Welcome and PRO Consortium Update

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14th Annual
Patient-Reported Outcome Consortium Workshop

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CRITICAL PATH INSTITUTE

Acknowledgments

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- 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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Introduction and Welcome

Cheryl D. Coon, PhD

Vice President, Clinical Outcome Assessment (COA) Program (as of February 27, 2023)

As VP, COA Program, Cheryl will oversee the PRO Consortium, the Electronic COA Consortium, and the Rare Disease COA Consortium. She is a psychometrician with two decades of experience developing and evaluating COAs for use in constructing patient-centered endpoints.

Introduction and Welcome

Daniel M. Jorgensen, MD, MPH, MBA

CEO of Critical Path Institute
(as of October 1, 2022)

Dr. Jorgensen is an accomplished physician executive with more than 24 years of experience in the biopharmaceutical industry, in both small and large companies, public and private, including C-level positions for the past 11 years.

CLINICAL OUTCOME ASSESSMENT PROGRAM

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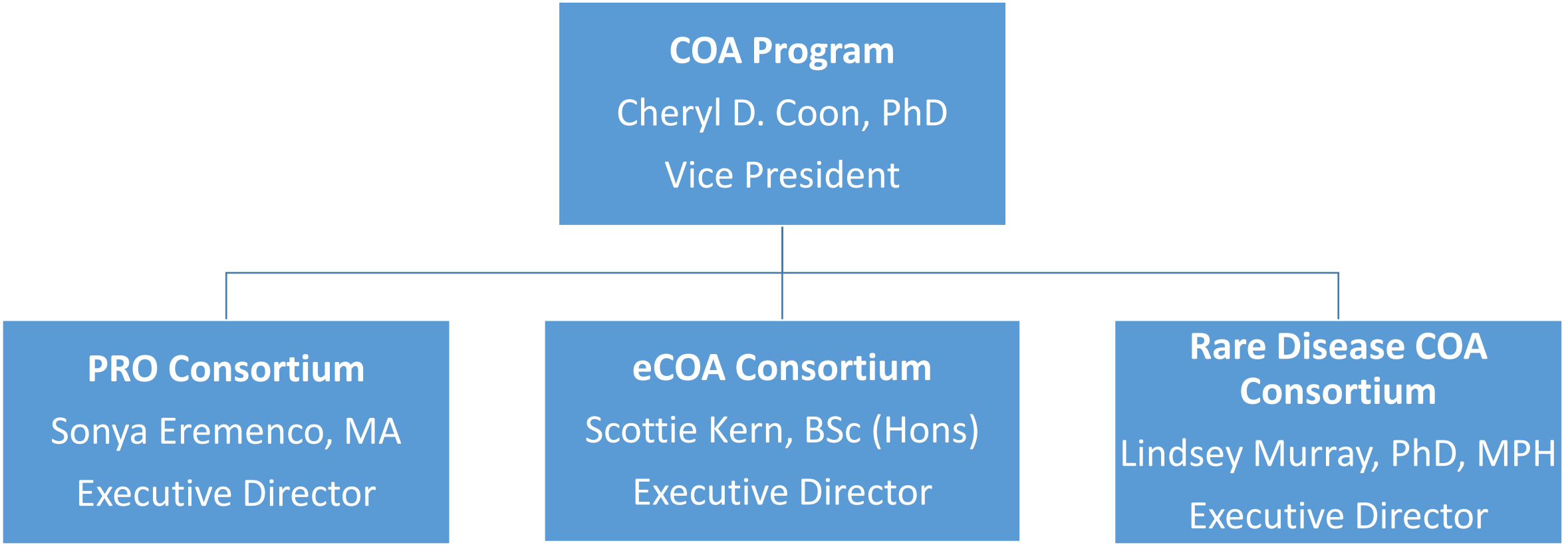
PRO CONSORTIUM

eCOA CONSORTIUM

RD-COA CONSORTIUM

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
  - 25 members (pharmaceutical firms)

- Additional Participants
  - Representatives of governmental agencies (FDA, NIH)
  - Clinical consultants, patients, patient advocacy organizations, 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

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Genentech
A Member of the Roche Group

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Jazz Pharmaceuticals

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MERCK

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REGENERON
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sanofi

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 produce and/or compile the necessary evidence to enable new or existing COAs to be qualified by 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

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- **IBS WG** - Obtained FDA qualification of *Diary for Irritable Bowel Syndrome Symptoms – Constipation (DIBSS-C)* – December 18, 2020
- **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/

Active Working Groups (slide 1 of 8)

- **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*)

  Context of use: adults with a clinician-confirmed history of CHF for ≥3 months with New York Heart Association class II to IV symptoms for ≥4 weeks, with preserved ejection fraction (HFpEF) or with reduced ejection fraction (HFrEF)

  Since April 2022…
  - **Qualification Plan for *CHF-SS* was submitted to FDA on March 24, 2023**
  - **Qualification Plan for *CHF-IS* in progress; target submission to FDA is Q3 2023**
  - **Informal meeting with FDA representatives to discuss activity monitor metrics held on October 6, 2022. Qualification Plan in progress for an activity monitor-based endpoint measure; target submission to FDA is Q4 2023.**

Active Working Groups (slide 2 of 8)

- **Cognition WG** – Working toward qualification of the *Virtual Reality Functional Capacity Assessment Tool-Short List Mild Cognitive Impairment (VRFCAT-SL MCI)*

  Context of use: Persons 50 years of age and older in clinical stages 2-3 of biomarker-confirmed Alzheimer's disease

  Since April 2022…

  - **The working group's research partner, WCG Clinical Endpoint Solutions (formerly VeraSci), is completing study start-up process with the Duke Memory Disorders Clinic to conduct a qualitative research study. This research is a necessary precursor to the development of the Qualification Plan for the *VRFCAT-SL MCI*.**

Active Working Groups (slide 3 of 8)

- **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*)

  Context of use: Persons 18 years and older with a diagnosis of major depressive disorder, who are being treated in ambulatory settings

  Since April 2022…

  - **Qualification Plan for *SMDDD* submitted to FDA on July 29, 2022**
    - **Determined to be reviewable by FDA on April 17, 2023**
  - **Qualification Plan for *SMDDMA* submitted to FDA on March 24, 2023**

Active Working Groups (slide 4 of 8)

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

  Context of use: Adults diagnosed with functional dyspepsia

  Since April 2022…

  FDA accepted the Qualification Plan for the *FDSD* on April 29, 2022

Active Working Groups (slide 5 of 8)

- **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*)

  Context of use: Adults diagnosed with remaining IBS subtypes (IBS-D or IBS-M)

  Since April 2022…
  - **Qualification Plan for the *DIBSS-D* submitted to FDA on December 16, 2022**
    - **Determined to be reviewable by FDA on April 7, 2023**

Active Working Groups (slide 6 of 8)

- **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*])

  Context of use: Adults with any type of MS

  Since April 2022…
  - **FDA accepted the Qualification Plan for the *PROMIS FatigueMS—8a* on September 6, 2022; Full Qualification Package is in preparation, and target submission is Q3 2023**
  - **Qualification Plan for *PROMISnq PFMS—15a* determined to be reviewable by FDA on November 14, 2022**

Active Working Groups (slide 7 of 8)

- **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.]

  Context of use: children 4 through 11 years old with a clinical diagnosis of mild to severe persistent asthma requiring a daily long-term control medication

  Since April 2022…

  Qualification Plan for *PAD-O and PAD-C* was submitted to FDA on December 15, 2022

  - **Determined to be reviewable by FDA on March 30, 2023**

Active Working Groups (slide 8 of 8)

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

  Context of use: adults diagnosed with RA

  Since April 2022… **Submitted Full Qualification Package to FDA on January 10, 2023**

- **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

  Context of use: adults with clinician-confirmed diagnosis of limited or extensive stage SCLC

  Since April 2022… **a qualitative research study is in progress to evaluate the *NSCLC-SAQ* in persons with SCLC as a precursor to the development of the Qualification Plan**

Working Group Posters

- During Workshop breaks, please view the working group posters at in the hallway outside the meeting room.

- Join us for a reception in the Magnolia Ballroom from 5:30 pm – 7:00 pm this evening. The posters will remain on display in the hallway during the reception.

- All workshop presentations and posters will be posted to the PRO Consortium webpage by the end of May 2023

Publications Since April 2022

- Romero H, DeBonis D, O'Donohoe P, Wyrwich K, Arnera V, Platko J, Willgoss T, Harris K, Crescioni M, Steele S, Eremenco S, on behalf of the Electronic Patient-Reported Outcome Consortium and the Patient-Reported Outcome Consortium. Recommendations for the electronic migration and implementation of clinician-reported outcome assessments in clinical trials. *Value in Health* 2022. https://doi.org/10.1016/j.jval.2022.02.012

- Eremenco S, Chen WH, Blum SI, Bush EN, Bushnell DM, DeBusk K, Gater A, Nelsen L, Coons SJ, on behalf of the PRO Consortium's Communication Subcommittee. Comparing patient global impression of severity and patient global impression of change to evaluate test–retest reliability of depression, non-small cell lung cancer, and asthma measures. *Quality of Life Research* 2022. https://doi.org/10.1007/s11136-022-03180-5

- Newton L, Knight-West O, Eremenco S, Hudgins S, Crescioni M, Symonds T, Reasner DS, Byrom B, O'Donohoe P, Vallow S on behalf of the Patient-Reported Outcome Consortium and the Electronic Clinical Outcome Assessment Consortium. Comparability of a provisioned device versus bring your own device for completion of patient-reported outcome measures by participants with chronic obstructive pulmonary disease: qualitative interview findings. *Journal of Patient-Reported Outcomes* 2022. (https://doi.org/10.1186/s41687-022-00492-5)

Publications Since April 2022……cont.

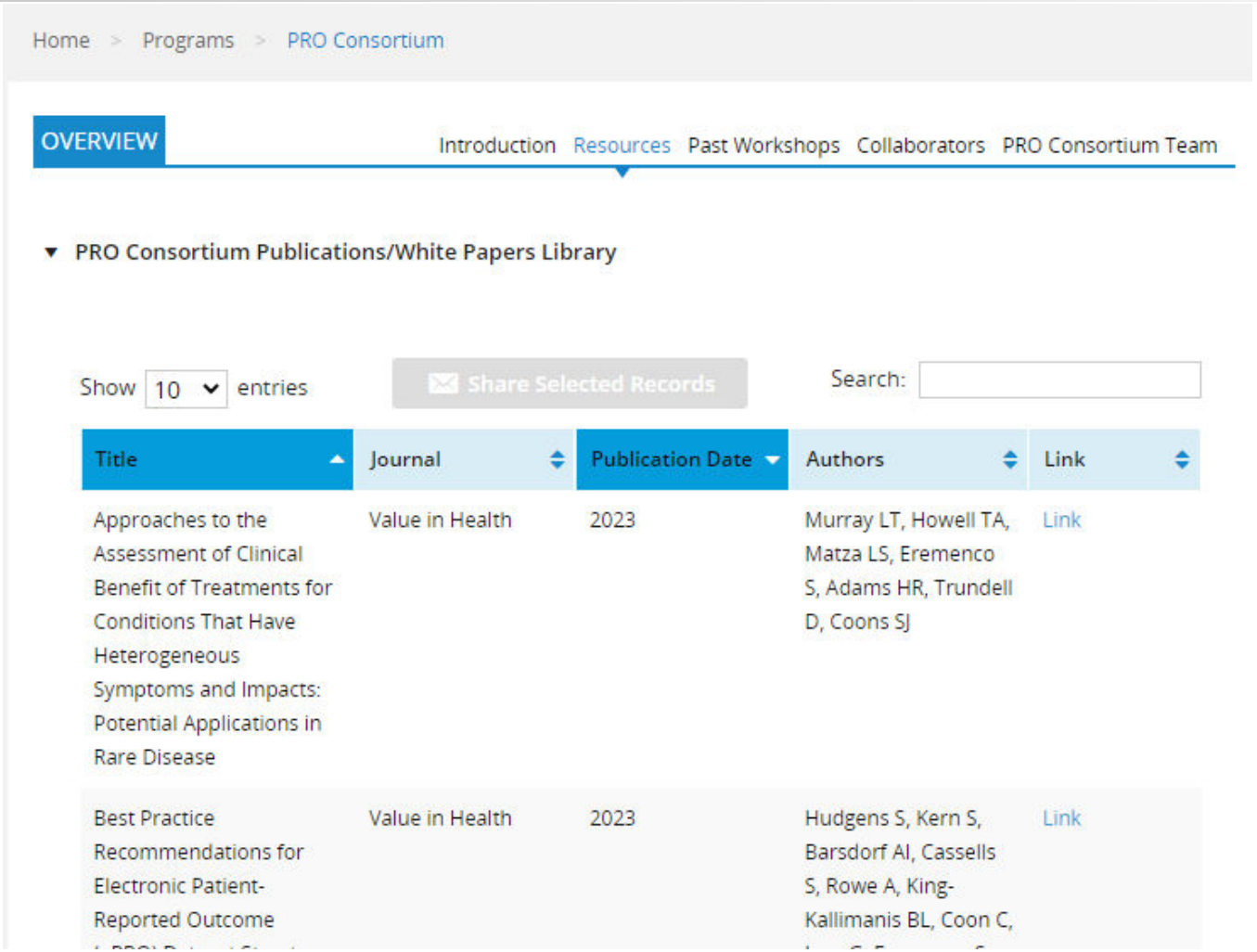
- Hudgens S, Newton L, Eremenco S, Crescioni M, Symonds T, Griffiths PCG, Reasner DS, Byrom B, O'Donohoe P, Vallow S on behalf of the Patient-Reported Outcome Consortium and the Electronic Clinical Outcome Assessment Consortium. Comparability of a provisioned device versus bring your own device for completion of patient-reported outcome measures by participants with chronic obstructive pulmonary disease: quantitative study findings. *Journal of Patient-Reported Outcomes* 2022. (https://doi.org/10.1186/s41687-022-00521-3)

- Murray LT, Howell TA, Matza LS, Eremenco S, Adams HR, Trundell D, Coons SJ. Approaches to the Assessment of Clinical Benefit of Treatments for Conditions That Have Heterogeneous Symptoms and Impacts: Potential Applications in Rare Disease. *Value Health* 2023. doi: 10.1016/j.jval.2022.11.012.

- Hudgens S, Kern S, Barsdorf AI, Cassells S, Rowe A, King-Kallimanis BL, Coon C, Low G, Eremenco S. Best Practice Recommendations for Electronic Patient-Reported Outcome (ePRO) Dataset Structure and Standardization to Support Drug Development. *Value Health* 2023. Feb 25:S1098-3015(23)00060-8. doi: 10.1016/j.jval.2023.02.011. Epub ahead of print.

Since Last Year's Workshop

PRO Consortium Publications Library



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OVERVIEW

Introduction Resources Past Workshops Collaborators PRO Consortium Team

▼ PRO Consortium Publications/White Papers Library

Show 10 entries Share Selected Records Search:

Title	Journal	Publication Date	Authors	Link
Approaches to the Assessment of Clinical Benefit of Treatments for Conditions That Have Heterogeneous Symptoms and Impacts: Potential Applications in Rare Disease	Value in Health	2023	Murray LT, Howell TA, Matza LS, Eremenco S, Adams HR, Trundell D, Coons SJ	Link
Best Practice Recommendations for Electronic Patient-Reported Outcome	Value in Health	2023	Hudgens S, Kern S, Barsdorf AI, Cassells S, Rowe A, King-Kallimanis BL, Coon C,	Link

eCOA: Getting Better Together Initiative Resources

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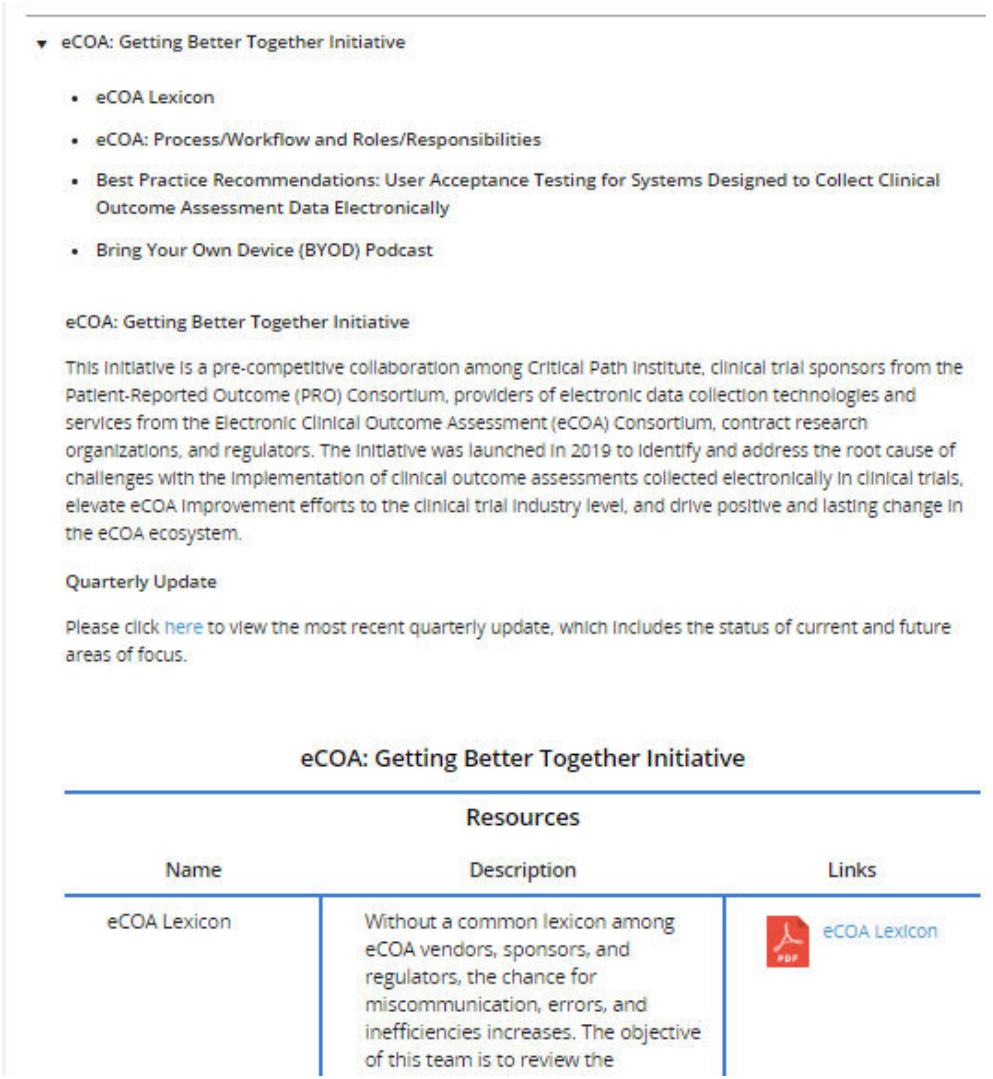

▼ eCOA: Getting Better Together Initiative

- eCOA Lexicon
- eCOA: Process/Workflow and Roles/Responsibilities
- Best Practice Recommendations: User Acceptance Testing for Systems Designed to Collect Clinical Outcome Assessment Data Electronically
- Bring Your Own Device (BYOD) Podcast

eCOA: Getting Better Together Initiative

This initiative is a pre-competitive collaboration among Critical Path Institute, clinical trial sponsors from the Patient-Reported Outcome (PRO) Consortium, providers of electronic data collection technologies and services from the Electronic Clinical Outcome Assessment (eCOA) Consortium, contract research organizations, and regulators. The initiative was launched in 2019 to identify and address the root cause of challenges with the implementation of clinical outcome assessments collected electronically in clinical trials, elevate eCOA improvement efforts to the clinical trial industry level, and drive positive and lasting change in the eCOA ecosystem.

Quarterly Update

Please click here to view the most recent quarterly update, which includes the status of current and future areas of focus.

eCOA: Getting Better Together Initiative

Resources

Name	Description	Links
eCOA Lexicon	Without a common lexicon among eCOA vendors, sponsors, and regulators, the chance for miscommunication, errors, and inefficiencies increases. The objective of this team is to review the	eCOA Lexicon

Summary of PRO Consortium Accomplishments Since April 2022

- 2 QPs accepted
- 1 QP determined to be reviewable and undergoing FDA review (submitted prior to April 2022)
- 5 QPs submitted between July 2022 and March 2023
  - 3 QPs determined to be reviewable and undergoing FDA review
  - 2 QPs undergoing reviewability assessment
- 1 FQP submitted and undergoing reviewability assessment
- 6 publications

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
- E-card for Stephen Joel Coons
- **Workshop Feedback Form Link:** https://forms.office.com/Pages/ResponsePage.aspx?id=pww68Rj7PEmvbepIXnH7MSOfB0LVOIdIszdh36Pbn6VUQllGVE9NVUo1U0hSOUoxVzNFVEszNjQxSi4u

* Emailed to registrants on Monday April 17, 2023

Workshop Agenda

Day 1 - Wednesday April 19, 2023: 7:30 am - 7:00 pm	
7:30 – 8:30 am	Registration and Breakfast – Cypress Ballroom
8:30 – 8:50 am	Welcome and Patient-Reported Outcome Consortium Update
8:50 – 10:20 am	Session 1: Update from FDA
10:20 – 10:45 am	Break
10:45 – 12:15 pm	Session 2: Patient Experience Data: Use in Regulatory Decision Making and Labeling
12:15 – 1:15 pm	Lunch
1:15 – 2:45 pm	Session 3: Qualitative Methods in Clinical Trials: Opportunities and Challenges
2:45 – 3:10 pm	Break
3:10 – 5:00 pm	Session 4: Applying Methodologies for Estimating Meaningful Within-person Change Thresholds: Considerations and Alternative Approaches
5:00 – 5:10 pm	Day 1 Closing Remarks / Adjourn
5:30 – 7:00 pm	Reception and Poster Session – Magnolia Ballroom

Day 2 – Thursday April 20, 2023: 7:30 am – 12:15 pm	
7:30 – 8:30 am	Registration and Breakfast – Cypress Ballroom
8:30 – 10:00 am	Session 5: Strategies for Use of COAs in Rare Disease Pediatric Populations
10:00 – 10:25 am	Break
10:25 – 11:55 am	Session 6: eCOA: COA Program Projects and Collaborations Update
11:55 – 12:15 pm	Workshop Wrap Up / Adjourn

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

Questions?

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

Please contact:

Sonya Eremenco: seremenco@c-path.org