Breaking Down Data Siloes: New Analytics Platform Sparks Opportunity for Rare Disease Field

C-Path, NORD and FDA have spent two years building the platform set to launch Sept. 14 at the 2021 RDCA-DAP Workshop.

TUCSON, Ariz. and **WASHINGTON**, September 1, 2021 — For two years, Critical Path Institute (C-Path), the National Organization for Rare Disorders (NORD) and the U.S. Food and Drug Administration (FDA) have joined with others throughout the rare disease community to create a novel, best-in-class platform to accelerate rare disease treatment innovation.

On Tuesday, September 14, 10 a.m. – 3:45 p.m. ET, the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), an FDA-funded initiative, will have its public premiere during the annual meeting for the project. The platform will host, standardize and share rare diseases data as part of its functionality.

In addition to the demonstration of the platform itself, presentations throughout the day will show how RDCA-DAP is breaking down data silos, provide insight into the platform's development and testing process, and showcase the importance of RDCA-DAP from the perspective of critical stakeholders, including academic, clinical, regulatory and patient communities.

"We invite colleagues and patients in the rare disease community to join us at the workshop to see the latest developments since launching the RDCA-DAP initiative in 2019," said Jeff Barrett, Ph.D., F.C.P., C-Path Senior Vice President and RDCA-DAP Lead. "It will be the public's first look at the platform's capabilities, showcasing how it can be used to generate solutions for rare diseases."

In collaboration with Aridhia Informatics, the team from C-Path's Data Collaboration Center has been designing and building the platform, which will allow researchers to develop advanced models to quantitatively describe relevant aspects of disease progression within and across diseases, capturing relevant sources of variability. Researchers can interact with these sophisticated models through a user-friendly interface to potentially submit them for regulatory review and endorsement as quantitative drug development tools, or for the purpose of running simulations intended to optimize clinical trial design.

"There is tremendous promise in the RDCA-DAP platform to empower patients and families to be a driving force in rare disease innovation," said Ed Neilan, M.D., Ph.D., Chief Medical and Scientific Officer, NORD. "The demonstration and workshop will showcase what's possible when the community comes together to share de-identified data for broader use to expedite the development of treatments."

NORD has worked with data custodians to contribute datasets from six different disease states with surveys housed in

NORD's IAMRARE® registry platform to RDCA-DAP and has agreements in place to contribute datasets in three additional disease areas. C-Path has secured 10 additional disease datasets, with existing datasets from C-Path's rare disease consortia having been integrated as well. Data from many more disease states are at an intermediate stage of the contribution process.

Those interested in attending the annual meeting may register for free here.

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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 dozens of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona and C-Path, Ltd. EU is headquartered in Dublin, Ireland, with additional staff in multiple other locations. For more information, visit c-path.org and c-path.eu.

About the National Organization for Rare Disorders (NORD)®
The National Organization for Rare Disorders (NORD) is the leading independent advocacy organization representing all patients and families affected by rare diseases?in?the United States. NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. Since then, the organization has led the way in voicing the needs of the rare disease community, driving supportive policies, furthering education, advancing medical?research, and providing patient and family services for those who need them most.?Together with over 330 disease-specific member organizations, more than 15,000 Rare Action Network advocates across all 50 states, and national and global partners, NORD delivers on its mission to improve the lives of those impacted by rare diseases. Visit rarediseases.org.

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