C-Path Rare and Orphan Disease Conference Highlights Unprecedented Collaborations and Breakthroughs in Rare Disease Research

TUCSON, Ariz., October 3, 2023 — The Critical Path Institute (C-Path) held its highly anticipated Rare and Orphan Disease Conference in September, bringing together leading experts, pharmaceutical industry leaders, regulatory agencies, and patient advocates to have a dynamic dialogue about the latest advancements in rare disease research and drug development. The three-day conference, held at the Marriott Metro Center in Washington, D.C., Sept. 11 – 13, featured a packed agenda of informative sessions, showcases, and networking opportunities.

In his opening address, C-Path CEO, Dan Jorgensen, M.D., MBA, extended a warm welcome to the diverse audience and in a special acknowledgment, Jorgensen expressed heartfelt gratitude to those in attendance who have a personal experience with a rare disease and recognized their enduring strength in the face of the daily challenges posed by their diagnosis. “You are the ones that endure the hardship of a rare disease daily and are hoping for new therapies. We admire your courage and resolve,” he said.

The conference kicked off with an effective dialogue that discussed the “Impact of Rare Disease Initiatives at FDA,” which set the stage for two days of insightful discussions and presentations. Key highlights from the

first day included:

- Industry Perspective on Drug Development in Rare Diseases: An engaging discussion on the industry's role in advancing treatments for rare diseases.
- Accelerating Access to Critical Therapies for ALS: An overview of the scientific landscape surrounding ALS and the potential impact of the "Accelerating Access to Critical Therapies for ALS Act."
- Rare Disease Endpoint Advancement (RDEA) Pilot Program: An in-depth look at the RDEA program and its potential to transform the development of rare disease therapies.
- Showcases: A series of showcases featuring advancements and innovations in rare disease research and drug development for Duchenne muscular dystrophy, ALS, drug repurposing and the Rare Disease Cures Accelerator-Data and Analytics Platform.

Day two of the conference continued to explore critical topics in rare disease research, with highlights including:

- Impact of Rare Disease Initiatives at EMA and Health Canada: An examination of initiatives and strategies at the European Medicines Agency and Health Canada.
- Patient-Focused Drug Development for Rare Diseases: A discussion on what patient-focused drug development means in the context of rare diseases.
- Rare Diseases Aggregated Databases: Insights into how aggregated data can inform drug development research, including a case example from the FA-ICD and RDCA-DAP.
- Regulatory Pathway and Applications in Drug Development: An exploration of regulatory pathways for rare disease drug development.
- De-risking Rare Disease Drug Development: A Livestream presentation that discusses strategies to de-risk rare disease drug development and measure its impact and outcomes.
- RDCA-DAP Showcases: Demonstrations of the power of "FAIR" data and ontology applications in rare epilepsies and Friedreich's ataxia (FA).

The second day ended with breakout sessions focused on Digital Health Technology Trials, increasing diversity and access for clinical trials, novel approaches to trial designs for rare diseases, and rare infections: new endpoints to inform trial design to treat virus BK infections.

Day three was flanked by a panel discussion on the "Global Rare Disease Initiative" led by Cécile Ollivier, M.S., Managing Director of C-Path in Europe. Additionally, the third day featured parallel sessions on diverse topics, providing attendees with a broad spectrum of insights. These breakouts covered disease simulation, informed consent, data sharing, regulatory considerations, and collaborative efforts between C-Path and patients with rare diseases.

The Critical Path Institute Rare and Orphan Disease Conference showcased the remarkable progress made in the field of rare disease research and underscored the importance of collaboration among stakeholders in advancing treatments for these often-overlooked conditions.

"C-Path's unwavering dedication to accelerating drug development and enhancing patient outcomes is resolute," emphasized Dr. Collin Hovinga, Ph.D., VP of Rare and Orphan Disease Programs. "This conference exemplifies our commitment to making a substantial impact within the rare disease community. We steadfastly prioritize collaboration and the sharing of valuable data in rare disease research, consistently advancing our mission one program, data point, tool, and partnership at a time."

The conference's main sessions can be accessed on C-Path's YouTube channel, here. For more information about C-Path and its ongoing initiatives, please visit 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 or email info@c-path.org.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 55% funded by the FDA/HHS, totaling $17,612,250, and 45% funded by non-government source(s), totaling $14,203,111. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.
Specific funding for the establishment of the Rare Disease COA Consortium was provided by FDA grant U01FD006.

Contact:
Kissy Black
C-Path
615.310.1894
kblack@c-path.org