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Book Reviews

Editor: Ananda Sen

Computational Actuarial Science with R
Arthur Charpentier
Chapman and Hall/CRC, 2014, 656 pages, $89.95, hardback
Series: Chapman & Hall/CRC The R Series
ISBN: 978-1-4665-9259-9

Readership: Students, researchers and practitioners with a background in actuarial science.

The main motivation of the book (and of computational actuarial science in general) is related to 'bring new life into the teaching of actuarial science in colleges and universities' and to 'provide an opportunity for students to move away from too much use of lecture–exam paradigm and more use of a laboratory paper paradigm in teaching' (statement rephrased from Kendrick *et al.* (2006)). In fact, the main objective of the book is that the reader gets interested in the topic and plays with the presented models and R codes in an active way. I have experienced that this goal can be easily reached for a large audience of readers, because the presentation of the various arguments encourages an active learning of the concepts 'without being burdened by the theory'.

The book starts with a preliminary chapter that introduces basic aspects of the R language, mentioning also more advanced methods such as parallel computing and C/C++ embedded codes. Then it is divided into four parts dealing with methodology (including Bayesian methods), life insurance, finance and non-life insurance. All the chapters are independent (at least in the computational part). Most of them present a list of exercises, which are very useful for didactic purposes. Although the emphasis is on the computational aspects, a long list of references to theoretical work is also provided at the end of the book.

In my opinion, this is a reference book for people interested in computational aspects of actuarial science. In order to understand its real value, it is necessary that the readers also 'get their hands dirty' and try the different codes and datasets that have been provided. To this end, the R codes presented in this book can be found on *github*, although some codes are still missing. Moreover, the package that contains all the datasets can be found on http://cas.uqam.ca/.

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An Introduction to STATA for Health Researchers
Svend Juul, Morten Frydenberg
Stata Press, 2014, 346 pages, £49.99, paperback
ISBN: 978-1-5971-8135-8

Readership: Researchers and analysts in the health industry with a range of experience using STATA.

The book is broken up into four main sections: The Basics of STATA, Data Management, Analysis and Graphs. There is also an additional, shorter section on advanced topics. The basics

section, comprising four chapters, is an overview of STATA that is comprehensive and well put together and will allow even those who have never opened STATA before to get comfortable with the software's setup. Data management is covered in detail, providing instruction on how to do the most common data management tasks. This section contains ten chapters. This part of the book, more than any, reads like a reference manual, and particularly for beginning users, it could benefit from a working example applying many of these concepts to a single dataset. Nevertheless, the details covered are useful and easy to follow, and the exercises in the Appendix allow some practice for beginners. I particularly enjoyed Chapter 10 'Taking good care of your data', which offers advice on using and managing data throughout a research project. This chapter is an overview of some 'best practice' tips that even experienced researchers would benefit from reviewing.

While impossible to cover every aspect of statistical analysis in a single book, this book does a nice job of covering the most common methods, from simple to complex, with an emphasis on the common methods seen in the field of health research. The authors do not make any assumptions about the knowledge level of the reader on these methods but provide enough details and interpretation to aid a variety of users. The five chapters in the analysis section also integrate information and sample syntax on how to produce relevant graphics for each analysis type. Following and making sense of these graphics statements is aided by first reading through the Graphs chapter (Chapter 16), which details common formats of graph commands and options to customise graphs, which I believe to be one of STATA's great features. The Graphs chapter also covers the generation of some commonly used graphs (histograms, boxplots, etc.) in further detail. The Advanced Topics chapter (Chapter 17) touches briefly on some of the programming capabilities that STATA has, and offers enough detail on topics such as macros and loops to get one started in programming within STATA.

Although this book claims to provide an introduction to STATA, I find that it would not only be useful for novice STATA users but would also be beneficial to experienced STATA users. The detailed table of contents and index make finding specific topics and examples within the book a breeze. The book is ideal to keep on hand as a reference manual.

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Adaptive Design Theory and Implementation Using SAS and R, Second Edition
Mark Chang
CRC Press, 2014, xli+ 664 pages, $89.96, hardback
ISBN: 978-1-4822-5659-8

Readership: Statistics graduate students and researchers with sufficient training in statistical hypothesis testing, modelling and computation, as well as clinical trial design.

With a heightened emphasis on 'personalised' and 'precision' medicine, now a part of the clinical trial landscape, it is appealing to make clinical trials seem less static and instead allow them to adapt to their accruing data. However, the word 'adaptive' is really an umbrella term for a multitude of designs that can use the accruing data to modify some aspect of a clinical trial. To that end, this text is a compilation of numerous adaptive trial designs that have been developed over the past few decades, including interim analyses, sample size re-estimation, biomarker enrichment and response-adaptive treatment assignment. It is also an update to the first edition published in 2008 and includes 12 new chapters, as well as reformulations of several other

chapters. Chapter 1 is an introduction to the concept of adaptive clinical trials, and Chapter 2 is a nice summary of what is commonly referred to as the 'classical' design of clinical trials. Each remaining chapter focuses on a specific example of an adaptive trial design and covers the statistical theory and computer code related to that design.

Two strengths of this text are the numerous examples that are included throughout and the inclusion of open-ended problems at the end of chapters to help readers work through some of the concepts on their own. Another strength of this text is its attempt to cover as many adaptive designs as possible. However, the encyclopaedic nature of this text is also its greatest weakness, as many of the topics are described without sufficient detail for the reader to fully understand and implement the designs successfully. For example, Chapters 21–24 cover Bayesian methods and adaptive designs that rely upon Bayesian statistics, but none of those chapters contain enough depth to make them useful for implementation. The coverage of adaptive dose-finding studies is also woefully out of date and seems to fit poorly with the rest of the text, which focuses on adaptive design suited only for larger-scale phase III trials.

Although there are some R codes included in the text, the majority of the statistical programmes included in this text are written in SAS. It would have been preferable for chapters to have parallel codes in both R and SAS so that all designs could be implemented in either language at the discretion of the reader. Much of the codes are also lacking in comments and explanations, so that only proficient users of SAS or R would be able to understand the programmes.

This text is certainly a useful reference that covers approaches to adaptive clinical trial design, but would only be suitable for those with sufficient graduate-level statistical training and background in clinical trial design and programming.

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Case–Control Studies
Ruth H. Keogh, D. R. Cox
CUP, 2014, £50.00, hardback
ISBN: 978-1-1070-1956-0

Readership: Statistics and biostatistics researchers, including graduate students pursuing research on statistical methodologies with application to biology, social, physical and health sciences.

Case–control design is a cornerstone of epidemiology studies. This design is also commonly used in other research areas including, but not limited to, social science, econometrics and political science. This book provides a lucid review of the fundamental concepts and recent novel developments in this retrospective sampling scheme. Throughout, the emphasis is on the concepts underlying various case/control ascertainment methods and on intuitive ways to write the corresponding likelihood functions using relevant conditional probabilities for estimating parameters such as odds ratios and risk ratios. Each chapter begins with an itemised list of the main results of that chapter. The notes given at the end of each chapter provide a comprehensive list of key related works corresponding to each section. Simple graphical displays of the design and examples based on published case–control studies facilitate a better understanding of the quantitative concepts.

This book can be a useful guide and a reference tool for case–control design and analysis methods for the statistically inclined researcher. It is an excellent collection of important results relating retrospective and prospective sampling schemes given in a succinct and well-organised fashion. It will be beneficial if the reader is already familiar with software packages for analysing case–control data, maximum likelihood estimation and inference.

This book has ten chapters. Chapter 1 is an introductory overview of the various types of case–control designs and biases arising under retrospective ascertainment. Chapters 2 and 3 describe the key characteristics of unmatched and matched case–control studies, respectively. Chapter 4 describes numerous novel concepts of the classic unmatched design under a logistic regression framework. Here, the reader is introduced to three main models: (i) the population model, which is based on the joint probability of outcome and risk factor(s); (ii) the sampling model, which corresponds to the conditional probability of risk factors given outcome and arises in retrospective sampling; and (iii) the inverse model, that is, the conditional probability of outcome given risk factors, which is usually the interpretable quantity of interest in practical settings and one that would arise naturally under a prospective sampling scheme. The interplay between these models is used to provide the intuition underlying several key results for the classic case–control studies, including the celebrated result about the equivalence of odds ratio of the risk factor under prospective and retrospective sampling. There is also an informative discourse on non-collapsibility of odds ratios when adjusting for versus marginalising over background variables. Chapter 4 also discusses fitting the inverse model under a non-logistic regression framework and provides insights into the relationship between odds ratio estimates under logistic and non-logistic models. There is a brief mention of Bayesian methods for estimating the odds ratio parameters with further details about the choice of priors relegated to the Appendix.

Chapter 5 is devoted to multiple outcomes, including multiple case subtypes and one or more control groups. Case-only and case-crossover designs are also introduced. Chapter 6 is devoted entirely to a summary of innovative extensions developed since the 1990s–particularly the two-stage (also known as two-phase) design to gain efficiency. A large cohort study or a large case–control study is established in stage 1. A random sample or a stratified sample (stratified according to some background variable of interest) is drawn from the stage 1 samples to measure risk factors that may be more expensive to measure in the complete sample. The parameter estimation methods and variance estimation are summarised using the sampling and inverse models. Chapter 6 also discusses matching in the second stage and ascertainment of family members as a way of obtaining matched controls.

Chapters 7 and 8 focus on case–control sampling that also takes time into account. Chapter 7 summarises the nested case–control sampling scheme and related analysis methods. Controls are selected from the risk set at every case event time. Several choices of controls are described, including random selection, matched and counter-matched controls. There is also a brief treatment of quota sampling. The case–subcohort description of Chapter 8 describes control selection at the beginning or end of the follow-up period so that the controls may be used to evaluate various types of outcomes. Throughout, the reader is shown how the probability of sampling the controls is an integral part of the likelihood function. While most of the discussion is based on proportional hazards regression, there is also a brief discussion of additive hazard and linear hazard models. Estimation of absolute risk is also discussed.

Chapter 9 is devoted entirely to issues surrounding measurement error and misclassification of exposures and outcomes. After describing these issues for binary exposure, regression calibration methods are described for measurement errors associated with continuous exposures. Here, the reader is also introduced to the need for external or validation data to obtain insights into the underlying error so that appropriate correction can be made to the odds ratio estimates. Other correction approaches based on missing data methods are also noted.

The concluding Chapter 10 is devoted to methods for conducting a synthesis of results across multiple strata or multiple studies. Both exact analysis and a random effects framework are introduced to obtain an odds ratio estimate by combining the results obtained from multiple studies. Here, the reader is also introduced to different analytical possibilities when individual level data are available from multiple studies.

The book by Breslow and Day (1980) provides a definitive account of the fundamentals of the classic unmatched and matched case–control designs, including estimation of odds ratios and standard errors and power calculations. In contrast, this book focuses on a variety of design issues and more recent developments for the statistician. This is a useful guide for graduate students and established statisticians pursuing research on the design and analysis of case–control studies. It will also be a useful resource for statistical researchers intending to provide guidance to epidemiologists and clinical practitioners on ways to identify risk factors related to an outcome by conducting a case–control study.

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Reference

Breslow, N.E. & Day, N.E. (1980). *Statistical Methods in Cancer Research: Volume 1 – The Analysis of Case–Control Studies.* Lyons: International Agency for Research on Cancer.

Introduction to High-dimensional Statistics
Christophe Giraud
Chapman and Hall/CRC, 2015, xv + 255 pages, £44.99, hardcover
ISBN: 978-1-4822-3794-8

Readership: Statistics graduate students and researchers, as well as practitioners.

This is an attractive textbook. It will prove a very useful addition to any library or personal reference collection.

Technological advancements over the past 20 years or so have resulted in the proliferation of high-throughput datasets in many fields, such as biotechnology, finance and astrophysics. The data produced by such technology are said to be *high dimensional* and give rise to the need for new statistical thinking and methodology. This book achieves well what it sets out to provide, an introduction to the mathematical foundations of high-dimensional statistics. It is targeted at advanced students and researchers seeking an introduction to the area and to the key mathematics involved. It is not intended as a comprehensive account of contemporary statistical methods for analysis of high-dimensional data and, as such, is likely to stand the test of time well.

Formally, a statistical model is said to be high-dimensional if its number of parameters p grows with the number of observations n. Conventional statistical theory describes the asymptotic behaviour of procedures for data analysis when n goes to infinity with p fixed, which now makes no sense. A different paradigm is required, to provide a theory that is valid for any value of n and p. This is achieved by replacement of the convergence theorems used in conventional statistics by more elaborate technical arguments based on ‘concentration

inequalities', which are the central tools for the non-asymptotic analysis of statistical methods in the high-dimensional setting.

Chapter 1 of the text provides a very lucid and accessible introduction to the key ideas and mathematical concepts involved in the analysis of high-dimensional data. To overcome the difficulties imposed by high dimensionality, the key is to exploit low-dimensional structure hidden in data, the saving assumption of sparsity that many of the p parameters might be negligible in magnitude relative to the others. Model selection presents the theory for tackling identification of low-dimensional structure. Chapter 2 considers the key setting of the Gaussian regression model. The general idea is to compare different statistical models, corresponding to different hidden structures, and then select the one most suited for the given data. An alternative to model selection, the idea of model aggregation instead of selecting one from among them, is described in Chapter 3. Both model selection and model aggregation are computationally prohibitive in many settings. Chapter 4 discusses the powerful strategy that has been successfully applied to circumvent computational difficulties, that of deriving estimators by efficient minimisation of a convenient convex criterion: the Lasso estimator is the most widely celebrated example of such a procedure. The operational difficulty with this strategy is that some sort of selection of the estimator to be applied, and also typically choice of some tuning parameter, is required. Chapter 5 provides a thorough account of how to decide among different estimation schemes and different tuning parameters.

Chapters 6 and 7 crank up the levels of statistical sophistication. Chapter 6 considers multivariate regression, with special focus on the situation where measurements lie in the vicinity of some (unknown) low-dimensional space. The essential notion is that correlations between statistical problems can be exploited to improve the accuracy of statistical procedures. Chapter 7 focuses on the specific problem of simultaneous estimation of the conditional dependencies among a large set of variables, using the theory of graphical models.

The final two chapters consider the issues arising in high-dimensional data classification. Chapter 8 is concerned with the mathematical foundations of procedures for multiple testing, with special consideration of control of the false discovery rate. The theory and key methods of supervised classification are discussed in Chapter 9. A very well-structured and clear series of mathematical appendices conclude a very substantial and authoritative account. Each chapter contains detailed exercises, with collaborative solutions on a wiki-site, although at the time of writing, this review contents of the site are rather sparse.

This is a dense text, which demands close study: it is not necessarily a book for dipping into. However, the technicalities are presented cleanly and comprehensibly. Simple but clear practical illustrations are effective and prevent the treatment from becoming dry. A natural comparison can be drawn with the textbook of Bühlmann and van de Geer (2011), which covers much the same ground, in an equally authoritative and accessible manner, and which, rather surprisingly, is not referenced here.

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Reference

Bühlmann, P. & van de Geer, S. (2011). *Statistics for High-Dimensional Data: Methods, Theory and Applications.* Springer.

Growth Curve Modelling: Theory and Applications
Michael J. Panik
Wiley, 2014, 454 pages, £89.50, hardcover
ISBN: 978-1-118-76404-6

Readership: Statistics graduate and advanced undergraduate students, as well as practitioners in biological sciences, economics, fishery management and public health who have a basic knowledge of probability and statistics.

The marine environment has undergone, and continues to undergo, major changes with respect to biodiversity, food-web structure and multispecies interactions. The availability of food for marine species continues to be an important determinant in species' mortality, species' range (both temporal and spatial) and ensuring food security for human populations.

The need for understanding the potential drivers of growth in marine species in terms of species abundance, body weight and numbers of organisms has motivated modellers for many years (O'Brien & Little, 2006). Many mathematical models have been developed and applied over the years to describe the processes of growth. However, a definitive text on the derivation, fitting and identification of suitable models to use in real-world applications has been missing in a single source. In this book, the author addresses these issues in a comprehensive and easily accessible way, providing a research resource that I am confident will be referred to for many years to come.

The most popular and often encountered growth curve models (linear, logarithmic reciprocal, logistic, Gompertz, Weibull, negative exponential, von Bertalanffy and Michaelis–Menten) are presented, along with some lesser known models (Chapman and Richards, generalised Ricker, generalised Beverton–Holt and Schnute).

Besides the author's detailed discussions of growth modelling and theory, the text provides SAS codes to illustrate the fitting of models and parameter estimation. Additionally, the author considers the detection and estimation of autocorrelated errors. One area the book does not appear to address is the important topic of model diagnostics–something to be considered in a second edition. The book will appeal to students and researchers alike, providing an excellent course text for anyone interested in developing an understanding of the variety and complexity of growth measurement techniques. The text will challenge students and provide the most able with a sound foundation from which to begin their own research.

Interesting theoretical properties of the models aside, it is important to understand how best to use such models for prediction and in decision-making. Fishery management, for example, has many applications of growth models and has been a rich area for model development, together with the need to provide practical solutions to the setting of catch limits and fishing quotas (Needle *et al*., 2003).

If you buy just one book this year, then make it this one; it is rare to find such an engrossing text from the first to last page!

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O'Brien, C.M. & Little, A.S. (2006). *Incorporation of Process Information into Stock-recruitment models.* ICES Cooperative Research Report, No. 282, 152.

Introduction to Probability
Joseph K. Blitzstein, Jessica Hwang
Chapman and Hall/CRC, 2015, xv + 580 pages, £49.99, hardcover
ISBN: 978-1-4665-7557-8

Readership: Beginning undergraduates.

Probability theory is one subject for which the once dire scarcity of good text books has now been supplanted by a plenty. The book under review, meant to serve as a first course for undergraduates, is a welcome addition to the set.

Borne out of an introductory course at Harvard University, the book quite deliberately pitches its ambition regarding spread not too high. The authors–wisely, in this reviewer's opinion–take special care to maintain a conversational tone to prioritise accessibility instead. The result is a very readable text with concepts introduced with a degree of clarity that should suit the beginner extremely well. However, this is not to say that non-essential 'additional' topics have been given a complete go by; in fact, three of them are dedicated full chapters: Markov chains, MCMC simulations and Poisson processes. Quite a few concepts are touched upon in the huge body of exercises: an extremely frugal partial list includes topics such as the Monty Hall problem, the bubble-sort algorithm, inverse sampling, the ROC curve, records, rudiments of estimation theory in both the Bayesian and non-Bayesian settings, non-homogeneous Poisson and Cox processes. Thus, coverage is indeed, in the ultimate analysis, considerable as well.

After the introductory chapter mostly on combinatorial probability followed by the axiomatic definition and its consequences, the authors devote a full chapter to conditional probabilities. This is followed in Chapter 3 by random variables and their distributions, but only discrete ones are considered here. The major univariate discrete distributions and their properties and some interconnections are treated in detail. The following chapter develops expectations for discrete random variables, going on to cover the means and variances of major discrete distributions. Only in the next are continuous random variables and distributions introduced.

Chapter 6 on moments goes over the concepts of skewness, kurtosis and so forth, followed by definitions and properties of generating functions like the moment generating function (mgf) and the probability generating function (pgf). The characterisation property of the mgf is stated without proof. Chapter 7 is on joint distributions, encompassing marginals, conditionals and dependence structures. Expectedly, the multinomial and multinormal distributions carry special emphasis here. The chapter is followed by one on transformations of random variables where change of variables is covered and interconnections between continuous distributions derived. Applications to order statistics follow. Chapter 9 is devoted to conditional expectations in the two cases separately, while the next chapter discusses the common inequalities followed by the standard limit theorems in their simplest settings.

The final three chapters pertain to finite Markov chains (MC), Markov chain Monte Carlo methods and Poisson processes, respectively. The ergodic theorem for MCs is stated without proof. An interesting feature is the inclusion of some discussion on reversibility, possibly with a view to the next chapter on MCMC, in which the two most popular MCMC methods, namely, the Metropolis–Hastings algorithm and the Gibbs sampler, are described.

Two appendices, one each on basic mathematical details and the R software, bring up the end.

Evidently, considerable thought has gone into the choices of topics to include and to leave out. It is easy to carp at omissions, for example. some mention of random variables that are neither discrete nor continuous, which do arise in Statistics; some modicum of geometric probabilities; characteristics functions; or a more detailed description of certain Markov chains like

branching chains, where personal taste is certain to enter. Even so, at nearly 600 pages, this is certainly no puny effort. On the other hand, quite a large number of interesting topics that might be considered to some extent exotic in a probability text have been touched upon in gist, such as Shannon's entropy in information theory, the PageRank measure sitting underneath the Google™ web search engine and Bayesian inference. The organisation of material is excellent too.

The spirit of easy accessibility has animated many of the proofs, where verbal common-sense reasoning conveyed in a conversational tone has often scored over mathematical rigour. In fact, the easy tone makes this book very suitable for self-learning as well.

An additional feature is the extensive use, and related instruction, of the R programming language for computations, simulations, approximations and so forth. While one should sound the caution that the book contains by no means a complete compendium of, or even a complete introduction to, all capabilities of R, what is discussed here is a pretty large set of R commands and libraries that students will find themselves continually using should they pursue studies in Statistics.

Quibbles are minor. A couple of them concern Chapter 5. For one, the definition of continuous random variables, while simplifying some arguments, leaves out many examples owing to the demand of differentiability of the cumulative distribution function everywhere. Next, in this reviewer's opinion, there is nothing to be gained by avoiding the use of the word exchangeability and calling this property simply symmetry, a word that has a different standard connotation. The introduction to the short discussion of linear regression in Chapter 9 could be somewhat confusing. But to reiterate, these shortcomings are quite minor compared with the many qualities of the book as an introductory text.

It needs reemphasising that the numerous problems are not just appendages meant for optional practice but also painstakingly develop many ideas in detail. Solving as many as possible will stand readers who pursue Statistics in good stead during learning quite a few topics subsequently. The carefully crafted attractive setting and language of many, like the ones analysing Shakespeare's vocabulary or games of chutes and ladders, should also kindle particular interest among early undergraduate learners.

In conclusion, beginning students opting for easy-paced learning will find the book highly suited to the purpose, while those desirous of greater depth may refer to such texts that give more stress to rigour in proofs and other mathematical niceties.

An e-book version of the book is available upon creating an account with the website vitalsource.com and redeeming a code provided with every print copy.

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Bayesian Methods for Management and Business–Pragmatic Solutions for Real Problems
Eugene D. Hahn
John Wiley and Sons, 2014, xv + 384 pages, €100.20, hardcover
ISBN: 978-1-118-63755-5

Readership: Advanced MBA students, business and management PhD candidates, undergraduate mathematics students and researchers in economics.

The interest in Bayesian statistics has been on a steady growth in the last decade or so. This was mainly attributed to two aspects of Bayesian methodology in the context of data analysis: relative methodological simplicity and new computational tools available through applications of the Monte Carlo Markov chain technique. This growth sparked a renewed interest in statistical methods among practitioners and researchers in many applied areas of research, in particular, in social science and economics. Traditionally and mostly because of historical reasons, a university course on Bayes methods requires as a prerequisite a course in frequentist statistical inference. Because some fundamentals in the probability theory are also necessary, courses in Bayesian statistics are often pushed further to more advanced stages in the university education. For this reason, available textbooks on the topic frequently assume that students already have a solid grasp of basic concepts of statistics. However, teaching Bayesian statistics can be carried out in parallel to standard statistics courses or even independently of them. This placement of a Bayesian, while benefiting those who are more interested in Bayesian statistics and not so much in the frequentist approach, calls for a textbook that explains concepts of statistical data analysis along with the Bayesian 'philosophy'.

In many respects, *Bayesian Methods for Management and Business* can be utilised as a textbook for the first course in Bayesian methods without prerequisites in statistics but with some fundamentals in probability. It has two prominent features that distinguish it from other comparable textbooks on the topic such as Hoff (2009) or Jackman (2009). Firstly, it has fully integrated R and WinBugs packages to assist a student in the learning process. This applies to the book as a whole, not just to Bayesian computing sections of the presented material. Secondly, a quite extensive set of empirical examples have been drawn from business and management contexts. Through these examples, the book convincingly demonstrates that Bayesian statistics can provide insights into the important issues facing business and management. The book also shows the integration of Bayesian statistics into data-rich environments, which is a very rapidly growing trend in data analysis.

The text covers a fairly broad range of topics: linear models, sensitivity analysis, Markov chain Monte Carlo, model comparison, hierarchical models, generalised linear models and latent variable. The topics are presented in an accessible manner although often at the cost of depth of coverage. A practical problem-solving approach is attractive for students, but the graphical presentation of the material is somewhat 'dr', and this could have some negative impact on its appeal to less advanced undergraduate students.

To summarise, the book should be very useful for management scholars and practitioners as well as business students who seek to acquire or broaden their methodological skill set for statistical analysis of complex dataset.

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Sample Size Calculations for Clustered and Longitudinal Outcomes in Clinical Research
Chul Ahn, Moonseong Heo, Song Zhang
Chapman and Hall/CRC, 2015, xv + 244 pages, £57.99, hardback
ISBN: 923-0-4651-9855-7

Readership: Statistics graduate and advanced undergraduate students, as well as practitioners.

'How many subjects do I need' is probably the most commonly asked question to the statistician. The researcher may not realise at the time that this is the invitation for the statistician to ask about what they really care about: study design, primary endpoints and proposed analyses. This books focuses on sample size estimation for diverse study designs and endpoints. Relatively little attention is paid, however, to the appropriateness of a design for a particular research question. While the subject matter may seem a little dry, this book is an excellent resource for both statisticians and practitioners undertaking prospective studies in human trials. The authors emphasise that sample size justifications should be simple, but design-specific. They encourage the use of closed-form expressions for sample size calculations, whether the study has a simple design or it is longitudinal or it uses clustered randomisation. Interestingly, they also consider complex designs where dropouts are anticipated, emphasising that blunt calculations based upon an overall dropout rate estimate may be too conservative.

The early chapters set the foundations nicely, so that even if one did not read beyond the first chapter, the essential information for a power analysis is explicit and clearly presented. Each subsequent chapter introduces another design twist that impacts the effect size. The reader is soon drawn into the overall pattern of thinking: identify the inferential statistic and estimate the expected effect size using assumptions of the first two moments. It is nearly always the variance estimate that poses the most challenges.

Their thorough exploration of sample size estimation in longitudinal and clustered randomised trials leads them to consider interesting questions. For example, if the assumed dropout rate can be fully specified using, for example, the marginal probability of missingness at each time point along with the joint probability of missingness at multiple time points for a patient, then one can derive a closed-form expression of the necessary sample size. The assumed correlation structure between time points within patients can be shown to interact with the assumed missing pattern, in interesting but expected ways.

The relationship between numbers of measurements per subject and number of subjects turns out also to be complicated. As a general rule, more measurements per subject reduce the number of subjects needed, but that relationship depends upon intra-subject correlation as well as the summary statistic being compared.

If I were writing the book, I would remind the reader that sample size calculations are very non-robust to deviations from assumptions. It is rare that the researcher has good information on variance, on interclass correlation or on the correlation between repeated measurements. Misspecifying the intraclass correlation has the largest impact on studies with large cluster sizes. 'Sample size calculation' should probably be termed sample size 'justification'. It provides a demonstration that the researcher has carefully considered all aspects of the study: from the study design and the endpoints to the anticipated attrition and the statistical analysis. A good sample size design makes research plan transparent not only to a reviewer but also to the researchers themselves.

A brief overview of the six chapters:

(1) *Sample size determination for independent observations*: This a revision of well-known results. It has a good chapter for people unfamiliar with the framework behind sample size calculations. It covers sample size estimation for one and two sample tests for comparing means (continuous outcome) with proportions. Equivalence studies as well as superiority studies are also considered.

(2) *Sample size determination for clustered outcomes*: This chapter picks up on the themes of the first chapter but considers the situation of clustered outcomes where the variance structure is slightly more complex. Assumptions about between-cluster and within-cluster variance are relevant to sample size determination. One-sample and two-sample tests for means and proportions are again considered, along with equal and unequal cluster sizes.
(3) *Sample size determination for repeated measurement outcomes using summary statistics* : This chapter introduces the additional complexity of repeated measures designs. The primary outcome of interest may be the rate of change or an average across time points. The impact of the assumed intra-patient correlation structure on the sample size calculation and common correlation matrices is discussed. This chapter also includes a section on how assumptions about attrition and missingness can be incorporated in determining the sample size.
(4) *Sample size determination for correlated outcome measurements using GEE:*: I found this to be the more interesting and also the most difficult chapter. The main ideas fit into the theme of identifying the treatment effect of interest (e.g. an average effect or an interaction) and finding the correct variance term. But they also explored the impact of the choice of correlation structure and the trade-off between increasing the number of subjects and increasing the numbers of visits per subject. Financial constraints are also considered, which may impact on the visits versus subjects trade-off.
(5) *Sample size determination for correlated outcomes from two-level randomised clinical trials*: This chapter reviews material from Chapters 2 and 3 and makes the important point about identifying the correct error term for inference. This reader would have preferred to see the ideas from this chapter integrated into the earlier chapters.
(6) *Sample size determination for correlated outcomes from three-level randomised clinical trials*: This chapter is for the die-hard practitioner who wants to show how well he or she understands variance components. To obtain the right mathematical formulation, the practitioner will need to assume variance components for between-level three units and between-level two units/within-level three units. While the authors focus on acquiring the sample size calculation correct, I would be more concerned about the consequences of misspecifying the variance structure and obtaining the right answer to the wrong set of assumptions.

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