

## "An Introduction to Statistics in Early Phase Trials"

Steven A. Julious, Say Beng Tan, and David Machin, 2010, 263 pages, Wiley-Blackwell, \$100

Review by Norman M. Goldfarb

"An Introduction to Statistics in Early Phase Trials" is an admirably concise and practical guide to the pertinent context, principles and formulae for statisticians inexperienced in the application of their discipline to Phase I and II clinical research.

The text is clearly written but assumes the reader is familiar with statistics, for example:

This book has been selected for  
[The First Clinical Research Bookshelf](#)  
Essential reading for clinical research professionals

### 3.2.2 ESTIMATE OF POPULATION VARIANCE

Given that we have a specified RPM, we need to have a planning value of the population variance,  $\sigma^2$ . What makes things convenient with early phase trials is that we would usually expect to have all the data to assess variability in-house, and so there is no need to perform an extensive literature review (this is not meant to imply that there should be no literature review at all). The bad news, however, is that there may not be much data upon which to make the estimate of the population variance. As the population variance may be the only thing that we would need to anticipate for the sample size calculations, it may be the one thing to which the sample size, and consequently the study design, is sensitive. It is recommended that this sensitivity is formally assessed at the design stage and we describe how to do this in Section 3.6.

The book consists of 15 chapters:

- Early phase trials
- Introduction to pharmacokinetics
- Sample size calculations for clinical trials
- Crossover trial basics
- Multi-period crossover trials
- First time into man
- Bayesian and frequentist methods
- First-time-into-new-population studies
- Bioequivalence studies
- Other Phase I trials
- Phase II trials: general issues
- Dose-response studies
- Phase II trials with toxic therapies
- Interpreting and applying early phase trial results
- Go/No-Go criteria

The book is available in bookstores.

**Reviewer**

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