Welcome to the 12th Annual Patient-Reported Outcome Consortium Workshop

Event will begin at 11:01 am US ET

April 14-15, 2021

Patient-Reported Outcome (PRO) Consortium Update

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12th Annual Patient-Reported Outcome Consortium Workshop

Held Virtually April 14 – 15, 2021

Acknowledgments

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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
  - 24 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

AMGEN®

AstraZeneca

AVROBIO

BAYER

Biogen

Boehringer Ingelheim

Bristol-Myers Squibb

Daiichi-Sankyo

EMD SERONO

Genentech
A Member of the Roche Group

GILEAD

gsk GlaxoSmithKline

Ironwood®

Janssen
PHARMACEUTICAL COMPANIES OF
Johnson & Johnson

Jazz Pharmaceuticals

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 that will be publicly available 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 COAs used as endpoint measures for specific concepts of interest and contexts of use

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/

Most Recent Qualification

- **Irritable Bowel Syndrome (IBS) WG** –
  - *Diary for Irritable Bowel Syndrome Symptoms – Constipation (DIBSS-C)*
- Qualification Date: December 18, 2020
- Information available at FDA's Qualified COAs website: https://www.fda.gov/drugs/clinical-outcome-assessment-coa-qualification-program/ddt-coa-000005-diary-irritable-bowel-syndrome-symptoms-constipation-dibss-c
- Licensing information will be available in the coming months at: https://www.c-pathcoas.org/

Active Working Groups (slide 1 of 7)

- **Chronic Heart Failure (CHF) WG –** Working toward qualification of an activity monitor-based endpoint measure of physical activity and two PRO measures developed by Amgen
  - *Chronic Heart Failure-Symptom Scale* (*CHF-SS*)
  - *Chronic Heart Failure-Impact Scale* (*CHF-IS*)

  Since April 2020…
  - **Qualification Plans for *CHF-SS* and *CHF-IS* in progress**
  - **Completed qualitative research study funded by FDA Drug Development Tool (DDT) Research Grant 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 7)

- **Cognition WG** – Working toward qualification of the *University of California San Diego Performance-based Skills Assessment – Three Domain* (*UPSA-3D*)

  Since April 2020…

  - **With the accelerated movement toward technology-enabled remote assessments, the WG is considering switching from the *UPSA-3D* to the *Virtual Reality Functional Capacity Assessment Tool (VRFCAT)*, which is a touchscreen computer-based assessment.**
  - **Planning underway for additional qualitative research and development of Qualification Plan**

Active Working Groups (slide 3 of 7)

- **Depression WG 2.0** – Working toward qualification of the *Symptoms of Major Depressive Disorder Diary* (*SMDDD*) and *Symptoms of Major Depressive Disorder Momentary Assessment* (*SMDDMA*)

  Since April 2020…

  - **Met with FDA to discuss *SMDDD* and *SMDDMA* qualitative research (cognitive interview) report and refine measures**
  - **Qualification Plan for *SMDDD* in progress**
  - **FDA DDT Research Grant was received to develop the Qualification Plan for the *SMDDMA*, also in progress**

Active Working Groups (slide 4 of 7)

- **Functional Dyspepsia (FD) WG** – Working toward qualification of the *Functional Dyspepsia Symptom Diary* (*FDSD*)

  Since April 2020… **prepared and submitted a Qualification Plan for the *FDSD***

- **Irritable Bowel Syndrome (IBS) WG** – Working toward qualification of
  - *Diary for Irritable Bowel Syndrome Symptoms – Diarrhea* (*DIBSS-D*)
  - *Diary for Irritable Bowel Syndrome Symptoms – Mixed* (*DIBSS-M*)

  Since April 2020… **developed a briefing package on urgency as an endpoint in IBS-D for submission to FDA; 507 transition updates for the *DIBSS-D* and *DIBSS-M* in progress; developing Qualification Plans for the *DIBSS-D* and *DIBSS-M***

Active Working Groups (slide 5 of 7)

- **Multiple Sclerosis (MS) WG** – Working toward qualification of
  - *PROMIS® Short Form v1.0—Fatigue-Multiple Sclerosis 8a* (*PROMIS FatigueMS—8a*)
  - *PROMIS®/Neuro-QoL™ Physical Function Measure for Multiple Sclerosis (PROMISnq Short Form v2.0 - Physical Function - Multiple Sclerosis 15a* [*PROMISnq PFMS—15a*])

Since April 2020… **submitted the Qualification Plan for the *PROMIS FatigueMS—8a to FDA;* FDA DDT Research Grant was received to develop the Qualification Plan for *PROMISnq PFMS—15a*, in progress**

Active Working Groups (slide 6 of 7)

- **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 April 2020… **initiated the cognitive interview study of both measures using web-based video interviews via Microsoft Teams, and completed Round 1 and 2 interviews with 10 children (8 through 11 years old) and 20 parents/caregivers of children (4 through 11 years old)**

Active Working Groups (slide 7 of 7)

- **Rheumatoid Arthritis (RA) WG** – Working toward qualification of *PROMIS®* *Fatigue Short Form 10a*

  Since April 2020… **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 an SCLC core symptom measure

  Since April 2020… **the measure was accepted into the COA Qualification Program, a research partner was selected, and a qualitative research study is in progress to evaluate the *NSCLC-SAQ* in persons with SCLC**

Working Group Posters

- More detail regarding the working groups and their April 2021 status is provided in the posters posted to the PRO Consortium webpage: www.c-path.org/12th-annual-patient-reported-outcome-consortium-workshop/

Rare Disease COA Consortium to be Launched in Late Summer 2021

C-Path, along with the National Organization for Rare Disorders, received an FDA grant (U01FD006882) to establish the Rare Disease Clinical Outcome Assessment Consortium, which was extended through August 31, 2021.

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 April 2020

- McKown S, Acquadro C, Anfray C, Arnold B, Eremenco S, Giroudet C, Martin M, Weiss D. Good practices for the translation, cultural adaptation, and linguistic validation of clinician-reported outcome, observer-reported outcome, and performance outcome measures. *Journal of Patient-Reported Outcomes* 2020. (https://doi.org/10.1186/s41687-020-00248-z)
- Gertel A, Raymond S, Vallow S, Arnera V, Crescioni M, Chassany O, Bodart S, Eremenco S on behalf of the Electronic Patient-Reported Outcome Consortium. Demystifying submissions of eCOA documentation for ethics committee review: are we making submissions more difficult than necessary? *Applied Clinical Trials* 2020. (https://www.appliedclinicaltrialsonline.com/view/demystifying-submissions-of-ecoa-documentation-for-ethics-review-are-we-making-submissions-more)

Questions?

We would be glad to answer any questions you may have regarding the information presented in these slides and posters.

Please contact:

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