Establishment of the Rare Disease COA Consortium

12th Annual PRO Consortium Workshop – Held Virtually on April 14-15, 2021

Rare Disease Clinical Outcome Assessment (COA) Consortium 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.

Rare Disease COA Consortium Vision Statement

- To be an essential catalyst in medical product development for measuring what matters to people with rare diseases.

Overview of Goals

- Continue the creation and curation of the Rare Disease COA Resource on publicly available COAs identified as potentially fit-for-purpose as efficacy endpoint measures for the identified domains in treatment trials across multiple rare diseases;
- Identify and prioritize other challenges related to the assessment of clinical benefit in rare disease trials and work toward attaining consensus-based solutions; and
- Advance the science of clinical outcome assessment in rare disease.

Membership will provide the opportunity to:

- Participate in a pre-competitive, multi-stakeholder collaboration with COA scientists, regulatory authorities, clinical experts, and patients/patient advocates;
- Collaborate with regulatory authorities and other key stakeholders on methodological challenges in rare disease clinical trials;
- Participate in learning opportunities with subject matter experts on issues specific to rare disease research (e.g., statistical, methodological, regulatory);
- Participate in the identification and prioritization of important and meaningful symptom and functional domains impacted by multiple rare diseases;
- Gain early insight into the creation and curation of the Rare Disease COA Resource;
- Contribute to consensus building efforts to address challenges related to the assessment for clinical benefit in rare disease trials; and
- Shape the future of rare disease COA research by participating in the publication of peer-reviewed manuscripts and review articles, as well as scientific presentations at major meetings.

Launch of the Rare Disease COA Consortium

- We anticipate launching the Rare Disease COA Consortium in July 2021. New members welcome! Please contact Lindsey Murray at lmurray@c-path.org for more information.

Rationale for Establishment of the Rare Disease COA Consortium

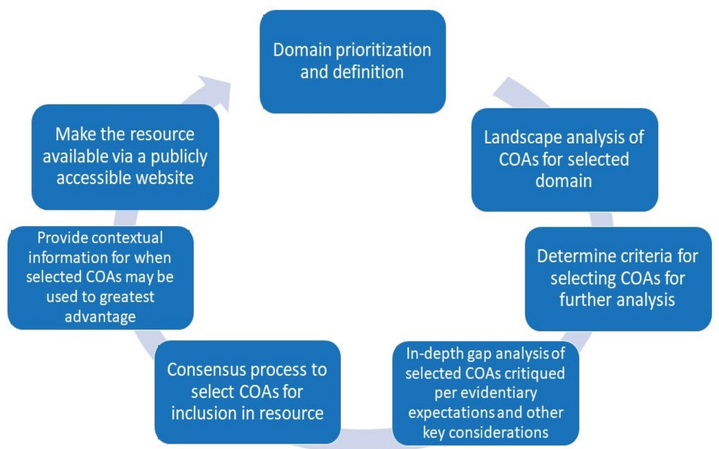
- The FDA's Center for Drug Evaluation and Research (CDER) awarded a one-year grant (U01FD006882) to Critical Path Institute (C-Path) with NORD as a sub-awardee on September 1, 2019. A no-cost extension was approved on July 17, 2019, extending funding through August 31, 2021.

  "This cooperative agreement will provide funding to establish a rare disease consortium focusing on clinical outcome assessments appropriate for use in drug development to demonstrate clinical benefit."
- The first step was formation of the Rare Disease Subcommittee within C-Path's Patient-Reported Outcome (PRO) Consortium.

Rare Disease COA Resource Development

- A **domain approach** is being used to identify COAs that might be fit-for-purpose for use as endpoint measures in treatment trials for multiple rare diseases.
- Initial efforts will focus on **pediatrics** and non-oncologic conditions; other areas will be addressed subsequently.
- **Daily function** was selected as the initial domain of interest; gap analyses for the subdomains of **self-care, gross motor function, and fine motor function** have been initiated, as well as landscape analyses for **communication/language**.

Overview of Rare Disease COA Resource Development



Addressing Methodologic Challenges

- *Assessing Clinical Benefit of Treatments for Conditions with Heterogeneous Manifestations*
- *COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials*

Rare Disease Subcommittee Participants

Grant Sub-Awardee
National Organization for Rare Disorders (NORD)

PRO Consortium Members	
AbbVie	Otsuka Pharmaceutical
Amgen	Sanofi
AstraZeneca	Takeda Pharmaceuticals
Daiichi Sankyo, Inc.	UCB Pharma
Genentech/Roche	
Advisory Members	
Aeglea BioTherapeutics, Inc	Horizon Therapeutics
Agios Pharmaceuticals Inc.	Ionis Pharmaceuticals, Inc.
Akcea Therapeutics	Lysogene
Apellis Pharmaceuticals, Inc.	MeiraGTx, LLC
Applied Therapeutics	Momenta Pharma
argenx US, Inc.	Neurocrine Biosciences, Inc.
Astellas Pharma Inc.	Ovid Therapeutics, Inc.
Audentes Therapeutics	PellePharm, Inc.
BioMarin Pharmaceutical	REGENXBIO Inc.
bluebird bio, Inc.	Sangamo Therapeutics
BridgeBio	Sarepta Therapeutics
Cabaletta Bio, Inc.	Ultragenyx Pharmaceutical, Inc.
Harmony Biosciences, LLC	

FDA Representation
Division of Clinical Outcome Assessment
Division of Neurology Products I
Office of Biostatistics
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine; Division of Rare Diseases and Medical Genetics
Office of Combination Products
Office of Strategic Programs
Patient-Focused Drug Development Program

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