and PRO Consortium collaboration will be included:

- O'Donohoe et al. "Considerations for requiring subjects to provide a response to electronic patient-reported outcome instruments"
- Fleming et al. "Optimizing electronic capture of clinical outcome assessment data in clinical trials: the case of patient-reported endpoints"

In the Coming Year…

Special section of *Therapeutic Innovation & Regulatory Science* to feature proceedings from the Sixth Annual PRO Consortium Workshop

Also edited by Joe Cappelleri and published in early 2016

Thanks Joe!

In the Coming Year…

Proposed PRO Consortium Webinar Series

- Determining clinically meaningful change
- Quantitative assessment of cross-cultural differences
- Optimizing qualitative and quantitative research (mixed methods) in COA instrument development
- Use of computer-adaptive testing (CAT) for assessment of efficacy endpoints

In the Coming Year…

Proposed consensus development workshops

- Assessment of physical function in oncology trials
- Use of PRO-CTCAE (Common Terminology Criteria for Adverse Events) in oncology trials

Consensus development initiative

- Agreement on essential evidence necessary to support COA translations for multinational trials

In the Coming Year…

Creation of two new working groups

- **Multiple Sclerosis** – to qualifying PRO measures of symptoms and functional impact to be used as co-primary or secondary endpoints

- **Myelofibrosis -** to gain stakeholder consensus on one harmonized *Myelofibrosis Symptom Assessment Form* for use as a secondary endpoint
    - This may lead to qualification although not the initial goal

Thank You!

Workshop Session Planning Team

Co-Chairs: Katarina Halling (AstraZeneca) and Ashley Slagle (FDA)

- Cheryl Coon – Adelphi Values
- Adam Gater – Adelphi Values
- Chad Gwaltney – ERT
- Sarrit Kovacs - FDA
- J. Jason Lundy – past Director, ePRO Consortium
- Linda Nelsen - GlaxoSmithKline
- Elektra Papadopoulos – FDA
- Jean Paty - Quintiles
- Liz Piault-Louis – Genentech
- Peter Trask – Genentech
- David Reasner - Ironwood
- Susan Vallow - GSK
- Jessica Voqui – FDA

Thank You!

C-Path's PRO Consortium Team

- Mabel Crescioni, DrPH, JD, LLM – Assistant Director
- Theresa Swentesky – Project Coordinator
- Theresa ("T") Griffey, PMP – Senior Project Manager
- Charlie Lynn, PMP – Senior Project Manager
- Margo Panke – Senior Project Manager
- Mira Patel, BS – Graduate Research Assistant

Thank you

for being here!