

Preface

The study of new medical treatments, and sequences of treatments, is inextricably linked with statistics. Without statistical estimation and inference, we are left with case studies and anecdotes and do not have a formal means of extracting meaning from noise. The types of questions that can be answered continue to be pushed forward. One direction that has seen great momentum is in the field of statistics for precision medicine, an area of medical treatment focused on the personalization of care using patient covariates, which may be demographic, clinical, or biological.

This book brings together both introductory topics and new research on statistical methods for the estimation of precision medicine, or dynamic treatment regimes. The statistical study of precision medicine with a particular focus on optimizing outcomes over several stages of treatment is less than two decades old, and the field continues to evolve rapidly. The chapters in this volume vary in their level of assumed statistical knowledge, and thus while all chapters are accessible to a wide audience of statisticians and computer scientists with a focus on machine learning, many chapters are also appropriate for epidemiologists and medical researchers with some statistical training.

Two websites may be of particular interest to precision medicine researchers. The Innovative Methods Program for Advancing Clinical Trials (IMPACT), a collaborative endeavor between researchers at the University of North Carolina at Chapel Hill, Duke University, and North Carolina State University, provides publications of the affiliated researchers, software, and notification of events relevant to precision medicine: These are available at www.siam.org/books/sa21. Software and code cited in this book will be hosted on this site, and several of the R packages for implementing methods discussed in this book are also available on the Comprehensive R Archive Network (CRAN). There are also several pages on the website of Pennsylvania State's Methodology Center, <http://methodology.psu.edu/ra>, including examples of SMART studies and code (primarily SAS) relevant to such trial designs.

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Some of the data considered in this book are publicly available. The analysis of the CATIE data are based on the limited access data sets distributed from the NIMH-supported “Clinical Antipsychotic Trials of Intervention Effectiveness in Schizophrenia” (CATIE-Sz). This is a multisite, clinical trial of persons with schizophrenia comparing the effec-

tiveness of randomly assigned medication treatment. The study was supported by US NIMH Contract N01MH90001 to the University of North Carolina at Chapel Hill. The ClinicalTrials.gov identifier is NCT00014001. Analyses of the CATIE data presented in the book reflect the views of the authors and may not reflect the opinions or views of the CATIE-Sz Study Investigators or the NIH. Analyses of STAR*D were performed using the limited access datasets distributed from the NIMH-supported “Sequenced Treatment Alternatives to Relieve Depression” (STAR*D) study. The study was supported by NIMH Contract N01MH90003 to the University of Texas Southwestern Medical Center. The ClinicalTrials.gov identifier is NCT00021528. Analyses of the STAR*D data presented in the book reflect the views of the authors and may not reflect the opinions or views of the STAR*D Study Investigators or the NIH.

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