Welcome to
Tenth Annual
Patient-Reported Outcome
Consortium Workshop

April 24 – 25, 2019 ■ Silver Spring, MD
Network: Cypress-Guest     Passcode: Cyp24689
Welcome and PRO Consortium Update

Stephen Joel Coons, PhD
Executive Director, PRO Consortium

Tenth Annual Patient-Reported Outcome Consortium Workshop

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Introduction and Welcome

Joseph Scheeren, PharmD
President and CEO of Critical Path Institute (as of April 15, 2019)

Prior to joining C-Path, Dr. Scheeren was Senior Vice President and Senior Advisor, Research and Development at Bayer AG (Berlin) and is currently Chair of DIA’s Board of Directors.
Workshop Materials*

- Welcome Letter
- PRO Consortium Fact Sheet
- PRO Consortium Mission Statement and Objectives
- Workshop Agenda
- Workshop Agenda At A Glance
- Workshop Speaker and Panelist Information
- Pre-Registration List
- Workshop Feedback Form Link: https://www.surveymonkey.com/r/93FV2VJ

* Emailed to registrants on April 19, 2019
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Disclaimer

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Patient-Reported Outcome (PRO) Consortium

- Formed in late 2008 by C-Path in cooperation with FDA’s Center for Drug Evaluation and Research (CDER) and the pharmaceutical industry

- Membership
  - 22 members (pharmaceutical firms)

- Additional Participants
  - Representatives of governmental agencies (FDA, NIH)
  - Clinical consultants, patients, academic researchers, and contract research organizations partnering in the development of PRO measures and other clinical outcome assessment (COA) tools
PRO Consortium Members

Abbvie
Allergan
Amgen
AstraZeneca
AvroBio
Bayer
Boehringer Ingelheim
Bristol-Myers Squibb
Daiichi-Sankyo
EMD Serono
GlaxoSmithKline
Genentech
Ironwood
Johnson & Johnson
Lilly
Merck
Novartis
Otsuka
Sanofi
Sunovion
Takeda
UCB
PRO Consortium Mission

To establish and maintain a collaborative framework with appropriate stakeholders for the qualification of patient-reported outcome (PRO) instruments and other clinical outcome assessment (COA) tools that will be publicly available for use in clinical trials where COA-based endpoints are used to support product labeling claims.
PRO Consortium Goals

- Enable pre-competitive collaboration that includes FDA input and expertise
- Obtain FDA qualification of PRO measures and other COA tools for use in assessing primary or secondary clinical trial endpoints
- Avoid development of multiple endpoint measures for the same purpose
- Share costs of developing new endpoint measures
- Facilitate FDA’s review of medical products by standardizing COA-based endpoint measures that will be publicly available
Goal of Working Groups

To produce and/or compile the necessary evidence to enable new or existing COAs to be qualified by the FDA for use in clinical trials where COA-based endpoints can be used to support product labeling claims.
Asthma WG - Obtained FDA qualification of *Asthma Daytime Symptom Diary (ADSD)* and *Asthma Nighttime Symptom Diary (ANSD)* – March 2019

Depression WG – Obtained FDA qualification of *Symptoms of Major Depressive Disorder Scale (SMDDS)* – April 2018

Non-Small Cell Lung Cancer WG – Obtained FDA qualification of *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)* – November 2017

Myelofibrosis WG – Derived the consensus-defined *Myelofibrosis Symptom Assessment Form v4.0 (MFSAF v4.0)*
Qualification Statement

Qualification of Asthma Daytime Symptom Diary and Asthma Nighttime Symptom Diary: Patient-Reported Outcome Instruments for Measurement of Symptoms of Asthma

Date: March 28, 2019

DDT Type: Clinical Outcome Assessment (COA)

DDT Tracking Number: DDTCOA-000006

Referenced COA: Asthma Daytime Symptom Diary (ADSD); Asthma Nighttime Symptom Diary (ANSD)

Type of COA: Patient-Reported Outcome (PRO) Instrument

The Center for Drug Evaluation and Research has determined that the ADSD and ANSD have demonstrated adequate evidence of content validity and cross-sectional measurement properties (i.e., internal consistency reliability, test-retest reliability, convergent validity, and known-groups validity) to measure symptoms of asthma in the context of use described below.

Full qualification statement available at:
Working Groups that have Completed Initial Goal

- **Asthma WG** - Obtained FDA qualification of *Asthma Daytime Symptom Diary* (ADSD) and *Asthma Nighttime Symptom Diary* (ANSD) – March 2019

- **Depression WG** – Obtained FDA qualification of *Symptoms of Major Depressive Disorder Scale* (SMDDS) – April 2018

- **Non-Small Cell Lung Cancer WG** – Obtained FDA qualification of *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire* (NSCLC-SAQ) – November 2017

- **Myelofibrosis WG** – Derived the consensus-defined *Myelofibrosis Symptom Assessment Form v4.0* (MFSAF v4.0)

*The above measures are being actively licensed for use in clinical trials*
Active Working Groups (slide 1 of 4)

- **Chronic Heart Failure (CHF) WG** – Seeking acceptance into FDA’s COA Qualification Program for two PRO measures developed by Amgen
  - *Chronic Heart Failure-Symptom Scale (CHF-SS)*
  - *Chronic Heart Failure-Impact Scale (CHF-IS)*

  and an activity monitor-based endpoint measure assessing physical activity

  - Since last year’s workshop... submitted Letter of Intent to FDA’s COA Qualification Program

- **Cognition WG** – Working toward FDA qualification of the *University of California San Diego Performance-based Skills Assessment (UPSA-MCI)*

  - Since last year’s workshop... the Initial Briefing Package being prepared for submission to FDA in Q2 2019
Active Working Groups (slide 2 of 4)

- **Depression WG 2.0** – Working toward FDA qualification of the *Symptoms of Major Depressive Disorder Diary (SMDDD)* and *Symptoms of Major Depressive Disorder Momentary Assessment (SMDDMA)*
  - Since last year’s workshop... **accepted into FDA’s COA qualification program**

- **Functional Dyspepsia (FD) WG** – Working toward FDA qualification of the *Functional Dyspepsia Symptom Diary (FDSD)*
  - Since last year’s workshop... **received feedback from FDA’s qualification review team (QRT) on submitted Qualification Briefing Package and will meet with the QRT (via TC) in May 2019**
Active Working Groups (slide 3 of 4)

- **Irritable Bowel Syndrome (IBS) WG** – Working toward qualification of
  - *Diary for Irritable Bowel Syndrome Symptoms – Constipation (DIBSS-C)*
  - *Diary for Irritable Bowel Syndrome Symptoms – Diarrhea (DIBSS-D)*
  - *Diary for Irritable Bowel Syndrome Symptoms – Mixed (DIBSS-M)*
  - Since last year’s workshop... **submitted Full Qualification Package to FDA for DIBSS-C**

- **Multiple Sclerosis (MS) WG** – Working toward qualification of *PROMIS® Fatigue*$_{MS}$ as first step and then a *PROMIS®* physical function short form subsequently
  - Since last year’s workshop... **a substantial amount of qualitative research has been conducted by Merck KGaA that will facilitate preparation of Initial Briefing Package**
Pediatric Asthma WG – Working toward qualification of *Pediatric Asthma Diary-Observer (PAD-O)* and *Pediatric Asthma Diary-Child (PAD-C)* [Note: The initial development of these measures was conducted by Merck.]
- Since last year’s workshop... received FDA feedback that led to release of RFP for additional qualitative research and development of Initial Briefing Package

Rheumatoid Arthritis (RA) WG – Working toward qualification of *PROMIS® Fatigue Short Form 10a*
- Since last year’s workshop... submitted Qualification Plan to FDA
Working Group to be Established

- **Small Cell Lung Cancer (SCLC) WG** – Aimed at leveraging the work of the NSCLC WG (and member firms’ individual efforts) to qualify a SCLC core symptom measure
  - Since last year’s workshop... **PRO Consortium Coordinating Committee approved the establishment of the SCLC WG (along with CHF WG)**
• During Workshop breaks, please view the working group posters at the back of the meeting room.

• The posters will also be on display during the reception in Magnolia Ballroom (First Floor) from 5:30 pm – 7:00 pm this evening.
Since Last Year’s Workshop

In addition to the qualifications....

- Process to extend the PRO Consortium for another five-year term was completed on November 15, 2018

- Completed development of Electronic Implementation Specifications documents for NSCLC-SAQ and SMDDS
Since Last Year’s Workshop


Since Last Year’s Workshop


Active Participation During the Q&A Portion of Each Session is Encouraged

Before you speak, please go to the microphone or wait until a microphone is handed to you

The workshop is being audio recorded

Please turn off cell phones or set to vibrate