

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.

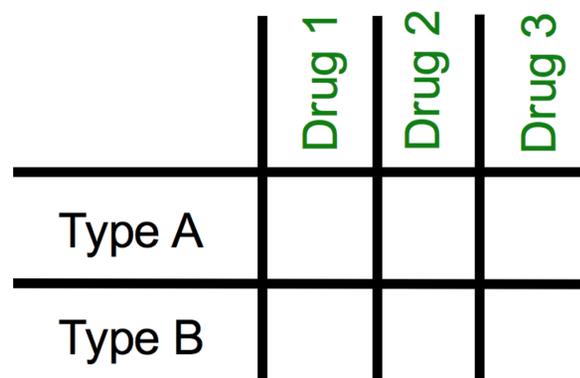


Figure 1: Platform Trial Design

REFLECTION

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

What is your preferred mucoactive therapy?

What is your preferred anti-inflammatory?

Backbone antibiotic: Adjunct antibiotic: Airway clearance therapy:

Mucoactive therapy: Anti-inflammatory:

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.

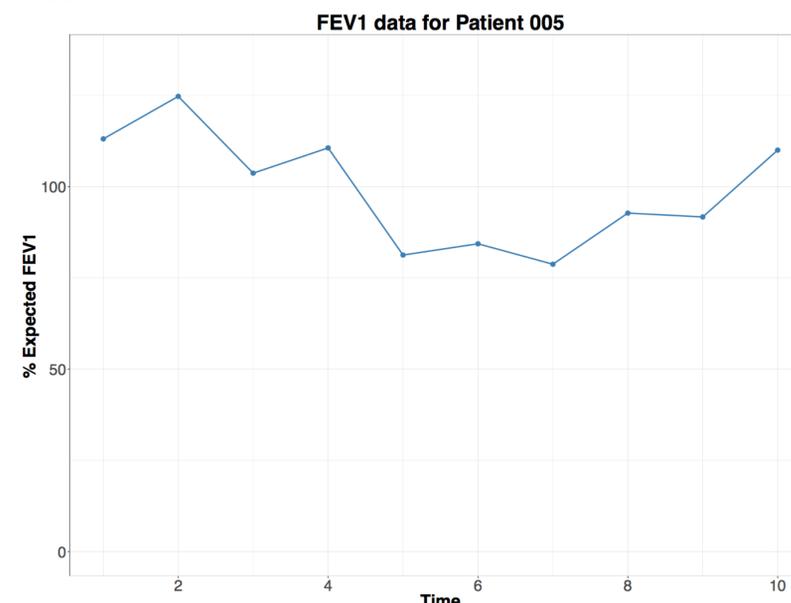


Figure 3: Forced Expiratory Volume in one second (FEV1) data

- 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

How well do you feel today? How would you rate your stress level?

Worst Ever Best Ever Worst Ever Best Ever

How much energy do you have today? How is your appetite?

All Time Low All Time High Worst Ever Best Ever

Figure 4: Adapted Patient App (Self-Assessment)

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