

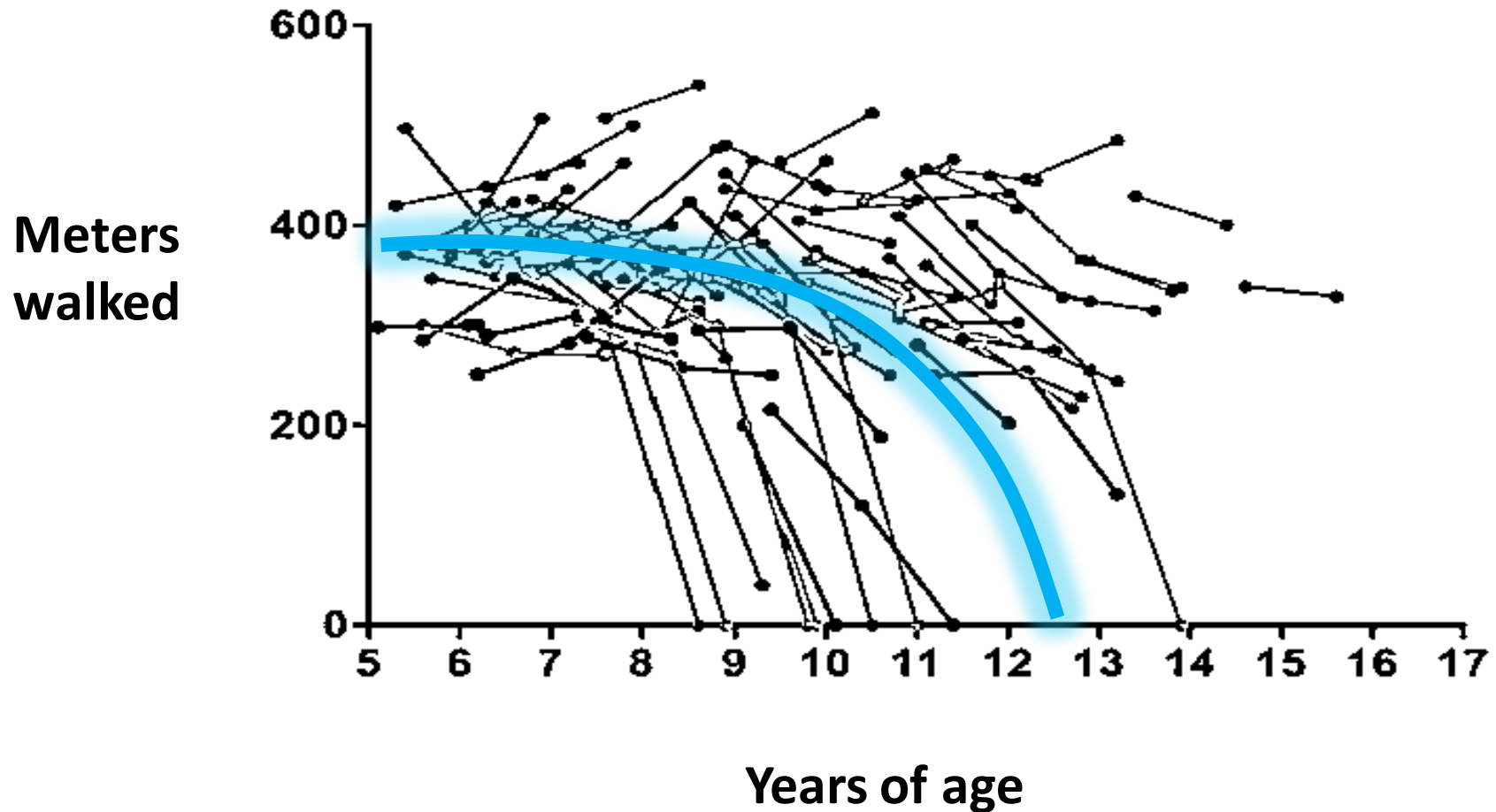
The Collaborative Trajectory Analysis Project (cTAP)

*Explaining variation in Duchenne disease progression
to accelerate drug development*

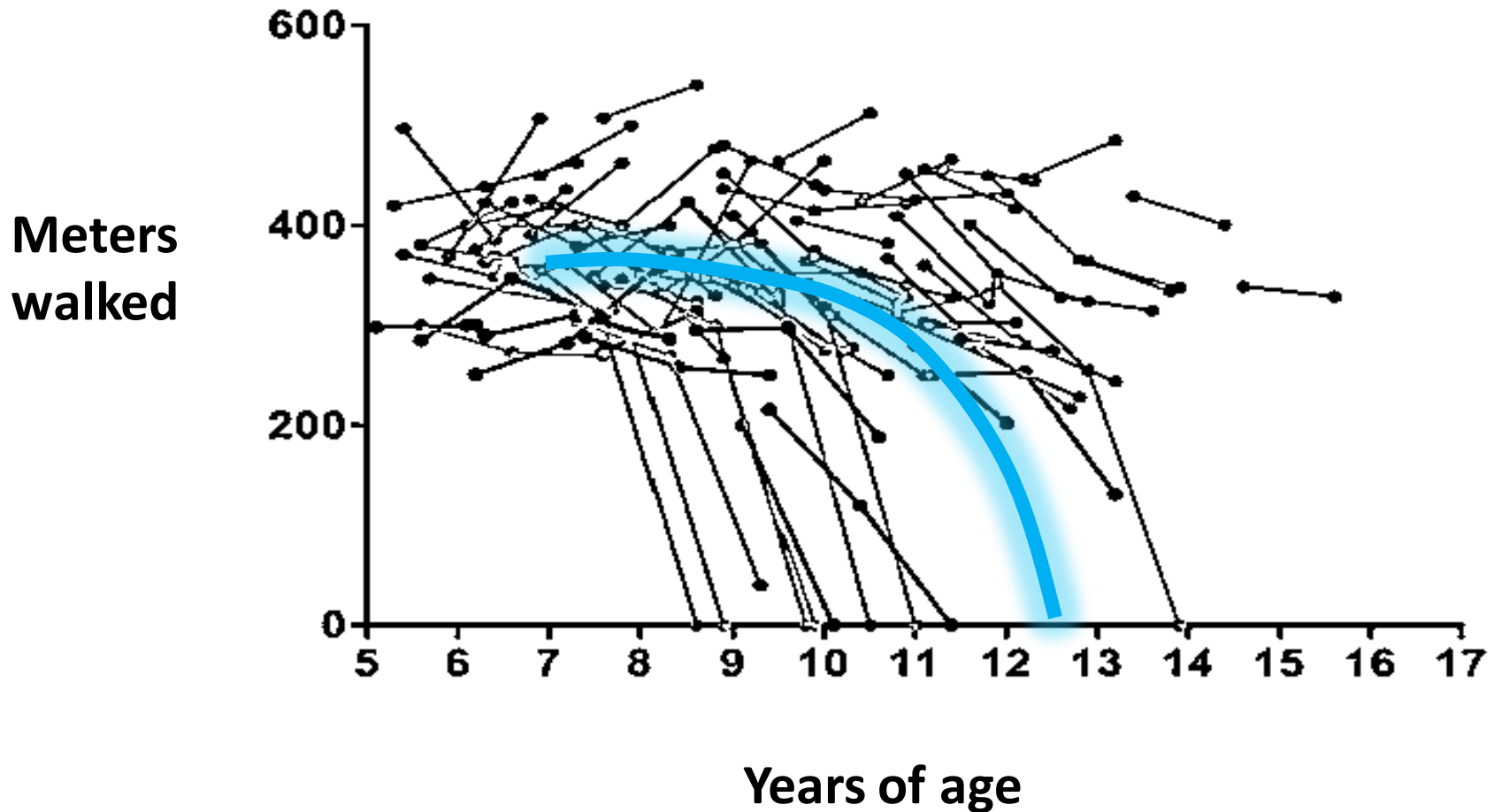
Individual trajectories of six-minute walk distance



Individual trajectories of six-minute walk distance



Individual trajectories of six-minute walk distance



Two kinds of phenotypic variation – and why we care

Unexplained variation

(we *don't* know what causes it)

Explained variation

(we *do* know what causes it)



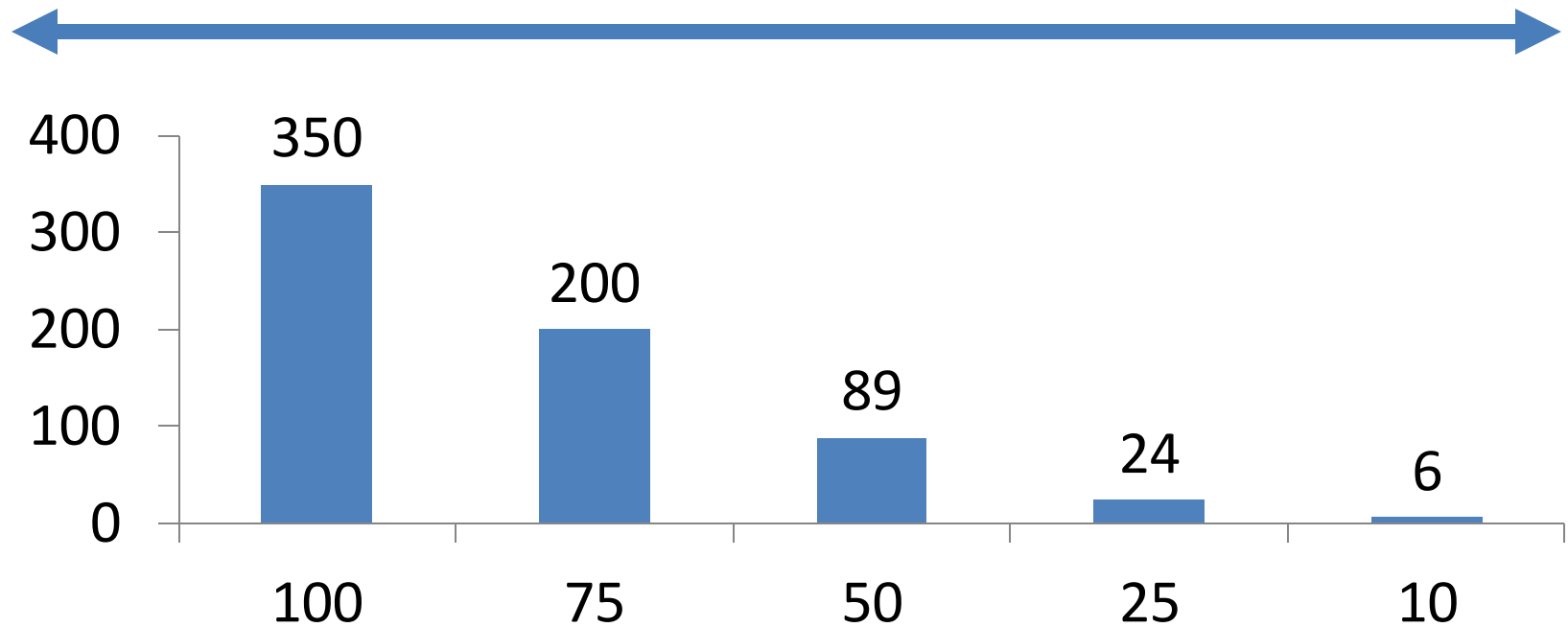
- Larger, longer trials
- Greater risk of not proving efficacy
- Disconnect between individual experiences and research on average outcomes

- Smaller, shorter trials
- Greater confidence in trial design
- Pathway for continually improving individualized care

“Ideal” number of boys in a hypothetical efficacy trial

Unexplained variation
(we *don't* know what causes it)

Explained variation
(we *do* know what causes it)



Unexplained variation in six minute walk distance (meters)

How can we explain more variation?

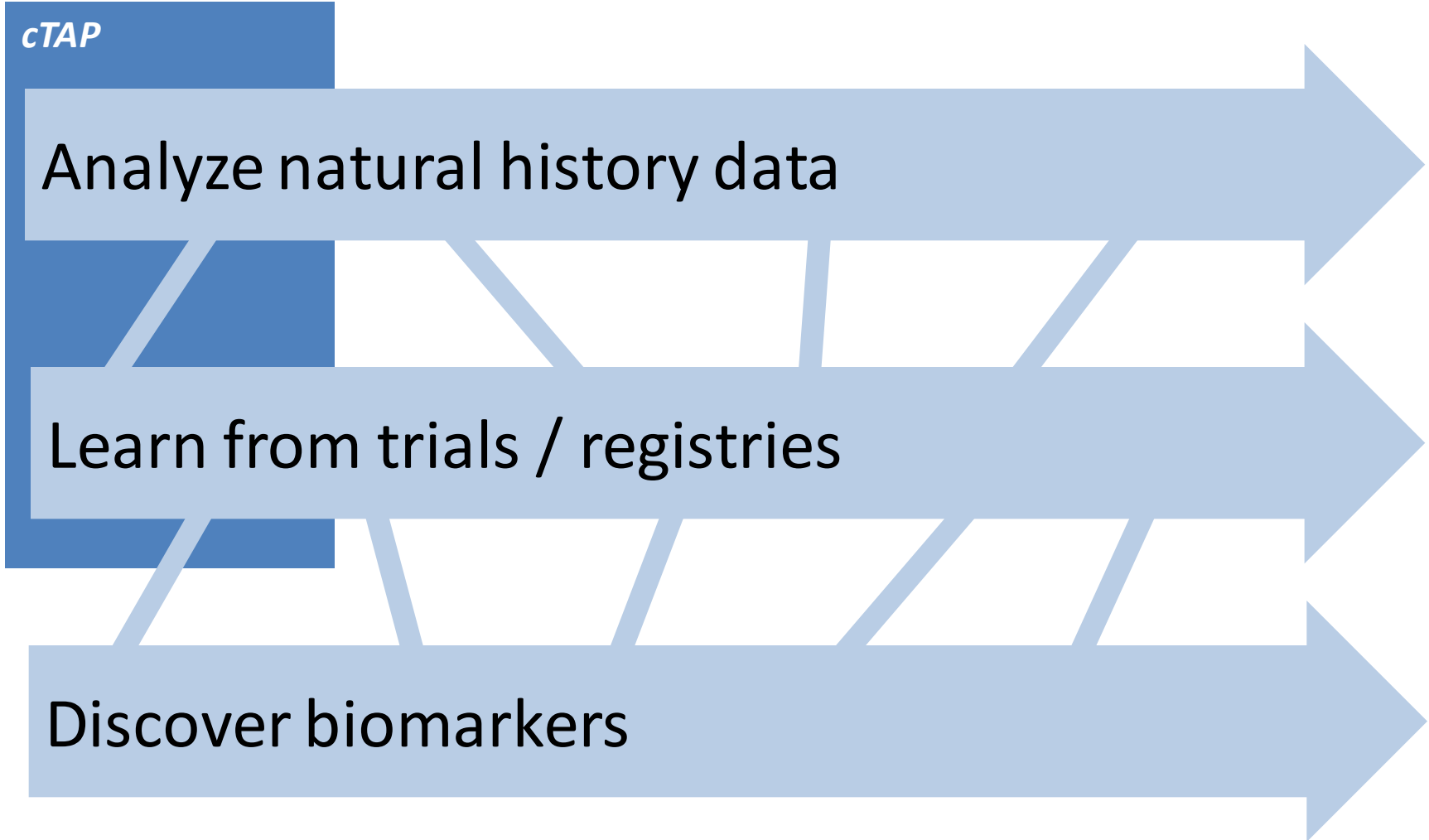


Analyze natural history data

Learn from trials / registries

Discover biomarkers

How can we explain more variation?



The Collaborative Trajectory Analysis Project (cTAP)

Mission

- Explain phenotypic variation in DMD
- Translate into tools for trial design/analysis
- Make the tools available to everyone in drug development
- Deliver near-term impact

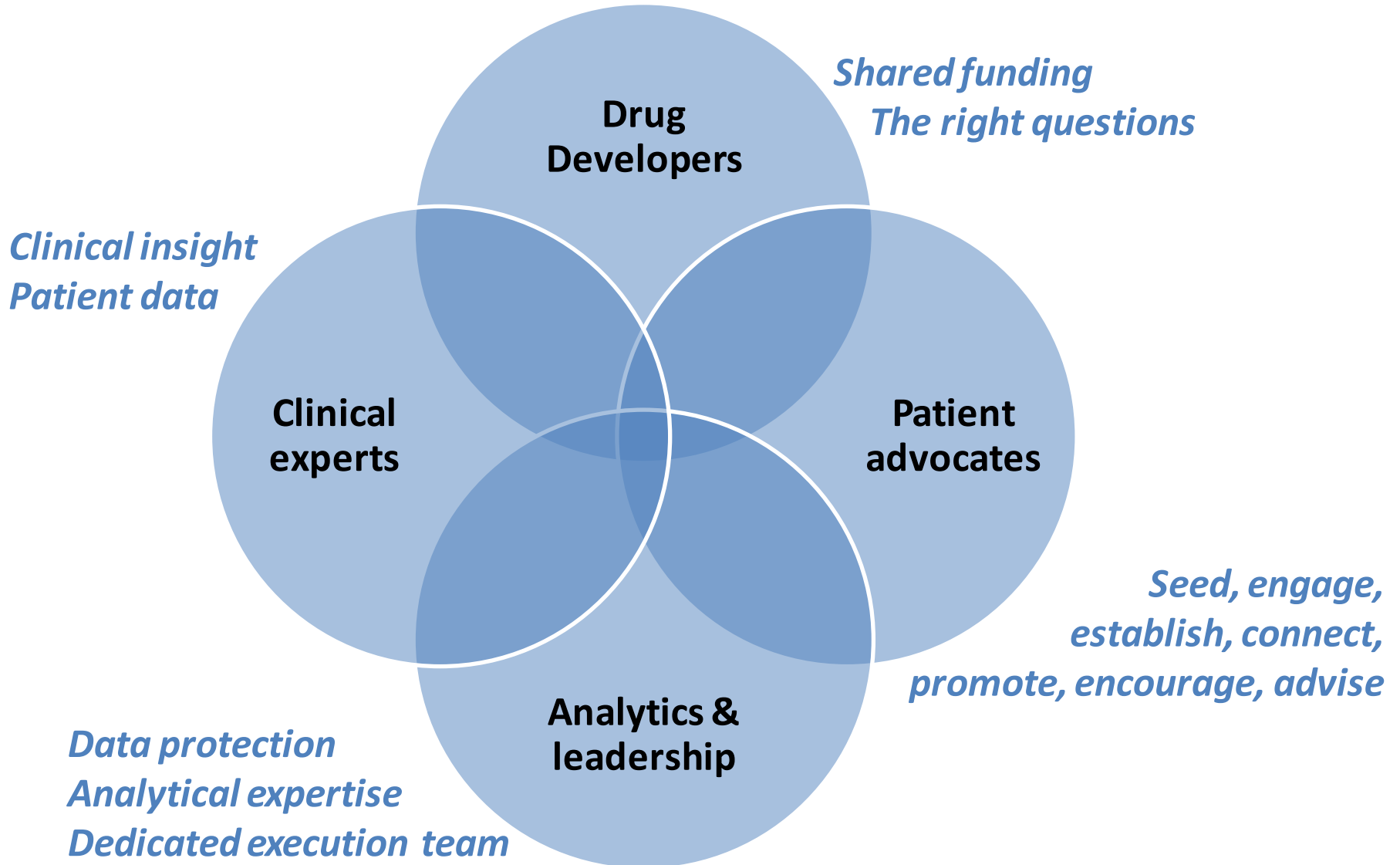
The benefit: accelerate effective treatments to patients

cTAP at year 1

who we are and what we've done

Clinical experts and registries	<ul style="list-style-type: none">• Mercuri; Telethon - 96 boys• Goemans; UZ Leuven - 65 boys• Muntoni; Northstar UK - > 500 boys... Wong (Cincinnati)... Vandeborne (Imaging DMD)
Drug developers	<ul style="list-style-type: none">• BioMarin (Prosensa)• Pfizer• PTC Therapeutics• Sarepta• Shire... Others in process
Patient advocates	<ul style="list-style-type: none">• Cure Duchenne• PPMD• Parents
Analytics, leadership	<ul style="list-style-type: none">• Analysis Group• Susan Ward

Collaboration: central to cTAP success



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The benefit: accelerate effective treatments to patients