

Rare Disease Clinical Outcome Assessment Consortium

13th Annual PRO Consortium Workshop – Held Virtually on April 13-14, 2022



Launch of the Rare Disease Clinical Outcome Assessment (COA) Consortium

- The Rare Disease COA Consortium was launched on January 1, 2022. The inaugural Coordinating Committee meeting took place on February 17, 2022.

Mission Statement

- To enable precompetitive, multi-stakeholder collaboration aimed at identifying scientifically sound tools and methodologies for collecting clinically meaningful outcomes data in treatment trials for rare diseases.

Vision Statement

- To be an essential catalyst in medical product development for measuring what matters to people with rare disease and their families.

Member Firms



FDA Liaison

Naomi Knoble, PhD, Division of Clinical Outcome Assessment

Clinical Experts

Heather Adams, PhD, University of Rochester

Kiera N. Berggren, MA/CCC-SLP, MS, Virginia Commonwealth University

Julie Eisengart, PhD, University of Minnesota

Other Representation

National Institutes of Health, National Center for Advancing Translational Sciences

National Institute of Mental Health

Patient-Centered Outcomes Research Institute

Rare Disease COA Resource Development

Gap Analyses for Selected COA Subdomains

- Self-Care, Gross Motor Function, Fine Motor Function
 - 68 COAs identified across these domains
 - Delivered in batches from March – June 2021
- Communication/Language
 - 23 COAs identified
 - Delivered in August 2021

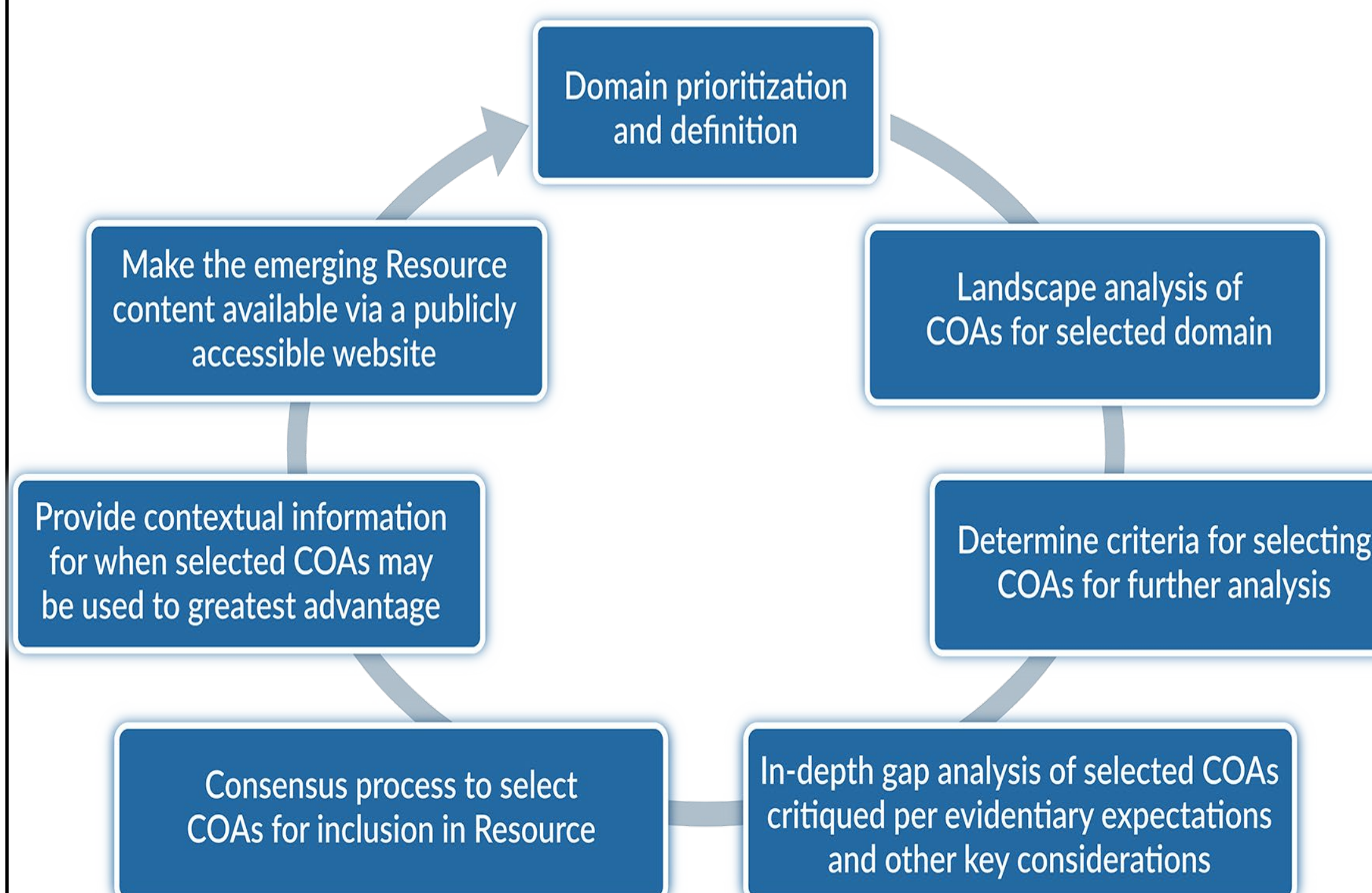
Review of Gap Analysis and Selected COAs

- A Rare Disease COA Resource Development Subcommittee was formed to select COAs for inclusion in the Rare Disease COA Resource
- Advisory Panels were convened to review results of gap analyses and selected COAs. Panel membership:
 - Self-Care, Gross Motor Function, Fine Motor Function Advisory Panel: Occupational Therapist, Physical Therapist, Caregiver Representative
 - Communication/Language Advisory Panel: Occupational Therapist, Speech and Language Pathologist, Caregiver Representative
 - A consensus process between each Advisory Panel and the Rare Disease COA Resource Development Subcommittee determined the final COAs to include in the Rare Disease COA Resource

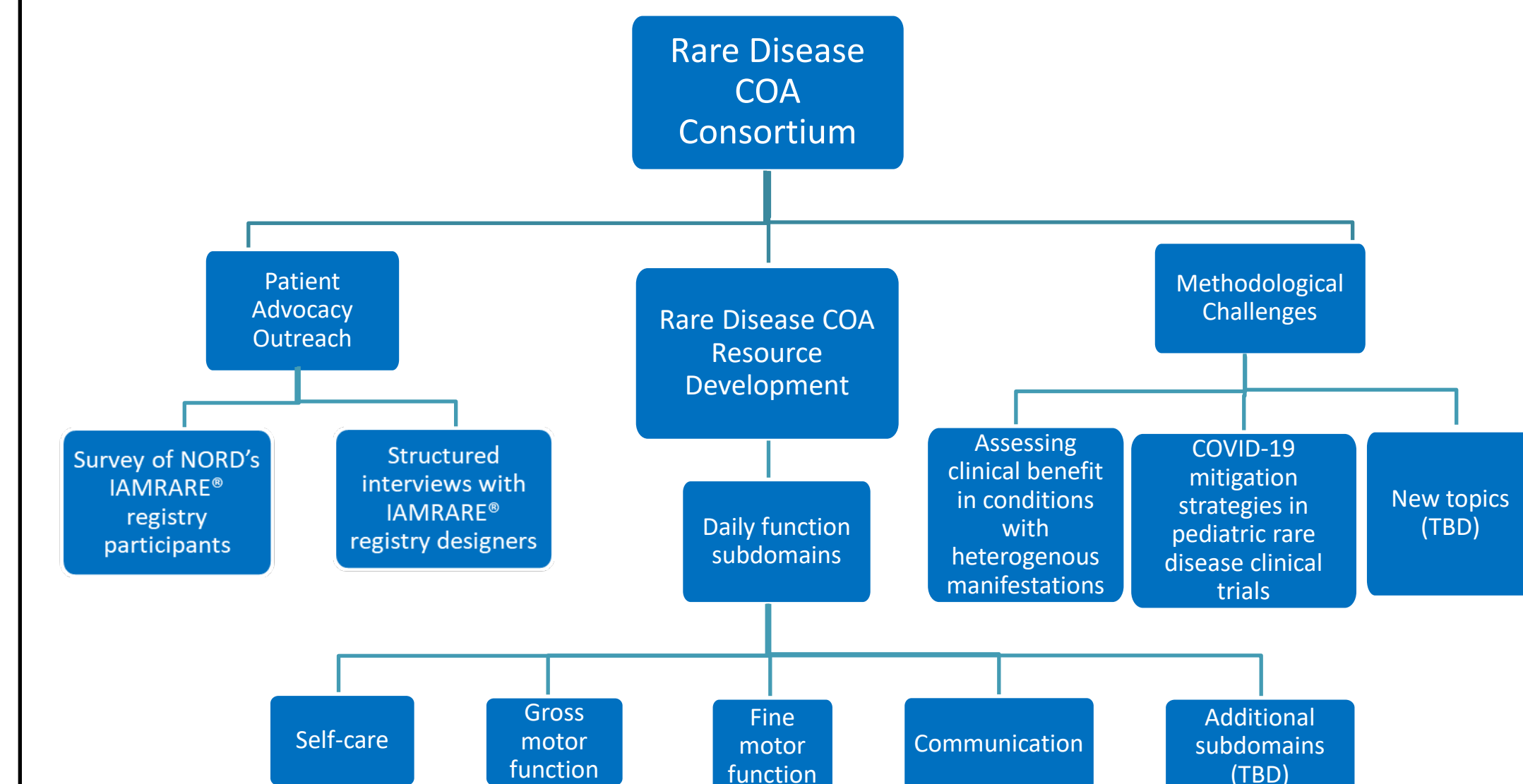
Rare Disease COA Resource Development

- Prototype developed for the Rare Disease COA Resource
- Next Steps: C-Path is working with a website developer to build the website that will house the Rare Disease COA Resource*

Rare Disease COA Resource: Ongoing Development Process



Rare Disease COA Consortium Work Structure



Patient Advocacy Outreach: Exploring NORD's IAMRARE® Registry Partnerships

Aim: To better understand how specific domains and COAs are selected for inclusion in patient registries

- Phase 1: NORD launched an online survey of their 40+ IAMRARE® registry partners in December 2020 (n=16 responded)
- Phase 2: Structured interviews with respondents of Phase 1 (n=14)
- Phase 3: Information from Phases 1 – 2 will be collated into a final report

Addressing Methodological Challenges

- Manuscript titled "Approaches to the Assessment of Clinical Benefit of Treatments for Conditions that Have Heterogeneous Symptoms and Impacts: Potential Applications in Rare Disease" was submitted to *Value in Health* and is currently under revision for resubmission.

COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials Team

- Established to identify challenges and successful mitigation solutions throughout the lifecycle of a pediatric rare disease clinical trial.
- May 2021: Virtual, live workshop presented to focus on in-person and remote assessment in pediatric rare disease clinical trials.
- Workshop recording and supporting documentation available at <https://c-path.org/view-now-covid-19-mitigation-strategies-in-pediatric-rare-disease-clinical-trials-virtual-workshop/>

Rare Disease COA Consortium Membership

- New members welcome! Please contact Lindsey Murray at lmurray@c-path.org for more information.