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Trial Design for Cancer Immunotherapy: A Methodological Toolkit

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Abstract:

Immunotherapy with checkpoint inhibitors (CPIs) and cell-based products has revolutionized the treatment of various solid tumors and hematologic malignancies. These agents have shown unprecedented response rates and long-term benefits in various settings. These clinical advances have also pointed to the need for new or adapted approaches to trial design and assessment of efficacy and safety, both in the early and late phases of drug development. Some of the conventional statistical methods and endpoints used in other areas of oncology appear to be less appropriate in immuno-oncology. Conversely, other methods and endpoints have emerged as alternatives. In this article, we discuss issues related to trial design in the early and late phases of drug development in immuno-oncology, with a focus on CPIs. For early trials, we review the most salient issues related to dose escalation, use and limitations of tumor response and progression criteria for immunotherapy, the role of duration of response as an endpoint in and of itself, and the need to conduct randomized trials as early as possible in the development of new therapies. For late phases, we discuss the choice of primary endpoints for randomized trials, review the current status of surrogate endpoints, and discuss specific statistical issues related to immunotherapy, including non-proportional hazards in the assessment of time-to-event endpoints, alternatives to the Cox model in these settings, and the method of generalized pairwise comparisons, which can provide a patient-centric assessment of clinical benefit and be used to design randomized trials.

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