DOCTORAL THESIS

Statistical inference for correlated binary data from bilateral studies

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Date of Award:
2009

Statistical Inference for Correlated Binary Data from Bilateral Studies

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A thesis submitted in partial fulfillment of the requirements

for the degree of

Doctor of Philosophy

Principal Supervisor: Dr. Tang Man Lai

Hong Kong Baptist University

July 2009

Abstract

In some medical comparative studies, outcomes are bilateral and dichotomous. Inference can be made by either treating subject as the fundamental unit or treating the paired outcomes as independent and ignoring the intra-class correlation information. The latter strategy is usually easier but may lead to inappropriate or wrong conclusions when correlation really exists. Taking the correlation into account can reduce variability and make comparison more precise. In my thesis, we base our statistical inference assuming that there is correlation in the bilateral binary data. In Chapter 2, we consider testing equality of the correlations of two paired binary responses from two treatment groups. Several statistics for testing the equality of correlations and sample size formulae for a two-arm randomized clinical trial are developed. Simulations are performed to evaluate the behaviors of various tests and the accuracy of different sample size formulae in terms of actual size and power. In Chapter 3, we construct and evaluate different confidence interval estimators for correlated proportion differences from bilateral binary data in two-arm randomized clinical trials under the dependence model with equal correlation coefficients between groups. We evaluate the performance of various confidence interval estimators with respect to exact coverage probabilities, exact confidence widths and non-coverage probabilities. In Chapter 4, we focus on model selection issues. We compare the performance of several goodness-of-fit tests for several dependence models used for bilateral binary data in the literature and demonstrate how one may choose an appropriate model in practice. In Chapter 5, we test the equality of the response rates between two treatments using combined unilateral and bilateral data. In particular, we propose several test statistics and investigate their performance. We also apply our proposed methods in each chapter to a real problem using real data sets. Chapter 6 contains conclusions and possible future work in this field.

Keywords: Correlated binary data, Paired data, Intra-class correlation, Score test, Wald test, Bootstrap method, Confidence interval estimation, Goodness of fit test, Model selection, Asymptotic tests, Systemic sclerosis, ophthalmology.

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