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## **Advancing Hope in Limb Girdle Muscular Dystrophies: Collaboration, Data, and Innovation During LGMDs Awareness Month**

Each September, Limb Girdle Muscular Dystrophies (LGMDs) Awareness Month shines a light on a devastating neuromuscular disease that remains widely underrecognized despite its profound impact on patients, families, and caregivers. LGMDs are a group of rare, inherited genetic disorders characterized by progressive weakness of the shoulder and hip girdle muscles due to loss of muscle fibers. Over time, people living with LGMD often experience increased difficulties with mobility and daily function, while facing a significant lack of effective treatment options.

For the LGMD community, September serves as a vital call to action to accelerate research, improve clinical understanding, and advance the development of therapies that can meaningfully change patients' lives. At Critical Path Institute® (C-Path), that mission is central to our work, and we take pride in it every day.

In September 2024, C-Path launched the Limb Girdle Muscular Dystrophies Task Force through its Rare Disease Cures Accelerator–Data and Analytics Platform (RDCA-DAP®). The initiative was created to address the urgent need for better tools, stronger collaboration, and more efficient drug development pathways for LGMDs.

This growing collaboration brings together leading organizations across the LGMDs ecosystem, including AskBio, Coalition to Cure Calpain 3, CureLGMD2i Foundation, Jain Foundation, Italfarmaco, LGMD Awareness Foundation, Inc., LGMD2D Foundation, LGMD2i Research Fund, BridgeBio, Newcastle University, Sarepta Therapeutics, Team Titin, The Dion Foundation, The Speak Foundation, University of Florida, University of Iowa and academic experts, all united by a shared goal: accelerating drug development and improving lives for people living with LGMDs.

Since its launch, the LGMDs Task Force has already achieved several important milestones. The initiative continues to expand a robust natural history and clinical trial database that is being made available to allow researchers to understand how LGMDs progress over time and inform their clinical trial design activities, including endpoint selection. In addition, the initiative is leveraging the database to support analytics of clinical endpoints and developing disease progression models that will inform future drug development tools designed to guide data-driven decision-making in therapeutic development.

Critical Path Institute is excited to announce the recent execution of a data contribution agreement with University of Newcastle upon Tyne of King's Gate and Jain COS consortium to confidentially share the "International Clinical Outcome Study for Dysferlinopathy" with RDCA-DAP's LGMD Task Force.

These advances are critical in a disease area where small patient populations, siloed and heterogeneous longitudinal data, and clinical complexity have historically slowed progress.

Through RDCA-DAP, C-Path enables the integration and analysis of patient-level data from diverse sources, helping stakeholders identify critical gaps in LGMD research while advancing biomarkers and clinical outcome assessments. By fostering collaboration among industry, academia, regulators, and patient advocacy organizations, the platform creates a shared scientific foundation that can reduce duplication, improve trial design, and accelerate the development of new treatments.

Equally important is keeping patients at the center of the research and drug development process. For rare neuromuscular diseases like LGMDs, collaboration between industry and patient advocates, as well as data sharing, are essential efforts needed to overcome longstanding hurdles. To ensure that the voice of those with lived experience is heard, RDCA-DAP provides the infrastructure and scientific framework needed to transform fragmented data into actionable insights that can ultimately improve clinical development and patient outcomes.

This LGMD Awareness Month, C-Path recognizes the resilience of the LGMD community and reaffirms its commitment to advancing innovative, collaborative solutions that bring hope closer to reality for those living with this devastating disease.

To learn more about C-Path's LGMD Task Force and ongoing efforts through RDCA-DAP, click [here](#).