

RARE DISEASE CURES
ACCELERATOR
**DATA AND ANALYTICS
PLATFORM**



December 15, 2022

RDCA-DAP® Quarterly Newsletter

Welcome to the RDCA-DAP quarterly newsletter. In between major announcements, webinars and meetings, this communication will serve to update you on the latest developments within the platform and overall initiative. None of RDCA-DAP's advancements are possible without the participation of stakeholders. Thank you for your continued support.



With the end of the year festivities coming around the corner, the RDCA-DAP team and extended rare disease family at C-Path would like to acknowledge our collaborators, data contributors and the patient advocacy groups, foundations and rare diseases advocates. We are grateful for your work, commitment and dedication to the thousands of patients and families living with a rare disease. We are also grateful for your trust, which we do not take lightly, as we continue our efforts to accelerate medical product development for patients.

Onward!

Data Developments

The platform continues to expand since going live September 2021, containing data for over 24 different rare diseases. More data will be accessible throughout 2022 and into 2023 as outreach efforts continue.

Interested in contributing data to RDCA-DAP? Fill out [this form](#) to be contacted by a team member.

RDCA-DAP currently contains data for the following diseases:

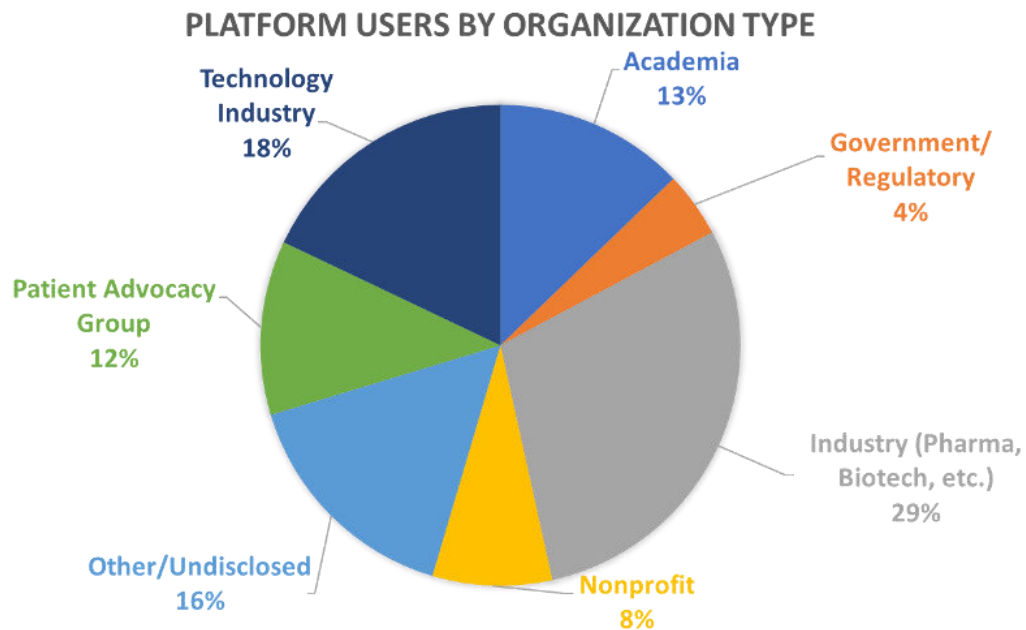
Angelman Syndrome	Necrotizing Enterocolitis*
Congenital Isolated Hyperinsulinism*	Pemphigus & Pemphigoid*
Desmoid Tumor*	Phenylketonuria (PKU)*
Duchenne Muscular Dystrophy*	Polycystic Kidney Disease
Facioscapulohumeral muscular dystrophy (FSHD)*	Prader-Willi Syndrome*
Friedreich's Ataxia	Progressive Supranuclear Palsy*
GNE Myopathy	Rare Epilepsies*
hnRNP related disorders*	RYR-1 gene mutation*
Kidney Transplant	Spinal Muscle Atrophy with Respiratory Distress*
KIF1A Associated Neurological Disorder*	Spinocerebellar ataxias type 1, 2, 3 & 6
Lennox-Gastaut Syndrome	Sturge-Weber Syndrome
Mitochondrial Disease	Tuberous Sclerosis

*Indicates disease with datasets that are currently discoverable on the platform

Platform Use

Since launching the platform, we've seen the following engagement from users:

- 282 approved platform requests
- 21 approved workspaces for external users



Check out the newly available data by accessing the platform now! You can also request access at portal.rdca.c-path.org

Recently Added/Updated Datasets:

- Lennox-Gastaut Syndrome clinical trial data from Eisai
- Prader-Willi Syndrome patient reported natural history data updated from the Foundation for Prader-Willi Research

Spotlight – Collin Hovinga, PharmD, MS, FCCP, Vice President, Rare and Orphan Disease Programs



Collin Hovinga, PharmD, MS, FCCP, serves as Vice President of the Rare and Orphan Disease Programs at the Critical Path Institute overseeing C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform and the Critical Path for Rare Neurodegenerative Diseases public-private partnership. Recognized as an expert in neuropharmacology, Dr. Hovinga completed his Bachelor of Science Degree in Biology and Doctor of Pharmacy degrees from Creighton University in Omaha, Nebraska. After which he pursued a Residency and Fellowship in Pediatric Pharmacotherapy with emphasis in Pediatric Neuroscience at the University of Tennessee, Memphis, LeBonheur Children's Medical Center. He has had a Fellowship at the FDA Office of Clinical Pharmacology and has a Masters of Epidemiology from the University of Tennessee Health Science Center. Dr. Hovinga has been active in studying factors that influence the efficacy, safety and the pharmacology of drugs in

children, patient adherence to medications, and acute seizure management. "I have long admired C-Path's vision to be a partner of excellence in transforming the medical product development process worldwide," said Hovinga. "I have been a collaborator in the RDCA-DAP initiative since it was launched in 2019 and I look forward to working with stakeholders to continue to build on the great impact C-Path has made in the rare disease drug development ecosystem."

Connect with Collin on [LinkedIn](#).

NORD Corner



NORD hosted four sessions on topics related to data and RDCA-DAP at the 2022 Rare Diseases and Orphan Products Breakthrough Summit: "Patient Voice at FDA: Tips for Impactful PFDDs and Listening Sessions", "Why Data Matters and What You Can Do with It", "Creating a Culture of Data Sharing", and an RDCA-DAP Lunch and Learn. Session recordings are now available to Summit registrants here: nordsummit.org

NORD has issued a request for applications from patient groups who are interested in starting a registry on the IAMRARE® platform and contributing data to the RDCA-DAP. Full details and a link to the application are available here: rarediseases.org/request-for-applications-new-patient-registries

Announcements

December 1, 2022: [C-Path and Ultragenyx Announce Data Sharing Agreement to Support Rare Disease Treatment and Novel Therapies](#)

November 10, 2022: [C-Path and EFACTS Announce Data Sharing Agreement Making RDCA-DAP the Largest Worldwide Database for Friedrich's Ataxia](#)

November 2, 2022: [C-Path and Eisai Data Sharing Collaboration to Include Lennox-Gastaut Syndrome Registry and Clinical Trial Data](#)

October 27, 2022: [C-Path Appoints Neuropharmacology Expert as New Vice president – Rare and Orphan Disease Programs](#)

October 20, 2022: [C-Path's Ramona Walls Promoted to Executive Director of Data Science](#)

October 20, 2022: [C-Path and Sturge-Weber Foundation Announce Data Sharing Agreement to Support Treatment Development for Sturge-Weber Syndrome](#)

Upcoming and Past Events

Save the Date - Rare Disease 2023 Workshop



Webinar Series 2023



2023 WEBINAR SERIES

March 16 – Clinical Outcome Assessments: Does one size fit all?

April 20 – Informed Consent: Considerations for Foundations and Patient Registries

June 22 – GUIDs and De-Identification Tools for Rare Diseases



**SAVE
THE
DATE**

On Demand Now - Webinar Series 2022

- March 16, in partnership with [Quinten Health](#). View here: [AI-powered real-world simulations for faster and value-based rare disease drug development](#)
- May 18, 2022, in partnership with [Replica Analytics](#). View here: [Generating Synthetic Longitudinal Data](#)
- June 15, 2022, in partnership with [Monarch Initiative](#). View here: [Using Ontologies to Standardize Rare Disease Data Collection](#)
- August 17, 2022, in partnership with [FSHD Society](#). View here: [Addressing the Gaps in Clinical Trial Readiness for Facioscapulohumeral muscular dystrophy \(FSHD\)](#)
- December 14, 2022, in partnership with [C-Path](#) and [NORD](#). View here: [Shared Stewardship in Collaborative Curation of Rare Disease Datasets](#)

For questions or additional information about participating in RDCA-DAP, please email rdcadap@c-path.org. For more information about the initiative, visit: c-path.org/programs/rdca-dap/.



#RDCADAP

