Modern Adaptive Randomized Clinical Trials Statis

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Targeted Learning in Data Science - Mark J. van der Laan 2018-03-28
This textbook for graduate students in statistics, data science, and public health deals with the practical challenges that come with big, complex, and dynamic data. It presents a scientific roadmap to translate real-world data science applications into formal statistical estimation problems by using the general template of targeted maximum likelihood estimators. These targeted machine learning algorithms estimate quantities of interest while still providing valid inference. Targeted learning methods within data science area critical component for solving scientific problems in the modern age. The techniques can answer complex questions including optimal rules for assigning treatment based on longitudinal data with time-dependent confounding, as well as other estimands in dependent data structures, such as networks. Included in Targeted Learning in Data Science are demonstrations with soft ware packages and real data sets that present a case that targeted learning is crucial for the next generation of statisticians and data scientists. Th is book is a sequel to the first textbook on machine learning for causal inference, Targeted Learning, published in 2011. Mark van der Laan, PhD, is Jiann-Ping Hsu/Karl E. Peace Professor of Biostatistics and Statistics at UC Berkeley. His research interests include statistical methods in genomics, survival analysis, censored data, machine learning, semiparametric models, causal inference, and targeted learning. Dr. van der Laan received the 2004 Mortimer Spiegelman Award, the 2005 Van Dantzig Award, the 2005 COPSS Snedecor Award, the 2005 COPSS Presidential Award, and has graduated over 40 PhD students in biostatistics and statistics. Sherri Rose, PhD, is Associate Professor of Health Care Policy (Biostatistics) at Harvard Medical School. Her work is centered on developing and integrating innovative statistical approaches to advance human health. Dr. Rose's methodological research focuses on nonparametric machine learning for causal inference and prediction. She co-leads the Health Policy Data Science Lab and currently serves as an associate editor for the Journal of the American Statistical Association and Biostatistics.
Randomization, Masking, and Allocation Concealment - Vance Berger 2017-10-30
Randomization, Masking, and Allocation Concealment is indispensable for any trial researcher who wants to use state of the art randomization methods, and also wants to be able to describe these methods correctly. Far too often the subtle nuances that distinguish proper randomization from flawed randomization are completely ignored in trial reports that state only that randomization was used, with no additional information. Experience has shown that in many cases, the type of randomization that was used was flawed. It is only a matter of time before medical journals and regulatory agencies come to realize that we can no longer rely on (or publish) flawed trials, and that flawed randomization in and of itself disqualifies a trial from being robust or high quality, even if that trial is of high quality otherwise. This book will help to clarify the role randomization plays in ensuring internal validity, and in drawing valid inferences from the data. The various chapters cover a variety of randomization methods, and are not limited to the most common (and most flawed) ones. Readers will come away with a profound understanding of what constitutes a valid randomization procedure, so that they can distinguish the valid from the flawed among not only existing methods but also methods yet to be developed.
Bayesian Analysis with R for Drug Development - Harry Yang 2019-06-26
Drug development is an iterative process. The recent publications of regulatory guidelines further entail a lifecycle approach. Blending data from disparate sources, the Bayesian approach provides a flexible framework for drug development. Despite its advantages, the uptake of Bayesian methodologies is lagging behind in the field of pharmaceutical development. Written specifically for pharmaceutical practitioners, Bayesian Analysis with R for Drug Development: Concepts, Algorithms, and Case Studies, describes a wide range of Bayesian applications to problems throughout pre-clinical, clinical, and Chemistry, Manufacturing, and Control (CMC) development. Authored by two seasoned statisticians in the pharmaceutical industry, the book provides detailed Bayesian solutions to a broad array of pharmaceutical problems. Features Provides a single source of information on Bayesian statistics for drug development Covers a wide spectrum of pre-clinical, clinical, and CMC topics Demonstrates proper Bayesian applications using real-life examples Includes easy-to-follow R code with Bayesian Markov Chain Monte Carlo performed in both JAGS and Stan Bayesian software platforms Offers sufficient background for each problem and detailed description of solutions suitable for practitioners with limited Bayesian knowledge Harry Yang, Ph.D., is Senior Director and Head of Statistical Sciences at AstraZeneca. He has 24 years of experience across all aspects of drug research and development and extensive global regulatory experiences. He has published 6 statistical books, 15 book chapters, and over 90 peer-reviewed papers on diverse scientific and statistical subjects, including 15 joint statistical works with Dr. Novick. He is a frequent invited speaker at national and international conferences. He also developed statistical courses and conducted training at the FDA and USP as well as Peking University. Steven Novick, Ph.D., is Director of Statistical Sciences at AstraZeneca. He has extensively contributed statistical methods to the biopharmaceutical literature. Novick is a skilled Bayesian computer programmer and is frequently invited to speak at conferences, having developed and taught courses in several areas, including drug-combination analysis and Bayesian methods in clinical areas. Novick served on IPAC-RS and has chaired several national statistical conferences.
Essentials of a Successful Biostatistical Collaboration - Arul Earnest 2016-10-14
The aim of this book is to equip biostatisticians and other quantitative scientists with the necessary skills, knowledge, and habits to collaborate effectively with clinicians in the healthcare field. The book provides valuable insight on where to look for information and material on sample size and statistical techniques commonly used in clinical research, and on how best to communicate with clinicians. It also covers the best practices to adopt in terms of project, time, and data management; relationship with collaborators; etc.
Modern Adaptive Randomized Clinical Trials - Oleksandr Sverdlov 2015-06-30
Is adaptive randomization always better than traditional fixed-schedule randomization? Which procedures should be used and under which circumstances? What special considerations are required for adaptive randomized trials? What kind of statistical inference should be used to achieve valid and unbiased treatment comparisons following adaptive randomization designs? Modern Adaptive Randomized Clinical Trials: Statistical and Practical Aspects answers these questions and more. From novel designs to cutting-edge applications, this book presents several new and key developments in adaptive randomization. It also offers a fresh and critical look at a number of already-classical topics. Featuring contributions from statisticians, clinical trialists, and subject-matter experts in academia and the pharmaceutical industry, the text: Clarifies the taxonomy of the concept of adaptive randomization Discusses restricted, covariate-adaptive, response-adaptive, and covariate-adjusted response-adaptive (CARA) randomization designs, as well as randomized designs with treatment selection Gives an exposition to many novel adaptive randomization techniques such as brick tunnel randomization, targeted least absolute shrinkage and selection operator (LASSO)-based CARA randomization, multi-arm multi-stage (MAMS) designs, to name a few Addresses the issues of statistical inference

following covariate-adaptive and response-adaptive randomization designs Describes a successful implementation of a single pivotal phase II/III adaptive trial in infants with proliferating hemangioma Explores some practical aspects of phase II dose-ranging studies and examines statistical monitoring and interim analysis issues in response-adaptive randomized clinical trials Modern Adaptive Randomized Clinical Trials: Statistical and Practical Aspects covers a wide spectrum of topics related to adaptive randomization designs in contemporary clinical trials. The book provides a thorough exploration of the merits of adaptive randomization and aids in identifying when it is appropriate to apply such designs in practice.

Design & Analysis of Clinical Trials for Economic Evaluation & Reimbursement - Iftekhar Khan 2015-11-11

Economic evaluation has become an essential component of clinical trial design to show that new treatments and technologies offer value to payers in various healthcare systems. Although many books exist that address the theoretical or practical aspects of cost-effectiveness analysis, this book differentiates itself from the competition by detailing

Statistical Remedies for Medical Researchers - Peter F. Thall 2020-03-12

This book illustrates numerous statistical practices that are commonly used by medical researchers, but which have severe flaws that may not be obvious. For each example, it provides one or more alternative statistical methods that avoid misleading or incorrect inferences being made. The technical level is kept to a minimum to make the book accessible to non-statisticians. At the same time, since many of the examples describe methods used routinely by medical statisticians with formal statistical training, the book appeals to a broad readership in the medical research community.

Cluster Randomised Trials - Richard J. Hayes 2017-07-06

Cluster Randomised Trials, Second Edition discusses the design, conduct, and analysis of trials that randomise groups of individuals to different treatments. It explores the advantages of cluster randomisation, with special attention given to evaluating the effects of interventions against infectious diseases. Avoiding unnecessary mathematical detail, the book covers basic concepts underlying the use of cluster randomisation, such as direct, indirect, and total effects. In the time since the publication of the first edition, the use of cluster randomised trials (CRTs) has increased substantially, which is reflected in the updates to this edition. There are greatly expanded sections on randomisation, sample size estimation, and alternative designs, including new material on stepped wedge designs. There is a new section on handling ordinal outcome data, and an appendix with descriptions and/or generating code of the example data sets. Although the book mainly focuses on medical and public health applications, it shows that the rigorous evidence of intervention effects provided by CRTs has the potential to inform public policy in a wide range of other areas. The book encourages readers to apply the methods to their own trials, reproduce the analyses presented, and explore alternative approaches.

Bayesian Methods for Repeated Measures - Lyle D. Broemeling 2015-08-04

Analyze Repeated Measures Studies Using Bayesian TechniquesGoing beyond standard non-Bayesian books, Bayesian Methods for Repeated Measures presents the main ideas for the analysis of repeated measures and associated designs from a Bayesian viewpoint. It describes many inferential methods for analyzing repeated measures in various scientific areas,

Artificial Intelligence for Drug Development, Precision Medicine, and Healthcare - Mark Chang 2020-05-07

Artificial Intelligence for Drug Development, Precision Medicine, and Healthcare covers exciting developments at the intersection of computer science and statistics. While much of machine-learning is statistics-based, achievements in deep learning for image and language processing rely on computer science’s use of big data. Aimed at those with a statistical background who want to use their strengths in pursuing AI research, the book: · Covers broad AI topics in drug development, precision medicine, and healthcare. · Elaborates on supervised, unsupervised, reinforcement, and evolutionary learning methods. · Introduces the similarity principle and related AI methods for both big and small data problems. · Offers a balance of statistical and algorithm-based approaches to AI. · Provides examples and real-world applications with hands-on R code. · Suggests the path forward for AI in medicine and artificial general intelligence. As well as covering the history of AI and the innovative ideas, methodologies and software implementation of the field, the book offers a comprehensive review of AI applications in medical sciences. In addition, readers will benefit from hands on exercises, with included R code.

Modern Adaptive Randomized Clinical Trials - Oleksandr Sverdlov 2015-06-30

Is adaptive randomization always better than traditional fixed-schedule randomization? Which procedures should be used and under which circumstances? What special considerations are required for adaptive randomized trials? What kind of statistical inference should be used to achieve valid and unbiased treatment comparisons following adaptive random

Multiregional Clinical Trials for Simultaneous Global New Drug Development - Joshua Chen 2016-04-21

In a global clinical development strategy, multiregional clinical trials (MRCTs) are vital in the development of innovative medicines. Multiregional Clinical Trials for Simultaneous Global New Drug Development presents a comprehensive overview on the current status of conducting MRCTs in clinical development. International experts from academia, in

Biosimilar Clinical Development: Scientific Considerations and New Methodologies - Kerry B. Barker 2016-11-25

Biosimilars have the potential to change the way we think about, identify, and manage health problems. They are already impacting both clinical research and patient care, and this impact will only grow as our understanding and technologies improve. Written by a team of experienced specialists in clinical development, this book discusses various potential drug development strategies, the design and analysis of pharmacokinetics (PK) studies, and the design and analysis of efficacy studies.

Bayesian Designs for Phase I-II Clinical Trials - Ying Yuan 2017-12-19

Reliably optimizing a new treatment in humans is a critical first step in clinical evaluation since choosing a suboptimal dose or schedule may lead to failure in later trials. At the same time, if promising preclinical results do not translate into a real treatment advance, it is important to determine this quickly and terminate the clinical evaluation process to avoid wasting resources. Bayesian Designs for Phase I–II Clinical Trials describes how phase I–II designs can serve as a bridge or protective barrier between preclinical studies and large confirmatory clinical trials. It illustrates many of the severe drawbacks with conventional methods used for early-phase clinical trials and presents numerous Bayesian designs for human clinical trials of new experimental treatment regimes. Written by research leaders from the University of Texas MD Anderson Cancer Center, this book shows how Bayesian designs for early-phase clinical trials can explore, refine, and optimize new experimental treatments. It emphasizes the importance of basing decisions on both efficacy and toxicity.

Applied Biclustering Methods for Big and High-Dimensional Data Using R - Adetayo Kasim 2016-10-03

Proven Methods for Big Data Analysis As big data has become standard in many application areas, challenges have arisen related to methodology and software development, including how to discover meaningful patterns in the vast amounts of data. Addressing these problems, Applied Biclustering Methods for Big and High-Dimensional Data Using R shows how to apply biclustering methods to find local patterns in a big data matrix. The book presents an overview of data analysis using biclustering methods from a practical point of view. Real case studies in drug discovery, genetics, marketing research, biology, toxicity, and sports illustrate the use of several biclustering methods. References to technical details of the methods are provided for readers who wish to investigate the full theoretical background. All the methods are accompanied with R examples that show how to conduct the analyses. The examples, software, and other materials are available on a supplementary website.

Modern Approaches to Clinical Trials Using SAS - Sandeep Menon 2015-12-09

Get the tools you need to use SAS(r) in clinical trial design! Unique and multifaceted, Modern Approaches to Clinical Trials Using SAS: Classical, Adaptive, and Bayesian Methods, edited by Sandeep M. Menon and Richard C. Zink, thoroughly covers several domains of modern clinical trial design: classical, group sequential, adaptive, and Bayesian methods that are applicable to and widely used in various phases of pharmaceutical development. Written for biostatisticians, pharmacometricians, clinical developers, and statistical programmers involved in the design, analysis, and interpretation of clinical trials, as well as students in graduate and postgraduate programs in statistics or biostatistics, the book touches on a wide variety of topics, including dose-response and dose-escalation designs; sequential methods to stop trials

early for overwhelming efficacy, safety, or futility; Bayesian designs that incorporate historical data; adaptive sample size re-estimation; adaptive randomization to allocate subjects to more effective treatments; and population enrichment designs. Methods are illustrated using clinical trials from diverse therapeutic areas, including dermatology, endocrinology, infectious disease, neurology, oncology, and rheumatology. Individual chapters are authored by renowned contributors, experts, and key opinion leaders from the pharmaceutical/medical device industry or academia. Numerous real-world examples and sample SAS code enable users to readily apply novel clinical trial design and analysis methodologies in practice.

Data and Safety Monitoring Committees in Clinical Trials, Second Edition - Jay Herson 2016-12-19

Praise for the first edition: "Given the author's years of experience as a statistician and as a founder of the first DMC in pharmaceutical industry trials, I highly recommend this book—not only for experts because of its cogent and organized presentation, but more importantly for young investigators who are seeking information about the logistical and philosophical aspects of a DMC." -S. T. Ounpraseuth, The American Statistician ? In the first edition of this well-regarded book, the author provided a groundbreaking and definitive guide to best practices in pharmaceutical industry data monitoring committees (DMCs). Maintaining all the material from the first edition and adding substantial new material, Data and Safety Monitoring Committees in Clinical Trials, Second Edition is ideal for training professionals to serve on their first DMC as well as for experienced clinical and biostatistical DMC members, sponsor and regulatory agency staff. The second edition guides the reader through newly emerging DMC responsibilities brought about by regulations emphasizing risk vs benefit and the emergence of risk-based monitoring. It also provides the reader with many new statistical methods, clinical trial designs and clinical terminology that have emerged since the first edition. The references have been updated and the very popular end-of-chapter Q&A section has been supplemented with many new experiences since the first edition. ? New to the Second Edition: Presents statistical methods, tables, listings and graphs appropriate for safety review, efficacy analysis and risk vs benefit analysis, SPERT and PRISMA initiatives. Newly added interim analysis for efficacy and futility section. DMC responsibilities in SUSARs (Serious Unexpected Serious Adverse Reactions), basket trials, umbrella trials, dynamic treatment strategies /SMART trials, pragmatic trials, biosimilar trials, companion diagnostics, etc. DMC responsibilities for data quality and fraud detection (Fraud Recovery Plan) Use of patient reported outcomes of safety Use of meta analysis and data outside the trial New ideas for training and compensation of DMC members ? Jay Herson is Senior Associate, Biostatistics, Johns Hopkins Bloomberg School of Public Health where he teaches courses on clinical trials and drug development based on his many years experience in clinical trials in academia and the pharmaceutical industry. ? ? ? ? ? ? ? ? ? ? ? ? ?

Clinical Trial Data Analysis Using R and SAS - Ding-Geng (Din) Chen 2017-06-01

Review of the First Edition "The goal of this book, as stated by the authors, is to fill the knowledge gap that exists between developed statistical methods and the applications of these methods. Overall, this book achieves the goal successfully and does a nice job. I would highly recommend it ...The example-based approach is easy to follow and makes the book a very helpful desktop reference for many biostatistics methods."—Journal of Statistical Software Clinical Trial Data Analysis Using R and SAS, Second Edition provides a thorough presentation of biostatistical analyses of clinical trial data with step-by-step implementations using R and SAS. The book's practical, detailed approach draws on the authors' 30 years' experience in biostatistical research and clinical development. The authors develop step-by-step analysis code using appropriate R packages and functions and SAS PROCS, which enables readers to gain an understanding of the analysis methods and R and SAS implementation so that they can use these two popular software packages to analyze their own clinical trial data. What's New in the Second Edition Adds SAS programs along with the R programs for clinical trial data analysis. Updates all the statistical analysis with updated R packages. Includes correlated data analysis with multivariate analysis of variance. Applies R and SAS to clinical trial data from hypertension, duodenal ulcer, beta blockers, familial andenomatous polyposis, and breast cancer trials. Covers the biostatistical aspects of various clinical trials, including treatment comparisons, time-to-event endpoints, longitudinal clinical trials, and bioequivalence trials.

Statistical Methods for Immunogenicity Assessment - Harry Yang 2015-07-17

Develop Effective Immunogenicity Risk Mitigation StrategiesImmunogenicity assessment is a prerequisite for the successful development of biopharmaceuticals, including safety and efficacy evaluation. Using advanced statistical methods in the study design and analysis stages is therefore essential to immunogenicity risk assessment and mitigation stra

Digital Therapeutics - Oleksandr Sverdlov 2022-12-13

One of the hallmarks of the 21st century medicine is the emergence of digital therapeutics (DTx)—evidence-based, clinically validated digital technologies to prevent, diagnose, treat, and manage various diseases and medical conditions. DTx solutions have been gaining interest from patients, investors, healthcare providers, health authorities, and other stakeholders because of the potential of DTx to deliver equitable, massively scalable, personalized and transformative treatments for different unmet medical needs. Digital Therapeutics: Scientific, Statistical, Clinical, and Regulatory Aspects is an unparalleled summary of the current scientific, statistical, developmental, and regulatory aspects of DTx which is poised to become the fastest growing area of the biopharmaceutical and digital medicine product development. This edited volume intends to provide a systematic exposition to digital therapeutics through 19 peer-reviewed chapters written by subject matter experts in this emerging field. This edited volume is an invaluable resource for business leaders and researchers working in public health, healthcare, digital health, information technology, and biopharmaceutical industries. It will be also useful for regulatory scientists involved in the review of DTx products, and for faculty and students involved in an interdisciplinary research on digital health and digital medicine.

Modern Approaches to Clinical Trials Using SAS: Classical, Adaptive, and Bayesian Methods - Sandeep Menon 2015-12-09

This book covers domains of modern clinical trial design: classical, group sequential, adaptive, and Bayesian methods applicable to and used in various phases of pharmaceutical development. Written for biostatisticians, pharmacometricians, clinical developers, and statistical programmers involved in the design, analysis, and interpretation of clinical trials, as well as students in graduate and postgraduate programs in statistics or biostatistics, it covers topics including: dose-response and dose-escalation designs; sequential methods to stop trials early for overwhelming efficacy, safety, or futility; Bayesian designs incorporating historical data; adaptive sample size re-estimation and randomization to allocate subjects to effective treatments; population enrichment designs. Methods are illustrated using clinical trials from diverse therapeutic areas, including dermatology, endocrinology, infectious disease, neurology, oncology and rheumatology. --

Bioequivalence and Statistics in Clinical Pharmacology - Scott D. Patterson 2017-03-27

Maintaining a practical perspective, Bioequivalence and Statistics in Clinical Pharmacology, Second Edition explores statistics used in day-to-day clinical pharmacology work. The book is a starting point for those involved in such research and covers the methods needed to design, analyze, and interpret bioequivalence trials; explores when, how, and why these studies are performed as part of drug development; and demonstrates the methods using real world examples. Drawing on knowledge gained directly from working in the pharmaceutical industry, the authors set the stage by describing the general role of statistics. Once the foundation of clinical pharmacology drug development, regulatory applications, and the design and analysis of bioequivalence trials are established, including recent regulatory changes in design and analysis and in particular sample-size adaptation, they move on to related topics in clinical pharmacology involving the use of cross-over designs. These include, but are not limited to, safety studies in Phase I, dose-response trials, drug interaction trials, food-effect and combination trials, QTc and other pharmacodynamic equivalence trials, proof-of-concept trials, dose-proportionality trials, and vaccines trials. This second edition addresses several recent developments in the field, including new chapters on adaptive bioequivalence studies, scaled average bioequivalence testing, and vaccine trials. Purposefully designed to be instantly applicable, Bioequivalence and Statistics in Clinical Pharmacology, Second Edition provides examples of SAS and R code so that the analyses described can be immediately implemented. The authors have made extensive use of the proc mixed procedures available in SAS.

Statistical Methods for Survival Trial Design - Jianrong Wu 2018-06-14

Statistical Methods for Survival Trial Design: With Applications to

Cancer Clinical Trials Using R provides a thorough presentation of the principles of designing and monitoring cancer clinical trials in which time-to-event is the primary endpoint. Traditional cancer trial designs with time-to-event endpoints are often limited to the exponential model or proportional hazards model. In practice, however, those model assumptions may not be satisfied for long-term survival trials. This book is the first to cover comprehensively the many newly developed methodologies for survival trial design, including trial design under the Weibull survival models; extensions of the sample size calculations under the proportional hazard models; and trial design under mixture cure models, complex survival models, Cox regression models, and competing-risk models. A general sequential procedure based on the sequential conditional probability ratio test is also implemented for survival trial monitoring. All methodologies are presented with sufficient detail for interested researchers or graduate students.

Methods in Comparative Effectiveness Research - Constantine Gatsonis 2017-02-24

Comparative effectiveness research (CER) is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care (IOM 2009). CER is conducted to develop evidence that will aid patients, clinicians, purchasers, and health policy makers in making informed decisions at both the individual and population levels. CER encompasses a very broad range of types of studies—experimental, observational, prospective, retrospective, and research synthesis. This volume covers the main areas of quantitative methodology for the design and analysis of CER studies. The volume has four major sections—causal inference; clinical trials; research synthesis; and specialized topics. The audience includes CER methodologists, quantitative-trained researchers interested in CER, and graduate students in statistics, epidemiology, and health services and outcomes research. The book assumes a masters-level course in regression analysis and familiarity with clinical research.

Textbook of Clinical Trials in Oncology - Susan Halabi 2019-04-24

There is an increasing need for educational resources for statisticians and investigators. Reflecting this, the goal of this book is to provide readers with a sound foundation in the statistical design, conduct, and analysis of clinical trials. Furthermore, it is intended as a guide for statisticians and investigators with minimal clinical trial experience who are interested in pursuing a career in this area. The advancement in genetic and molecular technologies have revolutionized drug development. In recent years, clinical trials have become increasingly sophisticated as they incorporate genomic studies, and efficient designs (such as basket and umbrella trials) have permeated the field. This book offers the requisite background and expert guidance for the innovative statistical design and analysis of clinical trials in oncology. Key Features: Cutting-edge topics with appropriate technical background Built around case studies which give the work a "hands-on" approach Real examples of flaws in previously reported clinical trials and how to avoid them Access to statistical code on the book’s website Chapters written by internationally recognized statisticians from academia and pharmaceutical companies Carefully edited to ensure consistency in style, level, and approach Topics covered include innovating phase I and II designs, trials in immune-oncology and rare diseases, among many others

Handbook of Methods for Designing, Monitoring, and Analyzing Dose-Finding Trials - John O'Quigley 2017-04-27

Handbook of Methods for Designing, Monitoring, and Analyzing Dose-Finding Trials gives a thorough presentation of state-of-the-art methods for early phase clinical trials. The methodology of clinical trials has advanced greatly over the last 20 years and, arguably, nowhere greater than that of early phase studies. The need to accelerate drug development in a rapidly evolving context of targeted therapies, immunotherapy, combination treatments and complex group structures has provided the stimulus to these advances. Typically, we deal with very small samples, sequential methods that need to be efficient, while, at the same time adhering to ethical principles due to the involvement of human subjects. Statistical inference is difficult since the standard techniques of maximum likelihood do not usually apply as a result of model misspecification and parameter estimates lying on the boundary of the parameter space. Bayesian methods play an important part in overcoming these difficulties, but nonetheless, require special consideration in this particular context. The purpose of this handbook is to provide an expanded summary of the field as it stands and also, through discussion, provide insights into the thinking of leaders in the field as to the potential developments of the years ahead. With this goal in mind we present: An introduction to the field for graduate students and novices A basis for more established researchers from which to build A collection of material for an advanced course in early phase clinical trials A comprehensive guide to available methodology for practicing statisticians on the design and analysis of dose-finding experiments An extensive guide for the multiple comparison and modeling (MCP-Mod) dose-finding approach, adaptive two-stage designs for dose finding, as well as dose–time–response models and multiple testing in the context of confirmatory dose-finding studies. John O’Quigley is a professor of mathematics and research director at the French National Institute for Health and Medical Research based at the Faculty of Mathematics, University Pierre and Marie Curie in Paris, France. He is author of Proportional Hazards Regression and has published extensively in the field of dose finding. Alexia Iasonos is an associate attending biostatistician at the Memorial Sloan Kettering Cancer Center in New York. She has over one hundred publications in the leading statistical and clinical journals on the methodology and design of early phase clinical trials. Dr. Iasonos has wide experience in the actual implementation of model based early phase trials and has given courses in scientific meetings internationally. Björn Bornkamp is a statistical methodologist at Novartis in Basel, Switzerland, researching and implementing dose-finding designs in Phase II clinical trials. He is one of the co-developers of the MCP-Mod methodology for dose finding and main author of the DoseFinding R package. He has published numerous papers on dose finding, nonlinear models and Bayesian statistics, and in 2013 won the Royal Statistical Society award for statistical excellence in the pharmaceutical industry.

Innovative Strategies, Statistical Solutions and Simulations for Modern Clinical Trials - Mark Chang 2019-03-20

"This is truly an outstanding book. [It] brings together all of the latest research in clinical trials methodology and how it can be applied to drug development.... Chang et al provide applications to industry-supported trials. This will allow statisticians in the industry community to take these methods seriously." Jay Herson, Johns Hopkins University The pharmaceutical industry's approach to drug discovery and development has rapidly transformed in the last decade from the more traditional Research and Development (R & D) approach to a more innovative approach in which strategies are employed to compress and optimize the clinical development plan and associated timelines. However, these strategies are generally being considered on an individual trial basis and not as part of a fully integrated overall development program. Such optimization at the trial level is somewhat near-sighted and does not ensure cost, time, or development efficiency of the overall program. This book seeks to address this imbalance by establishing a statistical framework for overall/global clinical development optimization and providing tactics and techniques to support such optimization, including clinical trial simulations. Provides a statistical framework for achieve global optimization in each phase of the drug development process. Describes specific techniques to support optimization including adaptive designs, precision medicine, survival-endpoints, dose finding and multiple testing. Gives practical approaches to handling missing data in clinical trials using SAS. Looks at key controversial issues from both a clinical and statistical perspective. Presents a generous number of case studies from multiple therapeutic areas that help motivate and illustrate the statistical methods introduced in the book. Puts great emphasis on software implementation of the statistical methods with multiple examples of software code (both SAS and R). It is important for statisticians to possess a deep knowledge of the drug development process beyond statistical considerations. For these reasons, this book incorporates both statistical and "clinical/medical" perspectives.

Statistical Methods for Healthcare Performance Monitoring - Alex Bottle 2016-08-05

Healthcare is important to everyone, yet large variations in its quality have been well documented both between and within many countries. With demand and expenditure rising, it’s more crucial than ever to know how well the healthcare system and all its components – from staff member to regional network – are performing. This requires data, which inevitably differ in form and quality. It also requires statistical methods, the output of which needs to be presented so that it can be understood by whoever needs it to make decisions. Statistical Methods for Healthcare Performance Monitoring covers measuring quality, types of data, risk adjustment, defining good and bad performance, statistical monitoring, presenting the results to different audiences and evaluating the monitoring system itself. Using examples from around the world, it

brings all the issues and perspectives together in a largely non-technical way for clinicians, managers and methodologists. Statistical Methods for Healthcare Performance Monitoring is aimed at statisticians and researchers who need to know how to measure and compare performance, health service regulators, health service managers with responsibilities for monitoring performance, and quality improvement scientists, including those involved in clinical audits.

Neuropsychological Rehabilitation - Barbara A. Wilson 2017-06-20

E) Rehabilitation in mainland China -- f) Rehabilitation in Hong Kong -- g) Rehabilitation in Brazil -- h) Rehabilitation in Argentina -- i) Rehabilitation in South Africa -- j) Rehabilitation in Botswana -- SECTION SEVEN Evaluation and general conclusions -- 42 Outcome measures -- 43 Avoiding bias in evaluating rehabilitation -- 44 Challenges in the evaluation of neuropsychological rehabilitation effects -- 45 Summary and guidelines for neuropsychological rehabilitation -- Index

Statistical Topics in Health Economics and Outcomes Research - Demissie Alemayehu, PhD 2017-11-22

With ever-rising healthcare costs, evidence generation through Health Economics and Outcomes Research (HEOR) plays an increasingly important role in decision-making about the allocation of resources. Accordingly, it is now customary for health technology assessment and reimbursement agencies to request for HEOR evidence, in addition to data from clinical trials, to inform decisions about patient access to new treatment options. While there is a great deal of literature on HEOR, there is a need for a volume that presents a coherent and unified review of the major issues that arise in application, especially from a statistical perspective. Statistical Topics in Health Economics and Outcomes Research fulfils that need by presenting an overview of the key analytical issues and best practice. Special attention is paid to key assumptions and other salient features of statistical methods customarily used in the area, and appropriate and relatively comprehensive references are made to emerging trends. The content of the book is purposefully designed to be accessible to readers with basic quantitative backgrounds, while providing an in-depth coverage of relatively complex statistical issues. The book will make a very useful reference for researchers in the pharmaceutical industry, academia, and research institutions involved with HEOR studies. The targeted readers may include statisticians, data scientists, epidemiologists, outcomes researchers, health economists, and healthcare policy and decision-makers.

Emerging Non-Clinical Biostatistics in Biopharmaceutical Development and Manufacturing - Harry Yang 2016-11-30

The premise of Quality by Design (QbD) is that the quality of the pharmaceutical product should be based upon a thorough understanding of both the product and the manufacturing process. This state-of-the-art book provides a single source of information on emerging statistical approaches to QbD and risk-based pharmaceutical development. A comprehensive resource, it combines in-depth explanations of advanced statistical methods with real-life case studies that illustrate practical applications of these methods in QbD implementation.

Exposure-Response Modeling - Jixian Wang 2015-07-17

Discover the Latest Statistical Approaches for Modeling Exposure-Response RelationshipsWritten by an applied statistician with extensive practical experience in drug development, Exposure-Response Modeling: Methods and Practical Implementation explores a wide range of topics in exposure-response modeling, from traditional pharmacokinetic-pharmacody

Statistical Testing Strategies in the Health Sciences - Albert Vexler 2017-12-19

Statistical Testing Strategies in the Health Sciences provides a compendium of statistical approaches for decision making, ranging from graphical methods and classical procedures through computationally intensive bootstrap strategies to advanced empirical likelihood techniques. It bridges the gap between theoretical statistical methods and practical procedures applied to the planning and analysis of health-related experiments. The book is organized primarily based on the type of questions to be answered by inference procedures or according to the general type of mathematical derivation. It establishes the theoretical framework for each method, with a substantial amount of chapter notes included for additional reference. It then focuses on the practical application for each concept, providing real-world examples that can be easily implemented using corresponding statistical software code in R and SAS. The book also explains the basic elements and methods for constructing correct and powerful statistical decision-making processes to be adapted for complex statistical applications. With techniques spanning robust statistical methods to more computationally intensive approaches, this book shows how to apply correct and efficient testing mechanisms to various problems encountered in medical and epidemiological studies, including clinical trials. Theoretical statisticians, medical researchers, and other practitioners in epidemiology and clinical research will appreciate the book's novel theoretical and applied results. The book is also suitable for graduate students in biostatistics, epidemiology, health-related sciences, and areas pertaining to formal decision-making mechanisms.

Analyzing Longitudinal Clinical Trial Data - Craig Mallinckrodt 2016-12-12

Analyzing Longitudinal Clinical Trial Data: A Practical Guide provides practical and easy to implement approaches for bringing the latest theory on analysis of longitudinal clinical trial data into routine practice.The book, with its example-oriented approach that includes numerous SAS and R code fragments, is an essential resource for statisticians and graduate students specializing in medical research. The authors provide clear descriptions of the relevant statistical theory and illustrate practical considerations for modeling longitudinal data. Topics covered include choice of endpoint and statistical test; modeling means and the correlations between repeated measurements; accounting for covariates; modeling categorical data; model verification; methods for incomplete (missing) data that includes the latest developments in sensitivity analyses, along with approaches for and issues in choosing estimands; and means for preventing missing data. Each chapter stands alone in its coverage of a topic. The concluding chapters provide detailed advice on how to integrate these independent topics into an over-arching study development process and statistical analysis plan.

Statistical Design and Analysis of Clinical Trials - Weichung Joe Shih 2015-07-28

Statistical Design and Analysis of Clinical Trials: Principles and Methods concentrates on the biostatistics component of clinical trials. Developed from the authors' courses taught to public health and medical students, residents, and fellows during the past 15 years, the text shows how biostatistics in clinical trials is an integration of many fu

Clinical Trial Optimization Using R - Alex Dmitrienko 2017-08-10

Clinical Trial Optimization Using R explores a unified and broadly applicable framework for optimizing decision making and strategy selection in clinical development, through a series of examples and case studies. It provides the clinical researcher with a powerful evaluation paradigm, as well as supportive R tools, to evaluate and select among simultaneous competing designs or analysis options. It is applicable broadly to statisticians and other quantitative clinical trialists, who have an interest in optimizing clinical trials, clinical trial programs, or associated analytics and decision making. This book presents in depth the Clinical Scenario Evaluation (CSE) framework, and discusses optimization strategies, including the quantitative assessment of tradeoffs. A variety of common development challenges are evaluated as case studies, and used to show how this framework both simplifies and optimizes strategy selection. Specific settings include optimizing adaptive designs, multiplicity and subgroup analysis strategies, and overall development decision-making criteria around Go/No-Go. After this book, the reader will be equipped to extend the CSE framework to their particular development challenges as well.

Elementary Bayesian Biostatistics - Lemuel A. Moyé 2007-07-27

Bayesian analyses have made important inroads in modern clinical research due, in part, to the incorporation of the traditional tools of noninformative priors as well as the modern innovations of adaptive randomization and predictive power. Presenting an introductory perspective to modern Bayesian procedures, Elementary Bayesian Biostatistics explores Bayesian principles and illustrates their application to healthcare research. Building on the basics of classic biostatistics and algebra, this easy-to-read book provides a clear overview of the subject. It focuses on the history and mathematical foundation of Bayesian procedures, before discussing their implementation in healthcare research from first principles. The author also elaborates on the current controversies between Bayesian and frequentist biostatisticians. The book concludes with recommendations for Bayesians to improve their standing in the clinical trials community. Calculus derivations are relegated to the appendices so as not to overly complicate the main text. As Bayesian methods gain more acceptance in healthcare, it is necessary for clinical scientists to understand Bayesian principles. Applying Bayesian analyses to modern healthcare research issues, this lucid introduction helps readers make the correct choices in the development of clinical research programs.

The Agile Approach to Adaptive Research - Michael J. Rosenberg

2010-02-18
Apply adaptive research to improve results in drug development The pharmaceutical industry today faces a deepening crisis: inefficiency in its core business, the development of new drugs. The Agile Approach to Adaptive Research offers a solution. It outlines how adaptive research, using already-available tools and techniques, can enable the industry to streamline clinical trials and reach decision points faster and more efficiently. With a wealth of real-world cases and examples, author Michael Rosenberg gives readers a practical overview of drug development, the problems inherent in current practices, and the advantages of adaptive research technology and methods. He explains the concepts, principles, and specific techniques of adaptive research, and demonstrates why it is an essential evolutionary step toward improving drug research and development. Chapters explore such subjects as: The adaptive concept Design and operational adaptations Sample-size reestimation Agile clinical development Safety and dose finding Statistics in adaptive research, including frequentist and Bayesian approaches Data management technologies The future of clinical development By combining centuries-old intellectual foundations, recent technological advances, and modern management techniques, adaptive research preserves the integrity and validity of clinical research but dramatically improves efficiency.

Data and Safety Monitoring Committees in Clinical Trials - Jay Herson 2016-12-19
Praise for the first edition: "Given the author's years of experience as a statistician and as a founder of the first DMC in pharmaceutical industry trials, I highly recommend this book—not only for experts because of its cogent and organized presentation, but more importantly for young investigators who are seeking information about the logistical and philosophical aspects of a DMC." -S. T. Ounpraseuth, The American Statistician In the first edition of this well-regarded book, the author provided a groundbreaking and definitive guide to best practices in pharmaceutical industry data monitoring committees (DMCs). Maintaining all the material from the first edition and adding substantial new material, Data and Safety Monitoring Committees in Clinical Trials, Second Edition is ideal for training professionals to serve on their first DMC as well as for experienced clinical and biostatistical DMC members, sponsor and regulatory agency staff. The second edition guides the reader through newly emerging DMC responsibilities brought about by regulations emphasizing risk vs benefit and the emergence of risk-based monitoring. It also provides the reader with many new statistical methods, clinical trial designs and clinical terminology that have emerged since the first edition. The references have been updated and the very popular end-of-chapter Q&A section has been supplemented with many new experiences since the first edition. New to the Second Edition: Presents statistical methods, tables, listings and graphs appropriate for safety review, efficacy analysis and risk vs benefit analysis, SPERT and PRISMA initiatives. Newly added interim analysis for efficacy and futility section. DMC responsibilities in SUSARs (Serious Unexpected Serious Adverse Reactions), basket trials, umbrella trials, dynamic treatment strategies /SMART trials, pragmatic trials, biosimilar trials, companion diagnostics, etc. DMC responsibilities for data quality and fraud detection (Fraud Recovery Plan) Use of patient reported outcomes of safety Use of meta analysis and data outside the trial New ideas for training and compensation of DMC members Jay Herson is Senior Associate, Biostatistics, Johns Hopkins Bloomberg School of Public Health where he teaches courses on clinical trials and drug development based on his many years experience in clinical trials in academia and the pharmaceutical industry.

Mathematical and Statistical Skills in the Biopharmaceutical Industry - Arkadiy Pitman 2019-07-15
Mathematical and Statistical Skills in the Biopharmaceutical Industry: A Pragmatic Approach describes a philosophy of efficient problem solving showcased using examples pertinent to the biostatistics function in clinical drug development. It was written to share a quintessence of the authors' experiences acquired during many years of relevant work in the biopharmaceutical industry. The book will be useful will be useful for biopharmaceutical industry statisticians at different seniority levels and for graduate students who consider a biostatistics-related career in this industry. Features: Describes a system of principles for pragmatic problem solving in clinical drug development. Discusses differences in the work of a biostatistician in small pharma and big pharma. Explains the importance/relevance of statistical programming and data management for biostatistics and necessity for integration on various levels. Describes some useful statistical background that can be capitalized upon in the drug development enterprise. Explains some hot topics and current trends in biostatistics in simple, non-technical terms. Discusses incompleteness of any system of standard operating procedures, rules and regulations. Provides a classification of scoring systems and proposes a novel approach for evaluation of the safety outcome for a completed randomized clinical trial. Presents applications of the problem solving philosophy in a highly problematic transfusion field where many investigational compounds have failed. Discusses realistic planning of open-ended projects.