Current drug development and trial designs in neuro-oncology: report from the first American Society of Clinical Oncology and Society for Neuro-Oncology Clinical Trials Conference

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Abstract

Successful drug development for people with cancers of the CNS has been challenging. There are multiple barriers to successful drug development including biological factors, rarity of the disease, and ineffective use of clinical trials. Based upon a series of presentations at the First Central Nervous System Clinical Trials Conference hosted by the American Society of Clinical Oncology and the Society for Neuro-Oncology, we provide an overview on drug development and novel trial designs in neuro-oncology. This Review discusses the challenges of therapeutic development in neuro-oncology and proposes strategies to improve the drug discovery process by enriching the pipeline of promising therapies, optimising trial design, incorporating biomarkers, using external data, and maximising efficacy and reproducibility of clinical trials.