
Rare Disease Workshop Highlights the Importance of Data Sharing in Drug Development

C-Path, NORD and FDA hosted an annual workshop September 13-14 to spotlight the impact of their innovative data and analytics platform on rare disease drug development

TUCSON, Ariz. and WASHINGTON, DC, September 15, 2022 — The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) initiative hosted an all-day workshop and annual meeting September 13 and 14 in Washington. The in-person and livestream event for rare disease stakeholders featured expert presentations, panel discussions, platform demonstrations, and was attended by more than 200 patients, researchers, clinicians, biopharmaceutical company representatives, regulatory reviewers and scientists.

RDCA-DAP was established through a partnership between [Critical Path Institute](#) (C-Path), [National Organization for Rare Disorders](#) (NORD®) and the U.S. Food and Drug Administration ([FDA](#)), and has grown to include dozens of collaborations between a variety of stakeholders throughout the rare disease community. The platform serves as a centralized and standardized infrastructure to host integrated and shared data and to support and accelerate rare disease medical product development.

The workshop's keynote address was delivered by Theresa Mullin, Ph.D., Associate Director for Strategic Initiatives, FDA, Center for Drug Evaluation and Research (CDER). "Building this platform was about better informing drug development programs to get drugs that are effective and safe to patients with rare diseases as soon as possible and as efficiently as possible," Mullin said. "It's taken a multistakeholder effort and now we're seeing tools and learnings that can be shared across rare diseases."

The keynote was followed by a panel discussion on the impact of RDCA-DAP, which featured representatives from each organization engaged in this effort: Klaus Romero, M.D., M.S. F.C.P. and Alexandre Bétourné, Ph.D., Pharm.D., from C-Path; Pamela Gavin, MBA, from NORD; and Theresa Mullin, Michelle Campbell, Ph.D. and Billy Dunn, M.D., from FDA.

Additional key speakers and panelists were featured from industry, academia and patient organizations. The robust agenda included case studies shared by users of the platform, sessions covering critical topics on the impact of RDCA-DAP on accelerating drug development for rare diseases, as well as active discussions around data sharing, privacy and standards, and much more. A significant portion of the agenda featured case studies on the use of the platform to further clinical research in sickle cell disease, Duchenne muscular dystrophy, Friedreich's ataxia and transplant therapeutics.

Following a [press release](#) by the FDA on Wednesday, C-Path announced it will serve as the convener of the [Critical Path for Rare Neurodegenerative Diseases \(CP-RND\)](#), a new public-private partnership (PPP) to benefit people across multiple rare neurodegenerative diseases.

"This year's RDCA-DAP annual workshop marked not only a return to in-person meetings, but also the strengthening of collaborations between RDCA-DAP across multiple rare diseases, with significant impact on accelerating medical product development," said C-Path Chief Science Officer and Executive Director of Clinical Pharmacology Klaus Romero. "As we look to next year, RDCA-DAP will leverage all of its strengths to contribute to the success of the new PPP for rare neurodegenerative diseases."

NORD's Aliza Fink, D.Sc. and Ed Neilan, M.D., Ph.D., gave a presentation titled Centrality of Patient Advocacy Organizations in Accelerating Treatments for Rare Disease, and the first day concluded with a panel on the future impact of RDCA-DAP. As part of the program for the second day of the conference, in-person participants attended breakout sessions on the role of RDCA-DAP in innovating data standards and data stewardship, regulatory science in rare diseases, and a practical session on how to use the platform.

Presentations throughout the event emphasized how RDCA-DAP continuously integrates data and breaks down silos, provides 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.

“There are many things that make rare diseases unique. The most obvious is that there are so few patients that can be studied which makes running clinical trials challenging,” said Ed Neilan, Chief Medical and Scientific Officer, NORD. “It's a special shame when data that get collected then become siloed. Patients and patient advocates want desperately for the data they contribute to be used to its fullest extent. For this reason, it's vital that tools like the RDCA-DAP exist. RDCA-DAP is in a favorable position as it's being co-developed by the FDA and is a prime locus for all rare disease data collection.”

A total of 28 speakers and panelists from patient organizations and regulatory, industry, and academic fields participated in the workshop. A recording of the workshop presentations and panel discussions will be available on C-Path's [YouTube](#) channel in the coming weeks.

RDCA-DAP released a new video resource at the end of the workshop entitled Data Literacy, [watch it on the NORD Video Library](#). To request access to RDCA-DAP, [apply directly on the platform](#). To submit critical rare disease data, contact the project team at rdcadap@c-path.org or visit [the website](#).



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.

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 54.2% funded by the FDA/HHS, totaling \$13,239,950, and 45.8% funded by non-government source(s), totaling \$11,196,634. 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.



About National Organization for Rare Disorders

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 300 disease-specific member organizations, more than 17,000 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.

Media Contacts:

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

Rohan Narayanan
NORD
571.255.0042
rnarayanan@rarediseases.org