C-Path Launches Rare Disease Clinical Outcome Assessment Consortium

The Rare Disease COA Consortium aims to advance patient-focused assessment of clinical benefit in treatment trials for rare diseases

TUCSON, Ariz., January 13, 2022 — Critical Path Institute (C-Path) today announced the launch of the Rare Disease Clinical Outcome Assessment (COA) Consortium, a public-private partnership focused on optimizing COA selection during medical product development for rare diseases. The Rare Disease COA Consortium is a collaborative effort among C-Path, the U.S. Food and Drug Administration (FDA), the National Organization for Rare Disorders (NORD), other government agencies, as well as key partners in the biopharma, clinical research and patient communities that are seeking treatments for rare diseases.

More than 350 million people worldwide are impacted by one of more than 7,000 rare diseases. These conditions result in substantial burden on patients, families, communities and global health care systems. Nearly 50% of rare diseases impact children, of whom 30% will die by age 5. It is currently estimated that less than 10% of rare diseases have approved treatments. COAs to measure clinical benefit of treatment do not exist or remain unidentified for the majority of rare diseases. With no approved therapies for most rare diseases, and considerable uncertainty around appropriate treatment trial endpoints, a significant unmet public health need remains unfulfilled. The Rare Disease COA Consortium seeks to address this need with the creation of the Rare Disease COA Resource that will identify and describe potentially fit-for-purpose publicly available COAs for use in treatment development programs across multiple rare diseases.

“With the increased emphasis on patient centeredness in clinical trials, selection of COAs that assess outcomes that are meaningful is critical,” said Lindsey Murray, Ph.D., Executive Director of the Rare Disease COA Consortium. “The vision of the Rare Disease COA Consortium is to catalyze medical product development by measuring what truly matters to people with rare diseases and their families.”

The underlying premise for the Rare Disease COA Resource is that, for rare diseases that share common characteristics, existing COAs may be used or modified for use as endpoint measures for treatment trials. By identifying existing measures that are potentially fit-for-purpose, the considerable time and cost associated with the development of new COAs may be reduced. With the support of funding provided by 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 daily function in pediatric, non-oncologic rare disease populations.

Additionally, work to identify and prioritize other challenges related to the assessment of clinical treatment benefit in rare disease therapeutic trials has been initiated, and will continue to be a focus of the new consortium. Initial efforts included identifying methods for assessing clinical treatment benefit in conditions with heterogeneous manifestations and impact profiles, and a webinar on strategies to mitigate the impact of COVID-19 in pediatric rare disease clinical trials.

“We are exceptionally grateful to FDA for the vision and funding that enabled the Rare Disease COA Consortium to be established,” said Stephen Joel Coons, Ph.D., Senior Vice President for C-Path’s Clinical Outcome Assessment Program. “It will now be supported by membership fees from biopharmaceutical firms and other stakeholders advocating for or

developing new drugs or biologics for the treatment or cure of rare diseases. We are very pleased this new consortium will join other C-Path consortia and programs aimed at enhancing the health and well-being of individuals with rare disorders."

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

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About C-Path

Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and dozens of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona and C-Path, Ltd. EU is headquartered in Dublin, Ireland, with additional staff in multiple other locations. For more information, visit c-path.org and c-path.eu.

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