Introduction to the Forum Report on Advances in the Design of Antifungal Clinical Trials

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Design of clinical trials to evaluate drugs has been moving to increasing levels of complexity. Multicenter, multinational trials have been required to determine the clinical indications for a new drug and to receive licensure across the global market. Problems in trial design, discovered after a large, expensive trial has been completed, can delay drug development and waste the limited resources of both the investigators and the industry. Compromises in trial design or size, made to expedite the development process, produce information that is of limited value and easily misinterpreted, to the detriment of patient care. Complex studies provide more information but require more clinician expertise to understand. Investigators conducting clinical trials have the obligation not only to improve the design of clinical trials but also to publish results in a way that makes both the findings and the limitations of the study apparent to the clinician. Clinical investigators, the pharmaceutical industry, and regulatory agencies have a shared responsibility for improving the process by which drugs are evaluated and explained to physicians involved in patient care.

Discussions at the Forum largely centered on design of drug trials but also included trials of a diagnostic test. The group focused on the analysis of certain recent pivotal trials with the intent to discern what these trials have to teach us about design of future trials. A session chairperson introduced each topic. A nominated speaker made a presentation, and further analysis was contributed by one or more discussants and by general discussion.

The Forum proceedings are presented in three parts. The first part concerns issues in clinical trials of empirical antifungal therapy to treat febrile neutropenic patients. The second part deals with issues in design of drug trials for invasive aspergillosis. The third part has three components: issues in the evaluation of diagnostic tests, use of historical controls, and merits of the current multicenter collaborative trial groups.

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