

# The Size of the Cochran-Armitage Trend Test in $2 \times C$ Contingency Tables: Two Multinomial Distribution Case<sup>†</sup>

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## Abstract

In this paper we show that the peak of the type I error rate of the Cochran-Armitage trend test could be greater than the nominal level when  $2 \times C$  contingency tables obtained from two multinomial distributions are extremely unbalanced. This result justifies the use of the exact Cochran-Armitage trend test in extremely unbalanced  $2 \times C$  contingency tables.

*Keywords:* Imbalance; type I error; exact test.

## 1. Introduction

Since the type I error rate of some popular asymptotic tests converges to the nominal level for given values of nuisance parameters, we might expect that the size (the supremum of the type I error rate over the nuisance parameter space) also converges to the nominal level as the sample size increases. However, it is not true in several hypothesis testing problems in contingency tables such as the test of independence, the test of homogeneity, and the test of the Hary-Weinberg law (Loh, 1989; Loh and Yu, 1993; Kang and Shin, 2004; Kang *et al.*, 2006; Kang and Lee, 2007). In this paper we deal with the Cochran-Armitage trend test in  $2 \times C$  contingency tables.

Ordered categorical data in  $2 \times C$  contingency tables may be obtained from two multinomial populations with  $C$  ordered multinomial outcomes or  $C$  binomial populations with  $C$  ordered groups. The Cochran-Armitage trend test is often implemented to detect a linear trend of ordered categorical data in  $2 \times C$  contingency tables. Recently, Kang and Lee (2007) showed that the sizes of the Cochran-Armitage trend test for  $C$  binomial populations with  $C$  ordered groups are always greater than the nominal level in infinite samples. The size is defined as the supremum of the type I error rate over the nuisance parameter space. Kang and Lee (2007) also showed that the peak of the type I error rate of the Cochran-Armitage trend test occurs at the extremes of the nuisance parameter space and that the type I error rate is very close to the nominal level for most values of the nuisance parameters. The results of Kang and Lee (2007) imply that the type I error rate of the Cochran-Armitage trend test could be greater than the nominal

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Table 2.1:  $2 \times C$  table with score  $S_j$  assigned to the  $j^{\text{th}}$  column

	$S_1$	$S_2$	...	$S_C$	total
	$X_{11}$	$X_{12}$	...	$X_{1C}$	$R_1$
	$X_{21}$	$X_{22}$	...	$X_{2C}$	$R_2$
total	$C_1$	$C_2$	...	$C_c$	$n$

level in unbalanced  $2 \times C$  contingency tables which are obtained from  $C$  binomial populations with  $C$  ordered groups, because the extreme values of the nuisance parameters make  $2 \times C$  contingency tables extremely unbalanced.

A next natural question is whether or not similar results hold for the Cochran-Armitage trend test for two multinomial populations with  $C$  ordered multinomial outcomes. Specifically, let us denote  $h(\mathbf{p}, n)$  by the type I error rate of the Cochran-Armitage trend test where  $n$  is the sample size and  $\mathbf{p} = (p_1, \dots, p_{C-1})$  are the nuisance parameters under the null hypothesis. In this paper we will show  $\lim_{n \rightarrow \infty} \sup_{H_0} h(\mathbf{p}, n) \geq \alpha$  and find the cases where the strict inequality hold. Note that  $\sup_{H_0} h(\mathbf{p}, n)$  is the size of the Cochran-Armitage trend test and represents the type I error rate at the worst possible case. We would like to emphasize  $\lim_{n \rightarrow \infty} \sup_{H_0} h(\mathbf{p}, n) \neq \sup_{H_0} \lim_{n \rightarrow \infty} h(\mathbf{p}, n)$ . Actually,  $\lim_{n \rightarrow \infty} h(\mathbf{p}, n) = \alpha$  for any parameters in the null hypothesis, so we have  $\sup_{H_0} \lim_{n \rightarrow \infty} h(\mathbf{p}, n) = \sup_{H_0} \alpha = \alpha$ . Therefore, in this paper we will show that the sizes of the Cochran-Armitage trend test for two multinomial populations with  $C$  ordered multinomial outcomes are always greater than or equal the nominal level in infinite samples.

## 2. Notation and Review

We assume that there are two independent multinomial samples. For  $i = 1, 2$ ,

$$\mathbf{X}_i \equiv (X_{i1}, X_{i2}, \dots, X_{iC}) \sim M(R_i, (p_{i1}, p_{i2}, \dots, p_{iC}))$$

where  $0 < p_{ij} < 1$ ,  $j = 1, \dots, C$ ,  $\sum_{j=1}^C p_{ij} = 1$ . In other words, we assume that the row margins ( $R_i$ ,  $i = 1, 2$ ) are fixed. Then, we have the following  $2 \times C$  contingency table. We would like to test  $H_0: p_{1j} = p_{2j} = p_j$  for an unknown  $p_j$ ,  $j = 1, \dots, C - 1$ ,  $0 < \sum_{j=1}^{C-1} p_j < 1$ . Here it should be noted that  $p_j$ 's,  $j = 1, \dots, C - 1$ , are the unknown nuisance parameters.

Since The Cochran-Armitage trend test is conducted based on large sample approximation, the test rejects  $H_0$  if

$$X^2 = \frac{A^2}{B} \geq \chi_{1, \alpha}^2,$$

where

$$A = \sum_{j=1}^C C_j (\hat{p}_j - \bar{p})(S_j - \bar{S}) = \sum_{j=1}^C X_{1j} S_j - R_1 \sum_{j=1}^C \frac{C_j S_j}{n},$$

$$B = \bar{p}(1 - \bar{p}) \left\{ \sum_{j=1}^C C_j S_j^2 - \left( \sum_{j=1}^C C_j S_j \right)^2 / n \right\},$$

$$\hat{p}_j = \frac{X_{1j}}{C_j}, \quad \bar{p} = \frac{R_1}{n}$$

and  $\chi_{1,\alpha}^2$  is the upper  $100 \times \alpha$  percentile of the chi-square distribution with one degree of freedom. Note that the asymptotic null distribution of  $X^2$  is the chi-square distribution which is free from the unknown nuisance parameters. However, under the null hypothesis the finite-sample distribution of  $X^2$  depend on the unknown nuisance parameter  $(p_j, 1 \leq j \leq C - 1)$  as given by

$$P_{H_0}(X^2 > \chi_{1,\alpha}^2) = \sum_{\mathbf{X}_1} \sum_{\mathbf{X}_2} R_1! R_2! \prod_{j=1}^C \frac{p_j^{X_{1j}+X_{2j}}}{X_{1j}! X_{2j}!} I_{[X^2(\mathbf{X}_1, \mathbf{X}_2) \geq \chi_{1,\alpha}^2]}. \tag{2.1}$$

In this paper we investigate the sizes of the Cochran-Armitage trend test in a  $2 \times C$  contingency table generated by two independent multinomial samples. The size of a test is defined by the supremum of the type I error rate over the nuisance parameter space under the null hypothesis. More precisely, the size of the level- $\alpha$  Cochran-Armitage trend test is the supremum of (2.1) over the parameter space of  $(p_j, 1 \leq j \leq C - 1)$ .

### 3. Main Results

In this section we examine size properties of the Cochran-Armitage trend test in large samples. Let  $E_h = \{\min_{ij} E_{ij} > h\}$  for a nonnegative number  $h$ , where  $E_{ij} = R_i C_j / n$ . Suppose that the test is only carried out if the event  $E_h$  occurs. This implies  $\min_{1 \leq j \leq C} C_j \geq 1$ . The conditional and unconditional sizes of the level- $\alpha$  test are defined by

$$\alpha_n(h) = \sup_{H_0} P(X^2 > \chi_{1,\alpha}^2 | E_h), \quad \bar{\alpha}_n(h) = \sup_{H_0} P(\{X^2 > \chi_{1,\alpha}^2\} \cap E_h),$$

where the supremum is taken over all possible values of  $(p_j, 1 \leq j \leq C - 1)$ .

**Theorem 3.1** Both the conditional and the unconditional sizes of the level- $\alpha$  Cochran-Armitage trend test in a  $2 \times C$  contingency table generated by two independent multinomial samples are greater than or equal to  $\alpha$  in large samples.

**Proof:** We use the following fact (Johnson and Kotz, 1969). Let a random vector  $(Y_1, Y_2, \dots, Y_C)$  follow a multinomial distribution  $M(N, (q_1, \dots, q_C))$  with  $\sum_{i=1}^C Y_i = N$ ,  $\sum_{i=1}^C q_i = 1$ . If  $(q_1, \dots, q_{C-1})$  go to zero and  $N$  tends to infinity in such a way that  $Nq_i = w_i > 0, i = 1, \dots, C - 1$ , then

$$P(Y_1 = y_1, \dots, Y_{C-1} = y_{C-1}) \longrightarrow \prod_{i=1}^{C-1} \frac{e^{-w_i} w_i^{y_i}}{y_i!}.$$

To obtain a lower bound of size, suppose that  $H_0$  holds with

$$p_j = \frac{w}{n}, \quad j = 1, \dots, C - 1, \quad p_C = 1 - (C - 1) \frac{w}{n}, \quad 0 < w < n.$$

Let  $f_i = R_i/n, i = 1, 2$  and  $f_* = \min_{1 \leq i \leq 2} f_i$ .

Since  $R_i p_j = f_i w$ ,  $i = 1, 2$ ,

$$X_{ij} \xrightarrow{d} Y_{ij},$$

where  $Y_{ij}$  follows independently Poisson distribution with mean  $f_i w$ ,  $i = 1, 2$ ,  $j = 1, \dots, C - 1$ . Let  $T_j = Y_{1j} + Y_{2j}$ ,  $j = 1, \dots, C - 1$ . Then  $T_j$ 's follow independently Poisson distribution with mean  $w$ . The conditional distribution of  $(Y_{1j}, Y_{2j})$  given  $T_j = t_j$  is binomial  $(t_j, f_1)$  for  $j = 1, \dots, C - 1$ . For the test statistic, we have

$$\begin{aligned} A &= \sum_{j=1}^C X_{1j} S_j - R_1 \sum_{j=1}^C \frac{C_j S_j}{n} = \frac{R_2}{n} \sum_{j=1}^C X_{1j} S_j - \frac{R_1}{n} \sum_{j=1}^C X_{2j} S_j \\ &= \frac{1}{n} \sum_{j=1}^{C-1} (S_j - S_C)(R_2 X_{1j} - R_1 X_{2j}) \xrightarrow{d} \sum_{j=1}^{C-1} (S_j - S_C)(f_2 Y_{1j} - f_1 Y_{2j}), \\ B &= \bar{p}(1 - \bar{p}) \left\{ \sum_{j=1}^C C_j S_j^2 - \left( \sum_{j=1}^C C_j S_j \right)^2 / n \right\} \\ &= \bar{p}(1 - \bar{p}) \left[ \sum_{j=1}^{C-1} (S_j^2 - S_C^2) C_j + n S_C^2 - \frac{1}{n} \left\{ \sum_{j=1}^{C-1} (S_j - S_C) C_j + n S_C \right\}^2 \right] \\ &\xrightarrow{d} f_1 f_2 \left\{ \sum_{j=1}^{C-1} (S_j^2 - S_C^2) T_j - 2 \sum_{j=1}^{C-1} (S_j - S_C) S_C T_j \right\} \\ &= f_1 f_2 \sum_{j=1}^{C-1} (S_j - S_C)^2 T_j. \end{aligned}$$

Now we obtain a lower bound of the unconditional size.

$$\begin{aligned} &\lim_{n \rightarrow \infty} \bar{\alpha}_n(h) \\ &\geq \lim_{n \rightarrow \infty} P_{H_0: p_j = \frac{w}{n}, j=1, \dots, C-1}(\{X^2 > \chi_{1, \alpha}^2\} \cap E_h), \quad \text{for all } w > 0 \\ &\geq P \left( \left( \frac{\left\{ \sum_{j=1}^{C-1} (S_j - S_C)(f_2 Y_{1j} - f_1 Y_{2j}) \right\}^2}{f_1 f_2 \sum_{j=1}^{C-1} (S_j - S_C)^2 T_j} > \chi_{1, \alpha}^2 \right) \cap \left\{ \min_{1 \leq j \leq C-1} T_j > h f_*^{-1} \right\} \right), \\ &\quad \text{for all } w > 0 \\ &= \sum_{\min t_j > h f_*^{-1}} P \left( \frac{\left\{ \sum_{j=1}^{C-1} (S_j - S_C)(f_2 Y_{1j} - f_1 Y_{2j}) \right\}^2}{f_1 f_2 \sum_{j=1}^{C-1} (S_j - S_C)^2 T_j} > \chi_{1, \alpha}^2 \mid T_1 = t_1, \dots, T_{C-1} = t_{C-1} \right) \end{aligned}$$

$$\begin{aligned} & \times P(T_1 = t_1, \dots, T_{C-1} = t_{C-1}) \\ &= \sum_{\min t_j > hf_*^{-1}} \sum_{Y \in A(t)} \prod_{j=1}^{C-1} t_j! \frac{f_1^{y_{j1}} f_2^{y_{j2}} e^{-w} w^{t_j}}{y_{j1}! y_{j2}! t_j!}, \end{aligned}$$

where  $t = (t_1, \dots, t_{C-1})$ ,  $Y = \{Y_{ij} : i = 1, 2, j = 1, \dots, C - 1\}$ .

$$\begin{aligned} A(t) &= \left\{ Y \mid \frac{\left\{ \sum_{j=1}^{C-1} (S_j - S_C)(f_2 Y_{1j} - f_1 Y_{2j}) \right\}^2}{f_1 f_2 \sum_{j=1}^{C-1} (S_j - S_C)^2 t_j} > \chi_{1, \alpha}^2 \right\} \\ &= e^{-(C-1)w} \sum_{\min t_j > hf_*^{-1}} w^{t_+} \sum_{Y \in A(t)} \prod_{j=1}^{C-1} \frac{f_1^{y_{j1}} f_2^{y_{j2}}}{y_{j1}! y_{j2}!}, \quad \text{where } t_+ = \sum_{j=1}^{C-1} t_j \\ &= H(\alpha, f, w, h), \quad \text{where } f = (f_1, f_2). \end{aligned}$$

Since the conditional distribution of

$$\frac{\left\{ \sum_{j=1}^{C-1} (S_j - S_C)(f_2 Y_{1j} - f_1 Y_{2j}) \right\}^2}{f_1 f_2 \sum_{j=1}^{C-1} (S_j - S_C)^2 t_j}$$

given  $T_1 = t_1, \dots, T_{C-1} = t_{C-1}$  follows asymptotically the chi-square distribution with one degree of freedom as  $w \rightarrow \infty$  and

$$P\left(\min_{1 \leq j \leq C-1} T_j > hf_*^{-1}\right) \rightarrow 1, \quad \text{as } w \rightarrow \infty,$$

the rightmost inequality in (1) is obtained. So we have

$$\lim_{n \rightarrow \infty} \bar{\alpha}_n(h) \geq \sup_{w > 0} H(\alpha, f, w, h) \geq \lim_{n \rightarrow \infty} H(\alpha, f, w, h) \geq \alpha.$$

□

We compute the large-sample lower bounds for the unconditional size  $H(\alpha, f, w, h)$  and display results in Table 3.1.

When  $(f_1, f_2) = (0.01, 0.99)$ , the lower bound is 0.099 which is much greater than the nominal level 0.05. It means that the size of the Cochran-Armitage trend test is greater than 0.099 in infinite samples. When  $(f_1, f_2) = (0.01, 0.99)$ , the ratio of the sample sizes of two multinomial distributions is 1:99, which implies that the  $2 \times C$  contingency table is extremely unbalanced. Since the size is the supremum of the type I error rate over the nuisance parameter space, the inflation of the size does not imply that the type I error

Table 3.1: The lower bound of the Cochran-Armitage trend test for the unconditional size ( $C = 3$ ,  $\alpha = 0.05$ ,  $h = 0$ ,  $S_i = i$ )

$(f_1, f_2)$	lower bound	$(f_1, f_2)$	lower bound
(0.5, 0.5)	0.05	(0.10, 0.90)	0.050
(0.6, 0.4)	0.05	(0.05, 0.95)	0.050
(0.7, 0.3)	0.05	(0.01, 0.99)	0.099
(0.8, 0.2)	0.05		

is inflated in all values of the nuisance parameters. Furthermore, the results in Table 3.1 hold in infinite samples, not in finite samples. Therefore, we conduct simulation studies to investigate the empirical type I error rates in finite samples at the various values of the nuisance parameters. Specifically we generate 50,000  $2 \times 3$  contingency tables for each combination of the row margins  $(R_1, R_2)$  and the values of  $(p_1, p_2, p_3)$  under the null hypothesis. The 5%-level Cochran-Armitage trend tests are conducted in the 50,000  $2 \times 3$  contingency tables and the frequency of rejecting the null hypothesis is counted. The empirical type I error rates are calculated as the proportion of rejecting the null hypothesis among the 50,000  $2 \times 3$  contingency tables. Simulation studies show that the empirical type I error rates are close to the nominal level for most values of the nuisance parameters, but the peak of the empirical type I error rates occurs at the extremes of the nuisance parameters. For example, we consider a  $2 \times 3$  contingency table whose row margins are  $(R_1, R_2) = (10, 990)$  and whose common success probabilities under the null hypothesis are  $(p_1, p_2, p_3) = (0.01, 0.01, 0.98)$ . Since the proof of Theorem 3.1 is obtained when  $p_1 \rightarrow 0$  and  $p_2 \rightarrow 0$ , we consider  $(p_1, p_2, p_3) = (0.01, 0.01, 0.98)$ .  $(R_1, R_2) = (10, 990)$  implies that the  $2 \times 3$  contingency table is extremely unbalanced in terms of row margins. Since  $p_3$  is much larger than  $p_1$  and  $p_2$ , the margin of the third column would be excessively larger than those of the first and the second column. Therefore, the  $2 \times 3$  contingency table is extremely unbalanced in terms of both row margins and column margins. In such case simulation studies show that the empirical type I error rate is 0.089 at 5% nominal level. Extreme imbalance in both row margins and column margins makes the Cochran-Armitage trend test very anti-conservative.

#### 4. Conclusions

In summary the type I error rates of the Cochran-Armitage trend test obtained from two multinomial distribution are close to the nominal level for most values of the nuisance parameters. But, when both column and row margins are extremely unbalanced, we show that the type I error rate of the Cochran-Armitage trend test could be much greater than the nominal level. In such cases the exact Cochran-Armitage trend test might be employed as an alternative, because the exact test guarantees that the type I errors are controlled under the nominal level. The exact Cochran-Armitage trend test can be implemented in commercial software StatXact (Cytel, 2007).

## References

- Loh, W. Y. (1989). Bounds on the size of the  $\chi^2$  test of independence in a contingency table, *The Annals of Statistics*, **17**, 1709–1722.
- Loh, W. Y. and Yu, X. (1993). Bounds on the size of the likelihood ratio test of independence in a contingency table, *Journal of Multivariate Analysis*, **45**, 291–304.
- Kang, S. H. and Shin, D. W. (2004). The size of the chi-square test for the Hardy-Weinberg law, *Human Heredity*, **58**, 10–17.
- Kang, S. H., Lee, Y. H. and Park, E. S. (2006). The sizes of the three popular asymptotic tests for testing homogeneity of two binomial proportions, *Computational Statistics & Data Analysis*, **51**, 710–722.
- Kang, S. H. and Lee, J. W. (2007). The size of the Cochran-Armitage trend test in  $2 \times C$  contingency table, *Journal of Statistical Planning and Inference*, **137**, 1851–1861.
- Johnson, N. L. and Kotz, S. (1969). *Distributions in Statistics: Discrete Distributions*, Jhon Wiley & Sons, New York.
- Cytel (2007). *StatXact*, Version 6.0. Software for exact nonparametric statistical inference with continuous or categorical data, Cambridge, Massachusetts: Cytel Software.

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