CRITICAL PATH INSTITUTE

Advancing Drug Development. Improving Lives. Together.

June 24, 2026

Welcome to the Rare/Orphan and Pediatric Disease Programs quarterly newsletter. In between major announcements, webinars and meetings, this communication serves to update you on the latest developments within C-Path's ROPD programs, as well as the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®). None of these advancements are possible without the participation of our members, collaborators, and data contributors. Thank you.

C-Path RARE/ORPHAN AND PEDIATRIC DISEASE PROGRAMS: QUARTERLY NEWSLETTER

As we move into summer, the Rare/Orphan and Pediatric Disease Programs team continues to advance collaborative efforts that support rare disease research and drug development. Through ongoing engagement with patient organizations, researchers, industry, and regulators, we remain committed to accelerating the development of therapies for individuals and families affected by rare diseases.

We're looking ahead to an exciting opportunity to connect in person. Join us once again for the C-Path Global Impact Conference, happening September 15-16 in Washington, D.C. The event will convene a global community of regulators, industry leaders, scientists, clinicians, those with lived experience, and advocates committed to accelerating the development of better treatments for people worldwide.

Additionally, the RDCA-DAP team will be hosting a free, in-person workshop, Got Data? Evaluating Real-World Data Suitability to Inform Regulatory Decision-Making. This interactive workshop will introduce a structured framework for evaluating whether real-world data (RWD) is fit for regulatory use. Participants will assess theoretical RWD sources across key dimensions, including relevance, observability, comparability, and reliability, and discuss opportunities to improve RWD quality and standardization, particularly for rare diseases. Join us on Monday, September 14, to put these principles into practice, engage in hands-on discussions, and gain practical insights into assessing the suitability of RWD for regulatory applications.

SPOTLIGHT: CP-RND Research Update

On June 5, 2026, the Critical Path for Rare Neurodegenerative Diseases team brought together partners from FDA, NIH, FNIH, and the broader community to share progress from collaborative efforts across ALS and other rare neurodegenerative diseases, including work supported under the Accelerating Access to Critical Therapies for ALS Act (ACT for ALS). Presentations demonstrated how coordinated approaches are improving how research is conducted, measured, and translated into action, while underscoring the role of strategic partnerships in accelerating research and advancing more patient-centered research and development.

A key focus of the program was the importance of engaging the lived experience community in research, with individuals impacted by rare neurodegenerative diseases sharing perspectives that have helped shape research priorities and drive meaningful change. The meeting also looked across diseases to identify common challenges, opportunities for collaboration, and priorities for the future. Together, these discussions highlighted the growing momentum in the field and reinforced the value of partnership and collective effort in advancing progress across the rare neurodegenerative disease ecosystem.

You can access the slides and recording here.

RDCA-DAP UPDATES

This quarter, RDCA-DAP saw a flurry of new data contributions. First, we continued to expand our data ecosystem through a new master data sharing agreement with the Across Healthcare Matrix registry platform, and additional transferred data from the CoRDS Registry. The team at CoRDS shared two new datasets including the registry for primary biliary cholangitis (PBC), a rare autoimmune liver disease from the PBCers Organization and the registry of the Hope for PDCD Foundation, a rare mitochondrial disease, and the fifth dataset shared by CoRDS to our platform. We have started to work with the Matrix team to develop an active exchange interface to facilitate data exchange to RDCA-DAP. This partnership creates a streamlined pathway for incorporating additional rare disease datasets into the platform, starting with registry data from the GLUT-1 Deficiency Foundation, which will be made available later this year along with three additional registry datasets in other diseases. Our long-time collaborator, NORD, provided new data updates for seven registries, with two new ones joining this summer.

Our collaboration with the Vivli platform and Biogen continued to yield results this year, with new trial data from a PSP trial, the sharing of three ALS clinical trials with a fourth underway, and more expected before year's end. Our CP-RND team's ALS database is gaining meaningful momentum, with twelve datasets shared to date, all either already accessible on our portal or currently moving through our curation pipeline and expected later this year.

RDCA-DAP
RARE DISEASE CURES ACCELERATOR DATA AND ANALYTICS PLATFORM

On the rare disease front, the RDCA-DAP team achieved a significant milestone with the addition of two primary biliary cholangitis datasets, marking our first foray into rare liver disease data sharing. The team also received datasets in limb-girdle muscular dystrophy from the University of Iowa and Team Titin, alongside MOVR registry data from MDA — a welcome broadening of our rare disease portfolio.

We are grateful to our collaborators, data contributors, and community partners whose commitment to data sharing makes this progress possible. Together, we are building a stronger foundation for rare disease innovation and discovery.

The platform currently hosts data for over 49 disease areas, including the largest database for Friedreich's ataxia. You can find a full list of diseases and platform engagement to date, here.

NORD CORNER

Innovative Clinical Trial Models for Rare Disorders

Do you understand Bayesian clinical trial designs and how to employ them? What about adaptive trials like the snSMART model? Are you aware of the alternatives to placebo controls and randomized controlled trials (RCTs)?

You can get educated on all of this and more, with specific case studies from today's leading rare disease researchers across disciplines, for as low as $25 when you register to access full session recordings from the 2026 National Organization for Rare Disorders (NORD®) Rare Disease Scientific Symposium. **Visit nordscience.org/on-demand-2026 to get started. A few short clips are also available on NORD's YouTube channel.**

The 2026 NORD Breakthrough Summit

The 2026 National Organization for Rare Disorders Rare Diseases and Orphan Products Breakthrough Summit® returns to Washington, D.C., October 25–27, bringing together voices from across the rare disease community — including hundreds of patient advocacy organizations as well as biopharma executives, government regulators, and medical leaders. Registration for this event is now open at nordsummit.org.

If you have conducted a research study, orphan product study, or public health project relevant to this field, you are invited to submit an abstract for inclusion in the Summit Poster Hall and share your work with leaders in this space. Read the submission guidelines and submit your abstract here. The deadline to submit is July 22.

WEBINAR SERIES 2026

On Demand Webinars

April 9: Hope on the Horizon for ADTKD Patients.

This webinar discusses autosomal dominant tubulointerstitial kidney disease (ADTKD), the second most common genetic kidney disease after PKD, affecting an estimated 75,000–100,000 people in the U.S. and up to two million worldwide. Discover how physician-scientists are advancing toward the first-ever treatments for UMOD and MUC1 — the gene variants responsible for most ADTKD cases — and why a clinical trial may be on the horizon. Learn how the Rare Kidney Disease Foundation is empowering patients, families, and advocates through education, community engagement, and advocacy efforts aimed at ending the impact of ADTKD on future generations.

March 19: Meeting Patients Where They Are: Validating Remote Digital Tools for Multi-Indication NMD Research.

In this session, Dr. Tina Duong explores the scientific, clinical, and regulatory considerations involved in validating video-based assessments and other digital tools across multiple NMD indications. Through both presentation and panel discussion, attendees gained valuable insights into how these approaches can enhance accessibility for patients regardless of location, reduce the burden of participation in clinical trials, generate reliable, regulatory grade data, ensure innovation remains grounded in patient-centered research, and more.

You can view all 2025 ROPD Webinars on demand here.

ANNOUNCEMENTS AND ADDITIONAL RESOURCES

May 29: ALS Awareness Month: Connecting the Dots in ALS Research

May 26: Huntington's Disease Awareness Month: Advancing Drug Development Through Lived Experience

May 22: Moving Treatments Across the Finish Line: A Focus for ALS Awareness Month

May 19: Advancing Hope in Progressive Supranuclear Palsy: Collaboration, Data, and Innovation During PSP Awareness Month

May 14: The Critical Path Institute Podcast: Accelerating Drug Development in Friedreich's Ataxia, with Ron Bartek

April 22: AIRNA Joins C-Path's Alpha-1 Antitrypsin Deficiency Consortium to Advance Innovative AATD Treatments

April 1: Advancing Rare Disease Innovation Through Responsible Data Sharing — Key Takeaways from the RISE Together: Data Sharing Across the Rare Disease Ecosystem

March 26: Critical Path Institute Launches 'One to Millions' to Reshape the Future of Individualized Medicine at Global Scale

For more information about Rare/Orphan and Pediatric Disease Programs, visit: https://c-path.org/area-of-focus/rare-and-orphan-diseases/.

Help support our mission. MAKE A GIFT TODAY

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FDA Acknowledgment