
C-Path's Duchenne Regulatory Science Consortium and Duchenne Data Foundation Announce Collaboration to Advance Solutions for Duchenne Muscular Dystrophy

TUCSON, Ariz. and VEENENDAAL, Netherlands, August 9, 2023 — [Critical Path Institute's](#) (C-Path) Duchenne Regulatory Science Consortium (D-RSC) and the Duchenne Data Foundation (DDF) are excited to announce a joint collaboration aimed at advancing research and improving healthcare for individuals with Duchenne muscular dystrophy (DMD) and other dystrophinopathies (conditions linked to mutations in the DMD gene).

The collaboration between D-RSC and DDF will focus on several key areas to address the challenges faced in the evaluation, development and implementation of solutions for Duchenne.

One of the pressing challenges in dystrophinopathies research is the lack of a unified data repository. Currently, diverse datasets related to various aspects of dystrophinopathies are dispersed in different formats and locations, hindering the ability of researchers and clinicians to fully utilize the potential value of the collected data.

To address these challenges, C-Path's Rare Disease Cure Accelerator–Data and Analytics Platform (RDCA-DAP®), provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical medical therapies, including DMD and other dystrophinopathies data. Through its integrated database of patient-level clinical data from DMD studies, D-RSC has been instrumental in generating standardized terminology and developing the [CDISC Duchenne Muscular Dystrophy Therapeutic Area User Guide](#), published in 2017.

D-RSC has also developed the first Clinical Trial Simulation tool for DMD, receiving a Letter of Support from the European Medicines Agency, and has undergone additional regulatory review processes towards quantitative solutions and biomarkers to advance drug development in DMD.

DDF aims to develop a global dystrophinopathy patient-level data repository with a two-fold purpose: acting as a digital catalog to identify and access relevant datasets from heterogeneous sources and providing long-term storage to ensure availability of dystrophinopathy data when needed. The DDF repository also aspires to provide advanced tools for data analysis, mining and decision-making in diagnosis, prognosis, therapeutic regimes and care. Compliance with GDPR and current security regulations ensures the privacy and security of the data within the DDF repository.

“We are excited for the opportunity to collaborate with the team of scientists and patient advocates at DDF and continue expanding our collaborations globally,” said Ramona Belfiore-Oshan, Ph.D., D-RSC Executive Director. “D-RSC and DDF both contribute to a shared mission, and we look forward to promoting vibrant discussions and mission-driven solutions to advance drug development for Duchenne and other dystrophinopathies.”

By combining the efforts and expertise of D-RSC and DDF, this collaboration aims to bridge knowledge gaps generated by fragmented information. This enables more effective research and drug development and ultimately improves healthcare outcomes for individuals affected by dystrophinopathies.

“We are thrilled to join forces with D-RSC in our shared mission to improve the lives of individuals with dystrophinopathies,” added George Paliouras, Ph.D., DDF chairman. “The collaboration will not only enhance our ability to develop a unified data repository but also accelerate the translation of research findings into effective treatments, giving hope to patients and their families.”

The collaboration between D-RSC and DDF represents a significant step forward in the fight against dystrophinopathies. By combining their expertise and dedication, the two organizations are poised to make a lasting impact in the field and bring us closer to a future where effective treatments for Duchenne muscular dystrophy and other dystrophinopathies are a reality.



About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the [FDA’s Critical Path Initiative](#). **C-Path’s mission is to lead collaborations that advance better treatments for people worldwide.** Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path’s global headquarters is located in Tucson, Arizona and C-Path’s Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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About Duchenne Regulatory Science Consortium (D-RSC)

D-RSC is a collaborative partnership between Critical Path Institute (C-Path) and Parent Project Muscular Dystrophy (PPMD). It focuses on advancing the science of drug development and regulatory evaluation for DMD and related neuromuscular diseases. For more information, visit c-path.org/d-rsc.

DUCHENNE

DATA FOUNDATION

About Duchenne Data Foundation (DDF)

DDF is dedicated to fostering convergence between stakeholders and developing a unified data repository for dystrophinopathies. The foundation aims to integrate heterogeneous data sources, facilitate collaboration, and

provide advanced tools for data analysis and decision-making in the field. For more information, visit <https://www.duchennedatafoundation.org>.

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