

Rare Disease Clinical Outcome Assessment Consortium

14th Annual PRO Consortium Workshop – April 19-20, 2023



Mission

- 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.

Scientific Strategy

- Expand the Rare Disease Clinical Outcome Assessment (COA) Resource into new domains of publicly available COAs identified as potentially fit-for-purpose efficacy endpoint measures in treatment trials across multiple rare diseases;
- Promote collaboration and education, and share learnings among member firms and consortium members to expedite innovations in rare clinical trial science; and
- Advance solutions for methodological and measurement challenges in rare disease by engaging teams of experts focused on dissemination.

Member Firms



FDA Liaison

Naomi Knoble, PhD, Associate Director, Rare Disease Measurement Science

Industry Co-Director

Dawn Phillips, PT, MS, PhD, REGENXBIO

Academic Partners

Heather Adams, PhD, University of Rochester

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

Julie Eisengart, PhD, University of Minnesota

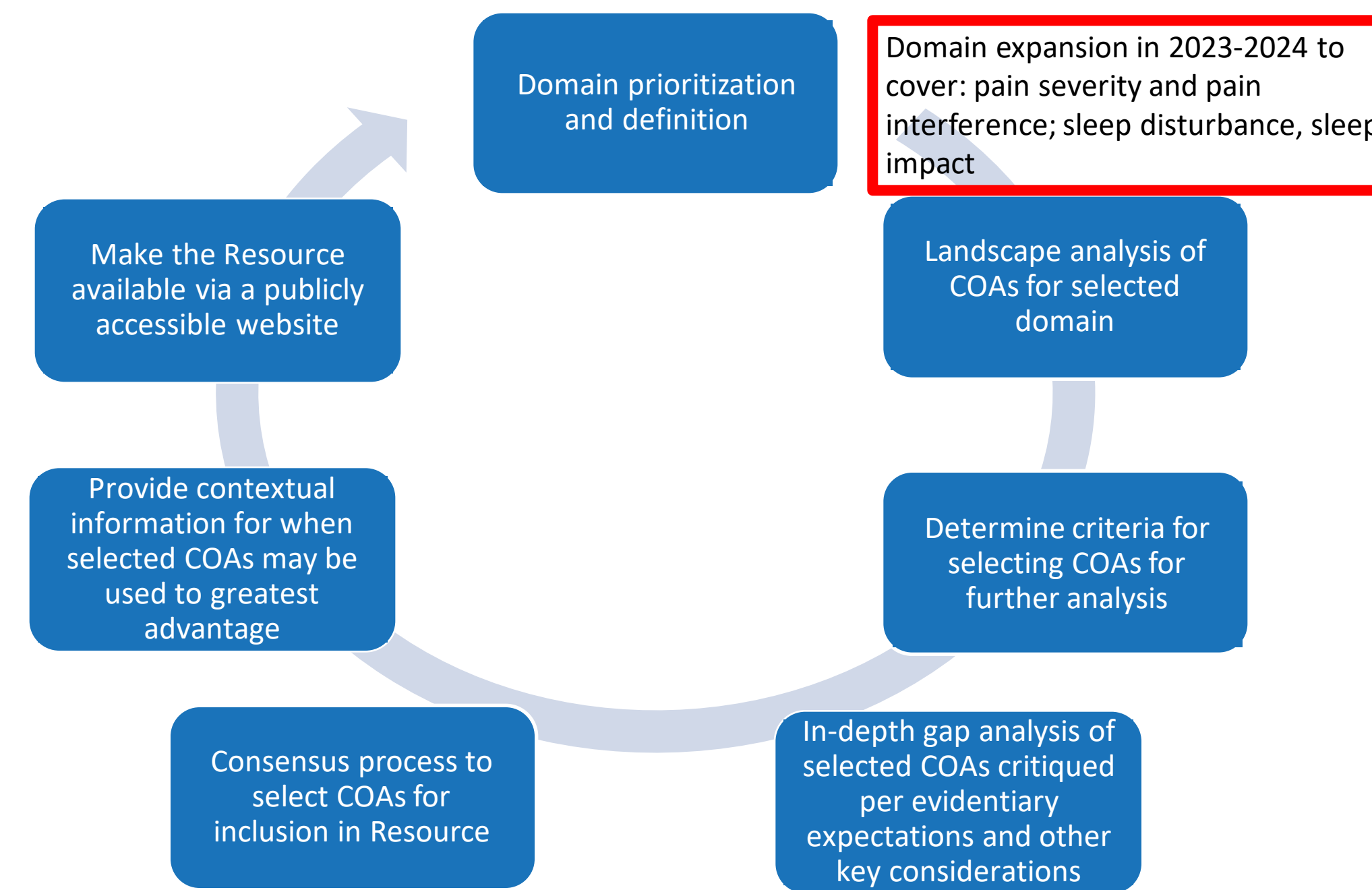
Other Representation

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 Process



Review of Gap Analysis and Selected COAs (Completed)

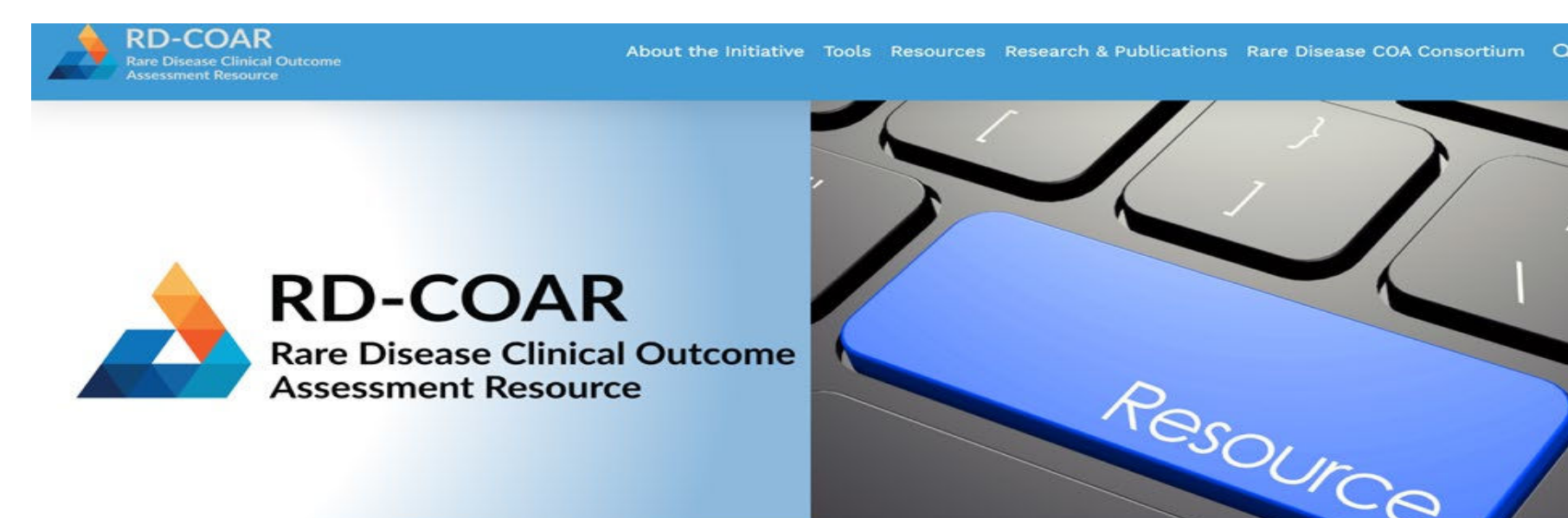
- A Rare Disease COA Resource Development Subcommittee was formed to select COAs for inclusion in the Rare Disease COA Resource
- Advisory Panels were convened to review results of gap analyses and selected COAs. Panel membership:
 - Self-care, gross motor function, fine motor function Advisory Panel: 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

Selected COA Subdomains of Daily Function (Completed)

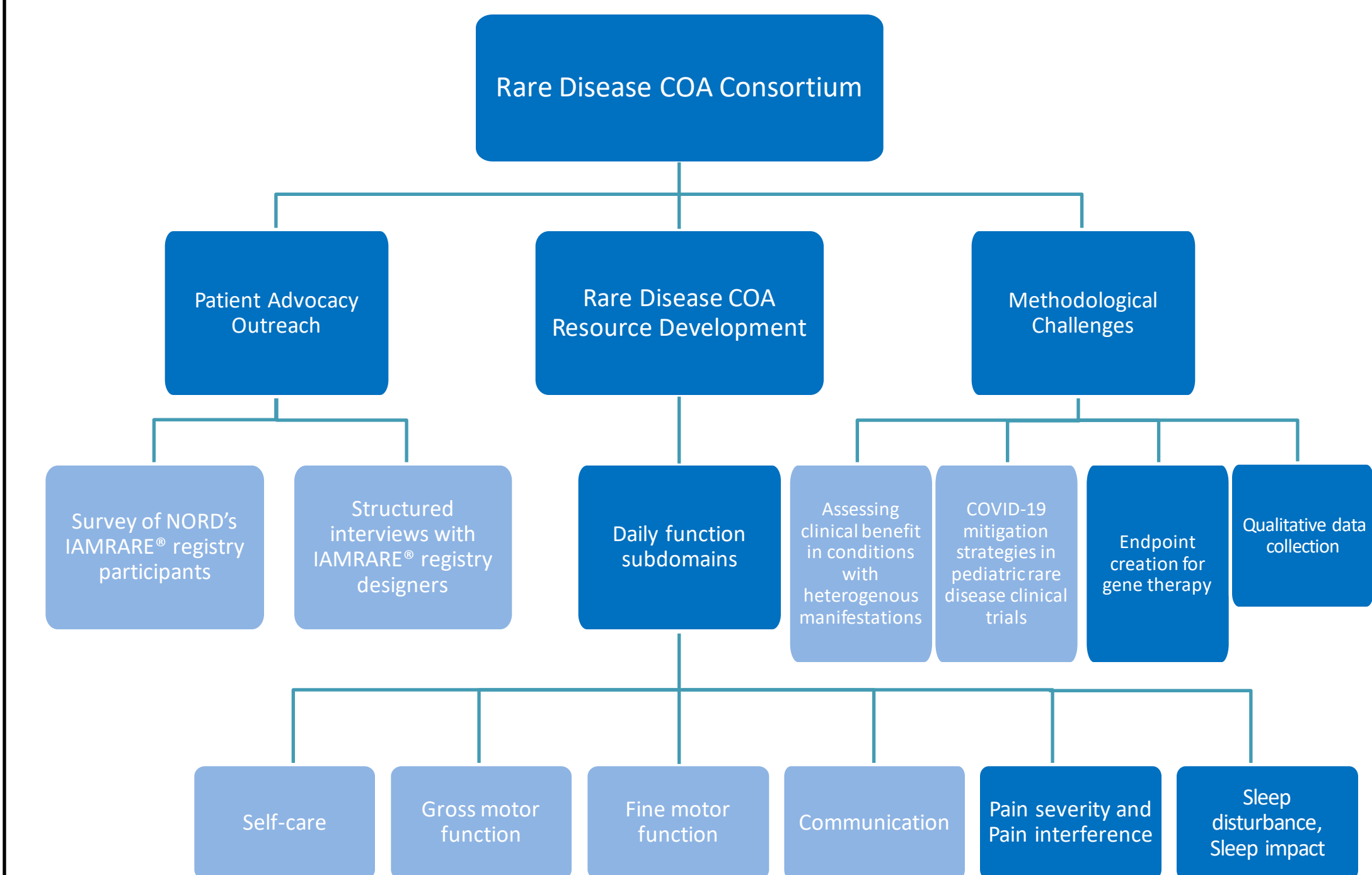
- Self-care, gross motor function, fine motor function
 - 46 COAs included
- Communication/language
 - 8 COAs included

Anticipated Launch of Rare Disease COA Resource

- Q2 2023



Rare Disease COA Consortium Work Structure



Grayed boxes indicate activity completion. Blue boxes indicate active efforts.

RD-COAC Teams

Qualitative Data Collection

- Aim: to provide recommendations for inclusion of exit interviews when designing a clinical trial. A publication is planned as a deliverable, which will include articulation of a clear definition of an exit interview, a template that outlines decision-making steps, points of consideration and best practices when including exit interviews in a clinical trial.

Crafting Endpoints for Gene Therapy Trials

- Aim: to provide recommendations for gene therapy clinical trial endpoints, specifically addressing the intersection of what is meaningful to patients and what is important to regulators.

Forthcoming Manuscript

- Establishment of the Rare Disease COA Consortium

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 accepted for publication by *Value in Health*. Anticipated publication in Spring 2023.

Rare Disease COA Consortium Membership

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