CRITICAL PATH INSTITUTE

NORD®
National Organization for Rare Disorders

RARE DISEASE CURES ACCELERATOR

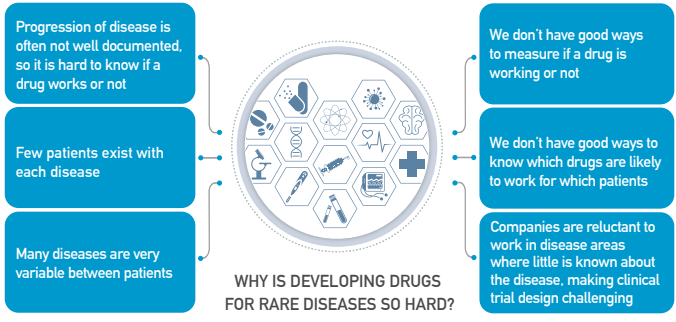
DATA AND ANALYTICS PLATFORM

The Challenge of Rare Disease Drug Development

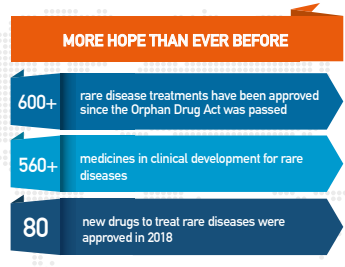
A rare disease affects fewer than 1 in 200,000 patients in the US

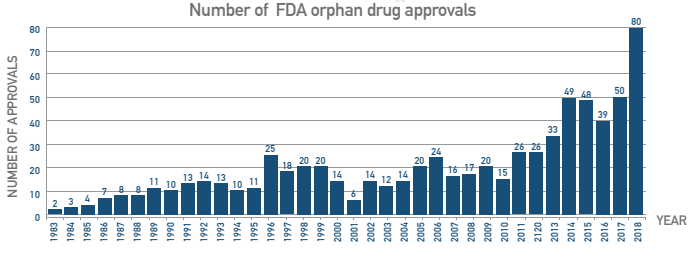
Over 7,000 rare diseases exist, so in aggregate, these affect over 350 million people in the world...

Of these, only 600 (<10%) have FDA-approved treatment options



The Orphan Drug Act, passed in 1983, provides incentives to develop drugs for rare disease. Despite huge successes since its passage, challenges in drug development for rare diseases still exist.





A Cooperative Approach to Drug Development is Needed to Accelerate Rare Disease Research

Recognizing the challenges in developing therapies for rare diseases the U.S. Food and Drug Administration (FDA) is developing plans for a Rare Disease Cures Accelerator (RDCA) with the goal of pushing for collaboration between stakeholders and development of shared infrastructure to accelerate clinical trial readiness, and make it easier and faster to test new therapies.
Some key components of RDCA include:

- Centralized standardized infrastructure to support and accelerate rare disease characterization — Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP)
- Standard core sets of Clinical Outcome Assessments (COA) measuring impacts that matter most to patients, ideally applicable to more than one rare disease
- A global rare disease clinical trials network

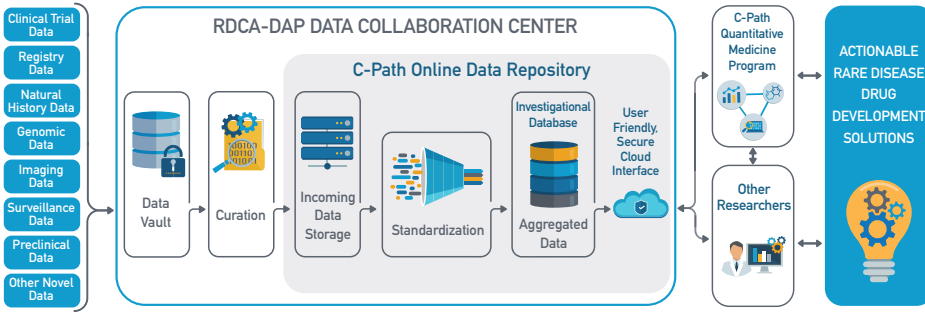
The Rare Disease Cures Accelerator-Data and Analytics Platform

RDCA-DAP is an integrated database and analytics hub that is designed to be used in building novel tools to accelerate drug development across rare diseases. It is being developed by the Critical Path Institute (C-Path) and the National Organization for Rare Disorders® (NORD) through a collaborative grant from the FDA* [Critical Path Public-Private Partnerships Grant Number U18 FD005320 from the U.S. Food and Drug Administration].

RDCA-DAP will promote the sharing of existing patient-level data, make such data available and provide an analytics platform to help interpret the data. RDCA-DAP will also encourage the standardization of new data collection.

How RDCA-DAP Works

RDCA-DAP will house anonymized data contributed by different organizations and companies around the world and share that data as widely as the data custodian allows. It will include an analytics platform that allows easy use of the data by qualified researchers and internal experts who will use the data to develop tools to accelerate drug development.



A Collaborative Effort

RDCA-DAP will not compete with ongoing efforts that are actively collecting (or plan to prospectively collect) patient-level data in rare diseases. It will contribute to the added value of such efforts, by providing a standardized platform for the integration of patient-level data that can also be shared with the community. RDCA-DAP will not sell data. Tools developed from the data by RDCA-DAP will be made publicly available.

Creating Value by Sharing and Integrating Data

By integrating available data in a regulatory-grade format suitable for analytics, the RDCA-DAP will accelerate the understanding of disease progression, clinical outcome measures and biomarkers, and facilitate the development of innovative clinical trial designs.

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Frequently Asked Questions

Why is RDCA-DAP important to the rare disease community?

RDCA-DAP will be a resource through which researchers and drug developers can access data about rare diseases and how they progress, leading to new insights about these diseases. It will also provide a way to develop new tools and methodologies to improve clinical trial design and empower the rare disease community. This will result in faster, more effective clinical trials and more rapid (and cheaper) development of new drugs. Our goal is to put the cures of tomorrow in patient hands today.

How can I be part of this project?

Any group with data relevant to the project may choose to share data with RDCA-DAP by contacting **rdcadap@c-path.org**. The governance structure will determine how researchers/companies/others will be able to access aggregated data from this platform. We anticipate that some anonymized patient-level data will be publicly available, as well as analysis of the data and tools developed from it.

How can data be contributed to RDCA-DAP?

Data may be contributed to RDCA-DAP from multiple sources. You may contact **rdcadap@c-path.org** if you would like to explore sharing data with the project. Data will be transferred through secure links in compliance with all relevant laws governing the sharing of patient-level data.

Will this compete with existing observational (natural history) studies, clinical data collections or patient registries?

RDCA-DAP will collaborate with patient groups through NORD's IAMRARE™ platform, which collects prospective data. Working with such groups ensures the highest possible data quality in new prospective studies, the ability to integrate such data in the future, and to ensure common use of data standards. RDCA-DAP can also convert existing data from prospective studies into regulatory-ready formats and share that information back with those collecting the data.

Can a patient affected by a rare disease participate directly in RDCA-DAP?

Unfortunately, RDCA-DAP is not able to accept data from individual patients, though our collaborator NORD may be able to. If you are interested in finding a suitable registry, contact us and we will refer you to a registry or open opportunity in your disease area.

Each rare disease is different. What is the value in aggregating data from multiple rare diseases?

Each rare disease is unique, and it is important to understand the progression of each one individually. However, there are many aspects of rare diseases (e.g. endpoints or outcome measures) that may be common to several disease states and we can apply learnings across diseases.

Will RDCA-DAP generate data prospectively?

RDCA-DAP is being developed to integrate existing data into a uniform regulatory-ready database. It will not generate new data nor compete with other data collection or aggregation efforts. RDCA-DAP will include data collected through other platforms which are set up to prospectively collect data, such as NORD's IAMRARE™ registry platform.

Will all rare diseases be included in RDCA-DAP? If so, how can my disease area get engaged?

If you are involved in a specific disease area and wish to engage with RDCA-DAP, contact rdcadap@c-path.org.

How will this project interact with other global data aggregation initiatives?

C-Path and NORD are reaching out to existing initiatives to ensure collaboration with established platforms, to avoid duplicating efforts, and to promote the interoperability of data.

For more information visit

c-path/rdca-dap

or contact

rdcadap@c-path.org

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