C-Path and Cure Mito Announce Data Sharing Agreement to Support Treatment Development For Leigh Syndrome and Mitochondrial Disease

TUCSON, Ariz., November 9, 2021 — Critical Path Institute (C-Path) and Cure Mito Foundation (Cure Mito) today announced a joint collaboration to significantly promote data sharing and accelerate Leigh syndrome and other rare mitochondrial disease data incorporation into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®).

Leigh syndrome, also known as Leigh disease, is a Mitochondrial disorder that affects about 1 out of 77,000 live births, with symptoms often appearing within the first year of life. It is a debilitating neurological disorder that can affect the development of mobility, posture and mental capacities in children, with these capabilities occasionally being lost after a period of seemingly normal growth. Though unusual, symptoms may not show until the early adult years, while others may show gradual signs throughout infancy.

"Progress towards the establishment of approved therapies for people with Leigh syndrome and other rare mitochondrial diseases is reliant on the participation of organizations like the Cure Mito Foundation," said RDCA-DAP Scientific Director Alexandre Betourne, Ph.D., Pharm.D. "This is a laudable step in that direction, and we are thrilled to be partnering with Cure Mito to promote data sharing, standardization and develop tools that will accelerate the development of treatments for Leigh syndrome and other mitochondrial disorders."

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.

Cure Mito was founded in 2018 as the Cure SURF1 Foundation by a group of families determined to fight for their children's lives with the primary focus of advancing research towards a cure for Leigh syndrome and eventually for mitochondrial disease as a whole.

"Patients enroll in registries and research studies hoping that their participation will matter," said Sophia Zilber, Cure Mito board member. "Cure Mito Foundation recognizes this and is committed to advancing the way data is collected, accessed and used, and empowering the patient community to be informed and active contributors of their data to advance research, treatments and eventually cures. We are proud to partner with C-Path in these important and much needed efforts."

The collaboration between C-Path and Cure Mito establishes the path for future integration of longitudinal data collected by the foundation, as available, along with other clinical outcome assessment data, to be used for the development of tools and solutions that ultimately lead to the development of new treatments for rare disease patients.

Groups interested in contributing data to RDCA-DAP, may visit, 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 **2021 RDCA-DAP Workshop** held on Tuesday, Sept. 14, click here; registration is not required.

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 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.

About Cure Mito Foundation

Cure Mito Foundation is a parent-led 501(c)(3) nonprofit organization that was founded in 2018 as a Cure SURF1 Foundation by a group of families determined to fight for their children's lives. In 2021, after successfully blazing the trail for SURF1 gene therapy at the University of Texas Southwestern Medical Center, the mission of the organization expanded and the name was updated. Cure Mito's primary focus is advancing research towards a cure for Leigh syndrome and eventually for Mitochondrial disease as a whole. Cure Mito is a volunteer organization with 100% of funds used towards research. Patients are at the heart of everything Cure Mito does. For more information please visit: curemito.org.

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