C-Path Welcomes Mucopolysaccharidosis Clinical Trial Data Contribution from Children's Hospital of Orange County into RDCA-DAP

TUCSON, Ariz., December 12, 2023 – C-Path (Critical Path Institute) is thrilled to announce a significant addition to its Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®). Children's Hospital of Orange County (CHOC) has generously contributed clinical trial data related to mucopolysaccharidosis (MPS), reinforcing the power of collaboration in advancing rare disease research.

Mucopolysaccharidosis refers to a group of rare genetic disorders characterized by the accumulation of specific complex carbohydrates known as mucopolysaccharides. These conditions impact thousands of individuals worldwide, and research efforts are crucial to understanding and ultimately treating MPS more effectively.

CHOC, a leading institution in pediatric healthcare, has taken a remarkable step by sharing clinical trial data that will significantly enhance the RDCA-DAP's efforts. This collaborative endeavor demonstrates CHOC's unwavering commitment to improving the lives of children and their families, particularly those affected by rare genetic disorders like MPS.

CHOC's Chief Medical Officer, Sandip Godambe, M.D., Ph.D., MBA, expressed his excitement about this collaborative effort, stating, "Our mission at CHOC is to nurture, advance, and protect the health and well-being of children. By sharing our MPS data with RDCA-DAP, we are taking a significant step toward fulfilling this mission. We are confident that our contribution will drive forward the development of innovative treatments for mucopolysaccharidosis."

RDCA-DAP, founded by C-Path and the National Organization for Rare Disorders with support from FDA, is a pioneering platform that fosters cooperation among diverse stakeholders, such as academic institutions, industry leaders, and healthcare organizations. It accelerates the development of treatments for rare diseases by facilitating data sharing, analysis, and collaboration.

In light of CHOC's collaboration with RDCA-DAP, the Critical Path for Lysosomal Diseases (CPLD) Consortium, part of C-Path's efforts, stands to benefit from this partnership. CPLD, which is committed to uniting industry leaders, researchers, regulators, and patient advocacy groups, aims to advance diagnostic and therapeutic approaches for lysosomal diseases. Kanwaljit Singh, M.D., MPH, Executive Director of CPLD, commended this effort, stating, "CHOC's data sharing with RDCA-DAP enhances our pursuit of new treatments and underscores the impact of collaborative innovation in rare disease research."

Collin Hovinga, Pharm.D., M.S., FCCP Vice President of C-Path's Rare and Orphan Disease Programs, expressed his appreciation for CHOC's involvement, saying, "CHOC's contribution of MPS clinical trial data is a significant milestone for RDCA-DAP. Their participation exemplifies the collaborative spirit necessary to drive innovative research and provide hope for those affected by rare diseases."

This collaborative effort between CHOC and RDCA-DAP not only advances research but also exemplifies the potential for groundbreaking discoveries when organizations work together for the greater good. It underscores CHOC's position as a leader in pediatric healthcare and their dedication to contributing to the advancement of rare disease research.

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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About Children's Hospital of Orange County

Children's Hospital of Orange County (CHOC), a pediatric healthcare system based in Orange County, California, is committed to being a leading destination for children's health by providing exceptional and innovative care. CHOC's growing community includes two state-of-the-art hospitals in Orange and Mission Viejo and a regional network of primary and specialty care clinics serving children and families in four counties. CHOC offers several clinical programs of excellence providing the highest levels of care for the most serious pediatric illnesses and injuries, physical and mental. CHOC's research and innovation institutes are focused on translating real patient needs into real-world treatments so every child can live the healthiest and happiest life possible.

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