PRO Consortium Update

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11th Annual
Patient-Reported Outcome Consortium Workshop

Cancelled due to COVID-19 (scheduled for April 22 - 23, 2020)

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

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

- Membership
  - 25 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 assessments (COAs)

PRO Consortium Members

abbvie

ALLERGAN

AMGEN

AstraZeneca

AVROBIO

BAYER

Biogen

Boehringer Ingelheim

Bristol-Myers Squibb

cyclerion

Daiichi-Sankyo

EMD SERONO

gsk GlaxoSmithKline

Genentech
A Member of the Roche Group

imbria PHARMACEUTICALS

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) measures and other clinical outcome assessments (COAs) 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 COAs 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 generate and/or compile the necessary evidence to enable new or existing COAs to be qualified by FDA for use in treatment trials where COA-based endpoints can be used to evaluate clinical benefit

The PRO Consortium has 15 COAs in CDER's Clinical Outcome Assessment Qualification Program

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
- **Non-Small Cell Lung Cancer WG** – Obtained FDA qualification of *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire* (*NSCLC-SAQ*) – April 2018
- **Depression WG** – Obtained FDA qualification of *Symptoms of Major Depressive Disorder Scale* (*SMDDS*) – 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 via the following website: https://www.c-pathcoas.org/

Active Working Groups (slide 1 of 6)

- **Chronic Heart Failure (CHF) WG** – Working toward qualification of 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… **all three measures accepted into FDA's COA Qualification Program; received FDA Drug Development Tool (DDT) Grant to conduct qualitative research with persons with CHF to explore meaningfulness of day-to-day physical activities potentially assessable with an activity monitor**

Active Working Groups (slide 2 of 6)

- **Cognition WG** – Working toward qualification of the *University of California San Diego Performance-based Skills Assessment* – Three Domain (*UPSA-3D*)
  - Since last year's workshop… **Initial Briefing Package submitted to FDA; planning underway for additional qualitative research and development of Qualification Plan**

- **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… **both measures accepted into FDA's COA qualification program; *SMDDD* and *SMDDMA* qualitative research (cognitive interview) report submitted to FDA**

Active Working Groups (slide 3 of 6)

- **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 met with QRT (via TC) in May 2019; obtained FDA funding to prepare Qualification Plan**

- **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 responses to Information Requests from FDA regarding the Full Qualification Package for *DIBSS-C***

Active Working Groups (slide 3 of 5)

- **Multiple Sclerosis (MS) WG** – Working toward qualification of
  - *PROMIS® Short Form v1.0—Fatigue-Multiple Sclerosis 8a* (*PROMIS FatigueMS—8a*)
  - *PROMIS® Short Form v2.1—Physical Function-Multiple Sclerosis 15a* (*PROMIS PFMS—15a*)
  - Since last year's workshop… **FDA DDT Grant was received to develop the Qualification Plan for the *PROMIS FatigueMS—8a;* submitted FDA DDT Grant application for development of Qualification Plan for *PROMIS PFMS—15a***

Active Working Groups (slide 5 of 6)

- **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… **C-Path submitted application in response to an FDA Broad Agency Announcement (BAA) and was awarded a 5-year contract to conduct the qualitative and quantitative research necessary to complete an Initial Briefing Package, a Qualification Plan, and a Full Qualification Package**

Active Working Groups (slide 6 of 6)

- **Rheumatoid Arthritis (RA) WG** – Working toward qualification of *PROMIS® Fatigue Short Form 10a*
  - Since last year's workshop… **FDA accepted Qualification Plan and development of Full Qualification Package in progress**

- **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… **Letter of Intent submitted to FDA on March 25, 2020**

Working Group Posters

- More detail regarding the working groups and their April 2020 status is provided in the accompanying posters.

Since Last Year's Workshop

C-Path, along with the National Organization for Rare Disorders, received an FDA grant (U01FD006882) to establish the Rare Disease Clinical Outcome Assessment Consortium

The Rare Disease Subcommittee was established within the PRO Consortium to serve as an incubator for the Rare Disease COA Consortium.

- The goal of the subcommittee (and, ultimately, the new consortium) is to help address critical unmet measurement needs for assessing clinical benefit in rare disease drug development.
- The initial focus is on the identification and evaluation of existing, publicly available COAs that have the potential to be used as efficacy endpoint measures for multiple rare diseases.

Publications Since Last Year's Workshop

- Papadopoulos E, Bush EN, Eremenco S, Coons SJ. Why reinvent the wheel? Use or modification of existing clinical outcome assessment tools in medical product development. *Value in Health* 2020;23:151-153. (https://doi.org/10.1016/j.jval.2019.09.2745)

- Walton MK, Cappelleri JC, Byrom B, Goldsack JC, Eremenco S, Harris D, Potero E, Patel N, Flood E, Daumer M. Considerations for development of an evidence dossier to support the use of mobile sensor technology for clinical outcome assessments in clinical trials. *Contemporary Clinical Trials* 2020;91:105962. (https://doi.org/10.1016/j.cct.2020.105962)

Questions?

Since we were unable to hold the 11th Annual PRO Consortium Workshop (April 22-23, 2020) due to the global pandemic, we would be glad to answer any questions you may have regarding the information presented in these slides and accompanying posters. Please contact:

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or Sonya Eremenco: seremenco@c-path.org

Our 12th Annual PRO Consortium Workshop is scheduled for April 14-15, 2021 and will be held virtually.