

## **The collaborative Trajectory Analysis Project (cTAP) – pioneering collaborative analytics for more effective clinical trials in Duchenne Muscular Dystrophy**

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**Background:** Longitudinal heterogeneity in disease progression in patients with DMD is primary driver of high variance in clinical trials, confounding interpretation of results, and resulting in a high rate of statistical failures. Although the DMD community has been prescient in curating high quality natural history clinical data, learning from patient clinical data has been constrained by significant barriers to sharing of de-identified patient data between researchers.

**Objectives:** 1) break the cycle of iterative failures in clinical trial design and analysis in DMD, 2) secure comprehensive access to clinical patient data, 3) develop insights and analytic tools to design more effective clinical trials, and 4) share findings with the entire community.

**Approach:** The foundational underpinning of cTAP's value is to accelerate learning from collective longitudinal data on thousands of patients.

**Results:** cTAP is the first initiative of its kind in DMD and is also the first between Europe and the US. cTAP has established collaborations to access patient level clinical data from large clinical centres treating DMD patients, clinical networks that curate data across multiple centres, and drug companies. It is the largest, and still growing, multi-national inventory of patient clinical data in DMD.

Since inception, cTAP has enabled therapy developers from 15 pharmaceutical and biotech companies to

- design trials that halve variance and reduce trial size
- pressure-test trial designs against real world outcomes before starting a study
- create matched natural history cohorts to augment placebo controls
- develop disease-based evidence to support pricing and access

In addition, cTAP has engaged with regulators to 'prime the pump' on translation of findings to trial design and interpretation.

**Conclusions:** cTAP models an effective means of promoting collaboration to advance drug development for DMD patients.