C-Path Receives Letter of Support from EMA on Duchenne Muscular Dystrophy Clinical Trial Simulation Platform

TUCSON, Ariz., Jan. 25, 2023 — Critical Path Institute (C-Path) today announced that the European Medicines Agency (EMA) has issued a letter of support for the Duchenne Regulatory Science Consortium's (D-RSC) Model-based Clinical Trial Simulation Platform for Duchenne Muscular Dystrophy (DMD).

On November 10, 2022, the EMA stated, "The EMA acknowledges the Applicant Critical Path Institute efforts in establishing their model-based Clinical Trial Simulation Platform (CTSP) for Duchenne muscular dystrophy treatments. The EMA has issued this Letter of Support to encourage the further development and validation of the CTSP, as well as encouraging sponsors to share patient-level data with the D-RSC team."

"This Letter of Support is an outstanding milestone and a testament to the D-RSC members' continued support in providing strong scientific leadership and pioneering data sharing for the benefit of the entire Duchenne community," said C-Path Chief Science Officer Klaus Romero, M.D., M.S., F.C.P. "Such a tremendous accomplishment is an example of C-Path's successful pre-competitive approach, and the D-RSC team's ongoing efforts to generate quantitative solutions to accelerate drug development for DMD," explained D-RSC's Executive Director, Terina Martínez, PhD.

Optimal design of clinical trials in DMD for rigorous, successful readouts remains a field-wide challenge due to inherent disease complexity, heterogeneity, and its rarity. This model-based clinical trial simulation platform — which is a first-of-its-kind for DMD — will help inform exploration of key design constructs including sample size, sampling scheme, patient enrollment criteria, dose and study design and accelerate medical product development.

DMD is a rare, muscle-wasting, fatal disorder associated with mutations on the X chromosome that affects predominately boys and impacts individuals worldwide. Diagnosis typically occurs around 2-5 years of age, and as the disease progresses, those affected with DMD experience loss of muscle tissue with consequent muscle weakness, loss of ambulation, loss of upper body function, scoliosis, respiratory impairment and cardiomyopathy.

D-RSC is a public-private partnership and was co-founded by C-Path and Parent Project Muscular Dystrophy to accelerate therapeutic progress and establish innovative drug development tools to better inform clinical trial protocols for DMD. D-RSC continues to pursue its mission with its collaborative membership spanning academic and clinical researchers, nonprofit research and patient advocacy groups, and the drug development industry.

The Letter of Support can be found on the EMA website here or on C-Path's website here.

About Critical Path Institute
Critical Path Institute (C-Path) is an independent, global nonprofit established in 2005 as a public-private partnership. As a neutral convener, C-Path's mission is to be a catalyst for innovation that accelerates the path to a healthier world. Through collaboration, we develop new approaches to advance the development of new therapies and regulatory science. C-Path provides an open, pre-competitive forum in which industry leaders, regulators, scientists, academic researchers, and patient groups work together on solutions that stand to benefit those with the greatest need. C-Path offices are located in Tucson, Arizona and Amsterdam, Netherlands with additional staff in multiple other locations. For more information, visit c-path.org.

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