COLLABORATIVE TRAJECTORY ANALYSIS PROJECT

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Press Release

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Eli Lilly and Company joins cTAP, helping to accelerate enriched clinical trial design for the provision of new treatments for Duchenne Muscular Dystrophy patients

Lilly to share data from Phase III trial of tadalafil in Duchenne, increasing knowledge of placebo-arm responses

CAMBRIDGE, Mass., December 21 (Business Wire) – Eli Lilly and Company, one of the top 15 global pharmaceutical companies, has joined the Collaborative Trajectory Analysis Project (cTAP), a unique partnership of many stakeholders all committed to accelerating scientific discovery and bringing new treatments to Duchenne Muscular Dystrophy (DMD) patients more rapidly.

"Lilly is proud to support the efforts of cTAP, which is making a real difference in our understanding of the progression of DMD, and how variations across patients make it particularly difficult to design effective clinical trials," said Kraig Kinchen, M.D., senior medical director of Lilly's Bio-medicines Core Team. "cTAP is a model that brings together the collective talent of a multi-stakeholder community. We all share a common interest in speeding the development of potentially effective therapies for Duchenne patients and their families."

Debra Miller, founder and CEO of CureDuchenne, a leading advocacy group in the fight against Duchenne, said "Advancing our scientific knowledge of Duchenne and the way in which its progression varies across patients is cTAP's mission. CureDuchenne was the founding advocacy group supporting cTAP and we are thrilled by Lilly's contribution."

"By sharing trial data, Lilly is showing real leadership," added Pat Furlong, founding president and CEO of Parent Project Muscular Dystrophy. "PPMD has a long history with

Lilly and their tadalafil trial, and we believe the data collected from our community will be valuable to the mission of cTAP."

Duchenne Muscular Dystrophy is a progressive, fatal disease of boys characterized by gradual weakening of muscles. It is the most common fatal genetic disorder diagnosed in childhood. Most Duchenne patients die in their 20s.

Though a Phase III human clinical trial of tadalafil, a phosphodiesterase inhibitor, in Duchenne ultimately failed to meet its targets for proving efficacy in slowing the decline in patients' ability to walk, the data generated by the trial are essential to expanding scientific understanding of the rate of decline in boys with Duchenne.

cTAP has characterized the variable rates at which Duchenne patients that exhibit similar symptoms often progress. This variation makes it difficult to design clinical drug trials that can definitively prove the efficacy of new treatments. Because Duchenne is a rare disease, with only about 20,000 new cases worldwide each year, it is virtually impossible to conduct large-scale trials with many patients, an approach that can be used to overcome variability in more prevalent disorders.

"Through our collaboration with cTAP we have been able to create new models that eliminate much of the statistical variation we see in patients in clinical trials of experimental Duchenne treatments," said Professor Nathalie Goemans, head of the Neuromuscular Reference Center for Children at the University Hospitals in Leuven, Belgium. "With these new data from Lilly, we will be able to assess how well our predictive tools – developed using natural history data - perform in the placebo arm of a clinical trial for a Duchenne treatment. That's why Lilly's participation is so critically important."

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About cTAP

The Collaborative Trajectory Analysis Project, or cTAP, is a novel group enabling leading clinical experts to solve the most urgent problems in Duchenne drug development. cTAP is a dynamic alliance of scientists, drug companies, patient advocacy organizations, and registries and clinical centers in DMD across Europe and the U.S. The collaboration also brings leaders in biostatistical outcomes research to the fight against Duchenne. cTAP is curating and growing what is already the largest natural history database of DMD. This enables it to develop near-term solutions for some of the key problems in designing clinical trials and analyzing their results. To learn more, please visit ctap-duchenne.org.

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 lost 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 therapies entering clinical development, with some receiving limited approval. Learn more about Duchenne at cureduchenne.org and parentprojectmd.org.