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Innovative strategies, statistical solutions and simulations for modern clinical trials (Chapman & Hall/CRC biostatistics series) 1st edition

Mark Chang, John Balser, Jim Roach and Robin Bliss, Boca Raton, FL, CRC Press, 2019, ISBN-13: 978-0815379447

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BOOK REVIEW

Innovative strategies, statistical solutions and simulations for modern clinical trials (Chapman & Hall/CRC biostatistics series) 1st edition by Mark Chang, John Balser, Jim Roach and Robin Bliss, Boca Raton, FL, CRC Press, 2019, ISBN-13: 978-0815379447

This is the first edition of a comprehensive book covering the most recent methodology on innovative clinical trial designs for drugs and biological products. It is a great reference book for statisticians, clinicians, and other stakeholders involved in drug discovery and development. The pharmaceutical industry's traditional approach of Research and Development has been challenged in order to optimize the drug development in a more efficient way in terms of resources and associated timeline. While the innovative approach has been considered and applied in some development programs, it has not been incorporated fully across the industry community for the development efficiency. Chang et al aimed to provide the statistical framework to reach the overall development program optimizations in this book. In addition, innovative methodology to mitigate the risks of failed efficacy, safety, strategy, commercial and operation failures have been described by Chang et al. Special techniques such as clinical trial simulations are highly recommended by the authors.

The first two chapters provide the overview of drug development, clinical development plan, and clinical trial design. Examples of drug discovery, preclinical, and clinical development with different therapeutic areas have given readers a nice and easy review. Chapter 2 introduces the concepts of clinical development plan (CDP). According to Chang et al, CDP is the combination of the strategic asset management plan and the highly tactical scientific and operational realms of the clinical trial process. Typical clinical trial design issues such as confounding factors, variability and bias are discussed in detail with examples. Chapter 3, Clinical Development Program Optimization, starts with the benchmarks in clinical development covering failure rates, costs of clinical trials, time to next phase, and rate of competitor emerging. With those identified issues, the 2nd part of this Chapter focuses on the steps of Clinical Development Program optimization. Stochastic decision process for a cancer clinical development program is provided step by step as an example. Chapter 4 is dedicated to the global optimal adaptive design. Examples are provided on how to design an adaptive design with global optimization. Chang et al recommended Bayesian application and other factors on safety to be considered in such designs.

The first 4 chapters above serve as the foundation to be considered and addressed for the global optimization in drug development. Chapter 5 to Chapter 8 cover specific topics on precision medicine, clinical trial with survival endpoint, multiple testing methods in clinical trials, and missing data handling. Readers can get the most recent methodology on missing data handling in Section 8.2.8. Change et al also noted the Guidance from FDA and the European Health Authority/CHMP on missing data handling in Section 8.2.9. Chapter 9 to Chapter 10 discuss special issues and resolutions such as adaptive design with mixed endpoints covered in Section 9.8. Issues and concepts on Data Monitoring Committee/DMC are discussed in depth in Chapter 10. Statistical methods for safety monitoring and statistical methods for interim efficacy analysis are covered in Section 10.6 and 10.7, respectively. Three key aspects, safety, efficacy, and data integrity need to be considered in trial monitoring according to Chang et al. Chapter 11 presents controversies in statistical science. The authors recommended the stakeholders rethink fundamental scientific concepts and basic statistical methods before putting all efforts into creating more complex statistical models. The book contains SAS and R code used in some of the examples, including power simulations and blinded treatment effect estimation, as well as a comprehensive list of references.

The statistical framework established by Chang et al in this book is aligned with FDA's position. FDA released multiple draft guidances to promote clinical trial design efficiency. There are two

guidance released in September 2018 and one guidance released in September 2019. The two draft guidances from 2018 are entitled: "Master Protocols – Efficient Clinical Trial Design Strategies to Expedite Development of Cancer Drugs and Biologics" and "Adaptive Designs for Clinical Trials of Drug and Biologics." The draft guidances from September 2019 are entitled "Interacting with the FDA on Complex Innovative Trial Designs for Drugs and Biological Products."

In summary, this is an excellent reference book for statisticians, clinicians, and all stakeholders involved in clinical development program with a common goal to reach clinical development optimization.

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