BEAT CF: OVERVIEW OF AN ADAPTIVE CLINICAL TRIAL IN RESPIRATORY MEDICINE

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BACKGROUND

- Cystic Fibrosis (CF), due to endocrine system malfunction, affects one in 2,500 babies born in Australia.
- Treatment for CF involves antibiotics, daily physiotherapy, and airway clearance therapy (CF Federation of Australia).
- Adaptive clinical trials use pre-defined decision rules to determine trial features such as allocation of patients to best performing treatment arms (response-adaptive) and early trial termination, ensuring that individual patients are more likely to receive the optimal treatment.

OBJECTIVE

Aim 1: To design a clinical trial that accounts for the heterogeneity in patient response

Aim 2: To elucidate the best combination of therapies for a unique patient based on accumulating data

METHODS

- Adaptive trials allow design parameters to change based on pre-defined rules and accumulating study data.
- Platform trials aim to find the best treatment for CF by randomly allocating patients to treatment combinations.
- Study design includes response-adaptive randomization and sample size recalculation based on different treatment response across patient subgroups.
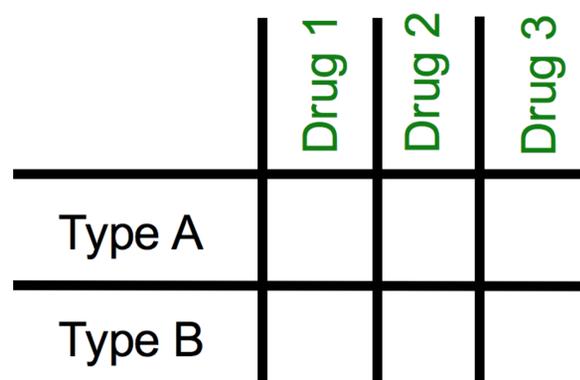

Figure 1: Platform Trial Design

REFLECTION

- Designed and developed an electronic data capture and randomization tool for clinicians and patients in the trial.

Figure 2: Adapted Clinician App (Treatment Randomization)

- App features data input fields via an accessible user-interface (UI) and real-time data visualization for smart devices.
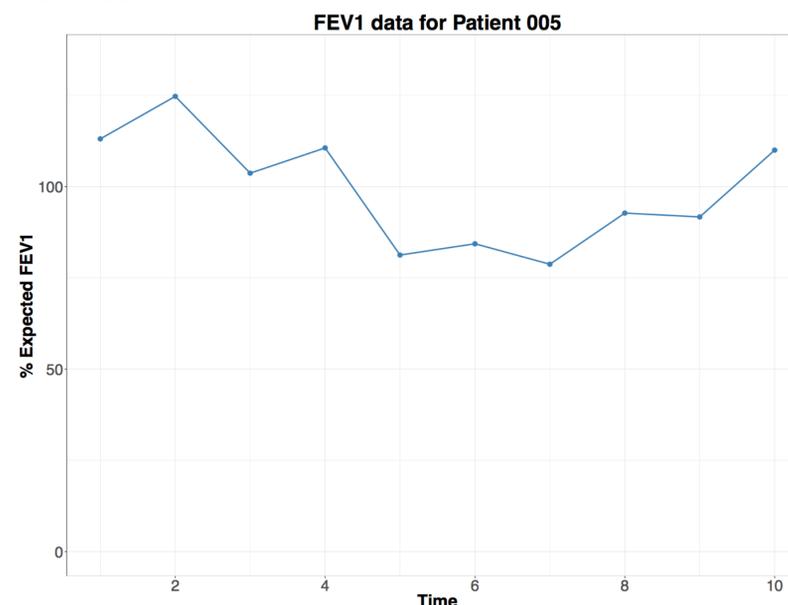

Figure 3: Forced Expiratory Volume in one second (FEV1) data

- Learned about different types of clinical trials and the importance of thoughtful study design to reduce biases.

LOOKING AHEAD

- Important to consider the heterogeneity of patient response (age, genetic biomarkers, etc.) when conducting research and prescribing treatments
- Considering MD/PhD career path

CONCLUSIONS

- Statistical simulation is important for these complex adaptive designs. However, electronic solutions are essential for real-time response adaptation.
- Response-adaptation minimizes the number of patients exposed to less effective treatments.
- Adaptive clinical trials change the focus from assessing if treatment A works to ensuring better overall treatment of patients.

FUTURE RESEARCH

- Clinical trials are expensive and resource intensive, and electronic data capture has the potential to offset these barriers to clinical research.
- More funding should be allocated to patient-centered medical research
- Electronic tools that capture patient's experience would allow individuals to input and track their own progression

Figure 4: Adapted Patient App (Self-Assessment)

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