Yale SCHOOL OF PUBLIC HEALTH
Biostatistics

Design of Dose-Response Clinical Trials

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ABSTRACT

In the process of drug discovery and drug development, understanding the dose-response relationship is one of the most challenging tasks. It is also critical to identify the right range of doses in early stages of clinical development so that Phase III trials can be designed to confirm some doses within this dose range. Usually at the beginning of Phase II, there is not a lot of available information to help guiding the study design. At this stage, Phase II clinical studies are needed to establish proof of concept (PoC), to identify a set of potentially effective and safe doses, and to estimate dose-response relationships.

Challenges in designing these studies include: selection of the dose frequency and the dose range, choice of clinical endpoints or biomarkers, and use of control(s), among others. Consequences of bad Phase II study designs may lead to the delay of the entire clinical development program or the waste of R&D investment. Misleading results obtained from poor designs could cause a Phase III program to confirm a wrong set of doses, or to stop developing a potentially useful drug. Therefore, it is critical to consider an entire drug development plan, to make best use of all the available information, and to include all relevant experts in designing Phase II dose response clinical trials. This presentation discusses some of these considerations.

12:00 Noon, Tuesday, October 9, 2018
47 College Street, Room 106B
11:45 AM - Lunch served outside Room 106B