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Press Release

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Non-Profits, Pharmaceutical Companies and Noted Clinicians Collaborate to Launch the Collaborative Trajectory Analysis Project (cTAP); Advancing Clinical Trial Design for Duchenne Muscular Dystrophy

Consortium Working Together to Bring Quality of Treatments to the Duchenne Community

NEWPORT BEACH, Calif., October 25, 2016 – The next wave in tackling Duchenne Muscular Dystrophy is getting a boost from a collaboration of non-profits, biopharmaceutical companies and noted clinicians who have joined together to advance the progress of drug development and bring quality of life treatments to the Duchenne community sooner.

Initially funded by CureDuchenne, the Collaborative Trajectory Analysis Project (cTAP) is a consortium that is unifying leaders in Duchenne muscular dystrophy research with leaders in health outcomes research, and teaming up with drug developers and non-profit organizations to unleash the power of collaborative data science on clinical trial design, potentially helping the entire community to bring effective new therapies to patients sooner.

“CureDuchenne was motivated to help create cTAP by the tremendous benefit to the trial and regulatory process that only a collaboration of this type could bring,” says Debra Miller, CEO and founder of CureDuchenne. “Key stakeholders are grateful to be involved and will be able to access this data for the potential benefit of the much broader community. As a mother of a son with Duchenne, I know this unprecedented level of cooperation will bring treatments to our sons sooner.”

“Clinical trials in Duchenne are challenging to design because the disease progresses at different rates in different patients,” said Professor Eugenio Mercuri, Neurology and Pediatrics, Università Cattolica del Sacro Cuore, Rome, Italy, a world-renowned expert in Duchenne and the founding academic collaborator in cTAP. “New tools to identify trajectories of progression can help to inform trial design in the community.”

The Collaboration members include:

- Professor Eugenio Mercuri, Neurology and Pediatrics, Università Cattolica del Sacro Cuore, Rome, Italy;
- Professor Francesco Muntoni, Director of The Dubowitz Neuromuscular Centre, University College London;
- Professor Nathalie Goemans, pediatric neurology clinic, University Hospitals in Leuven, Belgium;
- Dr. Brenda Wong and the Cincinnati Children’s Hospital Medical Center;
- Clinical Registries: The Italian DMD group supported with grants from Fondazione Telethon, and the Northstar Project UK supported by Muscular Dystrophy UK;

- Drug developers Pfizer Inc, BioMarin, PTC Therapeutics, Sarepta Therapeutics, Shire, Solid Biosciences, Catabasis Pharmaceuticals, and BMS;
- Dr. James Signorovitch, Analysis Group Inc;
- Dr. Susan J. Ward; The TAP Collaboration; and
- CureDuchenne and Parent Project Muscular Dystrophy (PPMD) support the Collaboration by providing insights from the Duchenne community, the members of which are most directly impacted by the need for more efficient and effective clinical trials.

Longitudinal data from multiple disease patient registries are analyzed by the Analysis Group Inc. under the direction of Dr. James Signorovitch, a leader in healthcare outcomes research. Databases include information on more than 1,300 patients, 15+ functional tests, and 10,000+ clinic visits.

The Collaboration is managed by Dr. Susan J. Ward, co-Founder of cTAP and previously a senior executive with Millennium Pharmaceutical, Wyeth, and Sterling-Winthrop.

For more information on cTAP, go to www.ctap-duchenne.org.

About CureDuchenne

CureDuchenne was founded in 2003 with a focus on saving the lives of those with Duchenne muscular dystrophy, a disease that affects more than 300,000 boys worldwide. With support from CureDuchenne, nine research projects have advanced to human clinical trials. CureDuchenne also brings physical therapy, and standard of care to local communities around the country through its [CureDuchenne Cares](#) program. For more information, please visit CureDuchenne.org and follow us on [Facebook](#), [Twitter](#) and [YouTube](#).

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