RDCA-DAP

RARE DISEASE CURES ACCELERATOR

DATA AND ANALYTICS PLATFORM

March 31, 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 stakeholders. Thank you for your continued support.

Introduction

As we enter the second quarter of the new year, the RDCA-DAP team has hit the ground running setting the groundwork for specialized, project focused task forces, newly executed data agreements and planning efforts for the 2023 Rare and Orphan Disease Conference.

We are also very excited to announce the promotion of our very own Scientific Director Alexandre Bétourné, PhD, PharmD to Executive Director of RDCA-DAP.

Dr. Bétourné works with the RDCA-DAP team to expand its reach into new disease areas, accessing new data and enhancing C-Path's relationships within the rare disease community. He holds both a PhD and a PharmD from the University of Toulouse in France, has three patents, and has written multiple papers that intersect with several different rare disease areas.

As Executive Director, Dr. Bétourné will continue expanding RDCA-DAP's disease representation, while also leading the vision for strategic solution-based projects.

Connect with Dr. Bétourné here.

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 2023 as outreach efforts continue.

RDCA-DAP currently contains data for the following diseases:

Angelman Syndrome	Necrotizing Enterocolitis*
Congenital 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

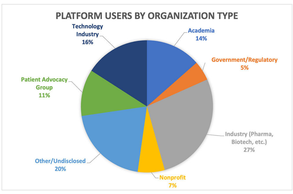
*Indicates disease with datasets that are currently discoverable on the platform

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

Platform Use

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

- 325 approved platform requests
- 35 approved workspaces for external users/research



Spotlight – RDCA-DAP to launch disease-specific Task Forces

RDCA-DAP continues its mission to address unmet needs and bottlenecks in drug development for rare diseases with the upcoming launch of the RDCA-DAP Task Forces. Similarly, to the FA-ICCD (our Friedreich's Ataxia collaborative effort with FARA), these Task Forces will work to help additional rare disease communities for which C-Path does not have an established effort. These groups will convene several industry participants, foundations and the C-Path team in a neutral environment, to aggregate data and work collaboratively to advance drug development solutions. We will focus on projects aiming at near-term deliverables and evaluate future expansion of the work as we go along.

The first RDCA-DAP task force will leverage our current database to develop models of disease progression for progressive supranuclear palsy (PSP), toward a drug development tool to inform clinical trial design for this rare neurodegenerative disorder. Shortly thereafter, we will launch a task force for Mucopolysaccharidosis type II (MPSII; Hunter Syndrome), working to create an aggregated database and build a comprehensive characterization of the disease progression using available endpoints. These two efforts will be conducted in concertation with our established pre-consortia or consortia (CP-RND for PSP and CP-LD for MPSII), as part of the RDCA-DAP Collaboratory.

RDCA-DAP always thrives for more and is currently investigating opportunities to build additional task forces, including rare epilepsies and mitochondrial disorders. These efforts rely heavily on data sharing: if you possess shareable PSP and MPS data or data that may help us create task forces for other rare diseases, please contact us.

NORD Corner

The National Organization for Rare Disorders (NORD) invites you to our 2023 Rare Impact Awards and 40th Anniversary Celebration on May 4, 2023. Hosted at the National Portrait Gallery in Washington, DC, this very special event will celebrate NORD's legacy and achievements and honor the individuals and organizations doing remarkable work on behalf of the rare disease community. Learn about this year's Rare Impact Award Honorees and secure your tickets today at rareimpact.org.

NORD Announces Four Patient Organizations to Join the IAMRARE Community and Submit Data to RDCA-DAP. Read the full post here.

Announcements

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

Upcoming and Past Events

2023 Rare and Orphan Disease Conference presented by Critical Path Institute

Webinar Series 2023

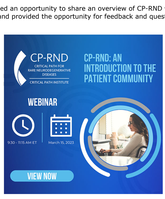
Register now for the first RDCA-DAP webinar of 2023!

In this webinar, learn more about the complexity of identifying appropriate clinical outcome assessments (COAs) for rare disease clinical trials, provide an overview of the Rare Disease Clinical Outcome Assessment Consortium and Highlight the synergy with RDCA-DAP and how data aggregation provides opportunities to refine, select and interpret COAs across rare diseases.

View On Demand Now

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

This webinar provided an opportunity to share an overview of CP-RND with the patient stakeholder group and provided the opportunity for 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.

RDCA-DAP | CRITICAL PATH INSTITUTE | NORD

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