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DESIGN EFFECTS OF TWO-STAGE SAMPLING

C. J. Skinner

Mathematics Research Center University of Wisconsin—Madison 610 Walnut Street Madison, Wisconsin 53705

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# UNIVERSITY OF WISCONSIN - MADISON MATHEMATICS RESEARCH CENTER

DESIGN EFFECTS OF TWO-STAGE SAMPLING

C. J. Skinner

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# ABSTRACT

The effect of a two-stage sampling design on statistical inference is discussed. A definition of a design effect is given. The structure of design effects for a class of statistics is investigated. Results have both a design-based and a model-based interpretation. The relation between design effects for multivariate statistics and design effects for univariate statistics is considered.

AMS (MOS) Subject Classifications: 62D05, 62H10

Key Words: Design Effect; Model Misspecification; Two-stage Sampling; Finite

Population; Sample Survey.

Work Unit Number 4 - Statistics and Probability

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### SIGNIFICANCE AND EXPLANATION

- B-

In sample surveys, the design effect of a statistic is usually defined as the ratio of its true variance under the given sample design to its variance had the sample been obtained by simple random sampling.

Empirical work suggests certain patterns for design effects of different types of statistics under different designs but theoretical work explaining these patterns is limited. This paper obtains general theoretical results on ( $s^{\dagger}a^{\dagger}is^{\dagger}icat$  interact) the structure of design effects for a broad class of statistics under a twostage sampling design. In particular, it discusses the relation between design effects of multivariate and of univariate statistics.

This relation is of practical interest because it is of relevance to the imputation of standard errors for multivariate statistics such as correlation coefficients or regression coefficients using design effects of univariate statistics. The latter quantities are often routinely derived on completion of the survey. The former may be difficult to compute by standard procedures, either because of the absence of the necessary design information or because

of software or degrees of freedom limitations.

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#### DESIGN EFFECTS OF TWO-STAGE SAMPLING

#### C. J. Skinner

#### 1. INTRODUCTION

The application of statistical methods such as regression analysis and multivariate analysis to sample survey data is now widespread. Such methods typically assume that the rows of an  $n \times q$  data matrix,  $x_n$ , are realizations of independent and identically distributed (IID) random vectors. A general question may therefore be raised as to the validity of inference procedures which make this assumption when the data is derived using a complex sample design. In particular, this paper is concerned with the effect of twostage sampling on the estimation of functions of population moments, such as correlation coefficients.

The term 'design effect' was originally introduced (Kish, 1965) as a measure of efficiency for comparing sample designs. More recently (e.g. Rao and Scott, 1981) it has also been used as a measure of the impact of a sample design on an inference procedure. We shall be concerned only with this latter concept.

We presume a basic acquaintance with the distinction between the <u>design-based</u> and the <u>model-based</u> approaches to survey-sampling inference (e.g. Sarndal, 1978). From the design-based viewpoint the interpretation of 'the effect of two-stage sampling' is clear. The IID assumption corresponds to the randomization distribution induced in  $x_n$  by simple random sampling with replacement from a finite population (or without replacement from an infinite population). Two-stage sampling induces a different distribution in  $x_n$  and consequently perturbs the distribution of estimators from that predicted by IID theory.

From the model-based viewpoint the effect of the sampling design on inference is much less clear. The model-based approach begins by specifying a model distribution for the matrix of values, x, of the population units. Inference then proceeds in one of two

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ways:

- (A) Inference is based only on the model distribution conditional on the units actually obtained in the sample and irrespective of any other sample that might have been selected.
- (B) Inference incorporates both the model distribution and the randomization distribution induced by the sample design.

The role of the sample design in model-based inference is by no means a subject of universal agreement (see, for example, the discussion of Royall and Cumberland, 1981). Sugden and Smith (1984) specify various conditions for choosing between (A) and (B). For example, an instance when it may be inappropriate to ignore the sample design occurs when sampling on the dependent variable in regression analysis (c.f. Nathan and Holt, 1980). In such cases the design has a direct effect on inference.

In Section 2 we adopt procedure (A). In this case the effect of the sample design is more indirect. For example, two-stage sampling presumes that the population is divided into clusters. Units within clusters usually tend to be more alike than units in different clusters. This implies that the IID assumption for  $x_n$  corresponds to an inappropriate model assumption. The 'effect of the design' is therefore really the effect of misspecifying the model (c.f. Scott and Holt, 1982, p. 850). For if the true model for  $x_n$ is in fact IID then two-stage sampling would have no effect under procedure (A). Conversely, if the true model is not IID and we happen to choose the same sample of units by (i) simple random sampling and (ii) two-stage sampling then the effect on inference is identical for (i) and (ii).

Our approach will be to define a distribution for  $x_n$  which has both a design-based and model-based interpretation and then to obtain results which may be interpreted as respectively design effects or misspecification effects. Because of the mathematical isomorphism between the results under the two approaches it will be convenient to use the single term design effect. We maintain, however, that this effect has distinct interpretations under the two approaches.

-2-

There is a further problem from the model-based viewpoint with the effect of design on statistical methods such as regression analysis. Suppose we take a two-stage sample and decide that the appropriate model allows for different regression relationships in different clusters. It may be argued (e.g. Pfefferman and Nathan, 1981) that the target parameters of interest are then the individual cluster regression coefficients rather than any overall population regression coefficient. We shall ignore this consideration here and assume that the target is a well defined population parameter. We view the design as an arbitrary selection process with no characteristic of substantive interest upon which we wish to 'condition' (c.f. Kish and Frankel, 1974).

We now introduce our basic definition of 'design effect'. We take  $x_n$  as a member of the infinite sequence  $\{x_n; n=1,2,\ldots\}$ . Let  $\pi_0 = \pi_0$  be the 'baseline' distribution of  $x_n$  under the IID assumption. Let  $\pi_1 = \pi_{1,n}$  be the true distribution of  $x_n$ . From the design-based viewpoint  $\pi_1$  is the randomization distribution induced by the complex sampling design. From the model-based viewpoint, assuming procedure (A) above,  $\pi_1$  is the true model distribution of x 'marginalized' to  $x_n$ .

<u>Definition 1.1</u>: Suppose  $t_n = t_n(x_n)$  is a scalar statistic obeying the following central limit laws as  $n + \infty$ .

 $n^{\frac{1}{2}}(t_n-\theta_0) \stackrel{L}{\rightarrow} N(0,\sigma_0^2) \text{ under } \pi_0$   $n^{\frac{1}{2}}(t_n-\theta_1) \stackrel{L}{\rightarrow} N(0,\sigma_1^2) \text{ under } \pi_1$ 

Suppose also that  $v_{0,n} = v_{0,n}(x_n)$  is consistent for  $\sigma_0^2$  under  $\pi_0$  and converges in probability to  $plim_{\pi_1}(v_{0,n})$  under  $\pi_1$ . Then the <u>design effect</u> of  $t_n$  is defined as

$$deff(t_n;\pi_1,v_{0,n}) = \sigma_1^2/plim_{\pi_1}(v_{0,n}) \quad . \tag{1.1}$$

#### Remarks

1. The traditional definition of a design effect (e.g. Kish, 1965, p. 265) as a measure of design efficiency is  $\sigma_1^2/\sigma_0^2$  in the above notation. Definition 1.1 is more natural as a measure of the impact of the design on estimation. It measures the effect of acting as if  $\pi_0$  is true when in fact  $\pi_1$  is true. Note that  $deff^{\frac{1}{2}}$ 

-3-

provides a multiplicative adjustment for the standard-error estimate,  $(n^{-1}v_{0,n})^{\frac{1}{2}}$ . 2. The design effect will usually be of secondary importance if  $t_n$  is inconsistent under  $\pi_1$ , that is if  $\theta_1$  is not the target parameter. If  $\pi_0$  is assumed to be the true distribution then  $t_n$  is usually chosen such that  $\theta_0$  is the target parameter. Then  $\theta_1 - \theta_0$  is the asymptotic bias.

3. Definition 1.1 does not depend on  $\pi_0$ , except as a heuristic device for deriving  $v_{0,n}$ . This makes this definition easier to use from the model-based approach than the definition  $\sigma_1^2/\sigma_0^2$ .

4. The design effect is unity when  $\pi_0 = \pi_1$ .

5. The asymptotic nature of the definition simplifies results but is not essential.

In this article we shall be interested in how design effects depend upon the survey design and upon the population. We adopt a theoretical approach as opposed to the empirical approach of, for example, Kish and Frankel (1974). The latter approach may be more realistic but lacks generalizability because of the enormous range of possible statistics and population structures. The theoretical approach must make strong assumptions to obtain useful results but the extent of possible generalization should be more apparent. Of course the two approaches should complement each other.

We shall be particularly interested in the relation between design effect of multivariate statistics and design effects of univariate statistics. Such relations are of practical interest for at least two reasons. Firstly, the survey data collection agency may publish design effects for univariate statistics but, for confidentiality reasons, may not make sufficient survey design information available on public use tapes for the data analyst to estimate standard errors in the usual way. Given suitable theoretical relations, the analyst could instead impute standard error for multivariate statistics using the published univariate design effects. Secondly, even if the analyst has available full design information it may still be desirable to impute standard errors because of computer software availability or degrees of freedom limitations. In Section 2 we outline the basic formal framework and define  $\pi_0$  and  $\pi_1$ . In Section 3 we apply Definition 1.1 to a general class of estimators under the given  $\pi_1$  and derive results on the form of design effects for the case of equal cluster sizes. An example is given in Section 4. The case of unequal cluster sizes is considered briefly in Section 5 and the implications of the results are discussed in Section 6.

# 2. FRAMEWORK AND ASSUMPTIONS

Consider a finite population, U, partitioned into K clusters. Let the j<sup>th</sup> unit in the i<sup>th</sup> cluster be labelled (i,j) for i = 1, ..., K,  $j = 1, ..., M_i$  where  $M_i$  is the size of the i<sup>th</sup> cluster. A sample is a subset, S, of  $U = \{(i,j) ; i = 1, ..., K,$  $j = 1, ..., M_i\}$ . We suppose that the sample is selected in such a way that each subset S of U has a known probability, p(S), of selection. Conventionally the sample is chosen in two stages: first, a sample of clusters is selected and then subsamples are selected

within each of the selected clusters. Without loss of generality we write the actual sample obtained as  $s = \{(i,j) ; i = 1,...,k, j = 1,...,m_j\}$ . The sizes of the sample and population are respectively:

$$n = \sum_{i=1}^{k} m_{i}, N = \sum_{i=1}^{k} M_{i}.$$

We suppose that a qx1 vector  $x_{ij}$  is associated with unit (i,j) in U and let

$$\mathbf{x} = (\mathbf{x}_{11}, \dots, \mathbf{x}_{\mathrm{KM}_{\mathrm{K}}})^{\mathrm{T}} \quad , \quad \mathbf{x}_{\mathrm{n}} = (\mathbf{x}_{11}, \dots, \mathbf{x}_{\mathrm{km}_{\mathrm{K}}})^{\mathrm{T}}$$

be respectively the  $N \times q$  matrix of finite population values and  $n \times q$  observed data matrix discussed in Section 1, where T denotes transpose.

For simplicity we make the following assumption in Sections 2 - 4.

Assumption 1: There is no auxiliary information to distinguish the clusters, in particular the cluster sizes are equal:  $M_i = M$ , i = 1, ..., K.

We consider the case of unequal  $M_{\rm i}$  in Section 5. We now define the distributions  $\pi_0$  and  $\pi_1,$  of  $x_{\rm n}.$ 

-5-

Definition 2.1: The true distribution of  $x_n$ , denoted by  $\pi_1(x_n)$ , obeys the following conditions:

(i) conditional on (random) distribution functions,  $F_1, \ldots, F_k$ , the  $x_{ij}$  are

mutually independent and

$$x_{ij} | F_1, \dots, F_k \sim F_i$$
  $i = 1, \dots, k; j = 1, \dots, m_i$ 

(ii)  $F_1, \ldots, F_k$  are IID.

<u>Remark</u>: In (ii) the  $F_i$  are functions on  $\mathbb{R}^q$  and so the distribution of each  $F_i$  is infinite dimensional as in the theory of stochastic processes. More precistely we might follow Ferguson (1974) and let  $\Phi$  be a set of distribution functions on  $\mathbb{R}^q$ ,  $\Theta$  be a sigma-algebra of subsets of  $\Phi$  and  $\mathbb{I}$  be a probability measure on  $(\Phi,\Theta)$ . Then, equivalently to (ii), we assume  $(F_1, \dots, F_k)$  is an outcome of the product space  $(\Phi, \Theta, \mathbb{I})^k$ .

#### Design-based Interpretation of T1.

This distribution can be viewed as the randomization distribution of  $x_n$  induced by simple random sampling with replacement at both stages. Let  $G_{\alpha}$  be the 'empirical' distribution function of x in the  $\alpha^{th}$  cluster, i.e.  $G_{\alpha}$  assigns probability mass  $M^{-1}$ to each point  $x_{\alpha 1}, \dots, x_{\alpha M}$ . Let  $\Phi = \{G_1, \dots, G_K\}$  and let  $\Pi$  assign probability  $K^{-1}$  to each outcome  $G_{\alpha}$ . Hence each  $F_1$  is equal to a randomly chosen  $G_{\alpha}$ .

#### Model-based interpretation of $\pi_1$ .

Suppose x is a realization of the Nxp random matrix, X, with prior distribution  $\pi_1(x)$  obtained by extending Definition 2.1 by substituting K for k and M for  $m_1$ . Suppose that the sample design, p(S), is non-informative in the sense that S and X are independent. Then  $\pi_1(x_n)$  is the appropriate distribution of  $x_n$  for model-based inference conditional on S = s (Sugden and Smith, 1984). This is inference procedure (A) referred to in Section 1.

The distribution  $\pi_1(x)$  seems both a natural and a general non-parametric model for expressing the symmetry between clusters and between units within clusters. A simple example is the one-way random effects model (e.g. Scott and Smith, 1969). Here  $\phi$  is a

-6-

location family,  $\Phi = \{G(x-\varphi); \varphi \in \mathbb{R}^{Q}\}$  where G is a given distribution function and I defines a prior distribution for  $\varphi$ . For example,  $\Phi$  may correspond to the normal family  $\{N_q(\varphi, \Omega_W); \varphi \in \mathbb{R}^{Q}\}$  and I may correspond to  $\varphi \sim N_q(\mu, \Omega_B)$ . Other examples with scale parameters and higher-order cumulants varying between clusters are given by Leonard (1975) and Skinner (1981) respectively.

The distribution  $\pi_1(x)$  is also a special case of the two-stage exchangability/random permutation model of Bellhouse et al (1977). Their model is more general because, in particular, it allows for negative intra-cluster correlation as does the similar model of Royall (1976). However, it is less interpretable and, for example, would not permit Theorem 3.6., one of our main results. Furthermore, if we add the assumption that x is part of a doubly infinite sequence  $\{x_{ij}, i = 1, 2, ..., j = 1, 2, ...\}$  such that (i) and (ii) hold for any K and M then we would conjecture that this two-stage exchangeability model could be represented by  $\pi_1$ . (Aldous, 1981, proves a stronger result for a crossed rather than a nested doubly infinite array.)

Definition 2.2: The baseline distribution of  $x_n$ , denoted by  $\pi_0(x_n)$ , obeys the following condition:

(i)  $x_{11}, \ldots, x_{km_{k}}$  are IID.

# Design-based interpretation of $\pi_0$ .

This is the randomization distribution induced in  $x_n$  by simple random sampling with replacement from the whole finite population.

#### Model-based interpretation of To.

This is the 'textbook' IID assumption referred to in Section 1.

We assume the existence of the first two moments of  $x_n$  under both  $\pi_0$  and  $\pi_1$  and write

$$E_{\pi_{1}}(x_{ij}) = \mu$$
(2.1  

$$cov_{\pi_{1}}(x_{ij}, x_{i'j'}) = \Omega \quad i = i', j = j'$$

$$= \Omega_{B} \quad i = i', j \neq j'$$

$$= 0 \quad i \neq i' .$$

Note that  $\mu$  and  $\Omega$  are the finite population mean and covariance matrix under the design-based interpretation.

Finally, given the nature of Definition 1.1 we need to define an asymptotic framework. We follow Fuller (1975, Appendix A) in considering a sequence of finite populations and designs  $\{U_k, p_k\}$   $k = 1, 2, ...\}$  such that  $U_k$  contains  $K_k$  clusters,  $K_k > K_{k-1}$ , and  $p_k$  selects k clusters of sizes  $m_1, \dots, m_k$  from  $U_k$ . We assume  ${m_1, m_2, \ldots}$  is a fixed infinite sequence with  $1 \le m_i \le M$ . The limits  $k + \infty$  and  $n = \sum m_i + \infty$  are then equivalent but we use the latter notation to be consistent with Section 1. We assume that Definitions 2.1 and 2.2 can be extended for x = 1, 2, ... and that the common distribution of  $F_i$  in Definition 2.1 and the common distribution of ×ii in Definition 2.2 does not depend on k. From the design-based interpretation this implies further restrictions if  $\pi_0$  and  $\pi_1$  are not to depend on k via  $K_k$ . One approach (c.f. Brewer, 1979) is to assume that  $K_{\mathbf{k}}$  is an integer multiple of k, say  $K_k = Lk$ . Then suppose that  $x_{ij} = x_{i,j}$ ,  $i = L+1, \dots, K$ ;  $j = 1, \dots, M$  where  $i_0 = 0$ (i-1)mod L + 1, that is x consists of k 'reproductions' of the LM×p matrix  $(x_{11},...,x_{LM})$ . Then  $\Phi = \{G_1,...,G_L\}$  and  $\Pi$ , which assigns probability  $L^{-1}$  to each  $G_{\alpha}$ ,  $\alpha = 1, \dots, L$ , no longer depends on  $K_k$ . Alternatively one might introduce superpopulation assumptions as in Fuller (1975, Appendix A).

3. RESULTS

We consider the class of estimators of form

$$t_n = g(\overline{x}_n) \tag{3.1}$$

-8-

where  $\overline{x}_n = n^{-1} \sum_{g} x_{ij}$  and g is a given function  $g : \mathbb{R}^q + \mathbb{R}$ . This class includes, in particular, functions of second moments such as correlation coefficients and linear regression coefficients by defining x to include squares and products of the 'raw' survey variables (see, for example, Section 4 and Krewski and Rao, 1981). For simplicity we assume g is scalar-valued but results extend straightforwardly to vector-valued g. We assume the target parameter is

$$\theta_1 = g(\mu) \tag{3.2}$$

where  $\mu$  is defined in (2.1). For example, if  $t_n$  is the sample correlation coefficient then  $\theta_1$  is the finite population correlation coefficient under the design-based interpretation or the 'super-population' correlation coefficient under the model-based interpretation of  $\pi_1$ .

The main aim of this section is to apply Definition 1.1 to  $t_n$  in (3.1) for the model  $\pi_1$  of Definition 2.1. This will be done in Theorem 3.5 but first we need to establish the conditions of Definition 1.1 for  $t_n$  and  $\pi_1$ . We make use of the following <u>Condition C1( $\pi_1$ )</u>: For some  $\varepsilon > 0$ ,  $E_{\pi_1} | (x_{ij} - \mu)_{\ell} |^{2+\varepsilon}$  exists for  $\ell = 1, \dots, q$ , where  $(\cdot)_{\ell}$  denotes the  $\ell^{\text{th}}$  element of a vector.

<u>Condition C2( $\pi_1$ )</u>: The function g admits continuous partial derivatives at  $\mu$  at least one of which does not vanish at  $\mu$ .

Conditions C1( $\pi_0$ ) and C2( $\pi_0$ ) are defined analogously with  $\pi_0$  and  $E_{\pi_0}(x_{ij})$  replacing  $\pi_1$  and  $\mu$  respectively.

The corollary of the following lemma establishes one conditon of Definition 1.1 and gives the numerator of (1.1).

Lemma 3.1

If C1( $\pi_1$ ) holds then under  $\pi_1$  as  $n + \infty$  $n^{\frac{1}{2}} (\overline{x}_n - \mu) \stackrel{\Gamma}{=} N_q [0, (I_q + (m^* - 1)\Gamma)\Omega] \qquad (3.3)$ 

where  $\Gamma = \Omega_B \Omega^{-1}$ ,  $m^* = \lim_{n \to \infty} n^{-1} \sum_{i=1}^k m_i^2$ .

<u>Proof</u>: Let  $z_1 = kn^{-1} \sum_{j=1}^{m_1} (x_{1,j}-\mu)$ , Then  $\{z_1, z_2, \dots\}$  is a sequence of independent random

vectors with

$$E_{\pi_{1}}(z_{1}) = 0 , E_{\pi_{1}}(z_{1}z_{1}^{T}) = k^{2}n^{-2}m_{1}(I_{q} + (m_{1}-1)\Gamma)\Omega ,$$

$$E_{\pi_{1}}|(z_{1})_{\ell}|^{2+\epsilon} = (kn^{-1})^{2+\epsilon}E_{\pi_{1}}|\sum_{j=1}^{m_{1}}(x_{1j}-\mu)_{\ell}|^{2+\epsilon}$$

$$\leq m_{1}^{2+\epsilon}E_{\pi_{1}}|(x_{1j}-\mu)_{\ell}|^{2+\epsilon} \text{ by Minkowski's inequality}$$
and since  $n \geq k$ 

= 0(1) from C1( $\pi_1$ ) and since  $m_i \leq M$ .

Hence by using a central limit theorem such as Lemma 3.1 of Krewski and Rao (1981)

$$k^{1/2} (k^{-1} \sum_{\substack{i=1 \\ i=1}}^{k} z_i) \stackrel{L}{\rightarrow} N_q(0, \sigma_z^2)$$
  
where  $\sigma_z^2 = \lim_{n \to \infty} k^{-1} \sum_{\substack{i=1 \\ i=1}}^{k} E(z_i z_i^T)$   
 $= \lim_{n \to \infty} kn^{-1} (I_q + (n^{-1}\Sigma m_i^2 - 1)\Gamma)\Omega$ .

The result follows since  $\overline{x} - \mu = k^{-1} \Sigma z_i$ .

From standard asymptotic theory we obtain the following:

# Corollary 3.2

If  $C1(\pi_1)$  and  $C2(\pi_1)$  hold then under  $\pi_1$  as  $n + \infty$ 

$$n^{1/2}(t_n-\theta_1) \stackrel{L}{=} \mathbb{N}[0, (1 + (m^*-1)\rho_g)\sigma_g^2]$$
 (3.4)

where 
$$\sigma_{g}^{2} = \nabla_{g}(\mu)^{T} \Omega \nabla_{g}(\mu)$$
,  $\nabla_{g}(\mu) = \partial g(\mu) / \partial \mu$ 

$$\rho_{g} = \nabla_{g}(\mu)^{T} \Omega_{B} \nabla_{g}(\mu) / \sigma_{g}^{2}$$
(3.5)

#### Remarks

1. Under the model-based approach Corollary 3.2 would also hold if  $\theta_1 = g(\overline{x}_N)$  where  $\overline{x}_N = N^{-1} \sum_{U} x_{ij}$  provided  $n/N \neq 0$  as  $n \neq \infty$ . Fuller (1975, Appendix A) gives a result

corresponding to Lemma 3.1 for  $\overline{x}_n - \overline{x}_N$  where  $n/N + f \neq 0$ .

2. m\* exists because the  $m_i$  are bounded,  $1 \le m_i \le M$ .

3. The quantity  $\rho_{\mathbf{q}}$  is the intra-cluster correlation of

$$w_{ij} = \nabla_{g}(\mu)^{T} x_{ij}$$
(3.6)

since

$$\rho_{g} = \operatorname{corr}_{\pi_{1}}(w_{ij}, w_{ij}), j \neq j'$$
 (3.7)

We may write alternatively

$$\rho_{g} = var_{\pi_{1}} (\nabla_{g}(\mu)^{T} \mu_{1}) / var_{\pi_{1}}(w_{1})$$
(3.8)

where

$$\mu_{i} = E_{\pi_{1}}(x_{ij}|F_{i}) = \int x dF_{i}(x) . \qquad (3.9)$$

The delta-method or Taylor-series linearization estimator of the variance of  $t_n$  under the assumption that  $\pi_0$  is true is  $n^{-1}v_{g,0,n}$  where

$$\mathbf{v}_{g,0,n} = \nabla_{g}(\overline{\mathbf{x}}_{n})^{T} \mathbf{v}_{0,n} \nabla_{g}(\overline{\mathbf{x}}_{n})$$

$$\mathbf{v}_{0,n} = (n-1)^{-1} \sum_{g} (\mathbf{x}_{ij} - \overline{\mathbf{x}}_{n}) (\mathbf{x}_{ij} - \overline{\mathbf{x}}_{n})^{T} .$$
(3.10)

The corollary of the following lemma establishes another condition of Definition 1.1 and gives the denominator of (1.1).

# Lemma 3.3

If  $C1(\pi_1)$  holds then as n + -

where + denotes convergence in probability under  $\pi_1$ .

Proof: We may write

$$\mathbf{v}_{0,n} = (n-1)^{-1} k \left[ k^{-1} \sum_{i=1}^{k} u_{i} \right] + (n-1)^{-1} n \Omega - n(n-1)^{-1} (\overline{x} - \mu) (\overline{x} - \mu)^{T}$$

where

$$u_{i} = \sum_{j=1}^{m_{i}} [(x_{ij} - \mu)(x_{ij} - \mu)^{T} - \Omega] .$$

Now  $\{u_1, u_2, \ldots\}$  is a sequence of zero-mean independent random matrices. If the

 $m_{1}$  are equal the  $u_{1}$  are IID and by Khinchine's version of the Weak Law of Large Numbers  $k^{-1} \Sigma u_{1} \stackrel{\pi}{}^{+1} 0$  even without  $C1(\pi_{1})$ . If the  $m_{1}$  are unequal then the application of Minkowski's inequality as in Lemma 3.1 and the use of  $C1(\pi_{1})$  together with the fact that  $m_{1} \leq M$  implies that the  $(1+\frac{1}{2}\varepsilon)^{th}$  moment of the absolute value of each element of  $u_{1}$  is bounded uniformly in i and so (e.g. Krewski and Rao, 1981, Lemma 3.2)  $k^{-1} \Sigma u_{1} \stackrel{\pi}{\to} 0$  from Lemma 3.1.

From the assumed continuity of  $\nabla_g$  and the fact that  $\overline{x}_n \stackrel{\pi_1}{\to} \mu$  we obtain: Corollary 3.4

If  $C1(\pi_1)$  and  $C2(\pi_1)$  hold then as n + -

$$v_{g,0,n} + \sigma_{g}^{*1}$$
 (3.11)

We are now in a position to derive our main result.

#### Theorem 3.5

If  $C1(\pi_0)$ ,  $C1(\pi_1)$ ,  $C2(\pi_0)$ ,  $C2(\pi_1)$  hold then  $deff[t_n; \pi_1, v_{g,0,n}] = 1 + (m^*-1)\rho_g . \qquad (3.12)$ <u>Proof</u>: The condition of Definition 1.1 hold from Corollaries 3.2 and 3.4 and by noting

that  $\pi_0$  is a special case of a model of form  $\pi_1$  with  $F_1 = \dots = F_k$ . The expression in (3.12) is obtained from (3.4) and (3.11).

#### Remarks

1. If  $m_1 = \ldots = m_k = m$  then  $m^* = m$  and the expression in Theorem 3.5 has the familiar form of the design effect of a mean (Kish, 1965). The IID-based estimator  $v_{g,0,n}$  underestimates the variance of  $t_n$  by an amount which depends on the subsample size m and the intra-cluster correlation  $\rho$ .

2. If the  $m_i$  are unequal note that

$$m^* = \lim_{n \to \infty} [\overline{m} + \Sigma(m_1 - \overline{m})^2/n] > \lim_{n \to \infty} \overline{m} \text{ where } \overline{m} = n/k$$
.

Hence expression (3.12) tends to be greater than the commonly used expression  $1 + (\overline{m}-1)\rho_g$ (Kish, 1965). Our expression for  $m^+$  is the limit of expressions appearing in Campbell (1977) and Rao and Scott (1981).

- 12-

3. Referring to Remark 2 under Definition 1.1 the asymptotic relative bias is zero under the design-based interpretation since  $\theta_0 = \theta_1 = g(\overline{x}_N)$  and should be negligible under the model-based interpretation because  $t_n - g(\overline{x}_N) \stackrel{\text{T}}{\to} 0$  under either  $\pi = \pi_0$  or  $\pi = \pi_1$ .

For the remainder of this section we examine the quantity  $\rho_{g}$  in (3.12). We may view  $\rho_{g}$  either as an intra-cluster correlation of  $w_{ij}$  as in (3.7) or as a measure of homogeneity of the  $\nabla_{g}(\mu)^{T}\mu_{i}$  as in (3.8). For example, if q = 1 and g is the identity function then  $t_{n}$  is the sample mean  $\overline{x}_{n}$  and  $\rho_{g}$  is the usual intra-cluster correlation of the  $x_{ij}$  which is a measure of homogeneity of the means  $\mu_{i}$  in the different clusters. In general, however, neither (3.7) or (3.8) are very easy to interpret because of their dependence on the rather artificial quantities  $w_{ij}$  and  $\nabla g(\mu)^{T}\mu_{i}$ . In order to obtain a more interpretable expression for  $\rho_{g}$  we impose a further condition on the distribution  $\pi_{1}$ . This condition is strictly only applicable under the model-based approach.

Referring to Definition 2.1 let  $F = E_{\pi}(F_i)$  be the marginal distribution of  $x_{ij}$ . We suppose each  $F_i$  is a mixture.

Condition C3:  $F_i = (1-\delta)F + \delta D_i$   $i = 1, \dots, k$ where  $0 \le \delta \le 1$  and  $D_1, \dots, D_k$  are IID distribution functions with  $E(D_i) = F$ .

One extreme  $\delta = 0$  then corresponds to  $\pi_0$  whilst the other extreme  $\delta = 1$  imposes no further structure on  $\pi_1$ . We shall suppose that  $\delta$  is small which we suggest is a natural non-parametric way of asserting that there is low intra-cluster correlation. This assumption may not be unreasonable in, for example, large-scale sample surveys where the clusters are geographical areas. In such surveys the intra-cluster correlation of variables is usually low (say < 0.1) by design, even though the design effect may be nonnegligible because of the value of  $m^*$ .

We need further regularity conditions.

<u>Condition C4</u>: The matrix  $H_g$  of second partial derivatives of g exists in a neighborhood of  $\mu$  and

$$\operatorname{ar}_{\pi} [(\mu(D_{i})-\mu)^{T}H_{\sigma}[\mu+\varepsilon(\mu(D_{i})-\mu)](\mu(D_{i})-\mu)]$$

is bounded as  $\varepsilon \neq 0$  where we use the functional notation  $\mu(D_{\underline{i}}) = \int x dD_{\underline{i}}(x)$  so that  $\mu_{\underline{i}} = \mu(P_{\underline{i}}), \ \mu = \mu(P).$ 

-13-

The following theorem gives an alternative approximate expression for  $\rho_g$  when the intra-cluster correlation is low.

# Theorem 3.6.

If C3 and C4 hold then as  $\delta \neq 0$ 

 $\rho_g = var_{\pi_1}[g(\mu_1)]/var_{\pi_1}(w_1) + O(\delta^3) = O(\delta^2)$ 

where  $\mu_i$  and  $w_{ij}$  are defined in (3.9) and (3.6).

Proof: Consider the Taylor Series expansion

$$g(\mu_{i}) = g(\mu) + \nabla_{g}^{T}(\mu)(\mu_{i}-\mu) + \frac{1}{2}(\mu_{i}-\mu)^{T}H_{g}(\mu^{*})(\mu_{i}-\mu)$$
(3.13)

where  $\mu^{+} = (1-\psi)\mu + \psi\mu_{i}$  and  $\psi$  is a scalar,  $0 \le \psi \le 1$ . Now

$$\mu_{i} - \mu = \mu(F_{i}) - \mu(F) = \mu(F_{i} - F)$$
  
=  $\mu[\delta(D_{i} - F)]$  from C3  
=  $\delta[\mu(D_{i}) - \mu]$  . (3.14)

The result follows by substituting (3.14) into (3.13) and using (3.8) and C4 with  $\varepsilon = \psi \delta$ .

The quantity  $g(u_i)$  is the cluster 'version' of  $t_n = g(\bar{x}_n)$  and  $\theta_1 = g(\mu)$ . For example, if  $t_n$  is the sample correlation coefficient and  $\theta_1$  is the population correlation coefficient then  $g(u_i)$  is the correlation coefficient in the i<sup>th</sup> cluster. A specific example is given in Section 4 where  $t_n$  is the sample variance and  $g(u_i)$  is the variance in the i<sup>th</sup> cluster. The quantity  $var(w_{ij})$  does not depend on the clustering in the population (in terms of C3 it depends on F but not on  $\delta$  or  $D_i$ ) and may be viewed as a standardizing quantity. Hence Theorem 3.6 permits  $\rho_g$  to be interpreted as a measure of homogeneity of the quantities  $g(u_i)$ , providing the overall level of intra-cluster correlation is 'low'. Combining with Theorem 3.5 suggests, for example, that the design effect of a sample correlation coefficient is mainly determined by the difference between the correlation coefficients within clusters.

#### 4. AN EXAMPLE: DESIGN EFFECT OF A SAMPLE VARIANCE

The low- $\delta$  approximation in Theorem 3.6 is examined here explicitly for the case where

$$t_n = n^{-1} \sum_{s} (y_{ij} - \overline{y}_n)^2 , \quad \overline{y}_n = n^{-1} \sum_{s} y_{ij}$$

We may write  $t_n$  in the form of (3.1) by letting q = 2,  $x_{ij} = (y_{ij}, y_{ij}^2)^T$ ,  $g[(x_1, x_2)^T] = x_2 - x_1^2$ . Hence  $\overline{x}_n = (\overline{y}_n, n^{-1} \sum y_{ij}^2)$ ,  $g(\overline{x}_n) = n^{-1} \sum y_{ij}^2 - \overline{y}_n^2 = t_n$ . Following (2.1) and (3.9) define the within-cluster and overall moments by

$$\mu_{i} = (\mu_{yi}, \mu_{yi}^{2} + \sigma_{yi}^{2})^{T} , \mu = (\mu_{y}, \mu_{y}^{2} + \sigma_{y}^{2})^{T} .$$

Then  $g(\mu_{i}) = (\mu_{yi}^{2} + \sigma_{yi}^{2}) - \mu_{yi}^{2} = \sigma_{yi}^{2}$  is the within-cluster variance corresponding to the sample variance  $t_{n}$  and the population variance  $g(\mu) = \sigma_{y}^{2}$ . Also  $\nabla_{g}(\mu) = (-2\mu_{y}, 1)$  obeys  $C2(\pi_{1})$  and from (3.6), up to an additive constant

$$w_{ij} = (y_{ij} - \mu_y)^2$$

The IID-based variance estimate of  $t_n$  given by (3.10) is

$$y_{g,0,n} = (n-1)^{-1} \sum_{\mathbf{x}} (w_{\mathbf{ij}}^* - \overline{w}^*)^2$$
 where  $w_{\mathbf{ij}}^* = (y_{\mathbf{ij}} - \overline{y}_n)^2$ ,  $\overline{w}^* = t_n$ .

The low- $\delta$  approximation to  $\rho_\sigma$  given by Theorem 3.6 is

$$\rho_{g}^{+} = var_{\pi_{1}}[g(\mu_{i})]/var_{\pi_{1}}(w_{ij}) = var_{\pi_{1}}(\sigma_{yi}^{2})/var_{\pi_{1}}(y_{ij}-\mu_{y})^{2}$$

which may be compared with the expression from (3.8)

$$\rho_{g} = var_{\pi_{1}} [\sigma_{yi}^{2} + (\mu_{yi} - \mu_{y})^{2}] / var_{\pi_{1}} (y_{ij} - \mu_{y})^{2} .$$

Hence we may write

$$g^{+} \leq \rho_{g} \leq \rho_{g}^{+} + 2(\eta \rho_{g}^{+})^{1/2} + \eta_{g}^{+}$$

where  $\eta = var_{\pi_1} [(\mu_{yi} - \mu_{y})^2] / var_{\pi_1} [(y_{ij} - \mu_{y})^2].$ 

Define the between-cluster and total coefficients of kurtosis by

$$\gamma_{B} = E_{\pi_{1}} (\mu_{yi} - \mu_{y})^{4} / [var_{\pi_{1}} (\mu_{yi})]^{2} - 3, \gamma = E_{\pi_{1}} (\gamma_{ij} - \mu_{y})^{4} / [var_{\pi_{1}} (\gamma_{ij})]^{2} - 3.$$
  
Let  $var(\mu_{i,i}) / var(\gamma_{i,i})$  be the conventional intra-cluster correlation of  $\gamma_{i,i}$ .

$$\eta = \frac{(2+\gamma_B)}{(2+\gamma)} \rho_y^2 .$$
 (4.1)

Then

Hence if  $\rho_g$  is small then n will be very small unless  $\gamma$  is very small (for example  $\gamma = -1.2$  for the very platykurtic uniform distribution) or  $\gamma_B$  is very large (for example  $\gamma_B = 6$  for the very leptokurtic exponential distribution). Thus if  $\rho_g$  is small and there is reasonable dispersion amongst the  $\sigma_i^2$  then  $\rho_g \stackrel{*}{=} \rho_g^+$  should be a fair

-15-

approximation. In terms of  $\delta$ , both  $\mu_{yi} \sim \mu_y$  and  $\sigma_{yi}^2 - E(\sigma_{yi}^2)$  are of  $O_{g_1}(\delta)$ ,  $\rho_g^+$ and  $\rho_y$  are of  $O(\delta^2)$  whilst  $\eta$  is of  $O(\delta^4)$ .

#### 5. UNEQUAL CLUSTER SIZES

The results in Sections 3 and 4 were based on the assumption of equal cluster sizes. If the  $M_i$  are unequal and Definition 2.1 (which does not involve the  $M_i$ ) still applies then these results will still hold (provided  $\{m_1, m_2, ...\}$  is a fixed bounded sequence).

From the design-based viewpoint, Definition 2.1 still holds under simple random sampling with replacement at both stages where the  $m_1$  are fixed and do not depend on the  $M_1$ .

From the model-based viewpoint, Definition 2.1 remains appropriate if the withincluster distributions  $F_{i}$  do not depend on the  $M_{i}$ . It does not matter here if the design p(S) is dependent on the  $M_{i}$  as for example in probability proportional to size sampling.

In general  $F_i$  may depend on  $H_i$  and the results of Section 3 will not hold. For example,  $\overline{x}_n$  may no longer even be a consistent estimator of  $\mu$ . A general discussion of inference under models for populations with unequal size clusters is given by Sundberg (1983). We suggest, however, that within strata, and in particular within size strata, our results should hold at least approximately. In fact, plots of  $\overline{x}_i = m_i^{-1} \sum_{j=1}^{m_i} x_{ij}$  and  $m_i^{-1} \sum_{j=1}^{m_i} (x_{ij} - \overline{x}_i)^2$  against  $M_i$  for various variables x and data sets in Skinner (1982) suggested little relation between  $F_i$  and  $M_i$ .

#### 6. DISCUSSION

Under given conditions, in particular when the number of sampled clusters is large, the design effect of two-stage sampling was shown in Theorem 3.5 to take the familiar form,  $1 + (m-1)\rho$ , for a broad class of statistics. This result has an interpretation both from the design-based viewpoint in terms of with replacement sampling and also from a model-

-16-

based viewpoint in terms of a fairly general non-parametric model for a clustered population.

For linear statistics, such as the sample mean,  $\rho$  may be interpreted as a measure of homogeneity of corresponding within cluster quantities, such as cluster means. For nonlinear statistics, such as the sample correlation coefficient, provided the overall level of intracluster correlation is not high, it was shown in Theorem 3.6 that  $\rho$  may also be interpreted as a measure of homogeneity of corresponding within cluster quantities, such as cluster correlation coefficients.

These results have rather negative implications for the existence of relations between design effects of multivariate and of univariate statistics as discussed at the end of Section 1. In general we conclude no necessary theoretical relation need hold. For example, the design effect of a correlation coefficient, being determined mainly by the heterogeneity of cluster correlations, has in general no necessary relation with the design effects of the means of the two variables, which are determined by the heterogeneity of the cluster means. Our conclusion agrees with that of Rao and Scott (1981) on the design effect involved in testing independence in a bivariate contingency table. They state that 'ideally we would like an approximation ... based on the marginal design effects' (that is the univariate design effects) but 'such an approximation does not seem possible in theory'.

Theoretical relations can be derived under restricted assumptions but such results can be misleading. For example, a regression model of y on z with errors correlated within clusters but regression slopes  $\beta$  constant across clusters is considered by Campbell (1977) and Scott and Holt (1982). They obtain the  $1 + (m-1)\rho$  result for the leastsquares estimator of the slope and show that  $\rho = \rho_z \rho_e$  where  $\rho_z$  and  $\rho_e$  are the intracluster correlations of z and of the residual  $e = y-\beta z$  respectively. Now if both  $\rho_z$ and  $\rho_e$  are small then  $\rho$  is very small which the authors take to correpond to Kish and Frankel's (1974) empirical observation that 'design effects for complex statistics tend to be less than those for means of the same variable'. However, this approach effectively assumes away the dominating  $O(\delta^2)$  term in Theorem 3.6 determined by the dispersion-

-17-

between cluster regression coefficients and just obtains the  $O(\delta^4)$  term analogous to n in (4.1). Hence we suggest the above formula could drastically underestimate the true design effect. Other examples of the application of Theorems 3.5 and 3.6 for specific statistics and under restricted assumptions are given in Skinner (1982).

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Rao and Scott (1981), following on from their statement above, suggest that 'it may be possible to find empirically-based approximations that work well in practice'. In another context, for example, Bebbington and Smith (1977) suggest an empirical relation between the design effect of a correlation coefficient and the minimum of the design effects of the corresponding means. The derivation of such empirical 'laws', whilst potentially useful, is no easy project without guidance from theory, given the infinite range of possible statistics, designs and population structures.

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