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Press Release

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cTAP ANNOUNCES TWO RESEARCH PUBLICATIONS CATEGORIZING AND PREDICTING DISEASE PROGRESSION IN DUCHENNE MUSCULAR DYSTROPHY

Cambridge, Mass (Business Wire) – October 31, 2016 – The Collaborative Trajectory Analysis Project (cTAP), a public-private partnership to accelerate data science solutions to critical problems in drug development for Duchenne Muscular Dystrophy (DMD), today announced the publication of two research studies with important implications for the design of effective clinical trials.

"cTAP is one of the best examples of international academic collaboration that has advanced the clinical understanding of Duchenne Muscular Dystrophy."

All patients with Duchenne eventually lose the ability to walk, but the rate at which ambulatory function declines can vary greatly between patients. This variability can cloud interpretation of clinical trials making it difficult to discern whether or not a drug is effective. The published studies announced here explain approximately half of the variability in disease progression in Duchenne, more than double that explained previously with conventional analyses.

"Without this understanding of the natural clinical progression of the various genetic causes for DMD, it would be extremely difficult to design the clinical trials or choose the appropriate endpoints necessary to develop novel drugs to use for DMD," said Dr. Edward Kaye, President, CEO and Chief Medical Officer of Sarepta Therapeutics. "cTAP is one of the best examples of international academic collaboration that has advanced the clinical understanding of Duchenne Muscular Dystrophy."

cTAP has connected leading clinical experts in Duchenne, drug developers and analytical experts with the shared goal of improving clinical trials in Duchenne by learning from patient data. The two published studies used statistical methods to quantify and predict disease progression in Duchenne, drawing from a growing database of more than 1,000 boys with Duchenne that, in total, includes functional assessments at more than 10,000 clinic visits.

Professor Eugenio Mercuri, a world-leading expert in Duchenne at the Pediatric Neurology at the Università Cattolica del Sacro Cuore, Rome, Italy, pioneered the collaborative access to registry data in Duchenne that made these studies possible. "As a first step, we wanted to quantify the different rates of disease progression in different patients," said Mercuri. Published in the September issue of *Neuromuscular Disorders*, the study by Mercuri and his colleagues identified statistically distinct groups of patients who had similar trajectories of ambulatory function over time; classifying patients into these groups explained more than half of the variability in trajectories of disease progression.

Building on these findings, a second study led by Duchenne expert Professor Nathalie Goemans, head of the Neuromuscular Reference Center for Children at the University Hospitals in Leuven, Belgium, was published in the October 18 issue of *PLoS One*. Goemans and her colleagues developed a prediction model for one-year changes in ambulatory function using a composite of patient characteristics and functional measures. The prediction model explained more than twice as much variability in ambulatory outcomes compared to patient measures that have been used to define eligibility for Duchenne clinical trials.

Both studies were conducted through cTAP and in scientific partnership with Dr. James Signorovitch, Vice President and outcomes research expert, and a team of researchers and data scientists at Analysis Group, Inc.

Funding

The collection and curation of patient data in Italy was supported by grants from Fondazione Telethon and Italian Duchenne Parent Project Onlus, and in Belgium with support from Fonds Spierzieke Kinderen. Funds to conduct the biostatistical analyses at Analysis Group Inc. were contributed by biopharmaceutical company sponsors - Pfizer, Shire, Biomarin, Sarepta, PTC Therapeutics, Solid Biosciences, Catabasis and BMS. Patient advocates CureDuchenne and Parent Project Muscular Dystrophy provided support for Dr. Susan J. Ward, Executive Director of cTAP, to manage the collaboration.

Works Cited

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About Duchenne Muscular Dystrophy

Duchenne is a uniformly fatal, progressive muscle-wasting disease affecting one in approximately 3,500-6,000 male births. Patients lack the ability to make dystrophin, a protein crucial to muscle function. As muscles deteriorate, patients progressively lose the ability to walk, to feed themselves, to turn over in bed, and ultimately to breathe. While there is no cure, the past decade has seen an explosion in research resulting in 15 or more therapies entering clinical trial development. The trials have proven challenging to design, giving rise to important initiatives that may enhance trial design and interpretation.
http://www.cureduchenne.org; http://www.parentprojectmg.org

About cTAP

cTAP is enabling leading clinical experts to solve the most critical problems in Duchenne drug development, NOW. A public-private partnership, the first of its kind in Duchenne, cTAP has forged a dynamic alliance between clinical experts, drug companies developing therapies, patient advocacy organizations and collaborating registries and clinical centers in Duchenne Muscular Dystrophy across Europe and the US, with data scientists expert in outcomes research. Curating the largest and still growing natural history clinical database in the Duchenne community, cTAP develops solutions through the application of advanced data science at the requisite scale and urgency necessary to impact clinical trial design and analysis, near-term.
http://ctap-duchenne.org

About Analysis Group

With more than 700 professionals, many with advanced degrees and expertise in health outcomes research, epidemiology, strategy, biostatistics, economics, and other quantitative disciplines, Analysis Group has established a leadership role in the science, economics, and business strategy of the global health care industry. The firm's 11 offices are located nationally in Boston, Chicago, Dallas, Denver, Los Angeles, Menlo Park, New York, San Francisco, and Washington, D.C.; and internationally in Montreal and Beijing.
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