C-Path and MitoAction Announce Data Sharing Agreement to Support Treatment Development in Mitochondrial Disease

TUCSON, Ariz., June 7, 2022 — Critical Path Institute (C-Path) and MitoAction today announced a joint collaboration to incorporate patient data for rare mitochondrial disorders into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®).

The data content is from MitoAction's own mobile app, which enables patients with mitochondrial disorders to track their diseases symptoms and medications, which provides invaluable data on the clinical manifestations and progression of these rare disorders. The data will be integrated into RDCA-DAP®, with work simultaneously continuing with MitoAction to bring in additional data, and develop data-driven solutions that will help accelerate drug development for these rare diseases. By giving patients the opportunity to share their experiences with rare disorders, patients can be empowered to take an active and meaningful role in research, by sharing their daily experiences.

"MitoAction developed their app to collect data directly from patients with rare mitochondrial disorders, which is incredible and our first dataset from a digital app," said RDCA-DAP Scientific Director Alexandre Betourne, Ph.D., Pharm.D. "Everyone working in the mitochondrial community recognizes the importance of data sharing among rare diseases and has been extremely collaborative."

Mitochondrial disease affects approximately 1 out of 4,000 individuals. These rare diseases are a group of debilitating metabolic disorders that can cause a wide range of symptoms such as debilitating physical, development, and cognitive disabilities, poor growth, loss of muscle coordination, muscle weakness and pain, seizures, vision and hearing loss, gastrointestinal issues, learning disabilities, and organ failure. These symptoms can present at any age from infancy until late adulthood. Mitochondrial disease can be difficult to diagnose and is often misdiagnosed.

"As a voice for the mitochondrial disease patient community, MitoAction recognizes how critical patient-reported data is to the therapy development process," said MitoAction CEO Kira Mann. "Through our mobile platform, in partnership with Care3, we are able to provide our families with a way to take an active role in research by simply sharing their day-to-day journey with mitochondrial disease. Our partnership with the RDCA-DAP® will allow us to harness the power of patient data and help keep us moving closer to having FDA-approved therapies. It takes all of us working together, collaborating, and MitoAction is honored to empower our patient community with another means to make a meaningful contribution to research."

C-Path's RDCA-DAP® provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical development. Additionally, the platform includes a framework that supports the rigorous conduct of natural history studies, with attention to established data quality standards, in order to be most useful to clinical trial design and regulatory review. It includes a robust,

integrated database and analytics hub that allows for the aggregation of rare disease data from various sources and the efficient and effective interrogation of that data.

“Accelerating the development of novel therapies for people with rare mitochondrial diseases, like Leigh syndrome requires collaboration, through the participation of organizations like MitoAction,” said Betourne. “This is a laudable step in that direction, and we are thrilled to be partnering with MitoAction. We were introduced to Kira Mann through our collaboration with Cure Mito, and hope to continue our efforts with both foundations to incentivize more data sharing and ultimately develop tools that will accelerate the development of treatments for Leigh syndrome and other mitochondrial disorders.”

MitoAction began in 2005 as an idea and has evolved from a small New England support group to a dynamic, active service organization helping thousands of patients and families across the globe.

Groups interested in contributing data to RDCA-DAP®, may visit, https://c-path.org/rdca-dap or email rdcadap@c-path.org. The platform is now OPEN and accepting applications for use; visit https://portal.rdca.c-path.org to apply and learn more. To access the 2022 RDCA-DAP Webinar series click here; all materials are on free and on demand.

About Critical Path Institute

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 hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) and is 54.2% funded by the FDA/HHS, totaling $13,239,950, and 45.8% funded by non-government source(s), totaling $11,196,634. 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.

About MitoAction

MitoAction is a Boston-based nonprofit whose mission is to improve the quality of life for children, adults, and families living with mitochondrial disease through support, education, outreach, advocacy, clinical research initiatives, and by granting wishes for children affected by mitochondrial disease. . Since its founding in 2005, MitoAction has served families from across the U.S. and the globe, working tirelessly to offer comprehensive, up-to-date, expert resources, information, and support at no cost to any patient, family, clinician, educator, or member of the community. Learn more at www.mitoaction.org.

Media Contacts:

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

Kira Mann
CEO
MitoAction
kira@mitoaction.org