RDCA-DAP®

RARE DISEASE CURES ACCELERATOR
DATA AND ANALYTICS PLATFORM

December 19, 2023

RDCA-DAP® Quarterly Newsletter

Welcome to the RDCA-DAP quarterly newsletter. In between major announcements, webinars and meetings, this communication serves 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 our supporters and data contributors. Thank you.

Introduction

As the holiday season is upon us, we want to take a moment to express our heartfelt gratitude for being part of our vibrant community. Your support and engagement have made this year truly special.

Let us cherish the special moments and progress made, reflect on the year gone by, and embrace the possibilities that lie ahead.

Thank you for being with us on this journey, and we look forward to connecting with everyone in the coming year.

Happy Holidays and a Wonderful New Year!

Warmest wishes,
C-Path's Rare & Orphan Disease Programs

Data Developments

The platform continues to expand since going live in September 2021, containing data for over 34 different rare diseases. More data will be accessible throughout 2023 and into 2024 as outreach efforts continue.

RDCA-DAP currently contains data for the following diseases:

Angelman Syndrome*	Lennox-Gastaut Syndrome*
CACNA1*	Mitochondrial Disease
Cerebellar Ataxia	Mucopolysaccharidoses (MPS)
Charcot-Marie-Tooth disease	Myotubular or centronuclear myopathy*
COL6-related dystrophies*	Necrotizing Enterocolitis*
Congenital Hyperinsulinism*	Ocular Melanoma
Desmoid Tumor*	Pemphigus & Pemphigoid*
Duchenne Muscular Dystrophy*	Phenylketonuria (PKU)*
Facioscapulohumeral muscular dystrophy (FSHD)*	Polycystic Kidney Disease
Fibrous Dysplasia disorders*	Prader-Willi Syndrome*
Friedreich's Ataxia*	Progressive Supranuclear Palsy*
GNE Myopathy*	Rare Epilepsies*
hnRNP related disorders*	Ryanodine Receptor-1 (RYR-1) Related Myopathies*
Kidney Transplant	Spinal Muscle Atrophy with Respiratory Distress*
KIF1A Associated Neurological Disorder (KAND)*	Spinocerebellar ataxias type 1, 2, 3 & 6
Kleefstra Syndrome	Sturge-Weber Syndrome
Leigh Syndrome and other mitochondrial disorders*	Tuberous Sclerosis*

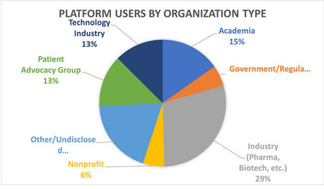
*Indicates disease with datasets that are currently discoverable on the platform

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

Platform Use

Engagement to date:

- 445 approved platform requests
- 26 approved workspaces for external users/research



NORD Corner

The National Organization for Rare Disorders (NORD®) has created a free online course in collaboration with the FDA's Center for Drug Evaluation and Research (CDER) and the Critical Path Institute (C-Path), designed to help patients, caregivers and patient advocacy groups understand the drug development process. The first modules of ***Rare Disease Drug Development: What Patients and Advocates Need to Know*** are now live, and additional modules will be released over the coming year. Each module is valuable on its own, and together they act as a comprehensive guide to the role of patient advocacy in rare disease drug development.

Some of the essential topics covered are:

- Stakeholder Roles in Drug Development
- Natural History Studies
- Transitioning from Pre-Clinical to Clinical Research
- Human Subjects Protections
- Regulatory Considerations

Start learning today at learn.rarediseases.org!

Announcements

December 12, 2023: C-Path's RDCA-DAP Program and PicnicHealth Announce Data Collaboration to Accelerate Rare Disease Research

December 12, 2023: C-Path Welcomes Mucopolysaccharidosis Clinical Trial Data Contribution from Children's Hospital of Orange County into RDCA-DAP

October 26, 2023: C-Path and Congenital Hyperinsulinism International Announce Data Sharing Agreement

August 15, 2023: NORDs' IAMRARE Registry Contributes Two New Datasets to C-Path's RDCA-DAP, Boosting Rare Disease Research and Therapeutic Advancement

July 19, 2023: The Foundation for Angelman Syndrome Therapeutics to Contribute Data to C-Path's RDCA-DAP, Accelerating Neurological Disease Research and Therapeutic Development

June 7, 2023: Newcastle University's John Walton Muscular Dystrophy Research Centre Contributes Data to C-Path's RDCA-DAP

June 2, 2023: New collaboration between Monarch Initiative and Critical Path Institute: Working towards improving drug discovery for rare diseases

May 31, 2023: CACNA1A Foundation to Contribute Data to C-Path's RDCA-DAP, Accelerating Neurological Disease Research and Therapeutic Development

February 28, 2023: Drug Repurposing Provides Big Impact for Patients

February 17, 2023: C-Path Stands with Bruce Willis, Neurodegenerative Disease Community After FTD Diagnosis

January 25, 2023: C-Path Receives Letter of Support from EMA on Duchenne Muscular Dystrophy Clinical Trial Simulation Platform

Webinar Series 2024

Please Save the date for our first webinar of 2024 on February 15! Details coming soon.

You can now view all 2023 RDCA-DAP Webinars on Demand

November 16: VCP disease(s), an integrated approach to neurodegenerative disorders

Attendees of this webinar left with an understanding of: diseases associated with VCP mutation and the Cure VCP Diseases foundation mission to advance treatment for neuromuscular and neurodegenerative diseases associated with VCP.

August 10: Teaching Patients and Caregivers: The Role of Education in Research and Data Sharing

This webinar focused on the role of foundational education in research concepts, such as data literacy and the research process, in creating a culture of research participation and data sharing in the rare disease space. Intended for patient advocacy groups and researchers who work with rare disease patients, the webinar provided knowledge and resources to help patients and caregivers understand and engage in the research process.

May 18: GUIDs and De-Identification Tools for Rare Diseases

This webinar discussed the need for rare disease communities to adopt common GUIDs and why it helps maximize the use of patient data integrated in RDCA-DAP. An overview of the de-identification solutions available to foundations and registries was also included.

April 27: Clinical Outcome Assessments - Does one size fit all?

This webinar discussed the complexity of identifying appropriate clinical outcome assessments for rare disease clinical trials and provided an overview of the Rare Disease Clinical Outcome Assessment Consortium.

March 15: Critical Path for Rare Neurological Diseases (CP-RND): An Introduction to the Patient Community

An overview of CP-RND with feedback and questions from participants.

View all RDCA-DAP 2022 Webinars here.

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.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 54% funded by the FDA/HHS, totaling $19,436,549, and 46% funded by non-government source(s), totaling $16,373,368. 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.

RDCA-DAP | CRITICAL PATH INSTITUTE | NORD

#RDCADAP