CACNA1A Foundation to Contribute Data to C-Path's RDCA-DAP, Accelerating Neurological Disease Research and Therapeutic Development

TUCSON, Ariz., May 31, 2023 — Critical Path Institute (C-Path) announced today that the CACNA1A Foundation, a parent-led nonprofit organization dedicated to creating awareness and finding a cure for CACNA1A-genetic related disorders, will be contributing data to the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) managed by C-Path.

CACNA1A is a gene that is essential to brain function. It plays a key role in the communication between neurons. A change in this gene affects the brain's electrical signals and can result in a variety of neurological disorders, including: developmental delays, impaired balance or coordination (ataxia), migraines (including those that are severe and preceded by weakness in one side of the body, also known as hemiplegic migraines), epilepsy, reduced muscle tone (hypotonia), eye movement disorders, reduction in the size and function in the cerebellum (the part of the brain that regulates coordination and balance) and intellectual disability. By contributing data to the RDCA-DAP, the CACNA1A Foundation will provide crucial information that will accelerate research and therapeutic development for these CACNA1A-related disorders.

RDCA-DAP provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical development. Additionally, the platform advances best practices to support 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.

"We are thrilled the CACNA1A Foundation chose to contribute their invaluable data to RDCA-DAP, and thank Dr. Wendy Chung at Columbia for her willingness to share data to our platform for the second time. The CACNA1A natural history study she conducted is a wonderful addition to our growing database in neurodevelopmental disorders, which will help further rare disease research for patients," said RDCA-DAP Executive Director Alexandre Betourne, Ph.D., Pharm.D. "Their commitment to raising awareness and finding a cure for CACNA1A genetic variants is in perfect alignment with C-Path's mission to accelerate drug development and effective treatments for rare diseases. We anticipate that this partnership will bring meaningful progress for patients and their families."

"As a foundation driven by the urgent needs of families impacted by CACNA1A–related disorders, we recognize the power of collaborative research and data sharing," said Lisa Manaster, President of the CACNA1A Foundation. "Contributing our data to RDCA-DAP enables us to join forces with C-Path and other stakeholders, transforming isolated research efforts into a collective push towards understanding and treating these complex neurological conditions. We believe that this partnership represents a significant stride towards our goal of finding a cure and improving the quality of life for those affected by CACNA1A variants. We're not just contributing data — we're growing hope for our community."

This collaboration between the CACNA1A Foundation and C-Path will enhance the understanding of these neurodegenerative diseases, ultimately facilitating the development of innovative treatments. By sharing the data, researchers worldwide can access comprehensive information to aid in their studies and improve patient outcomes.

Organizations interested in contributing data to RDCA-DAP can visit c-path.org/rdca-dap or email rdcadap@c-path.org. The platform is now open and accepting applications for use; visit portal.rdca.c-path.org to apply and learn more.

About CACNA1A Foundation
The mission of the CACNA1A Foundation is to find effective treatments and a cure for CACNA1A-related disorders by building a collaborative network of CACNA1A families, clinicians and scientists to collectively raise awareness and accelerate the understanding, diagnosis and treatment of CACNA1A-related disorders. The Foundation is a 501(c)(3) nonprofit established in 2020 by parents of children with CACNA1A-related disorders.

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.

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