cTAP Study Indicates Duchenne Muscular Dystrophy Clinical Trial Enrollment Criteria Should Expand Beyond Ambulatory Status

Study Co-Funded by CureDuchenne and cTAP Highlights Need for More Specific Enrollment Criteria That Could Increase Patient Participation and Drive More Comprehensive Therapeutic Evaluation

CAMBRIDGE, Mass., July 24, 2024 – The Collaborative Trajectory Analysis Project (cTAP) and international collaborators have announced evidence to support using a more sophisticated set of criteria than is typically used to determine eligibility for clinical trials in Duchenne muscular dystrophy (DMD), a rare disease characterized by progressive muscle weakness and atrophy. These findings, based on natural history data provided by global advocacy nonprofit CureDuchenne, could increase patient recruitment and eligibility for clinical trials by broadening the population eligible for trials and effective therapies for DMD.

Published in PLOS ONE, the study examined when upper limb and pulmonary functions start to decline relative to when a patient loses the ability to walk, with the goal of informing clinical trial designs for new therapeutics. The study was based on analyses of PRO-DMD-01, a prospective longitudinal natural history study of disease progression among boys with genetically confirmed DMD and conducted by an international team of leading clinical experts and care providers. The results show that more than half of all patients experienced clinically significant deficits in pulmonary and upper limb function *before* experiencing loss of ambulation. "These findings should help drug developers include more patients in clinical trials that test for upper-limb or pulmonary function benefits, rather than limiting to patients who cannot walk," said Dr. James Signorovitch, co-founder of cTAP and Partner at Analysis Group.

Professor Nathalie Goemans, UZ Leuven, Belgium, who led the study, commented, "At a time when many patients were enrolling in studies of exon-skipping therapeutics, this natural history study was challenging to conduct and is one of the only studies of its kind that tracked patients through their transition when ambulatory function is lost. We are so grateful to the patients, trial teams, and scientists who made this study possible."

"There is an urgent need for treatments for individuals of all ages and abilities facing this devastating disease. Research is progressing, but many trials focus on younger and ambulatory populations. We cannot leave older patients and young adults behind," said Debra Miller, founder and CEO of CureDuchenne, a founding patient advocacy partner and provider of initial seed funding to cTAP. "This study demonstrates our need to continuously improve clinical trials in order to best serve our community."

About cTAP

cTAP is a multinational, multistakeholder nonprofit collaboration dedicated to advancing clinical trial design, analysis, and data resources in rare neuromuscular diseases. Leveraging expansive natural history and placebo datasets, a highly engaged network of world-class experts, and a proven track record of

success in collaborative problem-solving, cTAP develops evidence-based advancements with real-world impact. With an initial focus on Duchenne muscular dystrophy, cTAP's rigorous, data-driven solutions hold promise for numerous rare, heterogeneous diseases and patient communities worldwide.

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