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"Key Statistical Concepts in Clinical Trials for Pharma"

J. Rick Turner, 2011, 61 pages, Springer, $49.95

Review by Norman M. Goldfarb

"Key Statistical Concepts in Clinical Trials for Pharma" efficently focuses on statistical concepts that are the most important for practical use in clinical trials. The booklet employs examples from cardiology to illuminate the explanations.

The following excerpt shows how the booklet presents statistical concepts in a practical context:

> **Subject Demographics and Accountability**
>
> Each clinical study report (CSR) has various sections, including subject demographics and accountability, safety data, and efficacy sections. An in-text table may be presented for demographic characteristics. Specific characteristics that are important can vary from study to study, but typical ones include age, sex, ethnicity and baseline data of relevance, e.g., weight, blood pressure, and heart rate. Information concerning the use of concomitant or concurrent medications and evaluations of subject adherence or compliance with the trial's treatment schedule is also atypically presented.
>
> Table 2.1 [not shown] provides an example of an in-text table from a hypothetical clinical trial that summarizes subject accountability.
>
> Several comments about these hypothetical data are appropriate. First, it is possible but unlikely that the number of subjects for the two treatment groups would be identical in a real study. Presenting percentages as well as absolute numbers is therefore useful, since the percentages allow for differing totals of subjects in each group. Second, the number of subjects in the individual categories must add up to the respective group totals. Third, explanation of (any) other reasons for premature withdrawal should be presented, either in text form above or below the table or in footnote form immediately underneath the table.
>
> Documentation of premature withdrawals from a study is important for various reasons. The implications of premature withdrawals are different in the analysis of safety and in the analysis of efficacy. From a safety perspective, these data relate to tolerability of the drug. From an efficacy perspective, dropouts lead to missing data, and the way(s) that missing data are addressed is important from the point of view of full interpretation of the analysis presented.

The booklet includes six chapters:

- Setting the Scene
- Analyzing Safety Data
- Assessing Efficacy Data
- Confidence Intervals: Additional Commentary
- Meta-Methodology
- Benefit-Risk Estimation

The booklet is available in bookstores.

Reviewer

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