

Concluding comments

We conclude with two overarching comments that bring together the various topics and considerations that we have discussed throughout the book.

First, when faced with the myriad challenges that occur during drug development, the discipline of Statistics is the knight in shining armor that rides to our assistance and facilitates the collection, analysis, and interpretation of optimum quality data as the basis for rational decision-making at all stages of the process. The discipline of Statistics as operationally defined in this book includes study design, experimental methodology, statistical analysis, and the interpretation of the findings of the trial. Very importantly, the interpretation involves addressing issues of both statistical significance and clinical significance. Our interest in Statistics, then, is a pragmatic one: The discipline provides the best way currently available to conduct clinical development programs.

Second, it is appropriate to remind ourselves that our ultimate interest is in providing a new, biologically active, pharmacological agent that will alter a patient's biology for the better. Statistical analyses can be performed on any kind of data (some extremely influential statistical methods were developed in the agricultural arena). In this book, however, the data of interest are biological data, both desirable biologically therapeutic effects and undesirable biological side-effects. Producing a drug that has an acceptable benefit–risk ratio is a long and complex process, and one in which statistical methodology is invaluable.

It is also good to remind ourselves frequently that the welfare of real patients is our ultimate concern. This may not be the first thought that pops into our heads when we are in the middle of a sample-size estimation calculation for an upcoming clinical trial, or when deciding upon which imputation methodology to use to deal with missing data in a clinical database. Nevertheless, this is why we do these things. The pharmaceutical industry is not immune to controversy; far from it. However, as Turner (2007, p 239) noted: “New drug development is a very complicated and difficult undertaking, but one that makes an enormous difference to the health of people across the globe. It is a noble pursuit.”

In this book, set in the context of drug development, we have taught you how to conduct an array of statistical analyses. While teaching such computational skills is appropriate in a statistics textbook, we also hope that we have been successful in providing you with a conceptual understanding of and appreciation for the contribution of the discipline of Statistics to the development of pharmaceutical drugs that may improve the health of your family members, your friends, and yourself.

Reference

Turner JR (2007). *New Drug Development: Design, methodology, and analysis*. Hoboken, NJ: John Wiley & Sons.

