Beyond Traditional Placebo Controls in Rare Disease Clinical Trials: New cTAP Evidence Supports Patient-Centric Alternatives

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Collaborative Trajectory Analysis Project (cTAP) →
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CAMBRIDGE, Mass., Feb. 7, 2023 /PRNewswire/ -- The Collaborative Trajectory Analysis Project (cTAP), a global coalition in rare disease, has announced evidence to support patient-centric alternatives to the conventional use of genetically matched placebo controls in clinical trials for RNA-editing therapies in Duchenne Muscular Dystrophy (DMD).

Published in Neurology, the large, multi-institution, multi-national study of more than 700 patients with DMD quantified the impact of genotype classes on observed disease. Results showed that less than 2 percent of change in motor function over 48 weeks, the typical duration of a placebo-controlled trial, is due to differences in genotype class.

"Our study presents an opportunity to address the growing practical and ethical challenges of enrolling genotype-matched placebo arms in DMD clinical trials of genotype-specific therapies," said Professor Francesco Muntoni, lead author and Chair of Paediatric Neurology at the UCL Great Ormond Street Institute of Child Health and the Great Ormond Street Hospital for Children, London. "We provide unbiased evidence that the contribution of individual DMD genotypes for clinical progression over the course of a clinical trial is minimal. The implications are important, providing a framework for using this knowledge to recruit more efficiently into these trials, simplify study execution, and reduce the number of treatment-eligible children receiving placebo."

"Our findings have significant implications for drug development," said James Signorovitch, co-founder of cTAP and a Managing Principal at Analysis Group. "Most importantly, clinical trials in DMD should consider patients' initial levels of motor function at least as carefully as their genotypes. Otherwise, we run an unnecessary risk of clouded treatment effects. Our findings also open the door to multi-genotype approaches, including mixed-genotype controls and platform trials, that can more efficiently test new therapies."

"cTAP strives to identify opportunities to make clinical trials more efficient, definitive, and patient-centric while still minimizing regulatory risk and upholding rigorous data integrity," said Susan J. Ward, co-founder and Executive Director of cTAP. "This study is an important addition to the unbiased evidence necessary to accelerate development of new therapeutics in rare disease."

About cTAP

cTAP makes tomorrow's improvements in clinical trial design and analysis possible today. It is a multi-national, multi-stakeholder collaboration that cuts to the heart of which questions drug developers need answered most to minimize variance and uncertainty in clinical trials and to achieve faster, more highly powered, more definitive results. Leveraging access to expansive natural history and placebo databases and its highly engaged network of world-class experts, cTAP applies advanced data science in the context of ongoing interaction with academic, drug developer, and patient stakeholders to develop rigorous, data-driven solutions with near-term, real-world application. cTAP's initiating focus targets critical unmet need in Duchenne muscular dystrophy, but its work bears implications and potential benefit to many rare, heterogeneous diseases and patient communities in need.

SOURCE Collaborative Trajectory Analysis Project (cTAP)