Innovative Data and Analytics Platform to Accelerate Drug Development for Rare Diseases

Launched by C-Path and NORD through an FDA grant, the goal of the new platform is to accelerate the development of cures by addressing the need to better characterize rare diseases.

TUCSON, Ariz. and **WASHINGTON**, September 19, 2019 — The Critical Path Institute (C-Path) and the National Organization for Rare Disorders® (NORD) launched the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) in Rockville, MD on Tuesday, Sept. 17. The platform, funded by a cooperative agreement through the Food and Drug Administration, [Critical Path Public-Private Partnerships Grant Number U18 FD005320], will provide data and analytics to aid in the understanding of rare diseases and to inform long-term drug development and support innovative trial designs.



Janet Woodcock, MD, Director for the Center for Drug Evaluation and Research (CDER), delivered opening remarks at the launch of the Rare Disease Cures Accelerator-Data and Analytics Platform on Tuesday, September 17, in Rockville, MD.

“People with rare diseases need treatments; we need to do what we can to make development of those treatments as efficient, effective and fast as possible,” said Center for Drug Evaluation and Research Director

Janet Woodcock, MD. “The way to do that is to have all the data we’ve been talking about brought to bear on how we test the interventions — the Rare Disease Cures Accelerator-Data and Analytics Platform is the vehicle that can deliver that data to the developers and the community.”

The launch meeting, attended by more than 150 individuals from patient groups, industry and regulatory agencies, plus hundreds more via live stream, served to inform the rare disease community about the new platform, and to seek input on its development. FDA representatives Theresa Mullin, PhD, Associate Director for Strategic Initiatives and Billy Dunn, MD, Director, Division of Neurology Products, explained how RDCA-DAP fits into the FDA’s vision for the future of drug development for rare diseases, and how it will provide tools to aid in understanding the trajectory of rare diseases and accelerate development of new treatments and cures. Dunn emphasized the importance of sharing and aggregating data, especially in the context of rare diseases, and how this helps to inform clinical trial design.

“We have tremendous experience with C-Path and NORD with regard to our approaches to data. It’s truly altruistic and it’s about bettering the community and allowing every member of the scientific and patient community to benefit from aggregated data,” Dunn said. “There’s increased recognition in the scientific community that being a good scientific citizen means sharing your data.”



Members of patient organizations, industry and regulatory agencies participated in a panel discussion on data integration in rare diseases and how they fit into the vision for RDCA-DAP.

Panels of representatives from industry and patient groups discussed problems encountered in rare disease drug development and the need for this infrastructure to help get past those bottlenecks. Several successful programs that have accelerated efforts to develop treatments in specific disease areas were highlighted, which will inform the development of this new pan-rare disease platform. RDCA-DAP is designed to collaborate with existing efforts in this space.

“Getting all the key opinion leaders, patients and key stakeholders involved is absolutely essential,” said Rosángel Cruz, MS, Director of Research and Clinical Affairs, Cure SMA. “Let’s get together and learn each other’s language. Sometimes in rare diseases we end up working in silos and as such, data ends up in silos, and there isn’t a way for us to all come together and share data and learnings from that data. RDCA-DAP is important to the entire rare disease community.”

NORD Director of Research Programs Vanessa Boulanger, MS, described the IAMRARE™ Registry Program that will serve as an initiation point for stakeholders looking to systematically collect natural history study and patient registry data. The data can be shared with the RDCA-DAP, as one way to ensure that the

rare disease community informs the development, utilization and impact of the platform. C-Path Director of Clinical Pharmacology and Quantitative Medicine Klaus Romero, MD, described the infrastructure that is already in place to aggregate data at C-Path and how it may be utilized by the rare disease community to accelerate drug development, as well as new features being designed specifically for RDCA-DAP.



C-Path Director of Clinical Pharmacology and Quantitative Medicine Klaus Romero, MD, MS, described the infrastructure that is already in place to aggregate data at C-Path and how it may be utilized by the rare disease community to accelerate drug development. The RDCA-DAP launch event was attended by more than 150 members of industry, patient groups and regulatory agencies, plus hundreds more via live stream.

Groups interested in contributing data to the effort, collaborating on the development of the platform or using the database may visit, c-path.org/rdca-dap or email rdcadap@c-path.org for more information. The platform is open to accept data immediately, but the user interface and analytics are still under development and will be available in the next six months, with more sophisticated analytics tools developed over time.

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 US 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. ?NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90% are still without an FDA-approved treatment or therapy. 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. For more than 35 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research and providing patient and family services for those who need them most. ?NORD is made strong together with over 275 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease community. For more information, visit rarediseases.org.

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