

# Biased Sampling, Over-Identified Parameter Problems and Beyond

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**Biased Sampling, Over-Identified Parameter Problems** and Beyond. Jing Qin. Singapore: Springer, 2017, xvi + 624 pp., \$159.99(H), ISBN: 978-9-81-104854-8.

The biased sampling mechanism is commonly encountered when the subjects in a target population are not given equal chances to be selected to a study, either accidentally by natural circumstances or intentionally by design. Although there is a substantial amount of literature on statistical methods for data subject to biased sampling, this is to my knowledge the first book to systematically review classical and up-to-date methods that properly account for biased sampling. The importance of the over-identified parameter problem has been well recognized in statistics and economics and has recently received a lot of attention when involving auxiliary information. This book is the first comprehensive overview of which I am aware that shows how statistical methods such as empirical likelihood and generalized method of moments can be appropriately and efficiently used in the over-identified parameter problem.

The book elegantly presents both basic and advanced issues, with numerous examples to help readers understand the methodology. It will be greatly beneficial for graduate students and junior researchers who are interested in the field of biased sampling and over-identified parameter problems. Most materials are presented at a level that can be understood by graduate students and researchers outside the advanced audience of top statistical journals. Readers should be aware that this is not a theorem-proof book, but a book focusing on statistical reasoning. The book's main contribution is to engage readers in a discussion of the use and development of statistical tools to tackle challenging problems arising from observed data.

Because of the wide array of related topics, this book consists of 26 cohesive but relatively independent chapters. Although a few chapters reference the preceding chapter, most chapters can be read out of order as self-contained. The first four chapters provide background information, including an introduction to general biased sampling with applications and reviews of the renewal process and parametric likelihood inference. In particular, Chapter 3 presents 18 excellent motivating examples from a wide range of applications. Those examples are referenced in subsequent chapters that describe how statistical methods can appropriately adjust for sampling biases. Chapter 4 introduces a wide variety of issues involved in classical maximum likelihood estimation, such as irregular cases, and covers other likelihoods, including profile and composite likelihoods. Chapters 5-9 review five statistical estimation and inferential methods: the optimal estimating function theorem, projection methods, the generalized method of moments, the empirical likelihood, and the Kullback-Leibler likelihood. After introducing the main concepts and theorems, these chapters describe how to apply these methods to account for biased sampling and over-identified parameters.

Chapters 10-23 primarily focus on statistical methods for different settings that are subject to biased sampling, which are initially introduced in Chapter 3 as motivating examples. Along this main line, the author also reviews some popular models and methods, including the maximum rank score and maximum rank correlation estimation. Chapter 10 is devoted to nonparametric distribution estimation for data subject to biased sampling with known or unknown weight functions. Chapters 11-14 mainly consider methods and theorems for casecontrol studies, and Chapter 15 presents methods for outcomedependent sampling with continuous outcomes. I particularly enjoyed reading Chapter 11, in which several methods are presented from different perspectives to derive the semiparametric maximum likelihood estimator given data from the case-control study. This significantly helps the reader to fully understand why the prospective likelihood can be used to estimate the odds ratio parameter. Chapter 16 reviews the noncentral hypergeometric and Poisson binomial models for two-by-two tables, which are often encountered in biomedical and epidemiological studies. Chapter 17 covers inference in semiparametric finite mixture models, in which only the density ratio model is assumed on the connection between different components to relax strong assumptions of parametric mixture models. Again, several examples in a wide range of applications are included in the chapter, which should certainly stimulate the reader's research interest. Chapter 18, one of my favorite chapters, nicely summarizes the connections among marginal, conditional, and empirical likelihoods. The author has earned his stripes in the field of likelihood-based inference, and provides illustrating examples and insightful discussions that are not easily found elsewhere.

Chapter 19 focuses on data that are missing at random, and Chapter 22 covers data not missing at random. Although a lot of books cover the issue of missing data, these two chapters offer original viewpoints that make them worthwhile additions to the literature. In Chapter 19, several methods are introduced to estimate the causal effects in causal inference as special cases of missing data. These include classical methods such as propensity score matching, and newly developed methods such as empirical likelihood-based imputation. For nonignorable missing data, Chapter 22 introduces parametric and semiparametric approaches. Conditional, profile, pseudo-, and empirical likelihoods are presented under different applications with missing data. In particular, the call back and Heckman's sample section models are discussed in detail through the use of different methods with several generalizations. Chapter 20 discusses the use of auxiliary information with a finite population in survey research. In earlier chapters (Chapters 10, 11, 17, and 18), the density ratio model with categorical covariates is introduced under different settings. To present a thorough review of the density ratio model, Chapter 21 covers the generalized density ratio model, including continuous covariates with two estimation methods, the pairwise conditional likelihood, and profile likelihood. This chapter also introduces two important applications in which the density ratio model is applied for testing the conditional independence assumption and handling highdimensional data. Chapter 23 discusses the biased sampling issue for data collected from capture-recapture experiments and inferential methods to estimate the population size with and without covariate information.

Chapters 24 and 25 deal with survival data. Although Chapter 24 is a review chapter, the contents and focus are quite different from those of traditional books about survival data analysis. Researchers who are new to survival data analysis are advised

to obtain basic knowledge from other books, such as that of Klein and Moeschberger (2005), before reading this chapter. One focus of this chapter is to apply several methods from different viewpoints to derive some popular estimators, providing deep insight into the estimators. For example, the Kaplan-Meier estimator for right-censored data is derived by three methods from different perspectives: nonparametric likelihood, imputation, and inverse probability weighting. Such a presentation greatly helps the reader to generalize the Kaplan-Meier estimator for other more complicated cases, such as length-biased right-censored data. For the commonly used semiparametric regression models, readers are shown how to estimate the regression parameters by using different methods. Using the Cox regression model as an example, at least five approaches, including the rank-based likelihood and projection methods, are presented for estimating the regression coefficients. Chapter 25 is devoted to the topic of length-biased right-censored data. Starting with the pioneering work by Vardi, this chapter reviews the current state of methodological development for statistical estimation and inference on nonparametric estimation and semiparametric modeling for length-biased right-censored data. These two chapters focus on statistical methods with heuristic arguments rather than theoretic proofs, such that readers without strong mathematical backgrounds will find them accessible. Readers who are interested in technical details, including asymptotic properties, are referred to specific references cited in these chapters.

The last chapter focuses on applications of isotonic regression and the pool-adjacent-violators algorithm (PAVA) and is one of the most interesting chapters in the book. It shows how to transfer some seemingly unrelated statistical problems to the isotonic regression framework, so that PAVA can be used to solve these otherwise computationally challenging problems. One illustrating example is to connect the transformation model for the current status data and isotonic regression. Given such a connection, PAVA paired with the EM algorithm provides a powerful computational tool for estimation. This chapter may motivate readers to explore the possibility of applying isotonic regression and PAVA in other applications.

It is certainly a great challenge to write a comprehensive book on biased sampling problems, over-identified parameter estimation, and other related topics because such topics have evolved significantly over many, diverse applications. In most chapters, however, the author starts with an introduction to the statistical concepts and methods and then illustrates how such statistical tools can be used in related applications. This book should shorten the substantial learning curve for younger researchers who are interested in working in this area and should motivate them to continue the work of developing novel approaches. With its extensive exercises and easy style, this book is suitable as an upper-level textbook for graduate students or as a reference book for workshops that target postdoctoral fellows and junior researchers. The format of this book makes it easy to jump into later chapters based on the reader's research interest without back-pedaling, which makes the book a very useful reference source. As a researcher already involved in biased sampling problems, I enjoyed reading the author's insightful discussions of methods and anticipate returning to the book many times as a reference source.

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**Elements of Nonlinear Time Series Analysis and Forecasting.** Jan G. De Gooijer. Switzerland: Springer International Publishing, 2017, xxi + 618 pp., \$159.99(H), ISBN: 978-3-31-943251-9.

This is an excellent addition to the library of books on time series analysis. The most attractive feature of this book is that it places importance on developing intuition about nonlinear time series rather than the more formal theorem-proof approach. It is abundant with data examples and simulations that enhance understanding of the stochastic properties of the models. In my opinion, the approach taken is the best pedagogical technique to learn about time series models. This book gives realizations from many models: bilinear (BL), nonlinear moving average (NLMA), self-exciting threshold autoregressive moving average (SETARMA), and so on. Visualizing these realizations is helpful to differentiate features and thus build intuition for choosing an appropriate class of models for particular s. The book is self-contained with pseudo-codes and theory and thus gives a multilayer approach to presenting the material, as technical details are placed at the end of each section to serve those readers with the need for formalities.

This book is an ideal resource for a serious student of nonlinear time series and even for a researcher with a good background on linear time series but with very little or no exposure to nonlinear time series. Its level and treatment of the subject is suitable for graduate students in statistics, econometrics, electrical engineering, and applied mathematics who have had proper coursework on mathematical statistics, linear time series, and regression analysis. The coverage of the book is comprehensive: classical nonlinear models; tests of linearity (in both spectral and time domain); model estimation and diagnostics; forecasting (semiparametric, nonparametric, and approximate methods); vector-valued nonlinear time series. The organization of each chapter gives the reader the freedom to explore the topics at various depths. The main body for each chapter gives a big picture exposition of the main ideas (e.g., models, tests, assumptions, methods) and thus attempts to build intuition, with in-depth formalities and theory provided at the end of each section. Any reader who is not familiar with the lay of the land of nonlinear time series will appreciate the summary of terminology and acronyms for the very large number of models. Each section also provides additional references with brief descriptions that will be helpful for digging deeper into the technical details.

I can see how this book can serve the needs of a semesterlong course on nonlinear time series that follows after a classic course on linear time series covering general linear processes, ARIMA models, GARCH models, spectral analysis, and non-stationary time series. For this course on nonlinear time series (14 weeks × 3 hr per week), I would cover Chapter 1 (Introduction with many data examples), Chapter 2 (Classical Nonlinear Models), Chapter 3 (Probabilistic Properties), Chapter 4 (Spectral Tests of Linearity), Chapter 5 (Time Domain Tests of Linearity), Chapter 6 (Model Estimation, Selection and Diagnostics), Chapters 9 and 10 (Forecasting), and Chapter 11 (Multivariate Time Series). I would assign three or four problem sets based on the theory and data analytic problems provided at the end of each section. I would also require an individual article that covers in-depth data analysis using the various models presented or in-depth simulation studies on the various tests and methods.

The book comes with a solutions manual for the instructor, which helps in organizing and preparing for a course. One weak point about this book is that the delivery of the codes could be made to be more friendly for the instructor. A good template would be the one provided in the book *Time Series Analysis and Its Applications: With R Examples* (4th ed.) by Shumway and Stoffer (http://www.stat.pitt.edu/stoffer/tsa4/). Moreover, this book could be improved in the future by adding a special section that compares and contrasts the different classical models in Chapter 2.

I would recommend this book to any scholar who is about to embark on serious research in nonlinear time series, along with (1) Nonlinear and Nonstationary Time Series Analysis by Priestley; (2) Nonlinear Time Series: Theory, Methods and Applications with R Examples by Douc, Moulines, and Stoffer; (3) Nonlinear Time Series: Nonparametric and Parametric Methods by Fan and Yao; and (4) Threshold Models in Nonlinear Time Series Analysis by Tong.

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Handbook of Methods for Designing, Monitoring, and Analyzing Dose-Finding Trials. John O'Quigley, Alexia Iasonos, and Björn Bornkamp, eds. Boca Raton, FL: Chapman & Hall/CRC Press, 2017, xiv + 306 pp., \$129.95(H), ISBN: 978-1-49-874610-6.

In recent years, model-based methods for dose-finding have become more commonly used and there is growing interest in methods that can be applied to more complex dose-finding trials. A book offering a comprehensive overview that clearly specifies the questions novel techniques can answer would be of great utility. The aim of the *Handbook of Methods for Designing, Monitoring, and Analyzing Dose-Finding Trials*, edited by John O'Quigley, Alexia Iasonos, and Björn Bornkamp, is to provide such an overview.

The editors have assembled a stellar group of authors, most of whom are leading experts in the field, to provide an overall picture of various methods that are useful when embarking on a trial to identify the correct dose for use in a further study. Given the enormous number of papers in this field, a book summarizing the most recent developments that can be used as the starting point for young researchers or as a reference for established researchers would be tremendously useful. Furthermore, a hands-on guide illustrating how to apply novel dose-finding methods would be invaluable. Unfortunately, we found the *Handbook of Methods for Designing, Monitoring, and Analyzing Dose-Finding Trials* to be uneven, achieving these needs in some chapters of the book but not in others.

The first part of the book, "Phase I designs," provides an overview of methods for single agent trials covering binary, ordinal, and time-to-event endpoints. The overview includes methods that become the basis for further developments described later and upon which more complex approaches are constructed. The first chapter also provides an extensive list of useful statistical software that is available to the reader and also provides references for studies comparing different dose-finding methods. A similar provision of relevant software and comparison studies would have been a useful addition to the remaining chapters in the first two parts of the book.

The second part of the book introduces more advanced dose-finding designs for clinical trials of increased complexity. Chapter 5 provides methods for evaluating both toxicity and efficacy of cytotoxic drugs simultaneously. The authors provide step-by-step guidelines for dealing with particular challenges in Phase I/II trials such as delayed responses or missing data. While the overview of the methods is extensive, it is not clear to what extent they are suitable for cytostatic drugs: Wages and Tait (2015) and Riviere, Dubois, and Zohar (2016) have argued that specialized methods for dose-finding are necessary for these studies. Chapter 6 describes dose-finding designs for dual combination trials. The authors indicate that there is a large number of model-based designs for the considered setting, provide an extensive list, and focus on the nonparametric Bayesian optimal interval (BOIN) design. Model-based designs are not discussed here in detail, so we believe that a reader of Chapter 6 would benefit from supplementing their study with the more comprehensive comparison provided by Riviere, Dubois, and Zohar (2015).

The subsequent chapters discuss the very flexible partial ordering continual reassessment method (POCRM) and its application to dose-schedule finding studies. In addition, these chapters discuss the crucial problem of heterogeneity of patients and introduce the nonparametric optimal benchmark: a useful tool to evaluate the performance of novel designs. Since this book has been published, Wages and Varhegyi (2017) have released a user-friendly implementation of the benchmark that



the reader might want to consider using. Chapter 10 provides the reader with experiences of practical implementations of the continual reassessment method (CRM, O'Quigley, Pepe, and Fisher 1990). The authors consider several aspects of the design and how they can be tailored to the needs of the trials. We believe that, given its title, the focus of this chapter should have been a bit broader than the specifics of the CRM and should have considered additional practical challenges in dose-finding studies (e.g., decision-making procedure, time required for analysis, etc.).

Given the title of the book, we were surprised to discover that no methods for phase I trials outside of oncology have been discussed in the first two parts of the book. We also found a notable absence of any methods based on a two-parameter logistic regression model (e.g., Whitehead and Williamson 1998). Although Chapter 10 claims that it "does not provide any benefits," methods based on a two-parameter model are widely used in practice and the statement about the lack of potential benefits is controversial at best. In particular, the Bayesian logistic regression method (Neuenschwander, Branson, and Gsponer 2008) is one of the most commonly used designs in the pharmaceutical industry, and providing it would give the reader a taste of currently employed methods. While we appreciate that it is impossible to cover every available method in a single book, in our opinion this omission limits the utility of the book.

The final part of the book provides a detailed description of Phase II dose-finding methods and we found this part particularly well constructed and a pleasure to read. Most of the chapters in this part include a step-by-step guide to applying these methods using the R package DoseFinding (Bornkamp, Pinheiro, and Bretz 2018), including the code necessary to implement the described designs. Additionally, the same motivating examples are used from chapter to chapter, which helps tremendously to keep the reader focused. These two features allow an investigator to conduct the initial simulation analysis for the planned clinical trial by the means provided in the book only. If the book had followed the same structure throughout, it would have become indispensable for any group undertaking early phase dose-finding studies.

Overall, we think that this book fills an important niche: it provides a good overview of novel dose-finding techniques that are motivated by practical clinical needs and at the same time relates these designs to applications in various stages of dose-finding. We identified some shortcomings, and the chapters are somewhat uneven in many respects: inconsistent reference styles, different presentational approaches, even different intended audiences. Still, the book proves to be a well written, engaging, and useful reference with plenty of motivating clinical trials and illustrations.

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# Statistical Analysis with Measurement Error or Misclassification: Strategy, Method, and Application.

Grace Y. Yi. New York, NY: Springer, 2017, xxvii + 479 pp., \$139.99(H), ISBN: 978-1-49-396638-7.

This book covers a wide range of topics in a unified framework where measurement error and misclassification problems receive careful treatments, from both practical and theoretical points of view. In particular, the author zooms in on five research fields that are popular playgrounds, especially among biostatisticians and epidemiologists: survival analysis, recurrent event data analysis, longitudinal data analysis, multi-state models, and case–control studies. These constitute Chapters 3 to 7 of the book, with more coverage on mismeasured covariates than on mismeasured responses.

Before starting this central part of the book, the author reviews widely applicable estimation methods in general contexts, without the complication of measurement error. Other concepts and issues to be revisited frequently in later chapters, such as identifiability, model misspecification, and asymptotics, are also briefly reviewed in Chapter 1. In Chapter 2, the author clarifies the types of measurement error to be considered, presents commonly entertained models for them, and outlines general strategies for taking measurement error into account when drawing inference. The first two chapters prepare one well for the methodology development in specific contexts in follow-up chapters. For more technically engaged readers, these reviews can quickly set them on the same page as the author in terms of notational conventions, and the backdrop of measurement error problems in general. Readers less familiar with parts of the background information can gain some intuition of key ideas conveyed in these reviews. For example, in Section 1.2.3, the convergence rate of an estimator is described as a "magnifier," by which one multiplies the difference between the estimator and the truth so that the resultant scaled difference follows a nondegenerate asymptotic distribution. This analogy

of convergence rates is intuitive enough to connect with readers at various levels of previous exposure to large sample theories.

After considering mostly mismeasured covariates in Chapters 3 to 7, the author devotes Chapter 8 to considerations of mismeasured responses, with error-free or error-prone covariates. Finally, a list of important topics in measurement error literature not systematically covered in this book is given in Chapter 9, along with a brief survey of existing works on each topic. Evidently, the author has a wide command of the literature on measurement error. Besides the survey of literature in Chapter 9, a good collection of existing works most relevant to materials discussed is available in each chapter in the section of Bibliographic Notes and Discussions. Readers who wish to try their wings in the field of measurement error may be able to find open research problems in these sections, besides Chapter 9.

In Chapters 3 to 7, five specific research fields are set as the stages for measurement error problems. In each chapter, the author follows a similar road map to present materials. Each of these five chapters begins with a succinct introduction of the research branch considered in that chapter, without measurement error in the picture just yet. These introductions are necessary for one to consider properties of naive inference based on error-prone data, that is, inference that ignores measurement error. Moreover, each introduction is an excellent read for someone unfamiliar with the corresponding research area. Following the introduction is investigation of the effects of measurement error. Then several inference methods adequately addressing measurement error and yielding consistent estimators are chosen to present in detail. In the opening remarks of a chapter, the author makes timely remarks on important distinctions in data structure, modeling, or sampling in the current context compared to the previous chapter(s). For example, it is made crystal clear at the beginning of Chapter 7 that, in case-control studies, both measurement error mechanisms and sample schemes differ from those seen in earlier chapters. Having a uniform coherent structure of presentation in this part of the book prevents readers from losing sight of the big picture even when one is in the midst of a long derivation, which is often inevitable when elaborating a complicated method. The author skillfully offers insights following technically involved derivations, which lead one to see through to the gist of potentially complex mathematical arguments.

Across this middle part of the book, one can see the reappearance of similar approaches for studying effects of measurement error and strategies for correcting naive inference in the presence of measurement error. For instance, the expectation correction method, the insertion correction method, and the conditional score method are frequently demonstrated in different contexts to account for measurement error in parameter estimation. Even though these methods are repeatedly used, the author captures novelties or tweaking that appear in each context to address complications caused by measurement error. An example of such clever adjustments of a routinely used method is the pseudo conditional score method unfolded in Section 6.5. Besides highlighting the novelties of a particular method, the author also gives insightful summaries of methods she chose to present in greater detail, such as the conditional score method for dealing with measurement error in matched case-control studies in Section 7.5. Readers who have gone through some earlier chapters before coming to Chapter 7 are most likely to have become familiar with the rationale behind the conditional score method. The author's summary comments on this particular conditional score method on page 335 underscore the trick of using the difference-covariate vectors to construct the likelihood function, which allows one to bypass nuisance parameters. Delicate technicalities like this one can be more fully appreciated by readers with a solid background in mathematical statistics. Readers who skip parts of the technical discussions may find similar themes in methodology development repeated without recognizing certain subtle differences.

I appreciate the author's unwavering effort in answering the question, "What if one ignores measurement error?" The effects of measurement error can get complicated and sometimes counterintuitive. The author strikes a nice balance between generality and interpretability when illustrating what naive inference produces. For instance, in Section 5.2.1, naively adopting the generalized estimating equations (GEE) method to carry out marginal analysis of longitudinal data with covariate measurement error amounts to using the GEE evaluated at error-prone data. Although writing down the naive estimation equations is trivial when one has the GEE in the error-free case, properties of estimators as solutions to the naive GEE are difficult to perceive by simply comparing the naive GEE and the error-free GEE. Following some generic discussions on the naive estimation function in Section 5.2.1, the author gives Example 5.2, where the model for the longitudinal response given covariates is more concrete and simplified. Assumptions are added to this longitudinal model until the naive GEE can be solved explicitly, making the connection between the naive estimators and the truth more transparent. This connection leads to interesting and insightful findings on which parameter may or may not be affected by measurement error, and the bias direction of an estimator compromised by measurement error.

This book can serve well as a textbook for a graduate-level course on measurement error in a (bio)statistics department, unless one is more interested in the Bayesian paradigm for solving measurement error problems. Besides ample real life applications presented in the book, from which students can appreciate practical relevance of measurement error problems, the Supplementary Problems at the end of each of Chapters 1 to 8 are carefully designed to help readers get acquainted with, and sometimes dig deeper on, concepts and methods introduced in that chapter. Most of the exercises directly relate to problems considered in published articles. A diligent student (with an adequate background in mathematical statistics) working on these exercises will comprehend related publications at an advanced level. By attempting these problems, graduate students can become careful, critical, and mindful readers.

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