C-Path Launches Clinical Trial Simulator for Duchenne Muscular Dystrophy Research

TUCSON, Ariz., March 28, 2024 — Critical Path Institute's (C-Path) Duchenne Regulatory Science Consortium (D-RSC) is excited to announce the launch of a groundbreaking model-based Clinical Trial Simulator (CTS), specifically designed to improve design of efficacy studies for potential therapies for Duchenne muscular dystrophy (DMD). This pioneering Drug Development Tool is set to positively impact the medical research community by significantly optimizing clinical trial design.

DMD is a rare, fatal, X-linked, muscle wasting, and progressive disease that predominantly affects boys but has been shown to manifest in some female carriers. Research and development in this area have been challenging due to the complexities associated with the disease.

Developed by C-Path's Quantitative Medicine Program and D-RSC, together with an extensive group of collaborators, this simulator utilizes advanced models of the longitudinal changes in the velocity at which individuals can complete specified, timed functional tests. These tests, which include the supine-stand, 4-stair climb, 10-meter walk/run test, or 30-foot walk/run test, are frequently used as clinical trial endpoints. Additionally, the tool models longitudinal changes in forced vital capacity and the North Star Ambulatory Assessment total score, providing a comprehensive approach to trial design for simulation. A key feature of the DMD Clinical Trial Simulator is its incorporation of relevant sources of variability such as baseline severity at study start, age, steroid use, genetic mutation and study type. This ensures that the simulations are as realistic and informative as possible, paving the way for more effective and efficient clinical trial designs.

In November 2022, D-RSC received a Letter of Support from the European Medicines Agency, and the Clinical Trial Simulator is currently being reviewed by the U.S. Food and Drug Administration (FDA).

"The launch of the DMD Clinical Trial Simulator represents a significant step forward in our fight against Duchenne muscular dystrophy," said Ramona Belfiore-Oshan, Ph.D., Executive Director for D-RSC. "By using quantitative methodologies that allow the improvement of trial design *in silico*, we can accelerate the development of effective therapies for DMD and provide a learning platform for application of model informed drug development in other rare diseases."

"The DMD Clinical Trial Simulator is poised to become a valuable resource for researchers and pharmaceutical companies involved in DMD research," commented C-Path CEO Klaus Romero, M.D. M.S., FCP. "Its ability to simulate various scenarios and outcomes will not only improve the accuracy of clinical trials but also reduce the time and resources required to bring new therapies to market."

The CTS will be made publicly available on c-path.org, here, and hosted on the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), C-Path's designated analytics hub for data analysis and generation of drug development tools across multiple rare diseases.

"We advance the RDCA-DAP platform as a place for innovation for rare disease drug development and are excited to offer open access to C-Path's drug development tools on RDCA-DAP. Granting access to the D-RSC Clinical Trial Simulator through RDCA-DAP creates a unique opportunity for innovative trial designs in DMD," said Alexandre Bétourné, Pharm.D., Ph.D., Executive Director, RDCA-DAP.

About D-RSC:
D-RSC (Duchenne Regulatory Science Consortium) is comprised of a team of experts dedicated to advancing the understanding and treatment of Duchenne muscular dystrophy through innovative research and technology solutions.

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 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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