Rare Disease Innovation and Data Sharing Accelerated by New RDCA-DAP Program

C-Path, NORD and FDA host annual meeting, demonstration and launch of new rare disease data and analytics platform.

TUCSON, Ariz. and **WASHINGTON**, September 15, 2021 — The Rare Disease Cures Accelerator-Data and Analytics Platform initiative (RDCA-DAP®) officially launched its next phase on Tuesday establishing itself as the leading platform to accelerate rare disease treatment innovation. The launch was part of an all-day virtual workshop and annual meeting of rare disease stakeholders, featuring a platform demonstration, industry expert presentations and attendance by over 400 patients, providers, researchers, clinicians, biopharmaceutical companies, regulatory reviewers and scientists.

RDCA-DAP is an FDA-funded initiative that will serve as a centralized and standardized base to host and share de-identified rare diseases data and to support treatment innovation. It was created through a partnership between Critical Path Institute (C-Path), the National Organization for Rare Disorders (NORD®) and the U.S. Food and Drug Administration (FDA) and has grown to include many others throughout the rare disease community.

“There is a lot of enthusiasm for seeing this platform become all it can be for patients and drug developers. It is not just about the data. This is a place to generate solutions in a highly collaborative way,” said Jeff Barrett, Ph.D., F.C.P., C-Path Senior Vice President and RDCA-DAP Lead. “We expect RDCA-DAP to be a place to promote candid dialogue, optimize patient selection and trial design, and co-develop the tools to further de-risk decision making for rare disease drug development.”

After a public premiere showcasing the functionality of the platform, presentations throughout the day emphasized how RDCA-DAP will curate data to break down silos, provided insight into the platform’s development and testing and showcased the importance of RDCA-DAP from the perspective of critical stakeholders, including academic, clinical, regulatory and patient communities.

On the importance of incorporating patients’ perspectives through RDCA-DAP, Theresa Mullin, Ph.D., Associate Director for Strategic Initiatives, Center for Drug Evaluation and Research (CDER), FDA, said, “Identifying the burdens of disease and current therapies that matter most, what can be measured, and what would constitute a meaningful change, can provide insight on the clinical context for regulatory review and potentially more direct evidence of drug benefits and risks when collected using valid and reliable measures and tools.”

A total of 31 speakers and panelists from patient organizations and regulatory, industry and academic fields participated in the workshop. Five patient profiles underscoring the importance of data sharing were also highlighted. Those interested in viewing the workshop and accessing supplemental information can do so here.

“Patient owned data collected by patient groups are less likely to get stuck in a silo,” said Ed Neilan, M.D., Ph.D., Chief Medical and Scientific Officer, NORD. “Of the over 7,000 estimated rare diseases, more 90% have no FDA approved treatment. And currently the attention of the pharmaceutical industry is on 100 or 200 of these rare diseases. RDCA-DAP may bring attention to rare diseases that might otherwise remain relatively neglected.”

To learn more and request access to RDCA-DAP, apply directly on the platform at https://portal.rdca.c-path.org. To submit critical rare disease data, contact the project team at rdcadap@c-path.org or visit c-path.org/programs/rdca-dap and rarediseases.org/rdca-dap.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) and is 56.5% funded by FDA/HHS, totaling $16,749,891, and 43.5% funded by non-government

source(s), totaling $12,895,366. 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. For more information, please visit FDA.gov.

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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