

C-Path's Rare Disease Clinical Outcome Assessment Consortium Launches Rare Disease COA Resource

The Rare Disease COA Resource aims to simplify COA selection for use in rare disease drug development.



TUCSON, Ariz., September 12, 2023 — The Rare Disease Clinical Outcome Assessment (COA) Consortium at [Critical Path Institute](#) (C-Path), a public-private partnership focused on optimizing COA selection during drug development for rare diseases, today announced the launch of the [Rare Disease Clinical Outcome Assessment \(COA\) Resource](#). The Rare Disease COA Resource provides information on published COAs that have the potential to be used to support efficacy endpoints in treatment trials for rare diseases.

More than 350 million people worldwide are living with a rare disease, half of whom are children, and less than 10% of the 7,000 known rare diseases or conditions have approved treatments. Identifying COAs to measure clinical benefit of treatment in rare disease clinical trials can be a difficult, resource intensive process. The Rare Disease COA Resource seeks to accelerate drug development by freely providing information on published COAs that may have applicability for use in drug development programs across multiple rare diseases.

“We’re excited to launch the new Rare Disease COA Resource to the public,” said Lindsey Murray, Ph.D., Executive Director of the Rare Disease COA Consortium. “We hope to dramatically reduce the considerable time and cost associated with identification of relevant COAs for a given outcome and make COA selection for clinical trials, natural history studies, and patient registries more efficient.”

Selected COAs were identified through an iterative process of landscape analysis, gap analyses, and advisory panels which included external clinical experts and caregiver representatives. Evidence from the extensive gap analysis on each COA included in this resource can be viewed for each tool individually or in comparison across several tools for a given outcome to facilitate COA selection for an individual research program. The Rare Disease COA Resource can also inform patient advocacy groups in the identification of COAs available to measure outcomes of interest in patient registries and natural history studies.

With funding support provided by the U.S. Food and Drug Administration (FDA), work toward developing the Rare Disease COA Resource has been underway since 2019. The first iteration of the resource focuses on the assessment of motor functioning (e.g., gross, fine), self-care, and communication (e.g., expressive, receptive language) in pediatric, non-oncologic rare disease populations. Inclusion of assessments of these outcomes in adult populations is planned for future Resource iterations.

At the Duke-Margolis Center for Health Policy Rare Disease Endpoint Advancement Pilot Program Workshop: Novel Endpoints for Rare Disease Drug Development, held on June 7, 2023, Naomi Knoble, Ph.D., Associate Director, Division of Clinical Outcome Assessment (DCOA), Center of Drug Evaluation and Research (CDER) at FDA, said, “The Rare Disease COA Resource reflects FDA’s commitment to accelerating rare disease medical product development through a dynamic precompetitive partnership of the C-Path Rare Disease COA Consortium, clinical experts, academic subject matter experts, industry members, NIH colleagues, and FDA contributing to the development of this resource. While a listing in this Resource is not a reflection of FDA’s endorsement of a COA, it is an essential starting point for a conversation, and creates critical efficiencies for measuring clinical outcomes that matter in rare disease clinical trials. We’re excited for the launch of the Rare Disease COA Resource and the positive impact it will ultimately have for people living with rare diseases, as they are the number one stakeholders for everything we are doing.”

“While the COA field has traditionally sought to develop and evaluate assessments for use in specific therapeutic programs, the Rare Disease COA Resource shows that in rare diseases, where time and funding are both limited, there may be existing assessments that could be considered. Leveraging such assessments in research programs may be a step towards the ultimate goal of making safe and effective therapies available to the individuals who need them more quickly,” said Cheryl Coon, Ph.D., Vice President for C-Path’s Clinical Outcome Assessment Program.

To learn more, visit c-path.org/programs/rd-coac.

To access the Resource, visit rdcoas.c-path.org.



About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the [FDA’s Critical Path Initiative](#). **C-Path’s mission is to lead collaborations that advance better treatments for people worldwide.** Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path’s global headquarters is located in Tucson, Arizona and C-Path’s Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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