Novel trial designs in neuro-oncology

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Abstract

Purpose of review: An important factor contributing to the low rate of success in identifying effective therapies for brain tumor patients is the slow, inefficient, and expensive process of drug development, as well as small patient numbers, low patient participation in clinical trials, and reluctance of patients to enroll in ineffective control arms. In recent years, a number of novel trial designs have been developed to try to address some of these issues.

Recent findings: Surgical 'window-of-opportunity' trials that evaluate tumor drug concentrations and pharmacodynamic effects provide invaluable early data early guiding the development of novel therapies. Basket and bucket trials facilitate the development of therapies that target specific biomarkers subsets. Platform trials utilizing Bayesian adaptive randomization and shared control arms such as the INSIGhT and GBM-AGILE trials increase the efficiency and cost-effectiveness of developing novel therapies. There is also growing interest in leveraging external control arms with patient level data to evaluate efficacy in single arm trials, and facilitate interim analysis and potentially reduce the number of control patients in randomized trials.

Summary: These novel designs will hopefully reduce the inefficiencies of developing novel therapies in neuro-oncology and facilitate the identification of more effective therapies for brain tumor patients.

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