

STATISTICAL METHODS FOR QUALITY OF LIFE STUDIES

Design, Measurements and Analysis

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Editors

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Statistical Methods for Quality of Life Studies

Design, Measurements and Analysis

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Statistical Methods for Quality of Life Studies

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Preface

On October 16 and 17, 2000, we hosted an international workshop entitled “Statistical Design, Measurement, and Analysis of Health Related Quality of Life.” The workshop was held in the beautiful city of Arradon, South Brittany, France with the main goal of fostering an interdisciplinary forum for discussion of theoretical and applied statistical issues arising in studies of health-related quality of life (HRQoL). Included were biostatisticians, psychometricians and public health professionals (e.g., physicians, sociologists, psychologists) active in the study of HRQoL.

In assembling this volume, we invited each conference participant to contribute a paper based on his or her presentation and the ensuing and very interesting discussions that took place in Arradon. All papers were peer-reviewed, by anonymous reviewers, and revised before final editing and acceptance. Although this process was quite time-consuming, we believe that it greatly improved the volume as a whole, making this book a valuable contribution to the field of HRQoL research.

The volume presents a broad spectrum of papers presented at the Workshop, and thus illustrates the range of current research related to the theory, methods and applications of HRQoL, as well as the interdisciplinary nature of this work. Following an introduction written by Sir David Cox, it includes 27 articles organized into the following chapters.

Chapter 1: Measurement, Scale Development and Study Design

Chapter 2: Analysis and Interpretation of Multiple Endpoints

Chapter 3: Item Response Theory and Rasch Models

Chapter 4: Joint Analysis of Quality of Life and Survival

Chapter 5: Quality-Adjusted Survival Analysis and Related Methods

Chapter 6: Methods for Informatively Missing Longitudinal Quality-of-Life Data

Chapter 1 covers many important issues related to developing a quality-of-life study, including the development and validation of tools for measuring HRQoL as well as study design considerations to ensure that, ultimately, the results from a HRQoL study will provide a useful interpretation. Chapter 2 describes methods for handling multiple

endpoints that arise in nearly all HRQoL studies because of the inherent multidimensional nature of quality of life. Chapter 3 focuses on item response theory and the distilling of responses on many individual items (e.g., questions on a questionnaire) into useful scales. Included in this chapter is the important Rasch model for item response. Chapter 4 presents methods for combining survival data and HRQoL data in a joint analysis. These methods are especially useful in studies of fatal diseases, where survival time is a critical endpoint and the HRQoL information must be interpreted in light of a patient's time of death. Chapter 5 describes techniques for evaluating survival time after adjustment for the quality of life experienced. These methods are useful for comparing interventions that differ in their impact on both quality of life and survival time. Finally, Chapter 6 presents a variety of methods for handling the important problem of missing data in quality-of-life studies, particularly the case where the missingness is related to the actual, unobserved quality of life at the time of the planned assessment.

While each paper in this book is an individual advancement in the field of HRQoL study, we believe that the full value of this volume exceeds the sum of its individual parts. In large part, owing to the valuable discussions in Arradon, this volume represents enhanced connections between biostatistics, psychometrics, and economics, with contributions from methodologists, clinicians and health scientists in academia, industry and regulatory agencies. Nevertheless, we cannot claim to exhaust, or come anywhere near exhausting, the creative ideas for new and useful methods. In particular, additional attention can easily be paid to the following (which of course is only a partial list): Bayesian analysis and subjective probability considerations, decision theoretic perspectives, optimal design for quality-of-life measurement in the longitudinal context with occurrence of death, and the responsiveness of a quality-of-life instrument under the item response modeling theory.

We sincerely hope that this volume will serve as a valuable reference for current HRQoL researchers as well as encourage new researchers to work in this important area.

Mounir Mesbah, Bernard F. Cole and Mei-Ling Ting Lee, editors
April, 2002

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Introduction

David R. Cox

INTRODUCTION

DAVID R. COX

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Professor Mounir Mesbah and his colleagues are to be warmly congratulated first on organizing in Arradon a most stimulating and enjoyable meeting and then, with remarkably few missing values, compiling and editing the papers into this comprehensive volume.

There is a temptation in these introductory remarks to try and comment on every paper. I have managed to resist this temptation. All the papers, however, merit careful discussion, as indeed occurred at Arradon. I shall instead concentrate on a few themes chosen if not at random certainly somewhat arbitrarily.

The great majority of the papers argue or implicitly accept that health-related quality of life is a multidimensional concept and that this multidimensionality is to be preserved in analysis and interpretation. Of course, in particular applications special aspects will be of concern, for example in patients undergoing hip surgery the issue of pain will predominate, although even then the analysis may require more than one dimension. See, in particular, the paper of S. Briançon et al. A different view is, of course, taken in health economic studies of resource allocation and in the overall assessment of the impact of health care, where reduction to units such as the QALY (quality-adjusted life year) or the DALY (disability-adjusted life year) is commonly made. The paper of C. Le Gallès discusses the application of multi-attribute utility theory to the construction of a single preference-based index. This is a very important if extremely difficult field, with implications for resource allocation far beyond the health fields. In particular, it seems desirable that if different groups of respondents have radically different and indeed conflicting disinterested priorities decisions taken should if possible respect the interests of various groups.

More broadly, dimension-reduction is a recurring theme in the analysis and interpretation of empirical data on complex phenomena. The extensive economic literature on index-numbers is rarely considered in the statistical world, by and large for good reason. P.K. Sen's contribution brings out parallels with the literature on poverty indices in welfare economics. A serious problem is that difficult but unavoidable choices of the relative weight to put on different features cannot really be bypassed by formalistic specifications of structure.

The broader implication is the need to distinguish distinct purposes for the use of these instruments. The helpful statement by C. Gnecco and P. Lachenbruch that the Food and Drug Administration's (USA) attitude to these issues inevitably and correctly involves some element of rigidity. It is to be hoped that this rigidity does not carry over into other areas of application. J. Wittes's very thoughtful paper