Poster # P.29

Accelerating clinical development of new therapeutics with patient data: evidence from the collaborative Trajectory Analysis Program (cTAP) in DMD

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Background

- Patients DMD and their families generously consent to research on their clinical data in the hope of accelerating potential new treatments
- Although it is widely believed that curated patient data are invaluable to drug development, the published literature is sparse on *how* patient data are used

Study Objectives

- Characterize how research on anonymized patient data aggregated by cTAP is used to accelerate clinical development of new therapeutics
- Provide clinical experts and developers with a framework for sharing the importance of curated patient data in drug development

Methods

Figure 1. Anonymized patient data

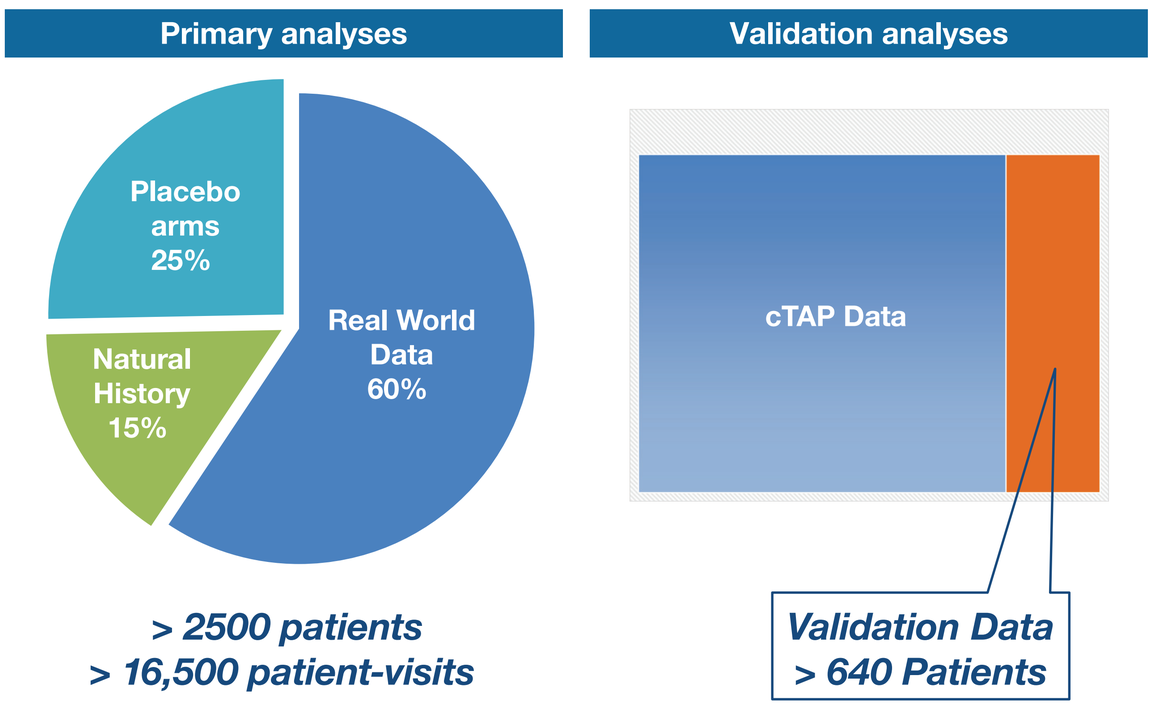
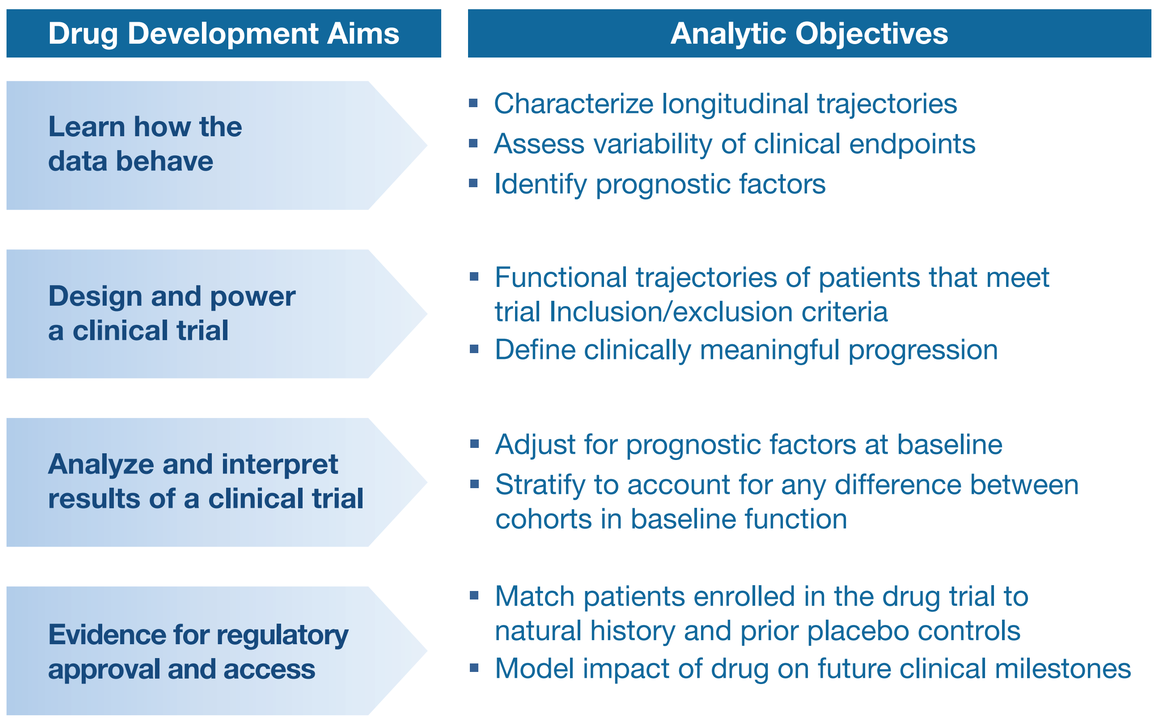


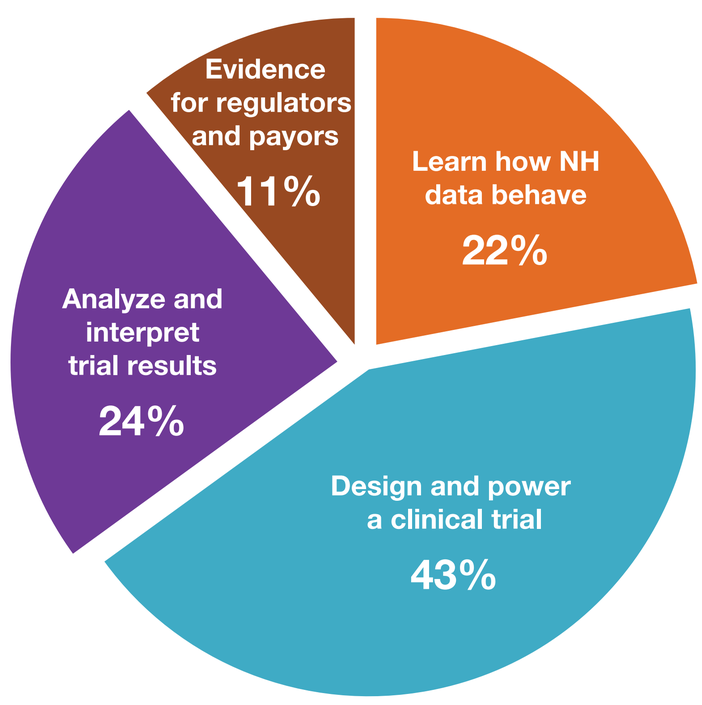
Figure 2. Categorizing the purposes of analyses of patient data



Results

- We analyzed the 78 discrete analytical studies conducted 2015 – 2021
- Approximately 65% of studies were conducted within the cTAP pre-competitive collaboration and approximately 35% applied these collaborative findings to company-specific clinical trials
- To date the majority of studies (43%) have focused on helping drug companies to optimize design and power of clinical trials (**Figure 3**)

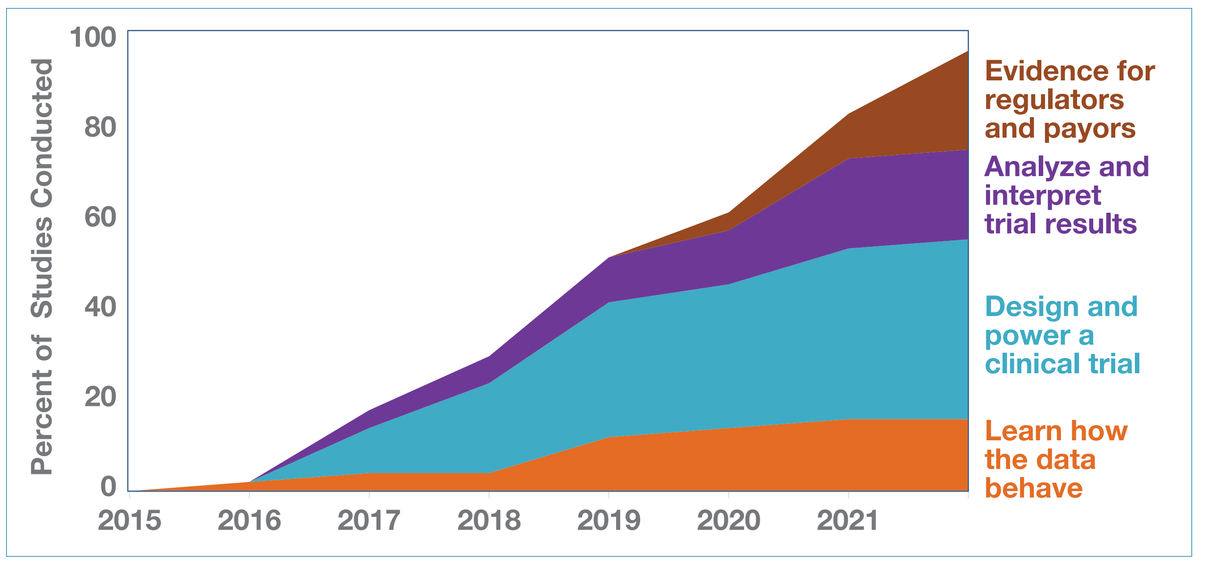
Figure 3: purpose of analyses of patient data 2015-2021



The purpose of cTAP analytical studies on patient data has evolved and expanded over time (Figure 4).

- The purpose of cTAP analytical on patient data has evolved and expanded over time (**Figure 4**).
- Initial studies focused on providing a richer characterization of natural history progression. Such studies continue to be important but have essentially plateaued since 2019 at ~ 20%
- Studies focused on clinical trial design, analysis, and interpretation have grown rapidly year on year since 2016
- In 2019, the first studies to support evidence-based drug effect for regulatory approval and patient access were conducted, and is growing rapidly
- Expansion of data use and evolution of scope parallels the growth in cTAP membership

Figure 4. Drug development focus of analytical studies over time



Limitations

- This study analysed the use of anonymized patient data by cTAP and collaborators to accelerate clinical development
- The study did not include studies directed at accelerating clinical development of new therapeutics that *may* have been conducted by other research groups

Conclusions

- Analysis of natural history and real-world clinical patient data can inform a wide range of important decisions drug developers need to make
- This study, which categorized patient data analyses that help accelerate drug development in DMD, may serve as a template to encourage publication and discussion other groups' work supporting clinical development in rare disease

Disclosures

This study was conducted within the collaborative Trajectory Analysis Project (cTAP), a precompetitive coalition of academic clinicians, drug developers, and patient foundations formed in 2015 to overcome the challenges of high variation in clinical trials in DMD. cTAP has received sponsorship from Astellas (Mitobridge), Avidity Biosciences, BioMarin Pharmaceutical, Bristol Meyers Squibb, Catabasis, Daiichi Sankyo, Edgewise Therapeutics, Entrada Therapeutics, FibroGen, Italfarmaco SpA, Marathon Pharmaceuticals, NS Pharma, Pfizer, PTC Therapeutics, Roche, Sarepta Therapeutics, Shire, Solid Biosciences, Summit Therapeutics, Ultragenyx, Vertex Pharmaceuticals, Parent Project Muscular Dystrophy, Charley's Fund, and CureDuchenne, a founding patient advocacy partner and provider of initial seed funding to cTAP.

Presented at the 27th International Hybrid Annual Congress of the World Muscle Society, October 11–15, 2022, Halifax, Nova Scotia, Canada