

Introduction to special issue on biomarker-based clinical trial designs in oncology

Recent years have seen an explosion in knowledge of the biologic drivers of cancer. Many (and likely soon most) tumors are now sub-classified by a molecular profile, with different treatment options and strategies for patients based on their tumor biomarker status. Therapeutic development is similarly now mostly targeted to subsets of patients within any given tumor type based on specific molecular and biologic characteristics. With the so-called basket trials, early phase therapeutic development of targeted agents is focused only on specific molecular aberrations, ignoring tumor type. Umbrella trials on the other hand focus on drug development targeting distinct molecular alterations in one tumor entity. These novel approaches to clinical drug development have resulted in a rapid evolution of methodologies to perform clinical trials in the setting of biomarkers and targeted therapies, where the paradigm of treating very large number of unselected patients is increasingly non-viable.

In this issue, we consider the topic of biomarker-based clinical trial design in oncology. A variety of thought-provoking clinical trials conducted internationally is considered. Reviews of past and ongoing biomarker-driven trials are provided for several of the most common malignancies, and specific high-profile trials such as NCI MATCH, LUNG-MAP, ALCHEMIST and FOCUS4 are reviewed in detail. The authors include clinicians and statisticians, who are integrally involved in these clinical trials and are expert in their respective fields. We hope that this series provides a useful overview of real clinical trials that are challenging the traditional clinical trial design paradigms. This is critical for advancing therapeutic development in this era of molecular medicine.



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