The Foundation for Angelman Syndrome Therapeutics to Contribute Data to C-Path's RDCA-DAP, Accelerating Neurological Disease Research and Therapeutic Development

TUCSON, Ariz., July 19, 2023 — Critical Path Institute (C-Path) announced today that the Foundation for Angelman Syndrome Therapeutics (FAST), a nonprofit organization dedicated to finding a cure for this genetic disorder, have contributed data from the Global Angelman Syndrome Registry to the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), managed by C-Path.

Angelman Syndrome (AS) is a rare genetic disorder characterized by developmental delays, epilepsy and problems with motor coordination and balance. Individuals with AS do not develop functional speech. Additionally, the seizure disorder in individuals with AS can be difficult to treat. Feeding disorders in infancy are common, persisting throughout childhood, with sleeping difficulties often noted as well. AS affects approximately one in 15,000 births.

RDCA-DAP provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical medical therapies. 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.

The Global Angelman Syndrome Registry collects data on over 2,000 individuals across 89 countries, and in multiple languages. By contributing data to RDCA-DAP, FAST will provide crucial information that will accelerate research and therapeutic development for AS.

"We are honored that FAST chose to contribute their invaluable data to RDCA-DAP and thank Meagan Cross at FAST Australia for her willingness to share the data," said RDCA-DAP Executive Director Alexandre Betourne, Ph.D., Pharm.D. "Data from the Global Angelman Syndrome Registry is a great addition to our growing database in neurodevelopmental disorders and our second Angelman dataset, which will help further accelerate drug development for individuals living with rare diseases."

"We are thrilled to embark on this collaboration with Critical Path Institute and RDCA-DAP," said Meagan Cross, FAST Board Chairperson. "This collaboration allows us to tap into the incredible potential of caregiver-entered data and create opportunities for significant advancements. Together, we look forward to positively impacting outcomes for patients with Angelman syndrome and other rare diseases."

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 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 .

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About FAST

FAST Australia launched in September 2010. Inspired by the mission and energy of FAST in the United States we are driven to address the lack of research and understanding into Angelman syndrome in Australia. Our combined energies create a powerful force in the search for therapeutic interventions and a cure to treat Angelman syndrome. In an age where Angelman syndrome therapeutics are being developed on a global scale, FAST is working to make sure communities across the world are organized and prepared to participate in studies for potential therapeutics. FAST has affiliates in Canada, France, Italy, Latin America (Argentina, Chile, Colombia), Spain and the UK. Learn more at https://cureangelman.org.au.

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