C-Path Partners with FARA to Fortify RDCA-DAP and Further Accelerate Drug Development with new Friedreich's Ataxia Data

The team also announced the closure of the FA-ICD portal

TUCSON, Ariz., June 20, 2024 — Critical Path Institute (C-Path), a leader in accelerating drug development for rare diseases, today announced the targeted integration of additional Friedreich's ataxia (FA) datasets into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) as part of a partnership with Friedreich's Ataxia Research Alliance (FARA).

This update includes data from two natural history studies; the FA-CHILD study, which focuses on pediatric patients with FA, and an expanded version of the FA-COMS study, which now encompasses over 20 years of data collection and spans multiple years among 1,450 patients with FA throughout the U.S., Canada, Australia, New Zealand and India.

In addition, the Friedreich's Ataxia Integrated Clinical Database (FA-ICD) platform for data access will be discontinued to centralize and improve access to FA data in RDCA-DAP, which provides advanced security and analytical tools. Conducted in collaboration with FARA, this comprehensive integration is set to enhance the impact and efficiency of research efforts directed at combating FA, a debilitating, life-shortening, degenerative neuromuscular disorder, exemplified by the contribution of the FARA data through RDCA-DAP to augment the regulatory evidence successfully presented for the first disease modifying treatment for this disease.

The FA-ICD, which contains critical data from numerous clinical trials and a detailed natural history study, was initially managed by C-Path as part of its online data repository. Under the new arrangement, the FA-ICD dataset along with other new FA datasets can be accessed by the research community. The full integration into RDCA-DAP strengthens the platform's capabilities, ensuring a more effective use of data for research and development.

RDCA-DAP, lauded for its robust infrastructure for data aggregation and analytics, supports the characterization and advancement of therapies for rare diseases.

"The incorporation of FA-ICD into RDCA-DAP substantially expands our data resources and sharpens our analytical precision," explained Alexandre Bétourné, Pharm.D., Ph.D., Executive Director of RDCA-DAP. "With this enhanced scope and clarity, we're better equipped to uncover insights that drive the development of effective therapies for Friedreich's ataxia."

Bétourné also highlighted the benefits of this strategic integration, noting, "By leveraging the comprehensive data from FA-ICD, now fully integrated within RDCA-DAP, we are better equipped to accelerate the pace of discovery, fostering the development of novel therapeutic approaches and substantially improving patient outcomes."

Jennifer Farmer, CEO of FARA, emphasized the significance of this partnership and importance of sharing patient contributed data. "FARA is excited to continue our collaboration with C-Path and foster data sharing from natural history studies and clinical trials to enhance research and analysis aimed at improving outcomes and developing treatments for Friedreich's ataxia. We believe in making patient contributions of data as

meaningful as possible and one way to honor this is by ensuring that such data is available to the research community through a secure and robust platform."

"RDCA-DAP's extensive resources can amplify the impact of the existing data and foster new discoveries in FA research. This collaboration will build upon our strong, sustained, and productive partnership with the C-Path team," said Farmer.

Looking ahead, C-Path anticipates that this integration will not only streamline research efforts but also enhance the global research community's access to high-quality, actionable data, potentially reducing the time and cost associated with bringing new therapies to market.

For more information about contributing data to RDCA-DAP or to learn more about this collaborative effort, please visit c-path.org/rdca-dap or contact rdcadap@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 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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Contacts:

Roxan Triolo Olivas
C-Path
520.954.1634
rolivas@c-path.org

Kissy Black
C-Path
615.310.1894
kblack@c-path.org