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Statistical and trial design considerations in central nervous system prophylaxis studies.

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The prognosis is very poor for patients with cancer who develop central nervous system (CNS) involvement. Prophylactic therapy may be a viable strategy for patients at high risk of CNS metastases. This article explores the rationale, feasibility, and ethics of prophylactic therapy. We discuss various study design considerations for CNS prophylaxis trials, with particular focus on statistical issues, and provide guidance to clinicians trying to decide how to investigate prophylactic therapy. The pool of patients eligible for inclusion in clinical trials is limited. To answer pressing clinical questions, innovative trial designs are needed, along with operational strategies that include risk factor enrichment, target-based end point selection and validation, flexible study accrual and monitoring, and techniques that permit early termination when initial treatment outcomes are poor. Two proposed trials for patients with glioblastoma multiforme and non-Hodgkin lymphoma are presented to highlight design considerations.

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