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.

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

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

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