

## MASTER'S THESIS

### Testing of non-unity risk ratio under inverse sampling

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# Testing of Non-unity Risk Ratio under Inverse Sampling

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Master of Philosophy

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# Abstract

Risk ratio, first proposed by Sheps (1958,1959), is defined as the risk or probability of an outcome among individuals who have a given specific characteristic divided by the risk or probability among individuals who lack this characteristic. When the interested disease is rare or chronic, we often investigate risk ratio via inverse sampling. Inverse sampling (also known as negative binomial sampling) scheme suggests that one continues to sample subjects until a pre-specified number of rare events of interest is observed (Haldane 1945). It is considered to be a more appropriate sampling scheme than the usual binomial sampling scheme when the subjects arrive sequentially, when the underlying response of interest is acute, and when maximum likelihood estimators of some epidemiologic indices are undefined. Statistical inferences and procedures for risk ratio under binomial sampling have been intensively studied, however hypothesis testing approaches for risk ratio via inverse sampling were exceptionally scattered.

In this thesis, we study various statistics for testing non-unity risk ratio in case-control studies under inverse sampling. They include the Wald, unconditional score, likelihood ratio and conditional score statistics. Four methods (namely, asymptotic, conditional exact, Mid- $P$  and approximate unconditional methods ) are adopted for  $P$ -value calculation. We evaluate the performance of different combinations of test statistics and  $P$ -value methods in terms of their empirical sizes and powers through Monte Carlo simulation.

In general, the (conditional and unconditional) score tests are preferable since their actual type I errors are well controlled around the pre-chosen nominal level while their powers are comparatively the largest under the asymptotic and approximate unconditional methods. Exact version of Wald test is recommended if one wants to control the actual type I error rate at or below the pre-chosen nominal level. If larger power is expected and fluctuation of type I error rate around the pre-chosen nominal level is allowed, Mid- $P$  version of Wald test will become a desirable alternative.

**Keywords:** Inverse sampling, Wald test, Score test, Likelihood ratio test.

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