
NORDs’ IAMRARE Registry Contributes Two New Datasets to C-Path’s RDCA-DAP, Boosting Rare Disease Research and Therapeutic Advancement

TUCSON, Ariz., August 15, 2023 — Critical Path Institute (C-Path) announced today that the National Organization for Rare Disorders (NORD), a founding partner of the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), is integrating two new datasets to the platform. The FD/MAS Alliance and A Cure in Sight (ACIS) join five other IAMRARE® registries already contributing data, expanding the rich resource for rare disease research and therapeutic development.

The FD/MAS Alliance is a dedicated community-led nonprofit organization that addresses the needs of individuals affected by fibrous dysplasia/McCune-Albright syndrome (FD/MAS), a condition impacting the bone, endocrine system, and skin. Concurrently, A Cure in Sight (ACIS) provides nationwide services, focusing on building public awareness, educating ocular melanoma (OM) patients and their caregivers, funding ongoing eye cancer research, and financially assisting OM patients to access necessary treatments.

By contributing their comprehensive datasets to the RDCA-DAP, both organizations bolster the acceleration of research and development of therapies for rare and complex diseases.

RDCA-DAP presents a unified and standardized infrastructure to accelerate rare disease characterization targeted for clinical development. The platform enhances best practices for rigorous conduct of natural history studies and offers a robust, integrated database and analytics hub. This allows the aggregation and effective interrogation of rare disease data from multiple sources, making it invaluable for clinical trial design and regulatory review.

“We are profoundly appreciative of our ongoing partnership with organizations such as NORD, which enabled collaborative data sharing with the FD/MAS Alliance, and ACIS and the continuous bolstering and diversification of our rare diseases database our data infrastructure,” said Alex Betourne, Ph.D., Pharm.D., Executive Director, RDCA-DAP. “Their unwavering dedication to enhancing the well-being of those afflicted with these rare disorders aligns seamlessly with C-Path’s own commitment to accelerating the development of potent therapies for rare diseases. Their generous contribution to our data platform represents a significant stride forward in the field of rare disease research.”

“Not only will the addition of these registries to RDCA-DAP catalyze much-needed research into ocular melanomas and fibrous dysplasia, they will also build on the overall platform’s potential for cross-disease analysis that serves the entire rare disease community,” said Ed Neilan, M.D., Ph.D., Chief Medical and Scientific Officer for NORD. “Expanding, deepening, and better synchronizing rare disease research through the RDCA-DAP is a core component of NORD’s mission to advance our understanding of rare diseases and develop more life-saving therapies for patients.”

The additional data from the FD/MAS Alliance and ACIS not only enhances the understanding of these specific rare diseases but also opens new paths for innovative treatment development. As more researchers worldwide access this comprehensive information, the shared data will enable improved patient outcomes and faster progress towards cures.

These two datasets join ongoing contributions from the following groups' IAMRARE registries: Desmond Tumor Research Foundation (DTRF), Foundation for Prader-Willi Research (FPWR), International Pemphigus & Pemphigoid Foundation (IPPF), National PKU Alliance (NPKUA), and Congenital Hyperinsulinism International (CHI).

Organizations interested in contributing data to RDCA-DAP can visit c-path.org/rdca-dap or email rdcadap@c-path.org. The platform is OPEN and accepting applications for use; visit portal.rdca.c-path.org to learn more.



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 located 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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About the National Organization for Rare Disorders (NORD)

With a 40-year history of advancing care, treatments and policy, the National Organization for Rare Disorders (NORD) is the leading and longest-standing patient advocacy group for the more than 30 million Americans living with a rare disease. NORD, a 501(c)(3) nonprofit, is dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 330 patient organization members, is committed to improving the health and well-being of people with rare diseases by driving advances in care, research, and policy. For more information, please visit <https://rarediseases.org/>.

Media Contact:

Kissy Black

C-Path

615.310.1894

kblack@c-path.org