

ABSTRACT for WMS 2017

The collaborative Trajectory Analysis Collaboration (cTAP) – accelerating the Translation of Natural History into more effective Clinical Trial Design

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Although the Duchenne community has collected a wealth of natural History data, translating those data in clinical trial design has proven challenging. Clinical trials have suffered from higher than anticipated variance, masking potential drug effects. The predominant driver of this variance is phenotypic heterogeneity in disease progression.

Founded in response to this challenge, cTAP is a dynamic and collaborative alliance forged between clinical experts, patient advocates, and a rapidly growing group of drug developers in Duchenne, who together access sophisticated analytics and data science to address the most important challenges to trial design, analysis and interpretation.

In just 2 years, cTAP collaborators have published methods to halve the unexplained variance impacting clinical trials, and developed a prognostic model for 6MWD that raises prognostic accuracy from ~ 20-25% to more than 60%. Drawing upon the largest aggregated clinical database of natural history and placebo data in Duchenne (1300+ patients, 10,000+ patient visits), cTAP has developed collaborative tools to compare outcomes under any trial conditions, in real time – particularly valuable to address regulator concerns on consistency across data-sources.

The cTAP model is predicated on a combination of stakeholder leadership and an enabling structure. Mercuri and the Italian Telethon group pioneered sharing of de-identified patient data outside of their network – a first in Duchenne. Drug developer funding for shared analytics is a first. The cTAP structure preserves a clinical network's ability to govern use of the data they collect and curate, and at the same time ensures the benefits are shared amongst drug developers, easily and equitably. cTAP makes no profit, and members share costs equally.

The cTAP experience reinforces the 'greater than the sum of its parts' value of collaboration between stakeholder groups.

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