

RDCA-DAP

Rare Disease Cures Accelerator-Data and Analytics Platform

How RDCA-DAP Can Help Overcome Rare Disease Challenges in the Drug Development Process



1 Discovery and Development

At this stage of drug development, researchers try to identify a compound that shows promise as a treatment for a particular disease. This involves studying the disease process and designing a product to stop, slow, or reverse progression. For rare diseases, this research can be challenging when the available data and clinical understanding of the disease are limited, making it difficult to identify potential treatment pathways. Even when scientists identify an approach that appears promising, further testing is needed. Developing or refining the models to support this step can be a lengthy process.

RDCA-DAP brings together rare disease data from various sources to help researchers better understand the symptoms and progression of disease. The tools provided by the data and analytics platform will support researchers as they seek new ways to treat the disease or its symptoms, opening new opportunities for scientific discovery.

2 Preclinical Research

In preclinical research, studies are done to test the most promising treatment candidates in the laboratory and in animals. These studies provide information on whether a product can safely be given to humans, determine the best way to deliver the treatment, and establish an initial dosing level for clinical trials. Limited funding opportunities for the study of rare diseases may result in delays as animal models may need to be built or refined to help determine the effectiveness and safety of a proposed therapy.

RDCA-DAP provides access to data in a centralized location to qualified researchers in industry, academia, and government to help lay the path towards efficient clinical development through understanding of biomarkers and endpoints.

3 Clinical Research

In clinical research, treatments are tested in humans to determine their safety and effectiveness. The small numbers of patients with a particular rare disease who are often geographically dispersed and managing serious medical conditions can make it challenging to design clinical trials. For many rare diseases, scientific understanding is evolving alongside therapeutic development and the outcome measures or endpoints that are used to demonstrate impact in trials may not be confirmed or well-defined. There are additional special considerations for pediatric trials.

RDCA-DAP provides researchers with the data and tools to develop a greater understanding of rare diseases and how diseases progress, encouraging innovative clinical trial designs that may require less time and resources to complete.

4 FDA Review

Once evidence is available to demonstrate that a treatment is safe and effective for its intended use, the FDA will review the application and data for completeness and make a decision to approve or not. With the challenges associated with rare disease clinical trials and limited data, it is not uncommon for the FDA to review applications with data that is inadequate for regulatory purposes often due to a limited understanding of the natural history of rare diseases. This can lead to delays in FDA reviews and may result in requests for additional analyses or studies to address open questions.

Data from RDCA-DAP will be formatted for regulatory submissions and will help both regulators and companies developing therapies to interpret and reinforce results in rare disease populations, supporting timely regulatory decisions.

5 FDA Post-Market Safety Monitoring

After a new product is approved and available to the public, FDA continues to monitor it for any safety issues that might become apparent over time. This ongoing surveillance includes programs that allow manufacturers, medical professionals, and consumers to track and report safety concerns with approved products.

RDCA-DAP is developed to combine existing data sources to create a more complete understanding and characterization of rare diseases. The existence of this centralized database, while not specific to post-market safety monitoring, may help FDA and drug companies better understand and resolve any concerns with new therapies as quickly as possible.

For more information, visit: rarediseases.org/rdca-dap

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For more information on the drug development process visit: www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/approvalapplications/newdrugapplicationnda/default.html

