CRITICAL PATH INSTITUTE

How RDCA-DAP Delivers Impact and Support for Rare Disease Treatment Innovation

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With over 350 million people in the world living with one of the more than 7,000 rare diseases, there is an urgent need to develop a clear understanding of how each rare disease progresses, as measured by defined outcome measures and/or biomarkers.

Meet C-Path Data Analyst Kurt Michels

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In 2019, C-Path data analyst Kurt Michels began working on a prototype project for RDCA-DAP creating r-shiny apps for later integration into the platform. His work allows people to better visualize data, similar to a

data interrogator. Then, coincidently, in 2021, Kurt was diagnosed with a rare disease himself, Huntington's disease. Now, as both a rare disease patient and analyst working on rare disease data, he strongly advocates for sharing data into RDCA-DAP. "I am speaking as someone is who is going to join and become a data point, or couple of data points. It may not necessarily help me, but if it can help someone else down that road, then I think that's really important."

"While rare disease populations are small, when you incorporate data from many rare disease datasets together, that's a sizable and significant number. You may be able to find solutions that you wouldn't have had without this platform. The more data points RDCA-DAP can generate, the more powerful and more meaningful answers you'll find."

In the United States, a rare disease is defined as one affecting fewer than 200,000 people and although the U.S. Orphan Drug Act, and similar laws in other countries, have created incentives for companies to develop rare disease drugs, only about 10% of these diseases have an FDA-approved treatment. Rare disease drug development is frequently slowed by the low numbers of patients with each disease and limited understanding of the variability and progression of each disease. This means that design of clinical trials that reliably evaluate the efficacy and safety of a potential therapy is extremely challenging.

To address this need, C-Path and the National Organization for Rare Disorders® (NORD), funded by a cooperative agreement through the U.S. Food and Drug Administration, launched the development of the Rare Disease Cures Accelerator Data and Analytics Platform (RDCA-DAP®) in 2019. The platform serves as a centralized and standardized base to host and share de-identified rare diseases data to support treatment innovation.

RDCA-DAP provides the infrastructure for a sustainable, shared scientific approach to clinical trial readiness in rare diseases by addressing the vast knowledge gaps present in the natural course of disease, the clinical evaluation of new treatments, and the patients' perspective on disease and treatment. RDCA-DAP can host clinical trial simulators that support the development of clinical trial protocols to efficiently determine if a new therapeutic is effective or not — accelerating clinical development, reducing costs, and encouraging new companies to step into rare disease drug development.

Since the initiative launched in 2019, C-Path has worked to curate sharable rare disease data from various sources, including clinical trials, observational studies, real world data, and patient registries — including those within NORD's IAMRARE® registry platform — that data owners are willing to contribute to the platform via a Data Contribution Agreement (DCA).

In 2021, RDCA-DAP launched its next objective to establish itself as the leading platform to accelerate rare disease treatment innovation. With more than 111 datasets in the platform and impressive capabilities for data search and analysis, RDCA-DAP is positioned to generate solutions for drug development, which are publicly available to qualified researchers in industry, government, regulatory agencies and academia. As such, the utility of the patient-level data is maximized and used to develop tools that will optimize and accelerate drug development across rare diseases.

Part of what makes the platform so unique is that it does not compete with ongoing efforts that are actively collecting (or plan to prospectively collect) patient-level data in rare diseases. RDCA-DAP is an added-value to such efforts by providing a standardized platform for the integration of patient-level data, with accessibility levels determined by each data contributor via C-Path's DCA. Additionally, RDCA-DAP will not hamper the ability of ongoing efforts to provide access to patient-level data in rare diseases. All data are formatted to the OMOP common data model, support FAIR data principles (**F**indable, **A**ccessible, **I** nteroperable, **R**eusable) and catalog entries are created for data sets in RDCA-DAP (highest level of the hierarchy). If data has been requested on the platform, a data dictionary (meta data level) is created to provide field and descriptors. If the data contributor wants a portion or a whole data set returned, a curated dataset will be initiated and provided upon request.

On the importance of incorporating patients' perspectives through RDCA-DAP, Theresa Mullin, Ph.D., Associate Director for Strategic Initiatives, Center for Drug Evaluation and Research (CDER), FDA, said, "Identifying the burdens of disease and current therapies that matter most, what can be measured, and what would constitute a meaningful change, can provide insight on the clinical context for regulatory review and potentially more direct evidence of drug benefits and risks when collected using valid and reliable measures and tools."

What's Next for RDCA-DAP?

The RDCA-DAP cohort builder will be made available in 2022 to facilitate detailed exploration and the development of data cohorts to be used for research and analysis. Beta testing of the cohort builder is less than six weeks away. Currently, the genomics task force is exploring options with experts in the field and with rare disease patient groups to identify the need and feasibility of implementing genomic analysis tools. Within the next year, a rare disease ontology will be integrated with the platform to enhance discovery, and the implementation of federated access with third-party platforms will be realized.

For more information on RDCA-DAP, visit https://c-path.org/programs/rdca-dap/ or email rdcadap@c-path.org.

To access the platform, visit: https://portal.rdca.c-path.org