Welcome to the 13th Annual Patient-Reported Outcome Consortium Workshop

Event will begin at 11:01 am US ET

April 13-14, 2022

Patient-Reported Outcome (PRO) Consortium Update

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13th Annual Patient-Reported Outcome Consortium Workshop

Held Virtually April 13-14, 2022

CRITICAL PATH INSTITUTE

Acknowledgments

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) and is 54.2% funded by the FDA/HHS, totaling $13,239,950, and 45.8% funded by non-government source(s), totaling $11,196,634. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.

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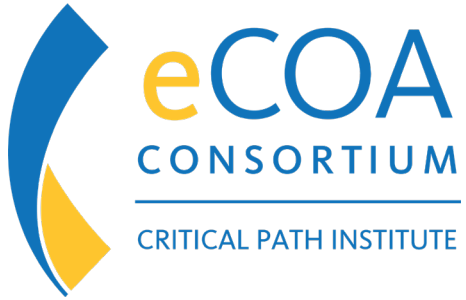
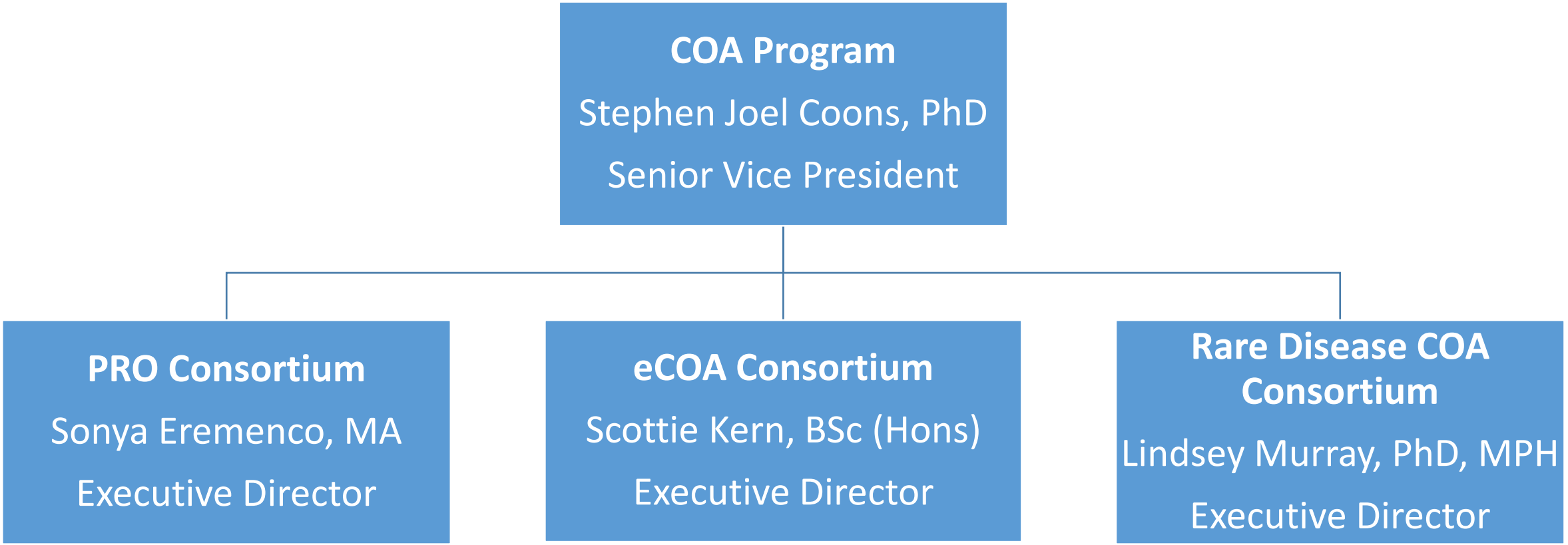
Clinical Outcome Assessment (COA) Program

Critical Path Institute (C-Path)



C-Path's COA Program Leadership



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
  - 26 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 COAs

PRO Consortium Members

abbvie

AMGEN®

AstraZeneca

AVROBIO

BAYER

Biogen

Boehringer Ingelheim

Bristol-Myers Squibb

Daiichi-Sankyo

EMD SERONO

Galápagos

Genentech
A Member of the Roche Group

GILEAD

gsk GlaxoSmithKline

Ironwood®

janssen
PHARMACEUTICAL COMPANIES OF
Johnson & Johnson

Jazz Pharmaceuticals

Lilly

MERCK

MindMed

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 9 active working groups with 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*) – included in COA Compendium

The above measures are actively being 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 2021…
  - **Qualification Plan for *CHF-SS* in progress; target submission to FDA is Q2 2022**
  - **Qualification Plan for *CHF-IS* in progress; target submission to FDA is Q3 2022**
  - **Advisory panel convened (December 2021; March 2022) with the goal of alignment on existing (or proposed novel) activity monitor metric(s) that best reflect/capture the meaningful aspects of physical activity identified by persons with CHF; Qualification Plan target submission to FDA is Q4 2022**

Active Working Groups (slide 2 of 7)

- **Cognition WG** – Working toward qualification of the *Virtual Reality Functional Capacity Assessment Too*l (*VRFCAT*)

  Since April 2021…

  - **After receiving support from FDA's Office of Neuroscience and Division of Clinical Outcome Assessment, the WG formally agreed in May 2021 to move forward with qualification of the *VRFCAT*, a touchscreen computer-based assessment, rather than the *UPSA-3D (University of California San Diego Performance-based Skills Assessment – Three Domain)***
  - **Study startup underway for additional qualitative research, followed by development of a 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 2021…

  - **Met with FDA to discuss combined *SMDDD* and *SMDDMA* quantitative pilot study protocol synopsis in July 2021; agreement to move forward**
  - **Qualification Plan for *SMDDD* in progress; target submission to FDA is Q2 2022**
  - **Qualification Plan for *SMDDMA* in progress; target submission to FDA is Q3 2022**

Active Working Groups (slide 4 of 7)

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

  Since April 2021… **FDA deemed the Qualification Plan for the *FDSD* reviewable; waiting for review determination from FDA**

- **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 2021… **submitted a briefing package to FDA on urgency as an endpoint in IBS-D; held teleconference with FDA to discuss, and now moving forward with developing the Qualification Plan for the *DIBSS-D***

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 2021…

- **submitted revised the Qualification Plan for the *PROMIS FatigueMS—8a* to FDA, deemed reviewable and under review as of March 8, 2022**
- **completed and submitted the Qualification Plan for *PROMISnq PFMS—15a* to FDA, undergoing reviewability assessment**

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 2021… **completed the cognitive interview study of both measures using web-based video interviews via Microsoft Teams and completed and submitted the Initial Briefing Package to FDA on March 31, 2022**

Active Working Groups (slide 7 of 7)

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

  Since April 2021… **Full Qualification Package being finalized for submission to FDA by Q2 2022**

- **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 2021… **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 2022 status is provided in the posters posted to the PRO Consortium webpage: https://c-path.org/13th-annual-patient-reported-outcome-consortium-workshop/

Rare Disease COA Consortium Launched in January 2022

C-Path, along with the National Organization for Rare Disorders, received an FDA grant (U01FD006882) to establish the Rare Disease Clinical Outcome Assessment (COA) Consortium, which was extended through August 31, 2022.

The Rare Disease Subcommittee was established within the PRO Consortium to serve as an incubator for the Rare Disease COA Consortium from 2019 to 2021.

- The goal of 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.
- See poster for full update: https://c-path.org/13th-annual-patient-reported-outcome-consortium-workshop/

Publications Since April 2021

- Bushnell DM, Atkinson T, McCarrier K, Liepa A, DeBusk K, Coons SJ. Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ): psychometric performance and regulatory qualification of a novel patient-reported symptom measure. *Current Therapeutic Research* 2021;95:100642. (https://doi.org/10.1016/j.curtheres.2021.100642)

- Gater A, Nelsen L, Coon C, Eremenco S, O'Quinn S, Khan A, Eckert L, Staunton H, Bonner N, Hall R, Krishnan J, Stoloff S, Schatz M, Haughney J, Coons SJ. Asthma Daytime Symptom Diary (ADSD) and Asthma Nighttime Symptom Diary (ANSD): Measurement properties of novel patient-reported symptom measures. *The Journal of Allergy Clinical Immunology: In Practice* 2021. (https://doi.org/10.1016/j.jaip.2021.11.026)

- Gordon S, Crager J, Howry C, Barsdorf A, Cohen J, Crescioni M, Dahya B, Delong P, Knaus C, Reasner D, Vallow S, Zarzar K, Eremenco S, on behalf of the Patient-Reported Outcome (PRO) Consortium and eCOA Consortium at the Critical Path Institute. Best practice recommendations: user acceptance testing for systems designed to collect clinical outcome assessment data electronically. *Therapeutic Innovation & Regulatory Science* 2022. (https://doi.org/10.1007/s43441-021-00363-z)

- Williams P, Burke T, Norquist JM, Daskalopoulou C, Speck RM, Samkari A, Eremenco S, Coons SJ. Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ): measurement properties and estimated clinically meaningful thresholds from a phase 3 study. *JTO Clinical and Research Reports* (2022). (https://doi.org/10.1016/j.jtocrr.2022.100298)

Questions?

I 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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