C-Path to Lead New Task Force Aimed at Accelerating Drug Development for Mitochondrial and Inherited Metabolic Diseases

TUCSON, Ariz., January 11, 2024 — Critical Path Institute (C-Path) today announced the launch of a task force focused on accelerating drug development for mitochondrial and inherited metabolic diseases. The task force will lay the groundwork for specific solutions, offering valuable insights that aim to contribute to regulatory decision-making.

C-Path's demonstrated expertise will be leveraged to ensure success, specifically its track record in generating tangible solutions that have accelerated drug development in rare and pediatric indications, utilizing the power of C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) to integrate multiple patient-level data sources. This collaborative task force will provide 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.

"Uniting stakeholders through a neutral convener such as C-Path will enable our community to collaborate better, share data, and accelerate the development of much-needed treatments," explained Sophia Zilber, Cure MITO Foundation board member. "We are grateful to C-Path for their partnership, and collaboration in assembling this initiative, and look forward to the next steps and achievements as this task force takes shape."

Mitochondrial and inherited metabolic diseases can affect one part of the body or many parts, and these effects can be mild or very serious. Symptoms range from fatigue and exercise intolerance to poor growth, muscle weakness, developmental delays, vision/hearing loss, stroke, seizures, heart failure, and kidney failure, among others.

"Through our ongoing experience within different consortia at C-Path, we now hope to accelerate the discovery and development of treatments for patients with mitochondrial diseases. We look forward to our work with C-Path and other members of this task force," says Dima Martini-Drew, M.D., Astellas Medical Affairs Mitochondrial and Rare Disease.

"Finding pathways to effective treatments from the mitochondrial research communities requires unique collaborations across institutions, industry, and researchers," says Gordon Freedman, the organizer of Mitochondria World, a global information source for the emerging field of mitochondrial biology. "C-Path brings the right tools and methods to speed the process for much-needed therapies."

"C-Path is uniquely positioned to lead this new task force," explained Amanda Klein, Pharm.D., C-Path's Executive Director of the Transplant Therapeutics Consortium and lead for this task force. "We thank the communities for recognizing the importance of collaborative projects. We look forward to leveraging our core competencies to provide strategic and tactical guidance, engage relevant stakeholders, and bring diverse expertise to generate the solutions to help patients and their families."

The Institute's core competencies, which include expertise in data management standards, biomarkers, modeling and analytics, regulatory science, and clinical outcome assessments, help de-risk decision-making in drug development and regulatory review of novel therapeutics.

To learn more and inquire about joining C-Path's mitochondrial and inherited metabolic diseases task force, contact rdca-dapadmin@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.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 54% funded by the FDA/HHS, totaling $19,436,549, and 46% funded by non-government source(s), totaling $16,373,368. 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 RDCA-DAP

The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP), a joint initiative by C-Path and NORD with support from FDA, offers a unique platform promoting cooperation among various stakeholders, including academic institutions, industry leaders, healthcare organizations, and patient advocacy groups. It accelerates the development of treatments for rare diseases through data sharing, analysis, and collaboration. For more information, visit c-path.org/rdca-dap.

About Astellas

Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions that combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into VALUE for patients. For more information, please visit our website at astellas.com/en.

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.

About Cure LBSL

Cure LBSL, formerly known as “A Cure For Ellie”, is an international non-profit foundation supporting patients and families affected by LBSL (Leukoencephalopathy with Brain Stem and Spinal Cord Involvement and Lactate Elevation). LBSL is an ultra-rare neurodegenerative disorder, also known as mitochondrial aspartyl-tRNA synthetase deficiency. The foundation mission is to bring awareness, support, and hope to patients and families, and to fund and accelerate research into potential treatments and cures. curelbsl.org

About Cure Mito Foundation

Cure Mito Foundation is a parent-led 501(c)(3) nonprofit organization that was founded in 2018 by a group of families determined to fight for their children’s lives. Cure Mito’s mission is to unite the global Leigh syndrome community to accelerate patient-centered research, treatments, and cures. Cure Mito has an international reach with patients from over 35 countries, currently a part of the Leigh syndrome global patient registry that Cure Mito has developed. Cure Mito funds promising research and strengthens and unites the community through such initiatives as its annual Leigh syndrome symposium and informational website AboutLeighSyndrome.com. Cure Mito is a member of Defense Health Research Consortium, COMBINEDBrain, Global Genes Foundation Alliance, Everylife Foundation Community Congress, and Indo US Rare Organization for Rare Diseases. Cure Mito has been rated as a Top-Rated Nonprofit by

@GreatNonprofits. For more information, please visit: curemito.org or follow Cure Mito on LinkedIn | Twitter | Facebook | Instagram.

About Hope for PDCD

Hope for PDCD was founded by PDCD parents in 2022 with an urgent mission: to cure a fatal childhood disease known as Pyruvate Dehydrogenase Complex Deficiency. All financial gifts are invested wisely: 100% of every dollar donated goes to research and advocacy efforts for PDCD. Hope for PDCD has quickly grown into a collective of volunteer parent board members, scientific advisors, and industry partners. Hope for PDCD aims to fund a multi-million dollar research project into new therapeutics for PDHA1 mutations, newborn screening, standards of care, the first ever PDCD-specific patient registry, biomarkers and clinical endpoints. Hope for PDCD serves the PDCD patient community by offering a state-by-state clinician directory, free genetic testing, resources on the ketogenic diet and more. Find out more at hopeforpdcd.org.

About Midwestern University

Midwestern University is a private, not-for-profit graduate and postgraduate educational institution specializing in the health sciences with 12 colleges located on two campuses. The Illinois campus, located on a 105-acre site in Downers Grove, is home to nearly 3,000 students and the Chicago College of Osteopathic Medicine; the College of Pharmacy, Downers Grove; the College of Health Sciences, Downers Grove; the College of Dental Medicine-Illinois; the Chicago College of Optometry; and the College of Graduate Studies, Downers Grove. The Arizona campus, located on a 156-acre site in Glendale, is home to over 3,900 students and the Arizona College of Osteopathic Medicine; the College of Pharmacy, Glendale; the College of Health Sciences, Glendale; the College of Dental Medicine-Arizona; the Arizona College of Optometry; the College of Veterinary Medicine; the College of Graduate Studies, Glendale; and the Arizona College of Podiatric Medicine. The University is accredited by The Higher Learning Commission, a Commission of the North Central Association of Colleges and Schools. For more information, visit midwestern.edu or call 623/572-3215.

About MitochondriaWorld

MitochondriaWorld is a nonprofit mitochondrial research and advocacy organization that brings together academic investigators, biopharmaceutical companies, clinical groups, and patients. It serves as a nexus for mitochondria specialists and patient groups to build community, drive research, and coordinate large-scale collaborations. The goal of MitochondriaWorld is to breakdown research silos and disseminate scientific insights from global mitochondrial research and clinical communities to accelerate breakthroughs in basic science and the development of metabolic therapies. Join our mission at MitoWorld.org.

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