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Sample Size Calculation for Clustered Binary Data with Sign Tests Using Different Weighting Schemes

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Abstract

We propose a sample size calculation approach for testing a proportion using the weighted sign test when binary observations are dependent within a cluster. Sample size formulas are derived with nonparametric methods using three weighting schemes: equal weights to observations, equal weights to clusters, and optimal weights that minimize the variance of the estimator. Sample size formulas are derived incorporating intracluster correlation and the variability in cluster sizes. Simulation studies are conducted to evaluate a finite sample performance of the proposed sample size formulas. Empirical powers are generally close to nominal levels. The number of clusters required increases as the imbalance in cluster size increases and the intracluster correlation increases. The estimator using optimal weights yields the smallest sample size estimate among three estimators. For small values of intracluster correlation the sample size estimates derived from the optimal weight estimator are close to that derived from the estimator assigning equal weights to observations. For large values of intracluster correlation, the optimal weight sample size estimate is close to the sample size estimate assigning equal weights to clusters.

Keywords

Proportion test; Intracluster correlation; Optimal weights; Simulation; Cluster size imbalance

1. Introduction

Clustered binary data often arise in medical studies such as dental and radiologic studies in which observations are taken from multiple observations of each subject (called “clusters”). For example, as many as 60 lesions may be observed through positron emission tomography (PET) in one patient since PET offers the possibility of imaging the whole body (Gonen et al. 2001). A major issue in the analysis of clustered data is the dependence among observations within each cluster. The degree of dependence is usually measured by the intracluster correlation coefficient. Application of the traditional statistical methods such as *t*-tests or chi-square tests developed for independent observations is invalid since observations within the same subject tend to be dependent. In other words, clustered binary data should be analyzed using statistical methods that take account of the dependence of within-subject observations.

While parametric methods have been extensively studied for the analysis of clustered binary data for the last three decades (Liang and Zeger 1986; Donner et al. 1981; Rao and Scott 1992; Lee and Dubin 1994; Jung and Ahn 2000), nonparametric statistical methods have recently received increasing attention for the analysis of clustered data (Larocque 2005; Rosner et al. 2006a, 2006b; Gerard and Schucany 2007; Larocque et al. 2007; Datta and Satten 2008). Larocque (2005) accounted for clustering in a signed-rank test with a variance estimate that is based on the sums of squares over independent clusters. To account for clustering, Rosner et al. (2006a) adjusted the variance in the signed-rank test by estimating it from the ranks of absolute observations with a common intracluster correlation. Datta and Satten (2008) derived a signed-rank test for clustered data in which the distribution of pairwise differences within a cluster depends on cluster size.

Jung et al. (2001) proposed a sample size calculation method for testing a proportion in clustered binary data using parametric statistical methods. Hu et al. (2010) derived the sample size formula for testing a proportion in clustered binary data with a sign test assigning equal weights to observations.

In this article we focus on sample size estimation for testing a proportion using a sign test with different weighting schemes; equal weights to observations, equal weights to clusters, and optimal weights that minimize the variance of the estimator. Here, the sample size refers to the number of clusters. Noether (1987) discussed sample size determinations for some common traditional nonparametric tests under the assumption of independence among observations, in which Noether's sample size formula for a sign test is a special case of the sample size formulas presented in this article.

We propose a nonparametric sample size calculation method for testing a proportion in clustered binary data incorporating the intracluster correlation and the distribution of cluster sizes. In this article our discussion is limited to testing a one-sample proportion in clustered binary data. In Section 2, we review weighted sign test statistics for testing a binomial proportion. In Section 3, we derive sample size formulas using three different weighting schemes. In Section 4, we apply sample size formulas to the design of a dental study. In Section 5, we conduct extensive simulation studies to evaluate the performance of the sample size formulas and to investigate the effects of cluster size imbalance and intracluster correlation.

2. Statistical Method

Let X_{ij} be the binary random variable ($X_{ij} = 1$ for success, -1 for failure) for the j th observation in the i th cluster, $j = 1, \dots, n_i$ and, $i = 1, \dots, m$. We can express the total as the difference between the total number of successes and the total number of failures for each cluster by coding $X_{ij} = 1$ and -1 for success and failure, respectively (Cox and Wermuth 1994; Molenberghs and Ryan 1999). Cluster size (n_i) may vary at random from a certain distribution with mass function $f(\cdot)$. We assume the common correlation model (Mak 1988), that is, observations in a cluster are assumed to be exchangeable in the sense that, given n_i , $X_{i1}, \dots, X_{in_i}$ have a common marginal response probability $P(X_{ij} = 1) = p$ ($0 < p < 1$) and a common intracluster correlation coefficient $\rho = \text{corr}(X_{ij}, X_{ij'})$ for $j \neq j'$. We test the null hypothesis $H_0: p = p_0$, versus $H_1: p = p_1$ for $p_0 \neq p_1$.

Let S_i be the difference between the total number of successes and the total number of failures in the i th cluster ($S_i = \sum_{j=1}^{n_i} X_{ij}$), and $(w_1, w_2, \dots, w_m)$ be a sequence of cluster weights such that $w_i \geq 0$ and $(1/m) \sum_{i=1}^m w_i n_i = 1$. Then we have a class of test statistics used in this sign test

$$T = \sum_{i=1}^m w_i S_i = \sum_{i=1}^m w_i (n_i^+ - n_i^-), \quad (1)$$

where n_i^+ and n_i^- are the total numbers of successes and failures in the i th cluster, respectively.

The expected value of T under the null hypothesis is given by

$$\mu_0(T) = \sum_{i=1}^m w_i E(S_i | H_0) = m(2p_0 - 1). \quad (2)$$

Under the null hypothesis, the variance of T is

$$\begin{aligned} \sigma_0(T)^2 &= \sum_{i=1}^m w_i^2 \text{Var}(S_i | H_0) \\ &= 4p_0(1 - p_0) \sum_{i=1}^m w_i^2 n_i \{1 + (n_i - 1)\rho\}, \end{aligned} \quad (3)$$

which can be consistently estimated by

$$\widehat{\sigma_0(T)^2} = 4p_0(1 - p_0) \sum_{i=1}^m w_i^2 n_i \{1 + (n_i - 1)\widehat{\rho}\}, \quad (4)$$

where $\widehat{\rho}$ can be estimated by the ANOVA method (Donald and Donner 1990). Through simulation, Ridout et al. (1999) evaluated the performance of various estimators of ρ for clustered binary data under the common-correlation model, $\rho = \text{corr}(X_{ij}, X_{ij'})$ for $j \neq j'$. They showed that the ANOVA estimator performed well through simulation.

The standardized weighted sign test statistic is defined by

$$Z = \frac{\sum_{i=1}^m w_i (n_i^+ - n_i^-) - m(2p_0 - 1)}{\sqrt{4p_0(1 - p_0) \sum_{i=1}^m w_i^2 n_i \{1 + (n_i - 1)\widehat{\rho}\}}}, \quad (5)$$

which is a asymptotically normal with mean 0 and variance 1.

The choice of $w_i = m / \sum_{i=1}^m n_i$ provides equal weights to observations, and the test statistic (1) can be expressed as

$$T_u = \frac{m}{\sum_{i=1}^m n_i} \sum_{i=1}^m (n_i^+ - n_i^-), \quad (6)$$

and its standardized test statistic from (5) is

$$Z_u = \frac{\sum_{i=1}^m (n_i^+ - n_i^-) - n(2p_0 - 1)}{\sqrt{4p_0(1 - p_0) \sum_{i=1}^m n_i \{1 + (n_i - 1)\hat{\rho}\}}}, \quad (7)$$

where $n = \sum_{i=1}^m n_i$.

The choice of $w_i = 1/n_i$ assigns equal weights to clusters, and the test statistic (1) becomes the nonparametric statistic of Datta and Satten (2008)

$$T_c = \sum_{i=1}^m \left(\frac{n_i^+ - n_i^-}{n_i} \right), \quad (8)$$

and its standardized test statistic from (5) is

$$Z_c = \frac{\sum_{i=1}^m \left(\frac{n_i^{(+)} - n_i^{(-)}}{n_i} \right) - m(2p_0 - 1)}{\sqrt{4p_0(1 - p_0) \sum_{i=1}^m \{1 + (n_i - 1)\hat{\rho}\}/n_i}}. \quad (9)$$

The variance of T , $\sigma_0(T)^2$, is minimized under the constraint of $(1/m) \sum_{i=1}^m w_i n_i = 1$ when w_i is given by

$$w_i = \frac{m\{1 + (n_i - 1)\hat{\rho}\}^{-1}}{\sum_{i=1}^m n_i \{1 + (n_i - 1)\hat{\rho}\}^{-1}}. \quad (10)$$

That is, the choice of the above w_i provides the minimum variance of T .

We refer the test statistic and the standardized test statistic using optimal weights as T_o and Z_o , respectively. Note that the optimal weights, w_i , depend on ρ . The standardized test statistic Z_o has the same form as (5) except that w_i needs to be estimated. If cluster sizes are constant, that is, $n_i = \bar{n}$, then all three standardized test statistics (Z_i , $i = u, c, o$) are the same. We reject H_0 if the absolute value of the standardized test statistic, (Z_i , $i = u, c, o$), is larger than $z_{1-\alpha/2}$, which is the $100(1 - \alpha/2)$ percentile of the standard normal distribution.

3. Sample Size Determination

Noether (1987) proposed a sample size formula for some common nonparametric tests such as a sign test. He showed that the sample size for power $(1 - \beta)$ of such a test can be estimated from the following equation,

$$\left\{ \frac{\mu_1(T) - \mu_0(T)}{\sigma_0(T)} \right\}^2 = (z_{1-\alpha/2} + rz_{1-\beta})^2, \quad (11)$$

where $r = \sigma_1(T)/\sigma_0(T)$.

We will extend Noether's nonparametric sample size formula to obtain sample size formula for clustered binary data. Noether (1987) stated that it will often be appropriate to assume that $\sigma_1(t)$ is close to $\sigma_0(T)$ for alternatives that do not differ too much from the alternative hypothesis. Here we assume that cluster sizes n_i 's are small relative to m so that asymptotic results can be attained with respect to m . The expected value of T under the alternative distribution is

$$\mu_1(T) = \sum_{i=1}^m w_i E(S_i | H_1) = m(2p_1 - 1).$$

Since n_i 's are independent and identically distributed random variables, from (3) we obtain three limits,

$$\begin{aligned} \frac{1}{m} \sigma_0(T_u)^2 &\rightarrow 4p_0(1-p_0)\{(1-\rho)E[N] + E[N^2]\rho\}/E[N]^2 \\ \frac{1}{m} \sigma_0(T_c)^2 &\rightarrow 4p_0(1-p_0)\{(1-\rho)E[1/N] + \rho\} \\ \frac{1}{m} \sigma_0(T_o)^2 &\rightarrow 4p_0(1-p_0) \frac{1}{E[N(1+(N-1)\rho)^{-1}]}, \end{aligned}$$

as $m \rightarrow \infty$, where N is the random variable with mean θ and variance τ^2 corresponding to the cluster size, and $E[\cdot]$ is the expectation with respect to the distribution of the cluster size. Then we have

$$\begin{aligned} \sigma_0(T_u)^2 &\rightarrow 4mp_0(1-p_0) \left\{ \frac{(1-\rho)}{\theta} + \rho + \frac{\tau^2}{\theta^2} \rho \right\} \\ \sigma_0(T_c)^2 &\rightarrow 4mp_0(1-p_0) \{(1-\rho)E[1/N] + \rho\} \\ \sigma_0(T_o)^2 &\rightarrow 4mp_0(1-p_0) \frac{1}{E[N(1+(N-1)\rho)^{-1}]}. \end{aligned}$$

Note that the limiting quantities of $\sigma_0(T_u)^2$ and $\sigma_0(T_c)^2$ are linear in ρ . The convergent quantity of $\sigma_0(T_c)^2$ is dependent on the distribution of cluster sizes only through its harmonic mean, $E[1/N]$ while that of $\sigma_0(T_u)^2$ depends on the distribution of cluster sizes through its first and second moments. With a power of $1 - \beta$, the sample size estimates from (11) (m_i , $i = u, c, o$) to test $H_0: p = p_0$ versus $H_1: p = p_1$ are

$$m_u = \frac{(z_{1-\alpha/2} + z_{1-\beta})^2}{(p_1 - p_0)^2} \left\{ \frac{(1-\rho)}{\theta} + \rho + \frac{\tau^2}{\theta^2} \rho \right\} p_0(1-p_0) \quad (12)$$

$$m_c = \frac{(z_{1-\alpha/2} + z_{1-\beta})^2}{(p_1 - p_0)^2} \{ (1 - \rho)E[1/N] + \rho \} p_0(1 - p_0) \quad (13)$$

$$m_o = \frac{(z_{1-\alpha/2} + z_{1-\beta})^2}{(p_1 - p_0)^2} \frac{1}{E[N\{1 + (N - 1)\rho\}^{-1}]} p_0(1 - p_0). \quad (14)$$

When cluster size is constant, the sample size formulas (12)–(14) are identical. If all responses are independent ($\rho = 0$) and all cluster sizes are equal to 1, then all the sample size formulas (12)–(14) reduce to Noether's formula (11).

4. Example

We use the data of Hujoel et al. (1990) to illustrate the sample size estimation for clustered binary data with the weighted sign test using different weighting schemes. An enzymatic diagnostic test was performed to investigate whether a site was infected by either *treponema denticola* or *bacteroides gingivalis*. An antibody assay was used as the gold standard to determine infected sites against the two organisms. There were different numbers of infected sites per subject. Table 1 shows the number of true positive test results (S_i) and the number of infected sites (n_i) in 29 subjects. Table 2 presents the observed distribution and the projected distribution of cluster size (N).

Suppose we want to use the data in Table 1 as pilot data to design a similar experiment to test the hypothesis $H_0: p = 0.6$ versus $H_1: p = 0.7$. From these data, we obtain the intraclass correlation coefficient estimate $\hat{\rho} = 0.2$ using the ANOVA method. Thus, we assume the intraclass correlation coefficient of $\hat{\rho} = 0.2$ for a new experiment. Sample size calculations are based on the projected distribution of the cluster size and the values $E(\frac{1}{N})$, $E(N(1 + (N - 1)\rho))^{-1}$, and τ^2 . We get $E[N] = 4.9$ by both the observed distribution and the projected distribution. Hence, the estimated sample sizes required in the experiment for 80% and 90% are $m_o = 70$ and $m_o = 94$ for T_o , respectively. Similarly, we obtain $m_u = 71$ (and 95) for T_u and $m_c = 71$ (and 96) for T_c for the power of 80% (and 90%) of power. Although the difference is small, m_o is the smallest.

5. Simulation Study

In this section, we report the results of a simulation study to investigate the performance of sample size formulas in terms of empirical powers. Since cluster sizes frequently exhibit considerable variations in medical studies, cluster sizes were generated using a negative binomial distribution truncated at zero as in Donner and Koval (1987) and Ahn et al. (2009). We refer the reader to Ahn et al. (2009) or Johnson et al. (1992) for details on the truncated negative binomial distribution.

The imbalance in cluster size is measured by the quantity $\kappa = 1/(1 + \sigma^2/\mu^2)$, where μ and σ are the mean and standard deviation of cluster size. The variation in cluster size decreases as κ increases. All cluster sizes are equal when $\kappa = 1$. Cluster sizes are generated from the truncated negative binomial distribution with mean cluster size of $\mu = 5, 10$, and 20 , and the imbalance parameter of $\kappa = 0.6, 0.8$, and 1.0 , which corresponds to severe, moderate, and no variability. We used p values of $0.05, 0.1, 0.3$, and 0.5 . Here, we test the null hypothesis $H_0: p = p_0$ against the alternative hypothesis $H_1: p = p_1$ with $\alpha = 0.05$, $1 - \beta = 0.9$ and $(p_0, p_1) = (0.5, 0.7), (0.6, 0.7)$ and $(0.7, 0.9)$. The required sample size (m) under three weighting

schemes is estimated using sample size formulas (12)–(14) for given values of $p_0, p_1, \rho, \kappa, \mu, \alpha$, and β . The correlated binary data are generated by the method of Lunn and Davies (1998) conditional on the estimated number of clusters and mean cluster size.

We conducted 10,000 experiments for each parameter combination. The intraclass correlation coefficient was estimated using the ANOVA estimator. Data with a sample size of $m_i (i = u, c, o)$ was generated from the distribution specified by p_0, p_1, ρ, μ and κ . We estimated empirical Type I errors and powers as the proportions of experiments rejecting H_0 and H_1 by the test statistics ($Z_i, i = u, c, o$) in 10,000 experiments, respectively.

Tables 3–5 present the sample size estimates and the corresponding empirical Type I errors and powers for testing $H_0: p = 0.6$ versus $H_1: p = 0.7$, $H_0: p = 0.5$ versus $H_1: p = 0.7$, and $H_0: p = 0.7$ versus $H_1: p = 0.9$ at 5% significance level and 90% power, respectively. Tables 3–5 show that all three estimators generally yield similar empirical powers even though m_o is always less than or equal to m_c or m_u . Tables 3 and 4 show that all the empirical powers are close to nominal power of 90% for all three estimators. Table 5 shows that all test statistics generally have lower empirical powers than the nominal power for testing $H_0: p = 0.7$ versus $H_1: p = 0.9$. That is, when p_1 is close to 1.0, these test statistics generally yield empirical powers lower than the nominal level. When a large sample size is required, for example testing $H_0: p = 0.6$ versus $H_1: p = 0.7$ as in Table 3, empirical Type I errors are close to the nominal level of 5%. Simulation results suggest that the all three weighted estimators yield empirical Type I errors and powers close to the nominal levels under large sample size ($m_i \geq 30$). However, there are cases that weighted estimators do not produce appropriate empirical Type I errors and powers when the estimated sample size is small ($m_i < 30$).

Overall, the required sample size estimate increases due to clustering effect for all three estimators as the intraclass correlation ρ increases. As κ decreases, cluster sizes vary more and more severely, the required number of clusters increases. When the mean cluster size μ increases, the required sample size decreases. If ρ is small and cluster size varies among clusters, m_o is closer to m_u . However, as ρ increases, m_o becomes closer to m_c . When $\kappa = 1$, estimated sample sizes for all three methods of assigning weights are equal. In practice, sample size estimation based on the optimal weight and use of the corresponding test statistic is recommended.

6. Discussion

In this article, we introduced sample size formulas for testing proportions using the weighted sign test when binary observations are dependent within a cluster. We illustrated the sample size calculation using the data of an enzymatic diagnostic test. We evaluated the finite sample performance of the test statistics ($Z_i, i = u, c, o$), and the sample size formulas (12)–(14) for the proportion test. All the empirical powers for testing $H_0: p = 0.6$ vs. $H_1: p = 0.7$ are within 2% of the nominal level of 90% power for all three weighted estimators. All the empirical powers for testing $H_0: p = 0.5$ versus $H_1: p = 0.7$ lie within 4% of the nominal level of 90% power for all three weighted estimators. However, all three weighted estimators become underpowered for testing $H_0: p = 0.7$ versus $H_1: p = 0.9$. That is, when p_1 is close to 1.0, the test statistics generally yield empirical powers less than the nominal level. The reason for low empirical powers needs to be investigated when p_1 is close to 1.0. Simulation results suggest that all three weighted estimators yield empirical Type I errors and powers close to the nominal levels under large sample size ($m_i \geq 30$). However, there are cases that weighted estimators do not produce appropriate empirical Type I errors and powers when the estimated sample size is small ($m_i < 30$).

An optimal estimator yields the smallest sample size estimate among three weighted estimators. The sample size estimate (m_o) is closer to m_u for smaller values of ρ while m_o is closer to m_c for larger values of ρ . When $\kappa = 1$, all three estimators yield the same sample size. The required sample size increases as the intracluster correlation ρ increases. As the variability in cluster size increases, the required number of clusters increases. When the mean cluster size μ increases, the required sample size decreases.

As a future study, we will investigate the asymptotic relative efficiency (ARE) of the weighted sign test using optimal weights with respect to equal weights to observations or clusters. The ARE will be computed for both the extent of correlation among observations within clusters and the various moments of the distribution of the number of observations. We will investigate at which distribution of the number of observations the maximum ARE occurs and what the maximum value is. We will investigate the sample size estimates in the two-sample cases.

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Table 1Pilot data of S_i/n_i from $m = 29$ subjects (clusters).

3/6, 2/6, 2/4, 5/6, 4/5, 5/5, 4/6, 3/4, 2/4, 3/4, 5/5, 4/4, 6/6, 3/3, 5/6, 1/2, 4/6, 0/4, 5/6, 4/5, 4/6, 0/6, 4/5, 3/5, 0/2, 2/6, 2/4, 5/5, 4/6

Table 2

Distribution of cluster size

	n_i				
	2	3	4	5	6
Relative frequency	2/29	1/29	7/29	7/29	12/29
Projected mass function	0.05	0.05	0.25	0.25	0.4

Table 3

Empirical powers (%), (sample size estimates), and Type I errors (%) for testing $H_0: p = 0.6$ versus $H_1: p = 0.7$ with $\alpha = 0.05$ and $\beta = 0.1$ from 10,000 simulations.

κ	ρ^a	μ^b	Power (sample size) Type I error		
			Z_u	Z_c	Z_o
0.6	0.05	5	91 (66) 5.4	92 (99) 5.2	91 (64) 5.3
		10	89 (43) 5.6	90 (62) 5.4	90 (40) 5.7
		20	89 (32) 5.9	90 (40) 5.3	89 (28) 5.6
	0.1	5	90 (84) 5.5	92 (106) 5.0	91 (77) 5.2
		10	90 (62) 5.4	90 (72) 5.2	90 (53) 5.7
		20	88 (51) 5.8	90 (51) 5.3	89 (41) 5.4
0.3	0.05	5	90 (154) 5.1	91 (136) 4.7	90 (121) 5.2
		10	89 (137) 5.4	89 (109) 5.2	90 (100) 5.1
		20	89 (129) 5.1	90 (93) 5.4	90 (88) 5.1
	0.1	5	89 (224) 4.9	90 (166) 5.4	90 (159) 5.3
		10	89 (212) 5.2	90 (146) 5.2	90 (142) 5.0
		20	90 (206) 5.3	90 (135) 5.3	90 (133) 5.3
0.8	0.05	5	89 (61) 5.5	90 (76) 4.5	90 (60) 5.2
		10	89 (38) 5.6	89 (44) 5.2	89 (37) 5.3
		20	88 (27) 5.5	89 (28) 5.4	88 (25) 5.7
	0.1	5	90 (74) 5.3	90 (84) 5.3	89 (71) 5.2
		10	89 (52) 5.5	90 (55) 5.7	90 (49) 5.5
		20	88 (41) 5.9	89 (39) 5.7	89 (37) 5.7
0.5	0.05	5	90 (124) 4.8	90 (119) 5.1	90 (112) 5.1
		10	89 (107) 5.2	90 (96) 5.4	89 (93) 5.4
		20	88 (99) 5.0	90 (84) 5.4	89 (83) 5.3
	0.1	5	90 (174) 5.2	90 (153) 5.1	89 (151) 5.2
		10	89 (162) 4.8	89 (137) 5.5	90 (136) 5.0
		20	88 (156) 5.2	89 (128) 5.1	90 (128) 5.3
1	0.05	5	90 (58) 5.2	90 (58) 5.2	90 (58) 5.2
		10	90 (35) 5.3	90 (35) 5.3	90 (35) 5.3

κ	ρ^a	μ^b	Power (sample size) Type I error			
			Z_u	Z_c	Z_o	
0.1		20	89 (24) 5.3	89 (24) 5.3	89 (24) 5.3	
		5	90 (68) 5.0	90 (68) 5.0	90 (68) 5.0	
		10	89 (46) 5.3	89 (46) 5.3	89 (46) 5.3	
		20	89 (35) 5.3	89 (35) 5.3	89 (35) 5.3	
0.3		5	89 (106) 5.1	90 (106) 5.1	89 (106) 5.1	
		10	89 (89) 4.9	89 (89) 4.9	89 (89) 4.9	
		20	89 (81) 5.2	89 (81) 5.2	89 (81) 5.2	
		5	90 (144) 5.2	90 (144) 5.2	90 (144) 5.2	
0.5		10	90 (132) 5.1	90 (132) 5.1	90 (132) 5.1	
		20	90 (126) 5.1	90 (126) 5.1	90 (126) 5.1	

^a ρ is an intracluster correlation coefficient

^b μ is the mean cluster size of a truncated negative binomial distribution below 1

^c Z_u , Z_c , and Z_o are the test statistics for equal weights to observations, equal weights to clusters and optimal weights

Table 4

Empirical powers (%), (sample size estimates), and Type I errors (%) for testing $H_0: p = 0.5$ versus $H_1: p = 0.7$ with $\alpha = 0.05$ and $\beta = 0.1$ from 10,000 simulations.

κ	ρ^a	μ^b	Power (sample size)			Type I error		
			Z_u	Z_c	Z_o	Z_u	Z_c	Z_o
0.6	0.05	5	89 (17)	5.4	91 (26)	4.6	90 (17)	5.5
		10	87 (11)	6.2	89 (16)	5.1	90 (11)	6.4
	0.1	20	89 (9)	8.5	90 (11)	5.5	86 (7)	8.8
		5	91 (22)	5.9	92 (28)	4.9	90 (20)	5.8
		10	88 (16)	6.9	90 (19)	5.4	88 (14)	6.7
		20	87 (13)	8.4	88 (13)	6.5	89 (11)	10
0.3	0.05	5	90 (40)	5.7	91 (35)	4.9	90 (31)	5.6
		10	90 (36)	5.7	89 (28)	5.5	90 (26)	5.6
	0.1	20	89 (33)	5.5	89 (24)	5.6	89 (23)	5.4
		5	90 (58)	5.2	91 (43)	4.9	90 (41)	5.4
		10	89 (55)	5.0	90 (38)	5.0	90 (37)	5.3
		20	89 (53)	5.4	89 (35)	5.2	89 (34)	5.3
0.8	0.05	5	88 (16)	5.2	90 (20)	5.0	89 (16)	5.0
		10	88 (10)	6.0	90 (12)	5.5	88 (10)	6.0
	0.1	20	86 (7)	7.3	91 (8)	6.0	88 (7)	6.9
		5	88 (19)	5.5	90 (22)	5.2	89 (19)	5.7
		10	89 (14)	6.1	89 (14)	5.9	88 (13)	6.1
		20	88 (11)	7.2	87 (10)	6.3	88 (10)	7.1
0.3	0.05	5	89 (32)	5.6	90 (31)	5.4	89 (29)	5.5
		10	89 (28)	5.5	90 (25)	5.3	89 (24)	5.4
	0.1	20	89 (26)	5.3	89 (22)	5.7	89 (22)	5.3
		5	90 (45)	5.0	91 (40)	5.3	90 (39)	5.3
		10	90 (42)	5.4	89 (35)	5.1	89 (35)	5.2
		20	89 (40)	5.3	89 (33)	5.3	89 (33)	5.0
1	0.05	5	89 (15)	5.2	89 (15)	5.2	89 (15)	5.2
		10	87 (9)	5.3	87 (9)	5.3	87 (9)	5.3

κ	ρ^a	μ^b	Power (sample size)			Type I error		
			Z_u	Z_c	Z_o	Z_u	Z_c	Z_o
0.1	20	5	86 (6)	7.8	86 (6)	7.8	86 (6)	7.8
			90 (18)	5.4	90 (18)	5.4	90 (18)	5.4
			10	88 (12)	6.0	88 (12)	6.0	88 (12)
	10	5	87 (9)	7.0	87 (9)	7.0	87 (9)	7.0
			90 (28)	5.4	90 (28)	5.4	90 (28)	5.4
			10	89 (23)	5.4	89 (23)	5.4	89 (23)
0.3	20	5	89 (21)	5.5	89 (21)	5.5	89 (21)	5.5
			90 (37)	4.8	90 (37)	4.9	90 (37)	4.9
			10	90 (34)	5.4	90 (34)	5.4	90 (34)
	10	5	90 (33)	5.2	90 (33)	5.2	90 (33)	5.2
			90 (33)	5.2	90 (33)	5.2	90 (33)	5.2
			90 (33)	5.2	90 (33)	5.2	90 (33)	5.2

^a ρ is an intracluster correlation coefficient

^b μ is the mean cluster size of a truncated negative binomial distribution below 1

Table 5

Empirical powers (%), (sample size estimates), and Type I errors (%) for testing $H_0: p = 0.7$ versus $H_1: p = 0.9$ with $\alpha = 0.05$ and $\beta = 0.1$ from 10,000 simulations.

κ	ρ^a	μ^b	Power (sample size)			Type I error		
			Z_u	Z_c	Z_o	Z_u	Z_c	Z_o
0.6	0.05	5	86 (12) 5.7	89 (17) 4.3	83 (11) 5.3			
		10	84 (8) 7.6	88 (11) 4.8	81 (7) 6.5			
		20	83 (6) 9.7	86 (7) 6.3	81 (5) 9.4			
	0.1	5	86 (15) 5.9	89 (19) 4.8	86 (14) 6.0			
		10	84 (11) 7.6	88 (13) 6.0	85 (10) 7.2			
		20	82 (9) 10.8	86 (9) 7.0	80 (7) 10.1			
0.3	0.05	5	87 (27) 5.8	89 (24) 5.3	87 (21) 5.4			
		10	86 (24) 5.7	87 (19) 5.9	86 (18) 6.0			
		20	86 (23) 5.9	85 (16) 6.2	87 (16) 6.3			
	0.1	5	88 (39) 5.6	89 (29) 5.3	89 (28) 5.5			
		10	87 (37) 5.4	88 (26) 5.3	88 (25) 5.8			
		20	87 (36) 5.6	88 (24) 5.7	87 (23) 5.6			
0.8	0.05	5	86 (13) 5.0	90 (20) 4.7	86 (11) 5.6			
		10	85 (7) 5.6	87 (8) 5.3	85 (7) 5.8			
		20	83 (5) 10.0	84 (5) 7.7	84 (5) 9.2			
	0.1	5	86 (13) 6.1	87 (15) 5.3	86 (13) 6.1			
		10	82 (9) 8.1	86 (10) 6.8	85 (9) 7.5			
		20	80 (7) 10.2	83 (7) 8.7	82 (7) 9.7			
0.3	0.05	5	87 (22) 5.7	87 (21) 5.6	87 (20) 5.7			
		10	85 (19) 6.0	86 (17) 6.1	84 (16) 6.2			
		20	84 (17) 7.0	85 (15) 6.5	85 (15) 6.6			
	0.1	5	87 (30) 5.6	88 (27) 5.4	87 (26) 5.6			
		10	87 (28) 5.5	87 (24) 5.7	88 (24) 5.7			
		20	86 (27) 5.5	86 (22) 5.7	86 (22) 5.7			
1	0.05	5	85 (10) 5.0	85 (10) 5.0	85 (10) 5.0			
		10	85 (6) 7.1	85 (6) 7.1	86 (6) 7.1			

κ	ρ^a	μ^b	Power (sample size)			Type I error		
			Z_u	Z_c	Z_o	Z_u	Z_c	Z_o
0.1		20	86 (5) 8.0	86 (5) 8.0	86 (5) 8.0	86 (5) 8.0	86 (5) 8.0	86 (5) 8.0
		5	85 (12) 6.0	85 (12) 6.0	85 (12) 6.0	85 (12) 6.0	85 (12) 6.0	85 (12) 6.0
		10	83 (8) 6.9	83 (8) 6.9	83 (8) 6.9	83 (8) 6.9	83 (8) 6.9	83 (8) 6.9
		20	78 (6) 9.8	78 (6) 9.8	78 (6) 9.8	78 (6) 9.8	78 (6) 9.8	78 (6) 9.8
0.3		5	87 (19) 5.9	87 (19) 5.9	87 (19) 5.9	87 (19) 5.9	87 (19) 5.9	87 (19) 5.9
		10	86 (16) 6.3	86 (16) 6.3	86 (16) 6.3	86 (16) 6.3	86 (16) 6.3	86 (16) 6.3
		20	83 (14) 7.5	83 (14) 7.5	83 (14) 7.5	83 (14) 7.5	83 (14) 7.5	83 (14) 7.5
		5	87 (25) 5.5	87 (25) 5.5	87 (25) 5.5	87 (25) 5.5	87 (25) 5.5	87 (25) 5.5
0.5		10	88 (23) 5.7	88 (23) 5.7	88 (23) 5.7	88 (23) 5.7	88 (23) 5.7	88 (23) 5.7
		20	87 (22) 5.5	87 (22) 5.5	87 (22) 5.5	87 (22) 5.5	87 (22) 5.5	87 (22) 5.5

^a ρ is an intracluster correlation coefficient

^b μ is the mean cluster size of a truncated negative binomial distribution below 1