Impact of Innovative trial designs on CNS Drug Development

Presenters: [1]Olugemo, K, [2]Gertsik, L, [1]Bussel, M, [1]Rudnyeva, O

Affiliations: [1]PAREXEL International, [2]California Clinical Trials Medical Group

Methodological Question Being Addressed: to quantify the impact of select innovative trials designs on CNS clinical trial efficiency, likelihood of drug launch and global patient access.

Introduction (Aims): Central nervous system (CNS) drugs take longer on average than drugs in other therapeutic areas and are half as likely to reach the market. As the costs of drug development increase and strain health systems, the need for innovative and more efficient drug development is imperative.

Methods: The Economist Intelligence Unit and a panel of experts from academia, healthcare policy, patient advocacy and the pharmaceutical industry convened to identify the most promising innovations in CNS trials, in order to stimulate discussion on rekindling productivity, improving efficiency, and restoring sustainability. An extensive literature review was conducted to identify key issues, possible innovations to study, and ways to measure success. Following a quantitative analysis, selected trial innovations included adaptive trial designs, patient-centric trials, precision medicine trials, and real-world data.

Retrospective analysis included 249 drugs from 372 phase II and III trials between 2012 and 2017 compared with a control group of 3999 trials from the same period that did not use the selected innovations. Metrics included impact of selected innovative trial designs on trial efficiency, drug launch and formulary addition in the United States, European Union and China.

Results: The likelihood of launch was increased in CNS trials across all the selected innovations compared to trials without innovation. Innovative CNS trials had an average of 13% less time spent on enrollment, and recruitment times were reduced by 50% in patient-centric trials. Reducing the proportion of time taken by enrollment means drug developers can choose to spend more time on treatment without necessarily lengthening total trial time. A positive trend for earlier listing of drugs using innovative methods in key formularies and national access lists was observed.

Adoption rates for innovation were surprisingly low. Conversations with experts across the industry reveal collective barriers to adoption, including a vast amount of fragmented data, inadequately trained workforce, and cultural barriers surrounding drug development and innovation.

Conclusions: The findings quantify the impact of the most promising innovations on CNS trial efficiency, success in launch, and obtaining formulary approval worldwide. This data is of key significance to multi-stakeholders and can inform early decision making during protocol development. Barriers to adoption of innovation and future enablers should be further explored.

Disclosures: Economist Intelligence Unit Report commissioned by PAREXEL. All authors, except Dr. Gertsik, are employees of PAREXEL International. Dr. Gertsik is an employee of California Clinical Trials Medical Group.