C-Path Receives Data Transfer from The Champ Foundation Registry

The data transfer is a first for natural history data on Pearson syndrome

TUCSON, Ariz., August 20, 2024 — Critical Path Institute (C-Path) has received its first data transfer of natural history data on single large-scale mitochondrial DNA (mtDNA) deletion syndromes from The Champ Foundation. The Champ Foundation Registry is the most comprehensive dataset of individuals with SLSMDS. The transferred data include patient-reported information of condition-specific symptoms, medical history, and standardized assessments of quality of life, care partner burden, and functional outcomes.?

The Champ Foundation is a patient advocacy group dedicated to supporting research to find treatment and a cure for SLSMDS, like Pearson syndrome. The Champ Foundation Registry seeks to improve the natural history understanding of these ultra-rare disorders. By sharing their data with C-Path, researchers will have an increased ability to gain novel insights about Pearson syndrome and SLSMDS.?

"We are excited to collaborate with C-Path to further research on Pearson syndrome and other single large-scale mitochondrial deletion syndromes (SLSMDS)," said Elizabeth Reynolds, Ph.D., Co-Founder and Executive Director of The Champ Foundation. "This data transfer represents a significant step towards better understanding these rare diseases and ultimately finding effective treatments."

C-Path Executive Director Amanda Klein, Pharm.D., from the Mitochondrial and Inherited Metabolic Diseases Task Force under the Institute's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) initiative, stated, "This collaborative task force provides a neutral environment for industry, regulatory agencies, academia, and the patient community to collaborate and actively contribute to advancing solutions aimed at facilitating therapeutic developments for these communities." Klein added, "We are grateful to have The Champ Foundation as a founding member of the Task Force and data contributor to RDCA-DAP."

Patient advocacy groups, like The Champ Foundation, play a crucial role in leading registry data efforts for mitochondrial and inherited metabolic diseases. Their work ensures that patient experiences and outcomes are central to research and regulatory decision-making, ultimately leading to more effective and patient-centered healthcare solutions.

For more information on collaborating with RDCA-DAP's Mitochondrial and Inherited Metabolic Diseases Task Force, and how to contribute data, please email RDCA-DAP at rdca-dapadmin@c-path.org.

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 and C-Path in Europe is headquartered in Amsterdam, Netherlands 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 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.

About the Mitochondrial and Inherited Metabolic Diseases Task Force

The MIMD Task Force is dedicated to three core objectives: standardizing and integrating mitochondrial and inherited metabolic disease data into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®); optimizing clinical trial design and endpoints; and fostering enhanced communication and collaboration among stakeholders, as well as the broader medical and research communities—all with the goal of improving the lives of those affected by these diseases.

Members of the MIMD Task Force include: Astellas Pharma, Inc; Azer Consulting, LLC; Barth Syndrome Foundation; The Champ Foundation, C-Path; CureARS; Cure LBSL Foundation; Cure Mito Foundation; Global Genes: RARE-X Platform; Hope for PDCD; LHON Collective; The Lily Foundation; MEPAN Foundation; Midwestern University; MitoAction; MitoCanada; MitochondriaWorld; National Institutes of Health; Sanford Research; University of Newcastle; Upon Tyne; and Washington University in St. Louis.

About The Champ Foundation

The Champ Foundation is a 501(c)(3) nonprofit organization that was founded in 2015 with a mission to find treatment and a cure for Pearson syndrome and single large-scale mtDNA deletion syndromes (SLSMDS). The Champ Foundation has funded over $2 million in research grants and activities, hosted international family-friendly research conferences, funded and launched a multi-site Natural History Study, and spearheaded the Champ Foundation Registry, the largest repository of data for individuals with SLSMDS. In 2020, The Champ Foundation was selected as part of the Rare As One cohort, and received a multi-year grant from the Chan Zuckerberg Initiative to establish a SLSMDS research network. For more information, please visit thechampfoundation.org or follow us on Facebook, Instagram, or Twitter.??

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