



Bayesian Adaptive Semiparametric Endpoints (Primary and Secondary) Pediatric ChatGPT (BASE-PedChatGPT) – A Web-based App



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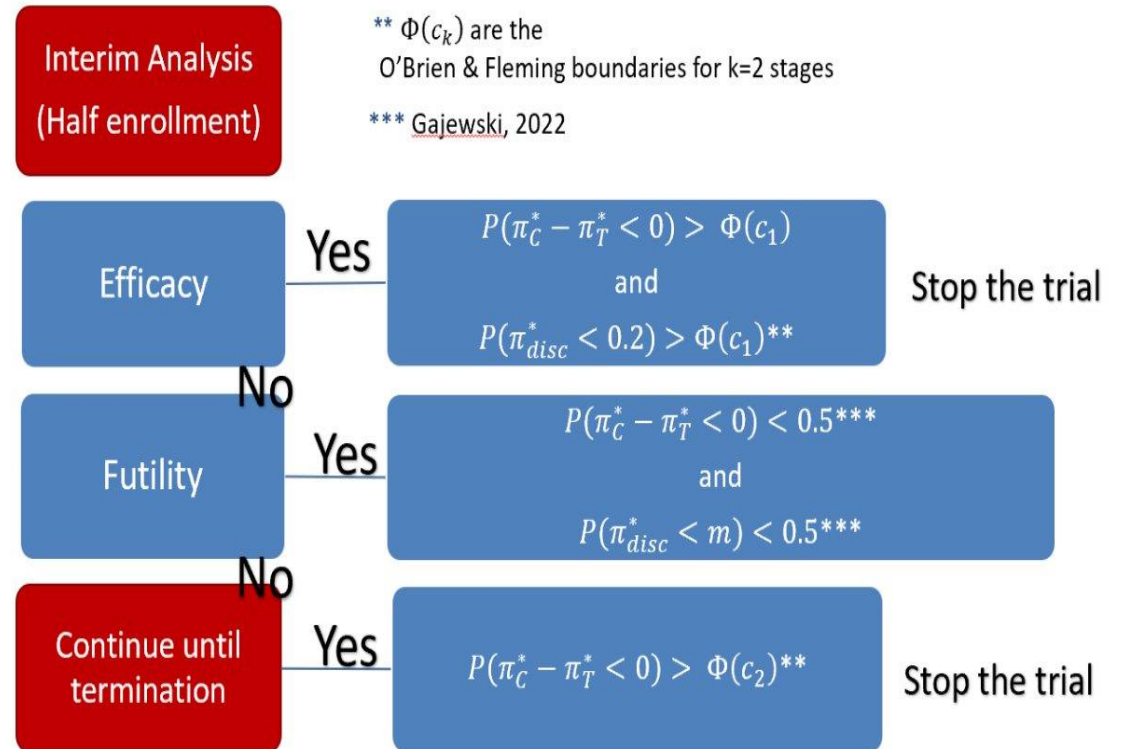
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Introduction to the Design

- ✓ Bayesian Adaptive Semiparametric Approach designed to address the challenges in pediatric randomized controlled trials (RCTs) conduction.
- ✓ This methodology is particularly pertinent in scenarios where sparse or conflicting prior data is present, a common occurrence in pediatric research, especially for rare diseases.
- ✓ To demystifies advanced statistical methodologies, making them accessible and understandable to clinicians and stakeholders in pediatric trials, we designed a web-based tool BASE-PedChatGP

Introduction to the BASE-PedChatGPT

- ✓ In order to address the communication barrier in complex trial designs, we introduce a web-based Shiny application interfaced with ChatGPT.
- ✓ BASE-PedChatGPT offers an intuitive platform for inputting trial parameters and instantly visualizing the implications of different design choices.
- ✓ Additionally, this web application offers a suggested paragraph to include in a study protocol. The paragraph, about the semiparametric approach for managing primary and secondary endpoints, is generated using the ChatGPT algorithm through its API interface.



Left Side Input Menu of BASE-PedChatGPT Web App

The left side of the screen features a web chat interface that allows users to input various parameters to evaluate the study design features

- ✓ Parametric or Semiparametric priors.
- ✓ Expert elicitation on event rate (treatment and control opinion)
- ✓ Design parameters such as Sample size per arm, Event rate in control arm, Absolute risk reduction (ARR), Secondary endpoint rate, Acceptability rate, Number of simulations and A parameter for prior informativeness

Priors

Parametric (Beta)
 Semiparametric (B-Spline)

Expert elicitation

Control Opinions

Number of Experts for Control: 5

Control Event rate for Expert 1: 0.23

Control Event rate for Expert 2: 0.25

Control Event rate for Expert 3: 0.35

Control Event rate for Expert 4: 0.26

Treatment Opinions

Number of Experts for Treatment: 5

Control Event rate for Expert 1: 0.46

Control Event rate for Expert 2: 0.38

Control Event rate for Expert 3: 0.31

Control Event rate for Expert 4: 0.33

Control Event rate for Expert 5: 0.42

Design parameters

Sample size per arm (n): 80

Event rate in control arm: 0.4

Absolute Risk Reduction (ARR): 0.18

Secondary endpoint rate: 0.18

Acceptability rate: 0.2

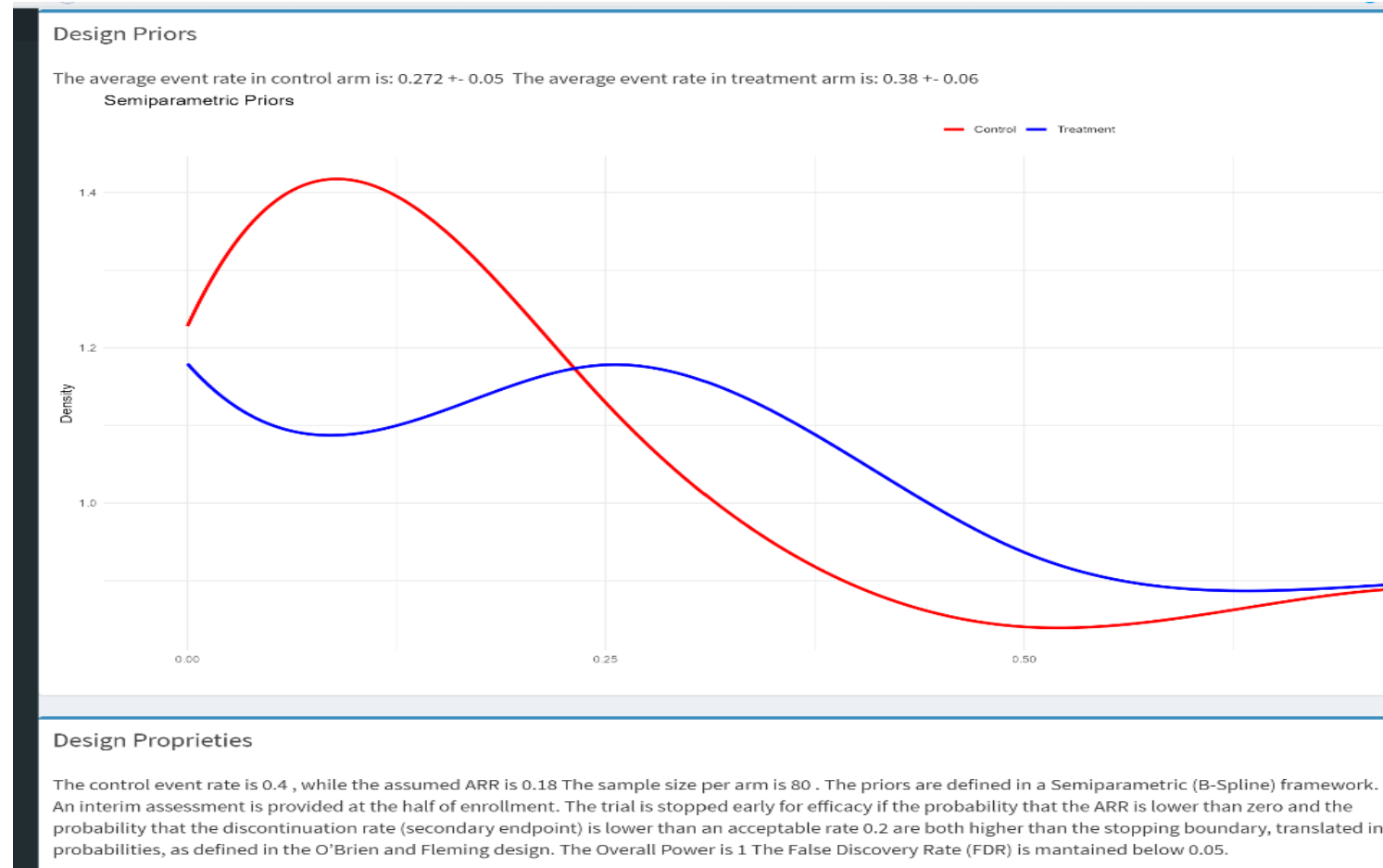
Number of Simulations: 10

Prior Informativeness parameter: 45

Calculate

Study Design Proprieties Output of BASE-PedChatGPT Web App

- ✓ After the user selects the input parameters, the web app displays the prior distribution densities for both the treatment and control arms.
- ✓ It also presents a summary of the study design characteristics, including empirical power and the FDR, as shown in Figure.



Study Design Paragraph Output of BASE-PedChatGPT Web App

- ✓ the user can specify further trial details on the left side of the screen, such as treatment names, endpoints, disease, and target population.
- ✓ This information is essential for generating a concise study design protocol paragraph using ChatGPT, as depicted in Figure

Write the protocol paragraph via ChatGPT:

Enter treatment name:
Dexametason+ Amoxicill

Enter control name:
Amoxicillin

Enter the disease name:
Urinary Tract febrile infer

Enter the target patients:
Pediatric

Enter the primary endpoint:
Difference in scar rate

Enter the secondary endpoint:
Discontinuation rate

Calculate

Sample size paragraph

The proposed protocol aims to investigate the effectiveness and safety of treatment with Dexamethasone and Amoxicillin compared to Amoxicillin alone in patients with Urinary Tract Febrile Infection, specifically in the pediatric population. The primary outcome of interest is the difference in scar rate between the treatment and control groups. A secondary outcome of interest is the discontinuation rate. The trial's design properties are defined by a Semiparametric (B-Spline) framework, with a sample size of 80 patients in each arm. The control event rate is 0.4, while the assumed Absolute Risk Reduction (ARR) is 0.18. An interim assessment will be conducted at the halfway point of patient enrollment. The trial will be stopped early for efficacy if the probability that the ARR is lower than zero and the probability that the discontinuation rate is lower than an acceptable rate of 0.2 are both higher than the stopping boundary, translated in probabilities, as defined in the O'Brien and Fleming design. The Overall Power of the study is 1, and the False Discovery Rate (FDR) is maintained below 0.05, providing robust results in the investigation of the treatment's effectiveness and safety.

Thank you for the attention!!

