

"Methods and Applications of Statistics in Clinical Trials"

N. Balakrishnan, editor, 2014, 1,896 pages, Wiley, \$195

Review by Norman M. Goldfarb

"Methods and Applications of Statistics in Clinical Trials" is a comprehensive, in-depth and up-to-date guide to statistics in clinical research. Most readers will have more than an introductory understanding of statistics. Most of the chapters are statistics-heavy but some chapters, like "Clinical Trial Misconduct," "Gene Therapy," "Placebos," and "Microarray" are not about statistics, but are of interest to statisticians.

This book has been selected for
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Essential reading for clinical research professionals

To fit all the material into two large volumes, the printer used a compact font similar to Times Roman 9. The first volume is about concepts, principles, trials and designs. The second volume is about planning, analysis and inferential methods.

The following excerpt illustrates the writing in the book:

5.3 The Alpha Spending Function

The initial (or classical) group sequential boundaries are formed by choosing boundary values such that the sum of the probability of exceeding those critical values during the interim analyses is exactly the specified alpha level set in the trial design, assuming the null hypothesis of no intervention effect. That is, the total available alpha is allocated or "spent" over the pre-specified times of interim analyses. The alpha spending function proposed by Lan and DeMets allocated the alpha level over the interim analyses by a continuous function, $\alpha(t)$, where t is the information fraction, $0 < t < 1$. Here t could be the fraction of target patients recruited (n/N) or the fraction of targeted deaths observed (d/D) at the time of the interim analysis. In general, if the total information for the trial design is I , then at the j -th analysis, the information fraction $t_j = I_j/I$. The total expected information I should have been determined by the trial design, if properly done. The function $\alpha(t)$ is defined such that $\alpha(0) = 0$ and $\alpha(1) = \alpha$.

The book consists of 135 articles, written by 208 contributors. There are too many articles to list here, but here are a few topics that struck the reviewer's eye:

- Alpha-Spending Function
- Biased Coin Randomization
- Preference Trials
- Bandit Processes and Response-Adaptive Clinical Trials: The Art of Exploration Versus Exploitation
- Bootstrap
- Over- and Underdispersion Models
- Treatment Interruption

The book is available in bookstores.

Reviewer

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