CRITICAL PATH INSTITUTE | NORD® National Organization for Rare Disorders

Advancing Drug Development. Improving Lives. Together.

RARE DISEASE CURES ACCELERATOR - DATA AND ANALYTICS PLATFORM

RDCA-DAP®

c-path.org/rdca-dap

Fostering Innovation

Critical Path Institute's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) is an FDA-sponsored initiative that provides a centralized and standardized infrastructure to support and expedite rare disease characterization, with the goal of advancing drug development solutions to accelerate the development of treatments for rare and orphan diseases.

Opportunities

RDCA-DAP leverages C-Path's core competencies to transform patient-level data into actionable solutions to accelerate drug development for rare diseases by:

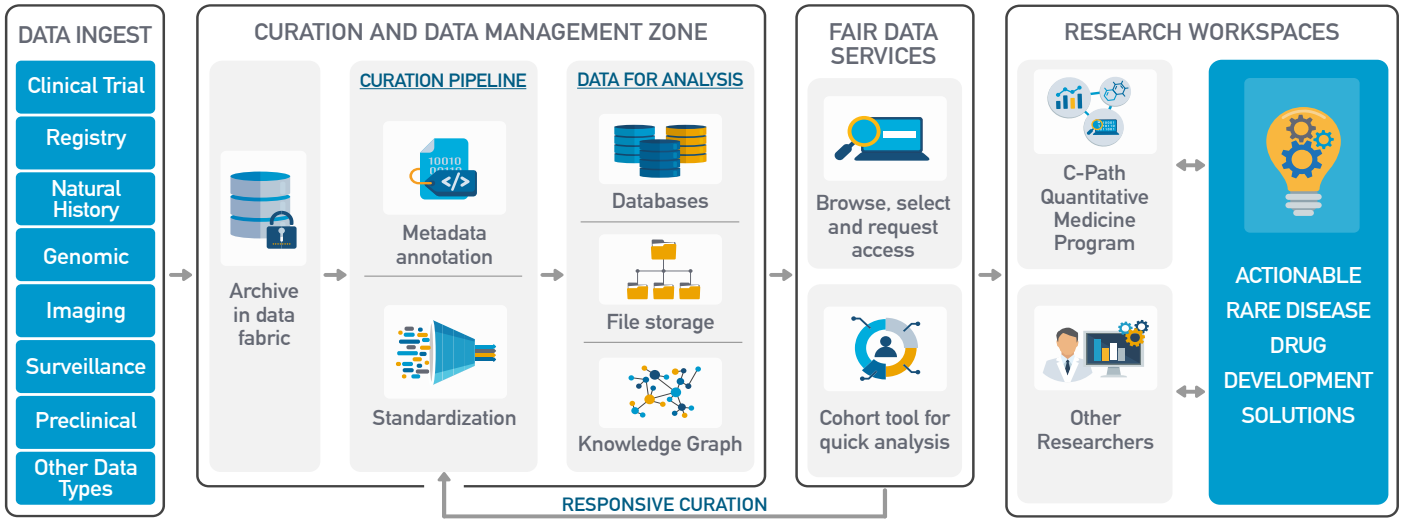
- Aggregating international patient-level data from any rare disease and any data type in a centralized, neutral, and GDPR-compliant hub.
- Enabling controlled sharing of rare diseases data through a secured interface that includes analytical tools available for free to our users.
- Increasing understanding of disease progression, patients' variability and clinical endpoints ability to track patients.
- Addressing bottlenecks in drug development.
- Developing tools to inform trial design, such as clinical trial simulation tools for Friedreich's ataxia and progressive supranuclear palsy.
- Coordinating with C-Path public-private partnerships to incubate projects to help more rare diseases populations.
- Creating bridges with external rare disease platforms, increasing the potential for data aggregation and data-driven solutions.

Solutions

RDCA-DAP integrates patient-level data across a multitude of rare diseases, contributed from academics, registries, clinical trials and hospital networks worldwide. Building on C-Path's expertise in data integration, analysis, model-informed drug development, and regulatory science, the RDCA-DAP team is focused on:

- Advancing disease progression modeling for rare diseases.
- Constructing tools to inform trial design and drug development.
- Developing rare diseases ontologies to facilitate data exploration and discoveries.
- The launch of rare disease task forces to expand activities and neutrally convene relevant stakeholders in each key disease area of focuss.

How It Works



RDCA-DAP Successes

- Critical Path Ontology (CPONT), our ontology application which facilitates mapping and linking between different standardized data vocabularies.
- Building a growing database in over 42 different diseases.
- The launch of our cohort builder tool. This tool allows interrogation of selected data to return summary level visualizations prior to submitting requests for access to patient level data, so researchers can more precisely identify data to support specific research needs.

RDCA-DAP Collaborators

DATA CONTRIBUTORS

- AllStripes
- CHI Global Registry
- CACNA1A Foundation
- CHAMP Foundation
- Children's Hospital of Orange County
- Columbia University Irving Medical Center
- Congenital Muscle Disease International Registry
- EFACTS
- Eisai Co. Ltd.
- Epilepsy Foundation
- FamilieSCN2A
- Foundation for Angelman Syndrome Therapeutics
- Foundation for Prader-Willi Research
- Friedreich's Ataxia Research Alliance
- Global Registry for COL6-related dystrophies
- Hereditary Neuropathy Foundation
- HNRNPH2-related Disorders
- International Niemann-Pick Disease Registry
- International Pemphigus and Pemphigoid Foundation
- John Walton Muscular Dystrophy Research Centre
- MitoAction
- ML Bio Solutions Inc.
- Myotubular and Centronuclear Myopathy Patient Registry
- National Organization for Rare Disorders
- National PKU Alliance
- Parent Project Muscular Dystrophy
- Rare Epilepsy Network
- Sanford CoRDS Registry
- Seattle Children's Hospital
- The Desmoid Tumor Research Foundation
- The Sturge-Weber Foundation
- TSC Alliance
- Ultragenyx
- University of Rochester Medical Center

FOUNDING MEMBERS

- Critical Path Institute
- National Organization for Rare Disorders

COLLABORATORS

- Cure Mito Foundation
- EATRIS
- Global Genes
- Quinten Health
- Vivli

Current Data

- Amyotrophic Lateral Sclerosis
- Angelman Syndrome*
- CACNA1*
- Charcot-Marie-Tooth Disease*
- Choroideremia
- COL6-related dystrophies*
- Congenital Hyperinsulinism*
- Cystic Fibrosis
- Desmoid Tumor*
- Duchenne Muscular Dystrophy*
- Facioscapulohumeral Muscular Dystrophy*
- Fibrous Dysplasia Disorders*
- Friedreich's Ataxia*
- GNE Myopathy*
- hnRNPH2-related disorders*
- Kidney Transplant
- KIF1A Associated Neurological Disorder*
- Kleefstra Syndrome*
- Leigh Syndrome and other Mitochondrial Disorders*
- Lennox-Gastaut Syndrome*
- Limb-Girdle Muscular Dystrophy
- Malan Syndrome
- Mitochondrial Disease*
- Mucopolysaccharidoses*
- Myhre Syndrome
- Myotubular or centronuclear myopathy*
- Necrotizing Enterocolitis*
- Niemann-Pick Disease
- Ocular Melanoma*
- Pemphigus & Pemphigoid*
- Phenylketonuria*
- Polycystic Kidney Disease*
- Prader-Willi Syndrome*
- Progressive Supranuclear Palsy*
- Rare Epilepsies*
- Ryanodine Receptor 1 Related Myopathies*
- SCN2A-related Disorders
- Spinal Muscle Atrophy with Respiratory Distress*
- Spinocerebellar Ataxias type 1, 2, 3, 6, 7, 8 & 10*
- Sturge-Weber Syndrome*
- SYNGAP1-related intellectual disability
- Tuberous Sclerosis*
- VCP Disease

Currently discoverable on the platform

portal.rdca.c-path.org

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c-path.org/fda-acknowledgement

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