Novel Adaptive Design in Schizophrenia: A Case Study in Constructing Point-of-Care Clinical Trial Design With Adaptive Population Enrolment

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INTRODUCTION

The introduction of clinical trial designs that consider variability in patient population and study settings have been identified as important ways to increase the generalizability of clinical trial findings. Indeed, the development of such a trial design would address many of the limitations identified with traditional design approaches. This manuscript presents the results of a simulated study designed to demonstrate the applicability of a point-of-care clinical trial (POC-CT) to schizophrenia research. This work draws on the recent discussions and recommendations by the ISCTM Adaptive Design Working Group to demonstrate the potential of such an approach to schizophrenia research.

METHODS

Study Design Parameters

- **Objective:** To evaluate the efficacy and safety of a novel antipsychotic treatment in patients with schizophrenia.
- **Study Population:** Inpatients or outpatients with unstable schizophrenia who are randomized to one of two treatment groups.
- **Study Endpoints:** Frequency of psychiatric hospitalization rates (HRs), cost, and burden of hospitalization.
- **Study Design:** Adaptive late futility/early success design.

Simulation Results

- **Simulation Format:** The simulations were performed in FACTS™.
- **Simulation Parameters:** The simulations provided examples of how designs behave and the metrics of performance.
- **Simulation Results:** The simulations allow for modifications of study designs to enhance their performance. The authors answer the question, “Do I design that trial–can we change it?”

CONCLUSIONS

- **Adaptive design:** The adaptive design allows for modifications of study designs to enhance their performance.
- **Futility Success:** The adaptive design allows for early termination due to either futility or success.
- **Early Stopping:** Allows for early termination due to either futility or success.
- **Probability of Success:** Set success criteria to ensure probability of success >0.5 for each case and as high as possible for all other cases.

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