cTAP Study Shows Real-World, Natural History Data Comparable to Placebo Data from Clinical Trials in Duchenne Muscular Dystrophy

*Results published in peer-reviewed journal Neurology indicate that real-world data (RWD) and natural history data (NHD) could augment or replace placebo controls in DMD clinical trials.*

*Use of RHD and NHD have potential to streamline and accelerate clinical programs targeting new treatments for DMD.*

**CAMBRIDGE, MA –** (September 8, 2020) – The Collaborative Trajectory Analysis Project (cTAP), a multi-stakeholder, global research coalition in Duchenne muscular dystrophy (DMD), today announced results from the largest ever multi-national, multi-collaboration study in DMD indicating that both real world data (RWD) and natural history data (NHD) are highly comparable to data from patients treated with placebo in multiple recent clinical trials. Results demonstrate that use of RWD or NHD could supplement or possibly even replace use of a placebo arm in future DMD clinical research, potentially helping to streamline efforts to develop new treatments.

“This study found a striking level of consistency in the six-minute walk distance assessment in DMD patients from six clinical trial placebo arms and patients from five different real world and natural history studies,” said Craig McDonald, a co-author of the study and Professor and Chair of the Department of Physical Medicine and Rehabilitation at the University of California Davis. “This rigorous study establishes a strong foundation for using natural history data as a substitute for placebo control in clinical trials and as a comparator to determine the effectiveness of prescribed drug treatments versus standard of care treatment.”

In the study, researchers brought together by cTAP analyzed disease progression in 383 patients with DMD randomized to placebo arms in six clinical trials compared with data observed in a real world setting for 430 patients from five clinical registries in the U.S. and Europe. Patient outcomes were assessed based on similar inclusion/exclusion criteria and adjusted for known prognostic factors. This is the first study to demonstrate the comparability of disease progression in NH/RWD and placebo arms of clinical trials in DMD. To access the article, click [here](#).

To date, the potential for bias in clinical trials versus clinical practice has limited the use of NH/RWD to supplement or replace placebo controls. This study demonstrates that the potential for bias is low, and as such provides a foundation for drug developers to now consider application of NH/RWD in registration trials.

“The results of this landmark research effort have profound implications for clinical research in DMD and potentially many other rare diseases. We applaud cTAP for supporting this research effort and look forward to sharing these insights with all of the stakeholders in DMD research including regulators, industry, clinicians and patient advocates,” said Francesco Muntoni, Professor and Chair of Pediatric Neurology at University College London. “This effort, which addresses a key priority for patient foundations, shows clearly that by working together we can identify better solutions to advance clinical research that can facilitate the development of new treatments for DMD.”
About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a uniformly fatal, progressive muscle-wasting disease affecting about one in 3,500-6,000 male live births. Patients with Duchenne lack the ability to make dystrophin, a protein crucial to muscle function. As their muscles deteriorate, they progressively lose the ability to walk, feed themselves, turn over in bed, and ultimately to breathe. While there is no cure, the past decade has seen an explosion in research resulting in more than 15 new therapies entering clinical development, with some receiving conditional approval. Learn more about Duchenne at http://cureduchenne.org, http://parentprojectmd.org and http://charleysfund.org.

About cTAP

The collaborative Trajectory Analysis Project (cTAP) was established in 2015 to solve critical problems in drug development in Duchenne muscular dystrophy with a sense of urgency for the thousands of patients who are waiting for treatments. cTAP’s dynamic global alliance of academic, industry, and patient stakeholders works collaboratively to identify and address priority questions crucial to the research community, regulators, and health authorities. Partnered with Analysis Group, cTAP’s advanced analytics platform unlocks the power of individual patient data to develop insights and tools with near-term application for more effective clinical trial design and analysis. With its large and continually growing multi-national database of natural history and clinical trial data sources, cTAP enables therapy developers to conduct smarter trials and bring new therapies to patients sooner. The cTAP approach offers potential application to any challenging disease state characterized by heterogeneous progression. To learn more about cTAP’s capabilities, visit http://ctap-duchenne.org.

About Analysis Group’s Health Care Practice

Founded in 1981, Analysis Group is one of the largest international economics consulting firms, with more than 1,000 professionals across offices in North America, Europe and Asia. Analysis Group’s health care experts apply analytical expertise to health economics and outcomes research, clinical research, market access and commercial strategy, and health care policy engagements, as well as drug safety-related engagements in epidemiology. Analysis Group’s internal experts, together with its network of affiliated experts from academia, industry, and government, provide our clients with exceptional breadth and depth of expertise and end-to-end consulting services globally. https://www.analysisgroup.com/practices/health-care/

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