C-Path to Lead Comprehensive Task Force Focused on Accelerating Drug Development for Limb-Girdle Muscular Dystrophies

TUCSON, Ariz., September 30, 2024 — Critical Path Institute (C-Path) today announced the formation of a new task force under its Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®), dedicated to advancing therapeutic development for limb-girdle muscular dystrophies (LGMDs). This initiative brings together leading organizations and experts in a concerted effort to tackle the challenges associated with LGMDs drug development. LGMDs are a group of disorders that affect voluntary muscles, primarily those around the hips and shoulders.

The confirmed members of the task force include ML Bio Solutions, the Coalition to Cure Calpain 3, the CureLGMD2i Foundation, the Jain Foundation, the LGMD2D Foundation, the LGMD2i Research Fund, and The Speak Foundation. Additionally, collaborations are underway with key thought leaders and patient advocacy groups to ensure comprehensive stakeholder engagement.

This task force will leverage the collective expertise of its members to expedite the development of new treatments for LGMDs. By integrating patient-level data from diverse sources through C-Path's RDCA-DAP — an FDA-funded initiative that provides a centralized and standardized infrastructure to support and accelerate rare disease characterization — the task force will work to identify and address the critical gaps in LGMDs research and development. The collaboration provides a neutral platform for stakeholders from industry, regulatory agencies, academia, and the LGMDs community to engage in meaningful dialogue and share data, enhancing the efficiency of therapeutic advancements for LGMDs.

"Limb-girdle muscular dystrophies are a devastating group of diseases with significant unmet medical needs," said Alexandre Betourne, Pharm.D., Ph.D., RDCA-DAP Executive Director. "By convening this task force, we are harnessing the collective strengths and knowledge of our members to drive forward the development of effective treatments for those affected by LGMDs."

Doug Sproule, M.D., M.Sc., Chief Medical Officer at ML Bio Solutions, commented on the task force's formation: "We are grateful for the opportunity to join the LGMDs Task Force to enhance collaboration between key stakeholders and advance the development of potential treatments for LGMDs, where there currently is a large unmet need. By providing our LGMD2I/R9 natural history data, we hope to increase the understanding of the progressive condition to improve the lives of those with LGMDs and their families."

The Speak Foundation Founder, Kathryn Bryant Knudson , emphasized the transformative potential of collaboration: "At the Speak Foundation, we understand that collaboration and shared knowledge are the cornerstones of groundbreaking drug development. This task force will harness data to forge powerful tools essential for innovation."

"I look forward to leading this effort with my colleagues from RDCA-DAP and Dr. Ramona Belfiore-Oshan, Executive Director for the Duchenne Regulatory Science Consortium (D-RSC)," stated RDCA-DAP Scientific Director Heidi Grabenstatter, Ph.D. "Leveraging data from multiple sources and across LGMD subtypes in addition to collaborative knowledge sharing within the task force will be key to developing a clinically impactful drug developmental tool that helps accelerate therapies for patients living with high unmet needs in the LGMD community."

For further information and to inquire about joining the RDCA-DAP task force on limb-girdle muscular dystrophies, interested parties are encouraged to contact rdca-dapadmin@c-path.org.

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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