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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CRITICAL PATH INSTITUTE
collaborate · innovate · accelerate

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

Acknowledgments

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Disclaimer

- The views and opinions expressed in the following slides are those of the individual presenters and should not be attributed to their respective organizations/companies, the U.S. Food and Drug Administration, or the Critical Path Institute.

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Patient-Reported Outcome (PRO) Consortium

PRO CONSORTIUM
CRITICAL PATH INSTITUTE

- 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

gsk GlaxoSmithKline

Genentech
A Member of the Roche Group

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.

Working Groups that have Completed Their 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*)

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:
https://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm626866.htm

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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**

Active Working Groups (slide 4 of 4)

- **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)**

Working Group Posters

- 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

- Naegeli AN, Hanlon J, Gries KS, Safikhani S, Ryden A, Patel M, Crescioni M, Vernon M. Literature review to characterize the empirical basis for response scale selection in pediatric populations. *Journal of Patient-Reported Outcomes* 2018;2:39. (https://doi.org/10.1186/s41687-018-0051-8)
- Safikhani S, Gries KS, Trudeau JJ, Reasner D, Rudell K, Coons SJ, Bush EN, Hanlon J, Abraham L, Vernon M. Response scale selection in adult pain measures: results from a literature review. *Journal of Patient Reported Outcomes* 2018;2:40. (https://doi.org/10.1186/s41687-018-0053-6)
- Gries K, Berry P, Harrington M, Crescioni M, Patel M, Rudell K, Safikhani S, Pease S, Vernon M. Literature review to assemble the evidence for response scales used in patient-reported outcome measures. *Journal of Patient-Reported Outcomes* 2018;2:41. (https://doi.org/10.1186/s41687-018-0056-3)
- Taylor F, Higgins S, Carson R, Eremenco S, Foley C, Lacy B, Parkman H, Reasner D, Shields A, Tack J, Talley N. Development of a symptom-focused patient-reported outcome measure for functional dyspepsia: the Functional Dyspepsia Symptom Diary (FDSD). American Journal of Gastroenterology 2018;113(1):39-48. (https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5770596/)

Since Last Year's Workshop

- Kluetz PG, Kanapuru B, Lemery S, Johnson LL, Fiero M, Arscott K, Barbachano Y, Basch E, Campbell M, Cappelleri JC, Cella D, Cleeland C, Coens C, Daniels S, Denlinger CS, Fairclough DL, Hillard JR, Minasian L, Mitchell SA, O'Connor D, Patel S, Rubin EH, Ryden A, Soltys K, Sridhara R, Thanarajasingam G, Velikova G, Coons SJ. Informing the tolerability of cancer treatments using patient-reported outcome (PRO) measures: summary of an FDA and Critical Path Institute workshop. *Value in Health* 2018;21:742-747. (https://doi.org/10.1016/j.jval.2017.09.009
- Qin S, Nelson L, McLeod L, Eremenco S, Coons SJ. Assessing test-retest reliability of patient-reported outcome measures using intraclass correlation coefficients: recommendations for selecting and documenting the analytical formula. *Quality of Life Research*. In press (https://doi.org/10.1007/s11136-018-2076-0)
- Bushnell DM, McCarrier KP, Bush EN, Abraham L, Jamieson C, McDougall F, Trivedi M, Thase M, Carpenter L, Coons SJ, on behalf of the Patient-Reported Outcome Consortium's Depression Working Group. Symptoms of Major Depressive Disorder Scale (SMDDS): performance of a novel patient-reported symptom measure. *Value in Health*. Accepted for publication

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