# Newcastle University's John Walton Muscular Dystrophy Research Centre Contributes Data to C-Path's RDCA-DAP

**TUCSON**, Ariz., June 7, 2023 — Critical Path Institute (C-Path) announced today that Newcastle University UK's John Walton Muscular Dystrophy Research Centre will contribute data to the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®) managed by C-Path. These data come from the Myotubular (MTM) and Centronuclear Myopathy (CNM) Patient Registry and the Global Registry for COL6-related dystrophies.

RDCA-DAP provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical development. Additionally, the platform advances best practices to support the rigorous conduct of natural history studies, with attention to established data quality standards, in order to be most useful to clinical trial design and regulatory review. It includes a robust, integrated database and analytics hub that allows for the aggregation of rare disease data from various sources and the efficient and effective interrogation of that data.

"We are excited to receive these valuable COL6-related dystrophies and MTM and CNM registry datasets from the John Walton Muscular Dystrophy Research Centre. The sharing of this data continues to enrich our database in rare dystrophies, which will help create new opportunities to advance research in rare muscular disorders" said RDCA-DAP Executive Director Alexandre Betourne, Ph.D., Pharm.D. "Our shared mission to accelerate the development of medical products and effective treatments for rare diseases aligns perfectly with C-Path's goals, and we look forward to the positive impact this partnership will have on patients and their families."

"Sharing data is an essential step to address knowledge gaps in rare muscular dystrophies and allow the creation of data-driven solutions, from the community, for the community," said Ramona Belfiore-Oshan, Ph.D., lead of the Duchenne Regulatory Science Consortium at C-Path. "We applaud Dr. Straub and collaborators for this data contribution that will aid in the generation of drug development tools for myotubular and centronuclear myopathy."

Launched in November 2014, the John Walton Muscular Dystrophy Research Centre at Newcastle University UK has an outstanding international reputation in research and care for neuromuscular diseases. The Centre focuses on five core activities: clinical care, clinical research, diagnostics, basic research, as well as strategic partnerships and networking.

The MTM and CNM Patient Registry is an open-ended, international research database with over 450 participants worldwide. Data is collected every 6 months from patients and their clinicians, and registration is open to anyone with a diagnosis of MTM or CNM, as well as X-linked MTM carriers. The registry is jointly funded by Myotubular trust, Muscular Dystrophy UK, and Astellas Gene Therapies (formerly Audentes Therapeutics) of Astellas Pharma Inc.

The Global Registry for COL6-related Dystrophies collects data from individuals with a diagnosis of Bethlem Myopathy, Ullrich Congenital Muscular Dystrophy, and intermediate forms of these diseases. There are currently over 200 participants represented on the registry. The registry is funded by Muscular Dystrophy

UK.

These registries were established to support clinical trial readiness and recruitment, as well as to enable future research and inform standards of care. Both registries are members of the TREAT-NMD Global Registry Network which encourages harmonization of data and sharing of best practice between neuromuscular patient registries around the world.

“Contributing our registry data to the RDCA-DAP represents another milestone in our efforts to advance research and improve the lives of individuals affected by neuromuscular conditions,” said Director of the John Walton Muscular Dystrophy Research Center, Professor Volker Straub, Ph.D., This partnership will amplify the research impact of our patient registries by increasing the visibility and accessibility of our data to the wider research community. By pooling data in this way, we hope to identify trends and carry out research that may have eluded individual research efforts. We hope to continue our collaboration with this exciting project, with plans to provide data from our other neuromuscular registries in the near future.”

The collaboration between Newcastle University UK’s John Walton Muscular Dystrophy Research Centre and C-Path will enhance the understanding of these rare diseases and ultimately facilitate the development of innovative treatments. By sharing the data from these registries, researchers worldwide can access comprehensive information to aid in their studies and improve patient outcomes.

Organizations interested in contributing data to RDCA-DAP can visit c-path.org/rdca-dap or email rdcadap@c-path.org. The platform is now OPEN and accepting applications for use; visit portal.rdca.c-path.org to apply and learn more.

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**About Critical Path Institute**
Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path’s mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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