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Analysis of the risk difference of marginal and conditional probabilities in an incomplete correlated 2×2 table

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Abstract

Incomplete correlated 2 × 2 tables are common in infectious disease studies and 2-step treatment studies in which one of the comparative measures of interest is the *rate/risk difference* (RD) between the marginal probability and the conditional probability. In this paper, we consider an interval hypothesis test about the RD in an incomplete correlated 2 × 2 table. We propose two asymptotic test statistics, a Wald-type (sample-based) test statistic and a constrained maximum likelihood estimation (CMLE-based) test statistic to test the interval hypothesis. The sample sizes based on the above two tests for two types of analyses: hypothesis testing and confidence interval estimation are proposed. The true type I error rates and powers of the two tests are computed by enumerating the exact probabilities in the rejection region. Our empirical results show that the CMLE-based test performs better than the sample-based test in the sense that its significance level is much closer to the prespecified nominal level, its confidence interval estimator generally possesses shorter confidence interval width with coverage probability close to the prespecified confidence level. Two real examples are used to illustrate the proposed methods.

Keywords: Constrained maximum likelihood estimator; Incomplete table; Risk difference; Power; Confidence interval; Score test; Sample size determination

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1. Introduction

Correlated 2×2 tables with a structural zero in one of the off-diagonal cells are often encountered in various clinical trails, epidemiological studies and biological studies. Structural zeros arise in situations where it may be inherent to the problem at hand or it is theoretically impossible for a particular cell to be observed. A typical example for the former is Agresti's (1990, p. 45) calves data. In this example, a sample of 156 calves born in Okeechobee County, Florida, was first classified according to whether they were infected by pneumonia within 60 days after birth. The same sample of calves were then classified according to whether they developed a secondary infection within 2 weeks after the first infection cleared up. In principle, calves who did not get a primary infection could not develop a secondary infection. Because we record those calves only if they are primary infected, we introduce a structural void in the cell of the summary 2×2 table that corresponds with secondary infection without primary infection. A typical example for the latter is the well-known two-step tuberculosis skin test program (Toyota et al., 1999; Tang and Tang, 2002). In the two-step tuberculosis (TB) skin test program, all subjects receive an initial TB test. Those subjects who are negative in an initial TB test are recommended to receive a second test 1–3 weeks later. Since those subjects who are strongly positive in the initial TB test will never be administered to the second test, the corresponding cell in the summary 2×2 table would necessarily contain a structural void. In these studies, one of the comparative measures of interest is the *rate/risk difference* (RD) between the probability of the initial negative response (or, the primary infection) and the conditional probability of the second negative response (or, the secondary infection), given the initial negative response (or, the primary infection).

Agresti (1990, p. 45) presented a hypothesis testing procedure for testing whether the RD is zero, i.e. testing whether the probability of the primary pneumonia infection of calves is equal to that of the secondary pneumonia infection of calves given the primary infection. Lui (2000) further studied the confidence interval estimators for RD. He proposed three asymptotic interval estimators based on Wald's test statistic, the likelihood ratio test and the basic principle of Fieller's theorem. It was found that the interval estimators based on the Wald test statistic and the Fieller theorem failed to attain the prespecified confidence level from small to moderate sample sizes, the probability failing to produce the desired confidence interval based on the likelihood ratio test is high even in moderate sample size. Hence, Tang and Tang (2003) proposed a score test procedure for detecting non-zero risk difference in an incomplete correlated 2×2 table. They compared the performance of the score test statistic and the likelihood ratio test in terms of the coverage probability, expected interval width, left-tail error rate and right-tail error rate. Their empirical results show that the score test and the likelihood ratio test perform equally well under general cases except that the former is undefined in only one scenario. Motivated by work of Lachenbruch and Lynch (1998) in assessing an equivalence between a new screening test diagnostic procedure and a currently available screening test diagnostic procedure without a gold standard procedure, we consider the following hypothesis test procedure: $H_0 : |RD| \geqslant \delta_0$ verse $H_1 : |RD| < \delta_0$, where δ_0 is a prespecified threshold for the difference of the two proportions for a primary infection (or, the initial negative response) and a secondary infection (or, the second negative response) given the primary infection (or, the initial negative response). The test