“Strategy and Statistics in Clinical Trials: A Non-Statistician’s Guide to Thinking, Designing and Executing”


Review by Norman M. Goldfarb

“Strategy and Statistics in Clinical Trials: A Non-Statistician’s Guide to Thinking, Designing and Executing” provides clear, non-technical explanations of the statistics in clinical trials, from hypothesis formulation through analysis.

Here is an excerpt from the book:

Equivalence

It is virtually impossible to show two products to be truly the same even if they are truly identical. Indeed, in statistics we assume that showing identity is impossible. For now, I ask that you take my word for it; an explanation is forthcoming. And because you cannot show that two products are truly equal, we define equivalence using a range of values rather than a single number. Thus, you will declare one product equivalent to another if the performance of the former is within a predetermined range of the latter. For example, you might specify your product is equivalent to another if your obtained rate of cure is within ±5% of the comparator. Thus, to demonstrate equivalence you specify upper and lower equivalence margins. If your study shows product outcome is within these margins relative to the comparator, you will conclude that products are equivalent.

So equivalence is defined by limiting your attribute at both ends. Using the example, you wish to show that the cure rate is ±5% relative to the comparator, which implies the following:

\[-5\% < T - R < +5\%\]

While the principle should be clear, the logic seems less so; why would you ever want to limit performance at the upper end? Why reject a product for being better? Well, there can be at least two answers to this question, one of which has primarily to do with wording. To this point I have equated “higher” with superior and “lower” with inferior, and this is not always the case. As anyone who cooks, bakes or carries out contracts for the Mafia knows, there is such a thing as “getting it just right.”

The book includes 15 chapters:

- Clinical Development and Statistics: The General View
- Questions For Planning Trials
- Medical Product Attributes
- Setting Research Objectives
- Statistical Thinking
- Estimation
- From Description to Testing: A Beginning
• Statistical Significance, Explanation and Prediction
• Exploratory and Confirmatory Clinical Trials
• One, Two, Three Testing: Hypothesis Testing and Multiplicity
• Elements of Clinical Trial Design I: Putting It Together
• Elements of Clinical Trial Design II
• Endpoints
• Sample Size
• Concluding Remarks

The book is available in bookstores.

Reviewer
Norman M. Goldfarb is Managing Director of First Clinical Research LLC, a provider of clinical research best practices information services. Contact him at 1.650.465.0119 or ngoldfarb@firstclinical.com.