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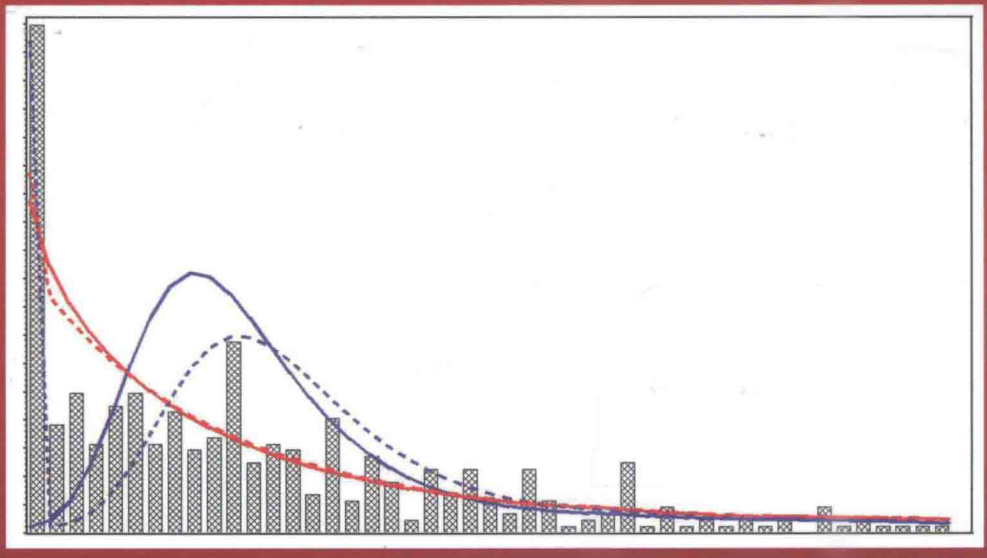

Wan Tang
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Applied Categorical and Count Data Analysis

Preface

This book focuses on statistical analysis of discrete data, including categorical and count outcomes. Discrete variables are abundant in practice, and knowledge about and ability to analyze such data is important for professionals and practitioners in a wide range of biomedical and psychosocial research areas. Although there are some excellent books on this general subject such as those by Agresti (2002, 2007), Long (1997), Long and Freese (2006), and Stokes et al. (2009), a book that includes models for longitudinal data, real data examples with detailed programming codes, as well as intuitive explanations of the models and their interpretations and differences thereupon will complement the repertoire of existing texts. Motivated by the lack of such a text, we decided to write this book five years ago when preparing a graduate-level biostatistics course on this topic for students within a medical school setting at the University of Rochester. The lecture notes from which this book has evolved have been used for the course over the past five years.

In addition to the classic concepts such as contingency tables and popular topics such as logistic and Poisson regression models, as covered by most available textbooks on categorical data analysis, this book also includes many modern topics. These include models for zero modified count outcomes, longitudinal data analysis (both parametric and semiparametric), reliability analysis, and popular methods for dealing with missing values. More importantly, programming codes are provided for all the examples in the book for the four major software packages, R, SAS, SPSS, and Stata, so that when reading the examples readers can immediately put their knowledge into practice by trying out the codes with the data in the examples using the statistical packages of their choice, and/or adapt and even extend them to fit settings arising from their own studies.

We view effective learning as a process of "reverse engineering" in the sense that one develops an in-depth appreciation of a concept, model, or approach by tracing its bumble beginnings that motivate its development in the first place. With this philosophy in mind, we try to describe the basic ideas underlying each concept, model, and approach introduced in this book so that even without rigorous mathematical arguments, readers can have a good grasp of the fundamentals of the concept and methodology. For the rather technical-savvy audience, we have also included a section in Chapter 1 to review some key results on statistical inference to help facilitate the discussion and understanding of the theoretical aspects of the models and inference methods introduced in the subsequent chapters, complemented by theory-oriented ex-

ercises at the end of each chapter. Readers should not be discouraged by such theoretical materials and exercises, since skipping such theoretical justifications will not hamper understanding of the concepts and models and principles of applying them in practice. The book is pretty much self-contained, with no prerequisite for using this book, although knowledge on statistics in general is helpful. Fundamental concepts such as confidence intervals, hypothesis tests, and p-values are briefly introduced as they first appear in the text so that people without former exposure to statistics may still benefit from the book.

The outline of the book is as follows. In addition to the review section mentioned above, Chapter 1 also presents various types of discrete random variables, together with an introduction of the study data that will be used throughout the book.

In Chapter 2, we first study individual random variables and introduce the popular discrete distributions including the binomial, multinomial, and Poisson models. Next we concentrate on the study of relationship between two categorical variables, i.e., the study of two-way contingency tables. This is followed in Chapter 3 by stratified two-way tables, controlling for potential categorical confounding variables.

When there are more than two categorical variables, or there are continuous variables present, regression analysis becomes necessary to study the relationship between such variables. In Chapter 4, we introduce regression models for categorical responses. We first discuss logistic regression for binary responses in detail, including methods to reduce bias for relatively small samples such as exact logistic models. Less popular models for binary responses such as the Probit and complementary log-log models are then discussed, followed by the models for general polytomous categorical outcomes to conclude this chapter.

Chapter 5 focuses on regression analysis of count responses. As the most commonly used models in this setting, the Poisson log-linear regression is first studied in detail, followed by a discussion on overdispersion, a common violation of the Poisson model, along with its detection and correction within the confines of this model using robust inference methods, such as the sandwich variance estimate. Alternative models that explicitly account for the sources of overdispersion and structural zero, another common violation of the Poisson, such as the negative binomial, hurdle, and zero-modified models, are then introduced to formally address such deviations from the Poisson. This chapter concludes with a systematic guide to modeling count responses using the different models introduced. Chapter 6 illustrates a major application of the Poisson log-linear regression, as it applies to general contingency tables to facilitate inference about the relationship between multiple variables, which is algebraically too complex using the classic methods discussed in Chapters 2 and 3. Also included in Chapter 6 is a section on model selection that introduces popular criteria for deriving optimal models within a given context.

Chapter 7 discusses analyses for discrete survival times. Survival analysis is widely used in statistical applications involving time to occurrence of some event of interest such as heart attacks and suicide attempts. We discuss non-

parametric life table methods as well as regression approaches.

The statistical methods covered in Chapters 2-7 are mainly for cross-sectional studies, where the data only include a single assessment point for every subject. This is not the case for longitudinal studies, where the same set of outcomes such as disease status is repeatedly measured from the same subject over time. Methods for longitudinal data must address the within-subject correlations in repeatedly measured outcomes over time. In Chapter 8, we introduce longitudinal data and models for such data, and focus on the popular parametric mixed-effects models and semiparametric generalized estimating equations.

Chapter 9 discusses validity and reliability analysis for diagnostic tests and measuring instruments. We discuss how to assess the accuracy of an ordinal test when the true status is known, using the theory of receiver operating characteristics (ROC) curves. We introduce measurement error models for assessing latent constructs, and discuss popular indices for addressing inter-rater agreement and instrument validity and reliability such as Cronbach's alpha coefficient and Kappa.

In Chapter 10, we discuss how to deal with missing values. Common approaches such as multiple imputation and inverse probability weighting methods are introduced. Since applications of the missing value concept really go beyond addressing the problem of missing values in study outcomes, we also illustrate how to apply the principles of such methods to a range of seemingly unrelated issues such as causal inference and survey sampling.

This book can serve as a primary text for a course on categorical and count data analysis for senior undergraduate, beginning as well as senior graduate students in biostatistics. It also serves well as a self-learning text for biomedical and psychosocial researchers interested in this general subject. Based on our own experiences, Chapters 1 through 7 can be covered in a one-semester course.

We would like to express our appreciation to all who have contributed to this book. We would like thank the students at the University of Rochester who took the course in the past five years, many of whom have provided countless helpful comments and feedbacks. We would also like to thank Dr. Yinglin Xia and Dr. Guoxin Zuo, who proofed many parts of the book, and offered numerous valuable comments and suggestions; Dr. Naiji Lu, who helped with some of the examples in Chapter 9 whose analyses are not supported by standard software packages; and Dr. Jun Hu, who proofread the entire book multiple times to help eradicate errors and typos. We are grateful to Drs. Linda Chaudron, Steve Lamberti, Jeffrey Lyness, Mary Caserta, and Paul Duberstein from the University of Rochester, and Dr. Dianne Morrison-Beedy from the University of South Florida for graciously sharing their study data for use in the book as real data examples. We are also thankful to editor David Grubbs for his patience and continuing support despite multiple delays on the project on our part, to one anonymous reviewer for his/her critical comments and constructive suggestions that have led to an improved presentation, and

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