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Web-based supporting information for "Impact of unequal cluster sizes for GEE analyses of stepped wedge cluster randomized trials with binary outcomes"

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Web Appendix A. Analytical inverse for nested exchangeable correlation structure under unequal cluster-period sizes

As noted in Section 2 of the main article, the nested exchangeable correlation structure characterizing the i-th cluster over J periods can be expressed by

$$\mathbf{R}_i(\boldsymbol{\alpha}_{\text{NEX}}) = (1-\alpha_0)\mathbf{I}_{n_i} + (\alpha_0-\alpha_1)\oplus_{j=1}^J \mathbf{J}_{n_{ij}} + \alpha_1\mathbf{J}_{n_i},$$

where $\boldsymbol{\alpha}_{\text{NEX}} = (\alpha_0,\alpha_1)^T$, α_0 is the within-period ICC, α_1 is the between-period ICC, $\mathbf{I}_s$ is the $s\times s$ identity matrix, $\mathbf{J}_s$ is the $s\times s$ matrix of ones, $n_i=\sum_{j=1}^J n_{ij}$ is the i-th cluster size, and '$\oplus$' is the block diagonal operator. We provide a derivation of the closed-form inverse of $\mathbf{R}_i$, extending the analytical results in Li et al. (2019) for $J=2$. Let $\mathbf{A}=(1-\alpha_0)\mathbf{I}_{n_i}+(\alpha_0-\alpha_1)\oplus_{j=1}^J\mathbf{J}_{n_{ij}}$ and $\mathbf{B}=\alpha_1\mathbf{J}_{n_i}$, by Henderson and Searle (1981), the inverse is

$$\mathbf{R}_i^{-1} = (\mathbf{A}+\mathbf{B})^{-1} = \mathbf{A}^{-1} - \mathbf{A}^{-1}\mathbf{B}(\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})\mathbf{A}^{-1}.$$

It is easy to verify that the $\mathbf{A}^{-1}$ has similar basis matrices as $\mathbf{A}$ and can be expanded as $\mathbf{A}^{-1}=x\mathbf{I}_{n_i}+\oplus_{j=1}^J y_j\mathbf{J}_{n_{ij}}$. Since

$$\mathbf{I}_{n_i} = \mathbf{A}\mathbf{A}^{-1} = (1-\alpha_0)x\mathbf{I}_{n_i} + (\alpha_0-\alpha_1)x\oplus_{j=1}^J\mathbf{J}_{n_{ij}} + (1-\alpha_0)\oplus_{j=1}^J y_j\mathbf{J}_{n_{ij}} + (\alpha_0-\alpha_1)\oplus_{j=1}^J y_j n_{ij}\mathbf{J}_{n_{ij}},$$

we must have

$$x=\frac{1}{1-\alpha_0}\; y_j = -\frac{\alpha_0-\alpha_1}{(1-\alpha_0)\psi_j},$$

where $\psi_j = 1+(n_{ij}-1)\alpha_0 - n_{ij}\alpha_1$. Since

$$\mathbf{A}^{-1} = \frac{1}{1-\alpha_0}\mathbf{I}_{n_i} - \frac{\alpha_0-\alpha_1}{(1-\alpha_0)}\oplus_{j=1}^J\frac{1}{\psi_j}\mathbf{J}_{n_{ij}},$$

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then

$$\mathbf{A}^{-1}\mathbf{B} = \frac{\alpha_1}{1-\alpha_0}\mathbf{J}_{n_i} - \frac{(\alpha_0-\alpha_1)\alpha_1}{(1-\alpha_0)}\Big(\oplus_{j=1}^J \frac{n_{ij}}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{ni} = \Big(\oplus_{j=1}^J \frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}.$$

Observe that

$$\mathbf{I}_{n_i} + \mathbf{A}^{-1}\mathbf{B} = \mathbf{I}_{n_i} + \Big(\oplus_{j=1}^J \frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i},$$

whose inverse shares the same basis matrices, and are denoted by $(\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})^{-1} = \mathbf{I}_{n_i}+[\oplus_{j=1}^J z_j\mathbf{I}_{n_{ij}}]\mathbf{J}_{n_i}$. Observe that

$$\begin{aligned}\mathbf{I}_{n_i} &= (\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})(\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})^{-1}\\ &= \mathbf{I}_{n_i} + \Big(\oplus_{j=1}^J z_j\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i} + \alpha_1\Big(\oplus_{j=1}^J \frac{1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i} + \alpha_1\Big(\oplus_{j=1}^J \frac{1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^J z_j\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\\ &= \mathbf{I}_{n_i} + \mathbf{C}.\end{aligned}$$

The (j,j)-th block of $\mathbf{C}$ is

$$0 = z_j\mathbf{J}_{n_{ij}} + \frac{\alpha_1}{\psi_j}\mathbf{J}_{n_{ij}} + \frac{\alpha_1}{\psi_j}\Big(\sum_{s=1}^J n_{is}z_s\Big)\mathbf{J}_{n_{ij}},$$

which implies

$$z_j + \frac{\alpha_1}{\psi_j} + \frac{\alpha_1}{\psi_j}\Big(\sum_{s=1}^J n_{is}z_s\Big) = 0.$$

Although the above condition is derived solely based on the diagonal block information, it turns out to be sufficient to ensure that $\mathbf{C} = 0$, and we can solve for z_j by noting that

$$\sum_{j=1}^J n_{ij}z_j + \sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j} + \Big(\sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j}\Big)\Big(\sum_{s=1}^J n_{is}z_s\Big) = 0,$$

and

$$\sum_{j=1}^J n_{ij}z_j = -\sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j}\Big/\Big(1+\sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j}\Big).$$

Define

$$\gamma_j = \psi_j\Big(1+\sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j}\Big),$$

we have,

$$z_j = -\frac{\alpha_1}{\psi_j}\Big(1+\sum_{j=1}^J n_{ij}z_j\Big) = -\frac{\alpha_1}{\psi_j}\Big/\Big(1+\sum_{j=1}^J \frac{n_{ij}\alpha_1}{\psi_j}\Big) = -\frac{\alpha_1}{\gamma_j}.$$

Then

$$\begin{aligned}(\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})^{-1}\mathbf{A}^{-1} &= \Big[\mathbf{I}_{n_i} - \Big(\oplus_{j=1}^J \frac{\alpha_1}{\gamma_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big]\Big[\frac{1}{1-\alpha_0}\mathbf{I}_{n_i} - \oplus_{j=1}^J \frac{\alpha_0-\alpha_1}{(1-\alpha_0)\psi_j}\mathbf{J}_{n_{ij}}\Big]\\ &= \frac{1}{1-\alpha_0}\mathbf{I}_{n_i} - \Big(\oplus_{j=1}^J \frac{\alpha_1}{(1-\alpha_0)\gamma_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i} - \oplus_{j=1}^J \frac{\alpha_0-\alpha_1}{(1-\alpha_0)\psi_j}\mathbf{J}_{n_{ij}}\\ &\quad + \Big(\oplus_{j=1}^J \frac{\alpha_1}{(1-\alpha_0)\gamma_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^J \frac{n_{ij}(\alpha_0-\alpha_1)}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\\ &= \frac{1}{1-\alpha_0}\mathbf{I}_{n_i} - \oplus_{j=1}^J \frac{\alpha_0-\alpha_1}{(1-\alpha_0)\psi_j}\mathbf{J}_{n_{ij}} - \Big(\oplus_{j=1}^J \frac{\alpha_1}{\gamma_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^J \frac{1}{\psi_j}\mathbf{I}_{n_{ij}}\Big).\end{aligned}$$

Further, routine calculations show that

$$\begin{aligned}
\mathbf{A}^{-1}\mathbf{B}(\mathbf{I}_{n_i}+\mathbf{A}^{-1}\mathbf{B})^{-1}\mathbf{A}^{-1} &= \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{(1-\alpha_0)\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i} - \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{n_{ij}(\alpha_0-\alpha_1)}{(1-\alpha_0)\psi_j}\mathbf{I}_{n_{ij}}\Big)\\
&\quad -\Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\gamma_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\\
&= \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{(1-\alpha_0)\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i} - \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{n_{ij}(\alpha_0-\alpha_1)}{(1-\alpha_0)\psi_j}\mathbf{I}_{n_{ij}}\Big)\\
&\quad -\Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\sum_{s=1}^{J}\frac{n_{is}\alpha_1}{\gamma_s}\Big)\Big(\oplus_{j=1}^{J}\frac{1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\\
&= \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{1-\alpha_1\sum_{s=1}^{J}n_{is}/\gamma_s}{\psi_j}\mathbf{I}_{n_{ij}}\Big).
\end{aligned}$$

The inverse is then given in closed form by

$$\mathbf{R}_i^{-1} = \frac{1}{1-\alpha_0}\mathbf{I}_{n_i} - \frac{\alpha_0-\alpha_1}{(1-\alpha_0)}\oplus_{j=1}^{J}\frac{1}{\psi_j}\mathbf{J}_{n_{ij}} - \Big(\oplus_{j=1}^{J}\frac{\alpha_1}{\psi_j}\mathbf{I}_{n_{ij}}\Big)\mathbf{J}_{n_i}\Big(\oplus_{j=1}^{J}\frac{1-\alpha_1\sum_{s=1}^{J}n_{is}/\gamma_s}{\psi_j}\mathbf{I}_{n_{ij}}\Big). \quad (1)$$

Li et al. (2019) derived a special case of (1) with $J = 2$ periods, and the above expression is more general and for any positive integer J. Furthermore, when $n_{ij} = n_{is} = m$ for all $j \neq s$, we have $\psi_j = \psi = 1 + (m-1)\alpha_0 - m\alpha_1$, and $\gamma_j = \gamma = \psi + Jm\alpha_1 = 1 + (m-1)\alpha_0 + m(J-1)\alpha_1$, which are two eigenvalues of the nested exchangeable correlation matrix with equal cluster-period sizes (Li et al., 2018). In this case, the inverse reduces to the formula presented in Teerenstra et al. (2010).

Web Appendix B. Proof of theorem 3.1

In this section, Theorem 3.1 in the main article will be proved. We first show the derivation of the GEE estimator of treatment effect under the working independence assumption.

Consider the cluster-period GEE

$$\overline{\mathbf{U}}(\boldsymbol{\theta}) = \sum_{i=1}^{I}\overline{\mathbf{D}}_i^T\widetilde{\mathbf{V}}_i^{-1}(\overline{\mathbf{Y}}_i - \overline{\boldsymbol{\mu}}_i) = 0$$

where $\overline{\mathbf{Y}}_i = (\overline{Y}_{i1},\ldots,\overline{Y}_{iJ})^T$, $\overline{\boldsymbol{\mu}}_i = (\mu_{i1},\ldots,\mu_{iJ})^T$, $\overline{\mathbf{D}}_i = \partial\overline{\boldsymbol{\mu}}_i/\partial\boldsymbol{\theta}^T$, $\boldsymbol{\theta} = (\beta_1,\ldots,\beta_J,\delta)^T$.

Under the working independence assumption, $\widetilde{\mathbf{V}}_i$ is a diagonal matrix of the variance functions $\nu_{i1},\ldots,\nu_{iJ}$ corresponding to each period within cluster i. Therefore, the independence GEE can be written as

$$\sum_{i=1}^{I}\overline{\mathbf{D}}_i^T\widetilde{\mathbf{V}}_i^{-1}(\overline{\mathbf{Y}}_i - \overline{\boldsymbol{\mu}}_i) = \sum_{i=1}^{I}\sum_{j=1}^{J}\begin{pmatrix}\mathbf{e}_j\\ X_{ij}\end{pmatrix}(\overline{Y}_{ij} - \mu_{ij}) = 0$$

where $\mathbf{e}_j$ is a sparse vector whose j^{th} element is one.

Then, the estimated parameter set $\hat{\boldsymbol{\theta}} = (\hat{\beta}_1,\ldots,\hat{\beta}_J,\hat{\delta})^T$ satisfies the system below.

$$\sum_{i=1}^{I}\left\{\overline{Y}_{ij} - \frac{\exp(\hat{\beta}_j + X_{ij}\hat{\delta})}{1+\exp(\hat{\beta}_j + X_{ij}\hat{\delta})}\right\} = 0 \text{ for } j = 1,\ldots,J$$

$$\sum_{i=1}^{I}\sum_{j=1}^{J} X_{ij}\left\{\overline{Y}_{ij} - \frac{\exp(\hat{\beta}_j + X_{ij}\hat{\delta})}{1+\exp(\hat{\beta}_j + X_{ij}\hat{\delta})}\right\} = 0$$

Given the binary value of the treatment indicator X_{ij}, we modify the system into

$$\sum_{i=1}^{I} X_{ij}\left\{\overline{Y}_{ij} - \frac{\exp(\hat{\beta}_j + \hat{\delta})}{1+\exp(\hat{\beta}_j + \hat{\delta})}\right\} + \sum_{i=1}^{I}(1-X_{ij})\left\{\overline{Y}_{ij} - \frac{\exp(\hat{\beta}_j)}{1+\exp(\hat{\beta}_j)}\right\} = 0 \text{ for } j = 1, \ldots, J$$

$$\sum_{i=1}^{I}\sum_{j=1}^{J} X_{ij}\left\{\overline{Y}_{ij} - \frac{\exp(\hat{\beta}_j + \hat{\delta})}{1+\exp(\hat{\beta}_j + \hat{\delta})}\right\} = 0$$

When restricting the scenario to SWDs with 3 periods, the equation system that solve $\hat{\boldsymbol{\theta}}$ can be written as the system followed.

$$\sum_{i=1}^{I}\left\{\overline{Y}_{i1} - \frac{\exp(\hat{\beta}_1)}{1+\exp(\hat{\beta}_1)}\right\} = 0$$

$$\sum_{i=1}^{I} X_{i2}\left\{\overline{Y}_{i2} - \frac{\exp(\hat{\beta}_2 + \hat{\delta})}{1+\exp(\hat{\beta}_2 + \hat{\delta})}\right\} + \sum_{i=1}^{I}(1-X_{i2})\left\{\overline{Y}_{i2} - \frac{\exp(\hat{\beta}_2)}{1+\exp(\hat{\beta}_2)}\right\} = 0$$

$$\sum_{i=1}^{I}\left\{\overline{Y}_{i3} - \frac{\exp(\hat{\beta}_3 + \hat{\delta})}{1+\exp(\hat{\beta}_3 + \hat{\delta})}\right\} = 0$$

$$\sum_{i=1}^{I} X_{i2}\left\{\overline{Y}_{i2} - \frac{\exp(\hat{\beta}_2 + \hat{\delta})}{1+\exp(\hat{\beta}_2 + \hat{\delta})}\right\} + \sum_{i=1}^{I}\left\{\overline{Y}_{i3} - \frac{\exp(\hat{\beta}_3 + \hat{\delta})}{1+\exp(\hat{\beta}_3 + \hat{\delta})}\right\} = 0$$

These solve

$$\frac{\exp(\hat{\beta}_2)}{1+\exp(\hat{\beta}_2)} = \frac{\sum_{i=1}^{I}(1-X_{i2})\overline{Y}_{i2}}{\sum_{i=1}^{I}(1-X_{i2})}$$

$$\frac{\exp(\hat{\beta}_2 + \hat{\delta})}{1+\exp(\hat{\beta}_2 + \hat{\delta})} = \frac{\sum_{i=1}^{I} X_{i2}\overline{Y}_{i2}}{\sum_{i=1}^{I} X_{i2}}$$

$$\hat{\delta} = logit\left\{\frac{\sum_{i=1}^{I} X_{i2}\overline{Y}_{i2}}{\sum_{i=1}^{I} X_{i2}}\right\} - logit\left\{\frac{\sum_{i=1}^{I}(1-X_{i2})\overline{Y}_{i2}}{\sum_{i=1}^{I}(1-X_{i2})}\right\}$$

When restricting the scenario to SWDs with 4 periods, the similar equation system can be written as

$$\sum_{i=1}^{I}\left\{\overline{Y}_{i1} - \frac{\exp(\hat{\beta}_1)}{1+\exp(\hat{\beta}_1)}\right\} = 0$$

$$\sum_{i=1}^{I} X_{i2}\left\{\overline{Y}_{i2} - \frac{\exp(\hat{\beta}_2 + \hat{\delta})}{1+\exp(\hat{\beta}_2 + \hat{\delta})}\right\} + \sum_{i=1}^{I}(1-X_{i2})\left\{\overline{Y}_{i2} - \frac{\exp(\hat{\beta}_2)}{1+\exp(\hat{\beta}_2)}\right\} = 0$$

$$\sum_{i=1}^{I} X_{i3}\left\{\overline{Y}_{i3} - \frac{\exp(\hat{\beta}_3 + \hat{\delta})}{1+\exp(\hat{\beta}_3 + \hat{\delta})}\right\} + \sum_{i=1}^{I}(1-X_{i3})\left\{\overline{Y}_{i3} - \frac{\exp(\hat{\beta}_3)}{1+\exp(\hat{\beta}_3)}\right\} = 0$$

$$\sum_{i=1}^{I}\left\{\overline{Y}_{i4}-\frac{\exp(\hat{\beta}_4+\hat{\delta})}{1+\exp(\hat{\beta}_4+\hat{\delta})}\right\}=0$$

$$\sum_{i=1}^{I}X_{i2}\left\{\overline{Y}_{i2}-\frac{\exp(\hat{\beta}_2+\hat{\delta})}{1+\exp(\hat{\beta}_2+\hat{\delta})}\right\}+\sum_{i=1}^{I}X_{i3}\left\{\overline{Y}_{i3}-\frac{\exp(\hat{\beta}_3+\hat{\delta})}{1+\exp(\hat{\beta}_3+\hat{\delta})}\right\}+\sum_{i=1}^{I}\left\{\overline{Y}_{i4}-\frac{\exp(\hat{\beta}_4+\hat{\delta})}{1+\exp(\hat{\beta}_4+\hat{\delta})}\right\}=0$$

By using the same strategy of reparameterizing the inverse logistic function (expit) terms and constructing a system of linear functions, we no longer gets sufficient number of equations to easily solve $\hat{\delta}$. The same problem makes it hard to handle all relevant cases with the number of periods larger than three.

Then, we show the derivation of the closed form variance of the treatment effect estimator under working independence. We first consider the case that the true correlation structure is nested exchangeable. The outcomes of interest can be continuous, binary, or count outcomes, which implies the canonical link function in the marginal model (2).

$$g(\mu_{ijk})=\beta_j+X_{ij}\delta \tag{2}$$

Assume there are I clusters being involved in a cross-sectional stepped wedge trial with 3 periods. I_1 clusters are randomized to the first treatment sequence where the clusters receive treatment in the second and the third period, and I_2 clusters are randomized to the second treatment sequence where the clusters would receive treatment only in the third period.

Theorem 2.1 in the main article shows the sandwich variance estimator of the parameter set $\boldsymbol{\theta}=(\beta_1,\beta_2,\beta_3,\delta)^T$ has the form

$$\overline{\boldsymbol{\Sigma}}_1^{-1}\overline{\boldsymbol{\Sigma}}_0\overline{\boldsymbol{\Sigma}}_1^{-1}=\left\{\sum_{i=1}^{I}\overline{\mathbf{D}}_i^T\widetilde{\mathbf{V}}_i^{-1}\overline{\mathbf{D}}_i\right\}^{-1}\overline{\boldsymbol{\Sigma}}_0\left\{\sum_{i=1}^{I}\overline{\mathbf{D}}_i^T\widetilde{\mathbf{V}}_i^{-1}\overline{\mathbf{D}}_i\right\}^{-1}$$

where $\overline{\mathbf{D}}_i=\partial\overline{\boldsymbol{\mu}}_i/\partial\boldsymbol{\theta}^T$, $\widetilde{\mathbf{V}}_i$ is the working covariance matrix for the cluster-period means, and

$$\overline{\boldsymbol{\Sigma}}_0=\sum_{i=1}^{I}\overline{\mathbf{D}}_i^T\widetilde{\mathbf{V}}_i^{-1}\mathrm{cov}(\overline{\mathbf{Y}}_i)\widetilde{\mathbf{V}}_i^{-1}\overline{\mathbf{D}}_i$$

. When the working correlation is independence,

$$\widetilde{\mathbf{V}}_i=\begin{pmatrix}\mathrm{var}(\overline{Y}_{i1}) & 0 & 0\\ 0 & \mathrm{var}(\overline{Y}_{i2}) & 0\\ 0 & 0 & \mathrm{var}(\overline{Y}_{i3})\end{pmatrix}=\begin{pmatrix}\frac{\phi\nu_{i1}}{n_{i1}} & 0 & 0\\ 0 & \frac{\phi\nu_{i2}}{n_{i2}} & 0\\ 0 & 0 & \frac{\phi\nu_{i3}}{n_{i3}}\end{pmatrix}$$

where for $j=1,2,3$, n_{ij} is the cluster-period sizes for the (i,j)-th cluster, ϕ is the dispersion parameter, and ν_{ij} is the variance function related to the mean for the (i,j)-th cluster. For continuous outcomes, $\nu_{ij}=1$. For binary outcomes, $\nu_{ij}=\mu_{ij}(1-\mu_{ij})$. For count outcomes, $\nu_{ij}=\mu_{ij}$. We can also write out the inverse of the working covariance matrix as

$$\widetilde{\mathbf{V}}_i^{-1}=\begin{pmatrix}\frac{n_{i1}}{\phi\nu_{i1}} & 0 & 0\\ 0 & \frac{n_{i2}}{\phi\nu_{i2}} & 0\\ 0 & 0 & \frac{n_{i3}}{\phi\nu_{i3}}\end{pmatrix}.$$

Besides, the true covariance structure for the cluster-period means is

$$\mathrm{cov}(\overline{\mathbf{Y}}_i)=\begin{pmatrix}\frac{\phi\nu_{i1}}{n_{i1}}[1+(n_{i1}-1)\alpha_0] & \sqrt{\nu_{i1}\nu_{i2}}\phi\alpha_1 & \sqrt{\nu_{i1}\nu_{i3}}\phi\alpha_1\\ \sqrt{\nu_{i1}\nu_{i2}}\phi\alpha_1 & \frac{\phi\nu_{i2}}{n_{i2}}[1+(n_{i2}-1)\alpha_0] & \sqrt{\nu_{i2}\nu_{i3}}\phi\alpha_1\\ \sqrt{\nu_{i1}\nu_{i3}}\phi\alpha_1 & \sqrt{\nu_{i2}\nu_{i3}}\phi\alpha_1 & \frac{\phi\nu_{i3}}{n_{i3}}[1+(n_{i3}-1)\alpha_0]\end{pmatrix}.$$

For $i = 1, 2, ..., I_1$, we then have

$$\overline{\mathbf{D}}_i = \begin{pmatrix} \nu_{i1} & 0 & 0 & 0 \\ 0 & \nu_{i2} & 0 & \nu_{i2} \\ 0 & 0 & \nu_{i3} & \nu_{i3} \end{pmatrix}$$

Alternatively, for $i = I_1 + 1, I_1 + 2, ..., I_1 + I_2$,

$$\overline{\mathbf{D}}_i = \begin{pmatrix} \nu_{i1} & 0 & 0 & 0 \\ 0 & \nu_{i2} & 0 & 0 \\ 0 & 0 & \nu_{i3} & \nu_{i3} \end{pmatrix}$$

Thus, the matrix expression of $\overline{\mathbf{D}}_i^T \widetilde{\mathbf{V}}_i^{-1} \overline{\mathbf{D}}_i$ for cluster i is either

$$\begin{pmatrix} n_{i1}\nu_{i1} & 0 & 0 & 0 \\ 0 & n_{i2}\nu_{i2} & 0 & n_{i2}\nu_{i2} \\ 0 & 0 & n_{i3}\nu_{i3} & n_{i3}\nu_{i3} \\ 0 & n_{i2}\nu_{i2} & n_{i3}\nu_{i3} & n_{i2}\nu_{i2} + n_{i3}\nu_{i3} \end{pmatrix}$$

or

$$\begin{pmatrix} n_{i1}\nu_{i1} & 0 & 0 & 0 \\ 0 & n_{i2}\nu_{i2} & 0 & 0 \\ 0 & 0 & n_{i3}\nu_{i3} & n_{i3}\nu_{i3} \\ 0 & 0 & n_{i3}\nu_{i3} & n_{i3}\nu_{i3} \end{pmatrix}$$

determined by the step in which the cluster is randomized.

Similarly, there are two forms of $\overline{\mathbf{D}}_i^T \widetilde{\mathbf{V}}_i^{-1} \text{cov}(\overline{\mathbf{Y}}_i) \widetilde{\mathbf{V}}_i^{-1} \overline{\mathbf{D}}_i$, depending on the value of i, namely,

$$\frac{1}{\phi} \begin{pmatrix} \nu_{i1} n_{i1}[1 + (n_{i1} - 1)\alpha_0] & \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2} \alpha_1 & \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 & A_1 \\ \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2} \alpha_1 & \nu_{i2} n_{i2}[1 + (n_{i2} - 1)\alpha_0] & \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 & A_2 \\ \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 & \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 & \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] & A_3 \\ A_1 & A_2 & A_3 & A_4 \end{pmatrix}$$

where

$$\begin{aligned} A_1 &= (\sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2} + \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3})\alpha_1 \\ A_2 &= \nu_{i2} n_{i2}[1 + (n_{i2} - 1)\alpha_0] + \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 \\ A_3 &= \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 + \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] \\ A_4 &= \nu_{i2} n_{i2}[1 + (n_{i2} - 1)\alpha_0] + \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] + 2\sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1, \end{aligned}$$

or

$$\frac{1}{\phi} \begin{pmatrix} \nu_{i1} n_{i1}[1 + (n_{i1} - 1)\alpha_0] & \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2} \alpha_1 & \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 & \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 \\ \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2} \alpha_1 & \nu_{i2} n_{i2}[1 + (n_{i2} - 1)\alpha_0] & \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 & \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 \\ \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 & \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 & \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] & \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] \\ \sqrt{v_{i1}\nu_{i3}} n_{i1} n_{i3} \alpha_1 & \sqrt{v_{i2}\nu_{i3}} n_{i2} n_{i3} \alpha_1 & \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] & \nu_{i3} n_{i3}[1 + (n_{i3} - 1)\alpha_0] \end{pmatrix}.$$

To express $\overline{\mathbf{\Sigma}}_1$ and $\overline{\mathbf{\Sigma}}_0$ more succinctly, we define a series of notation as followed.

$$
\begin{aligned}
a &= \sum_{i=1}^{I} \nu_{i1} n_{i1}/\phi,\ a_1 = \sum_{i=1}^{I_1} \nu_{i1} n_{i1}/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i1} n_{i1}/\phi \\
b &= \sum_{i=1}^{I} \frac{\nu_{i2} n_{i2}}{\phi},\ b_1 = \sum_{i=1}^{I_1} \nu_{i2} n_{i2}/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i2} n_{i2}/\phi \\
c &= \sum_{i=1}^{I} \frac{\nu_{i3} n_{i3}}{\phi},\ c_1 = \sum_{i=1}^{I_1} \nu_{i3} n_{i3}/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i3} n_{i3}/\phi \\
d &= \sum_{i=1}^{I} \nu_{i1} n_{i1}(n_{i1}-1)/\phi,\ d_1 = \sum_{i=1}^{I_1} \nu_{i1} n_{i1}(n_{i1}-1)/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i1} n_{i1}(n_{i1}-1)/\phi \\
e &= \sum_{i=1}^{I} \nu_{i2} n_{i2}(n_{i2}-1)/\phi,\ e_1 = \sum_{i=1}^{I_1} \nu_{i2} n_{i2}(n_{i2}-1)/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i2} n_{i2}(n_{i2}-1)/\phi \\
f &= \sum_{i=1}^{I} \nu_{i3} n_{i3}(n_{i3}-1)/\phi,\ f_1 = \sum_{i=1}^{I_1} \nu_{i3} n_{i3}(n_{i3}-1)/\phi = \sum_{i=1}^{I} X_{i2}\nu_{i3} n_{i3}(n_{i3}-1)/\phi \\
x &= \sum_{i=1}^{I} \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2}/\phi,\ x_1 = \sum_{i=1}^{I_1} \sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2}/\phi = \sum_{i=1}^{I} X_{i2}\sqrt{\nu_{i1}\nu_{i2}} n_{i1} n_{i2}/\phi \\
y &= \sum_{i=1}^{I} \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3}/\phi,\ y_1 = \sum_{i=1}^{I_1} \sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3}/\phi = \sum_{i=1}^{I} X_{i2}\sqrt{\nu_{i1}\nu_{i3}} n_{i1} n_{i3}/\phi \\
z &= \sum_{i=1}^{I} \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3}/\phi,\ z_1 = \sum_{i=1}^{I_1} \sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3}/\phi = \sum_{i=1}^{I} X_{i2}\sqrt{\nu_{i2}\nu_{i3}} n_{i2} n_{i3}/\phi,
\end{aligned}
$$

where X_{i2} denotes the indicator of whether the i-th cluster receives treatment during the second time period. Then, $\overline{\mathbf{\Sigma}}_1$ and $\overline{\mathbf{\Sigma}}_0$ can be expressed as

$$
\overline{\mathbf{\Sigma}}_1 = \begin{pmatrix} a & 0 & 0 & 0 \\ 0 & b & 0 & b_1 \\ 0 & 0 & c & c \\ 0 & b_1 & c & b_1 + c \end{pmatrix}
$$

and

$$
\overline{\mathbf{\Sigma}}_0 = \begin{pmatrix} a + d\alpha_0 & x\alpha_1 & y\alpha_1 & (x_1 + y)\alpha_1 \\ x\alpha_1 & b + e\alpha_0 & z\alpha_1 & b_1 + e_1\alpha_0 + z\alpha_1 \\ y\alpha_1 & z\alpha_1 & c + f\alpha_0 & c + f\alpha_0 + z_1\alpha_1 \\ (x_1 + y)\alpha_1 & b_1 + e_1\alpha_0 + z\alpha_1 & c + f\alpha_0 + z_1\alpha_1 & b_1 + c + e_1\alpha_0 + f\alpha_0 + 2z_1\alpha_1 \end{pmatrix}.
$$

Each cells of the matrix product $\overline{\mathbf{\Sigma}}_1^{-1}\overline{\mathbf{\Sigma}}_0\overline{\mathbf{\Sigma}}_1^{-1}$ can be obtained from direct calculation. Therefore, the sandwich variance of $\hat{\delta}$, which is the cell located at the bottom right corner of the obtained matrix can be calculated. The simplified variance of the treatment effect estimator is therefore given by

$$
\text{var}(\hat{\delta}) = \frac{(b_1^2 e - 2b_1^2 b e_1 + b^2 e_1)\alpha_0 + b_1 b^2 - b_1^2 b}{(b_1 b - b_1^2)^2} \tag{3}
$$

Likewise, when the true correlation structure is exponential decay, the same steps can be followed to derive the variance of the treatment effect estimator. The only difference there is that we have an alternative form of $\overline{\mathbf{\Sigma}}_0$ computed as following.

$$\begin{pmatrix} a+d\alpha_0 & x\alpha_0\rho & y\alpha_0\rho^2 & x_1\alpha_0\rho+y\alpha_0\rho^2 \\ x\alpha_0\rho & b+e\alpha_0 & z\alpha_0\rho & b_1+e_1\alpha_0+z\alpha_0\rho \\ y\alpha_0\rho^2 & z\alpha_0\rho & c+f\alpha_0 & c+f\alpha_0+z_1\alpha_0\rho \\ x_1\alpha_0\rho+y\alpha_0\rho^2 & b_1+e_1\alpha_0+z\alpha_0\rho & c+f\alpha_0+z_1\alpha_0\rho & b_1+c+e_1\alpha_0+f\alpha_0+2z_1\alpha_0\rho \end{pmatrix}$$

The simplified variance of treatment effect is exactly the same as (3) and thus omitted here for brevity.

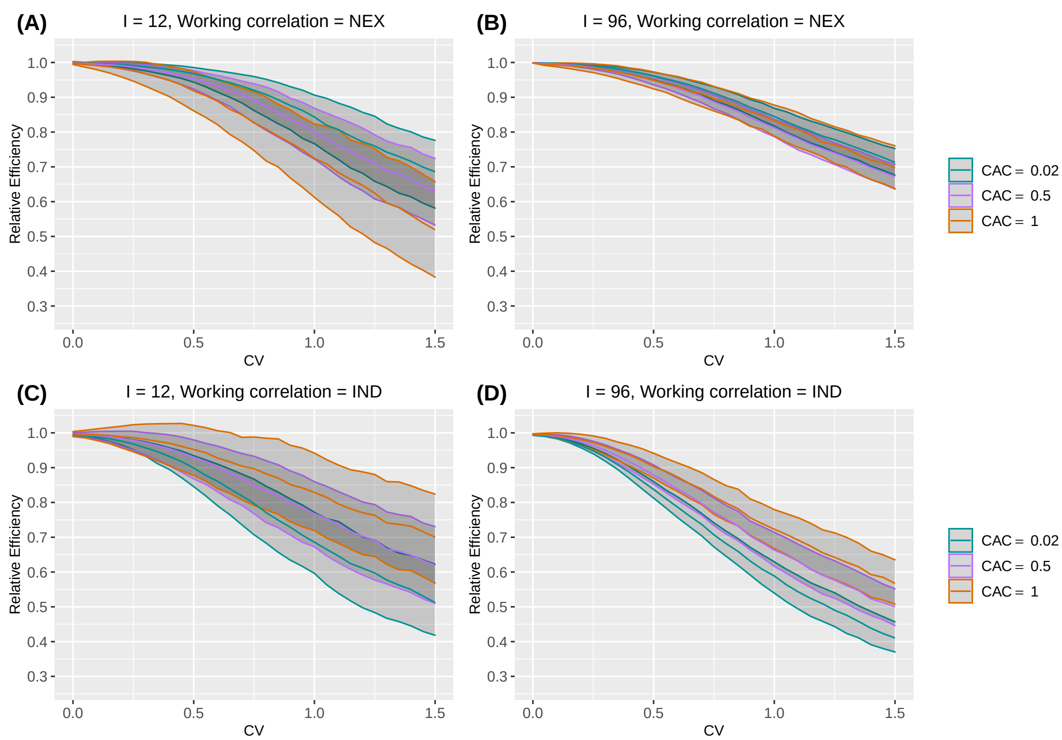
Web Appendix C. Supplementary simulation results under nested exchangeable true correlation structure

In this section, supplementary figures and tables related to the simulation results under the nested exchangeable true correlation structure are showed.

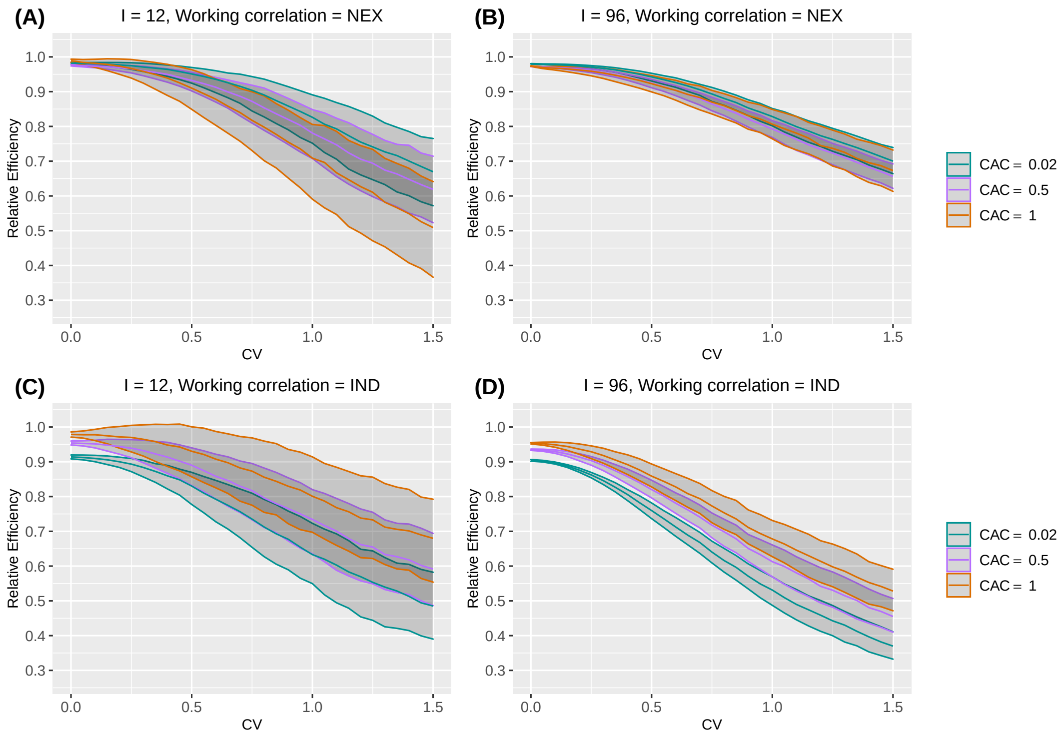
Web Appendix C.1. Cluster size variability

The following Figures are the counterparts to Figure 3 in the main text when pattern 1, 2, and 3 within-cluster imbalance are introduced.

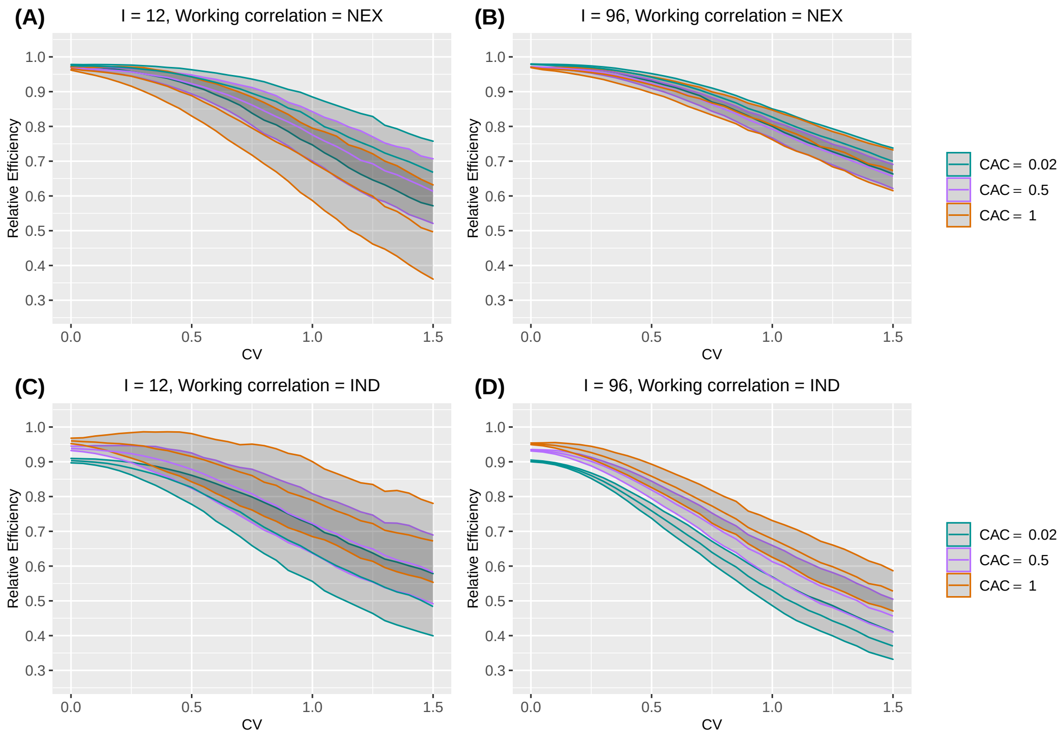
Web Figure 1 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 1: constant) is introduced.



Web Figure 2 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



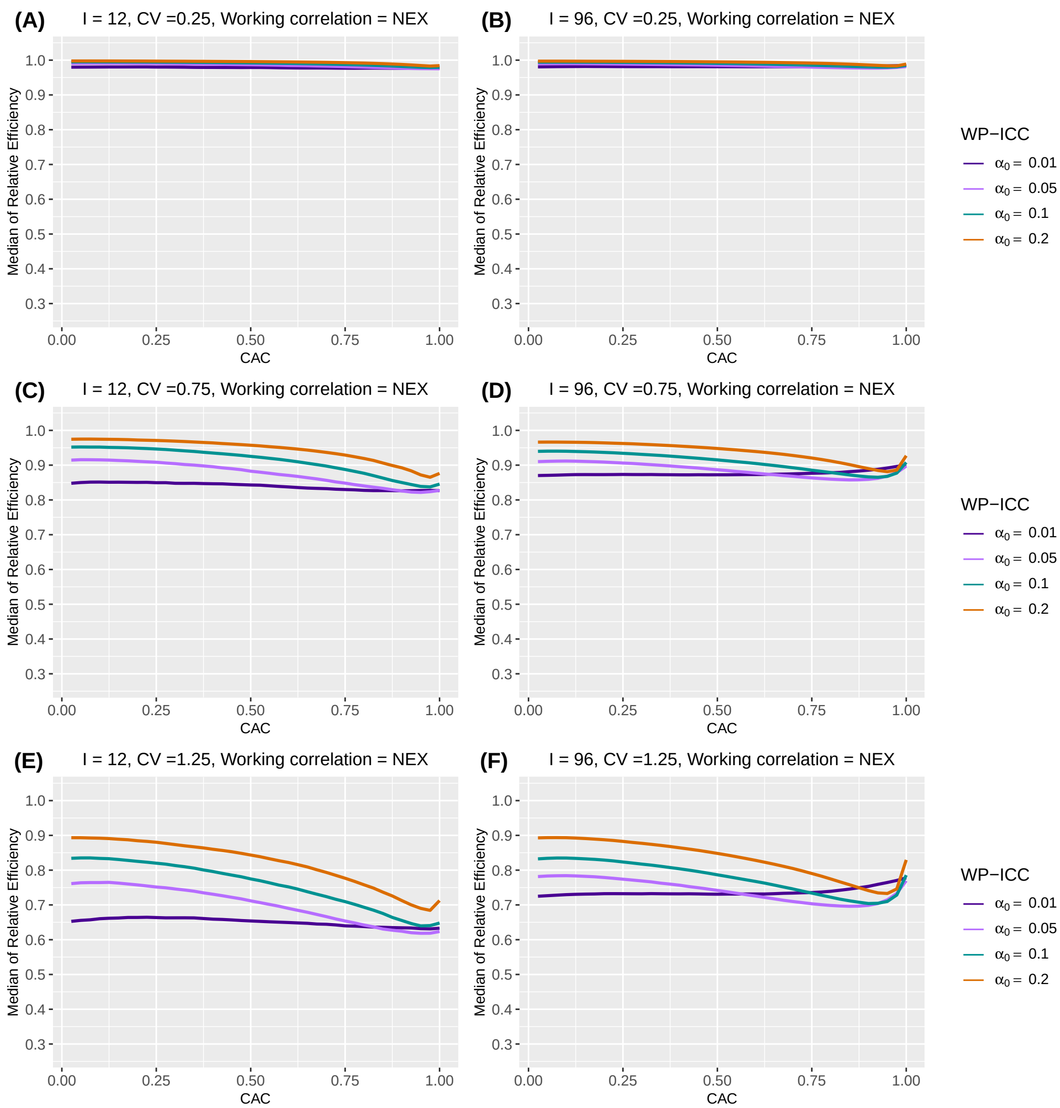
Web Figure 3 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



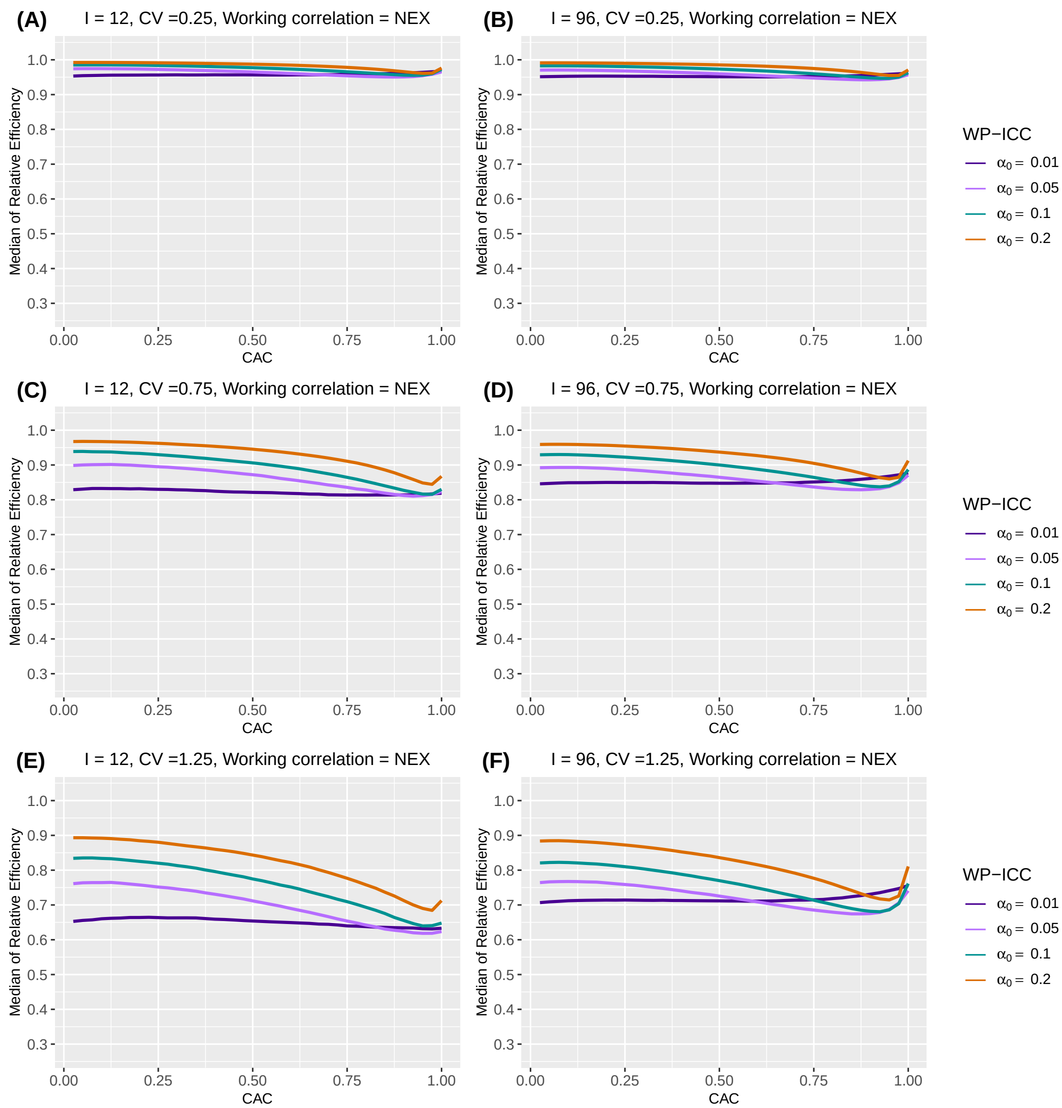
Web Appendix C.2. Intraclass correlation coefficients

The following Figures shows the counterparts to Figure 4 and 5 in the main article when pattern 1, 2, 3, and 4 within-cluster imbalance are introduced.

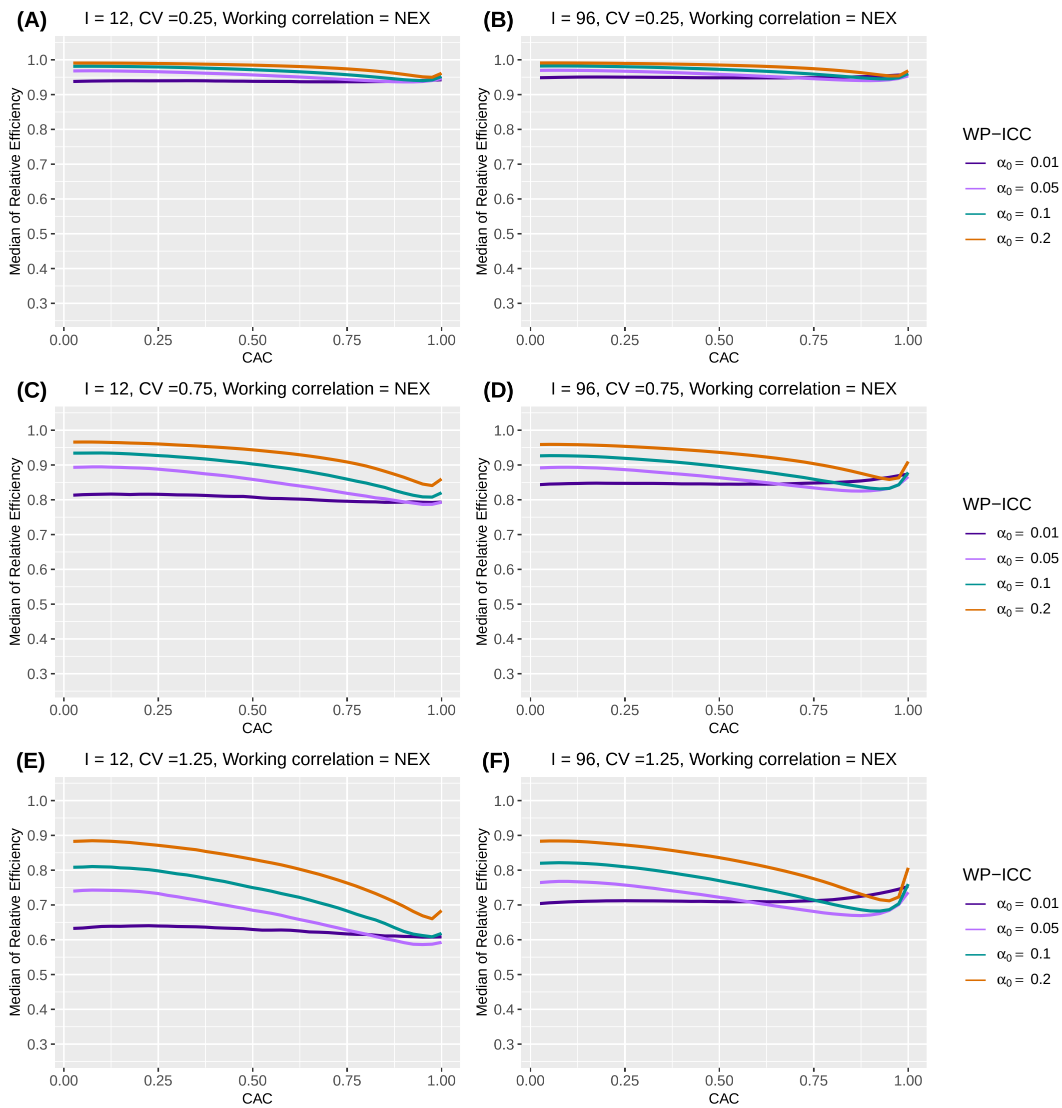
Web Figure 4 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 1: constant) is introduced.



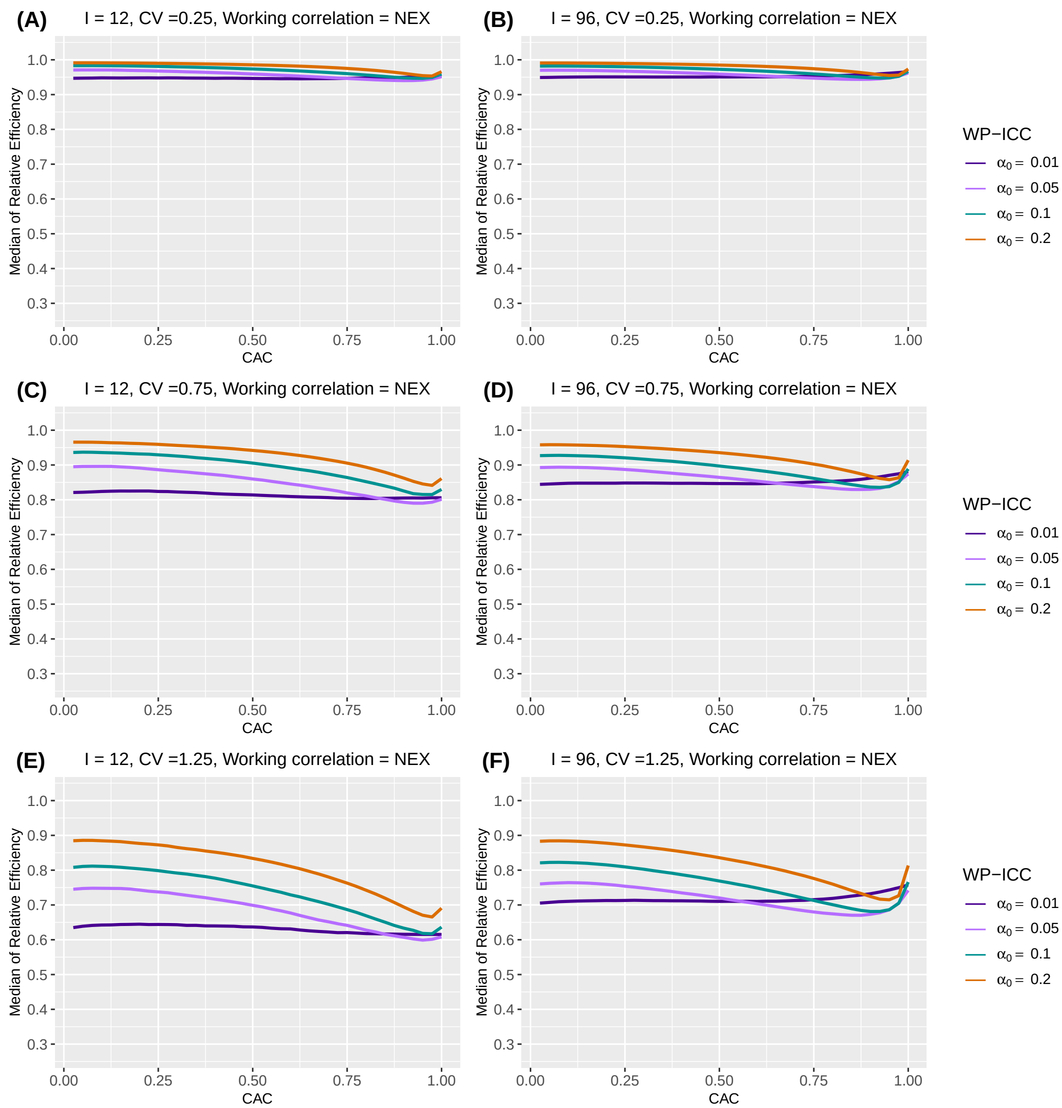
Web Figure 5 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



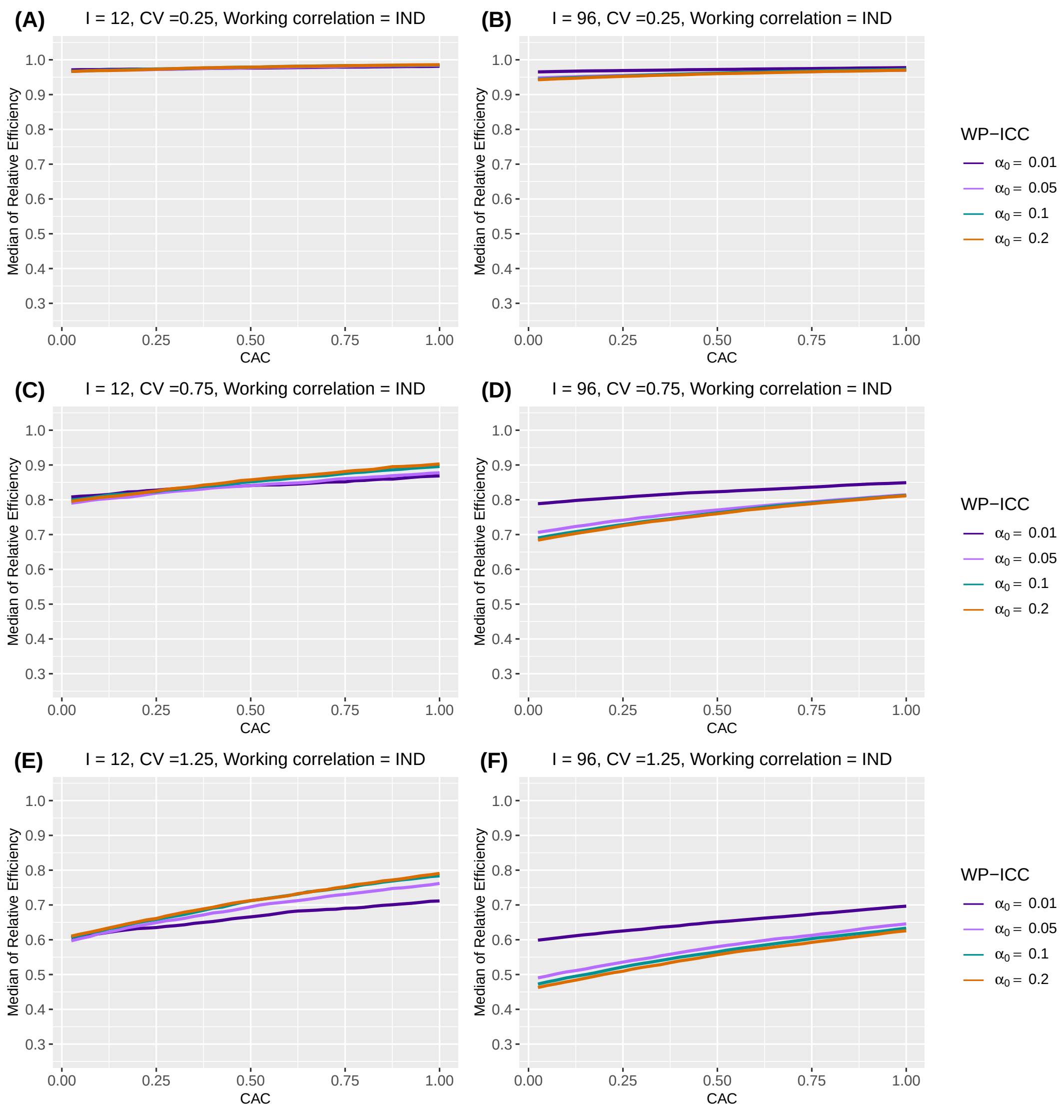
Web Figure 6 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



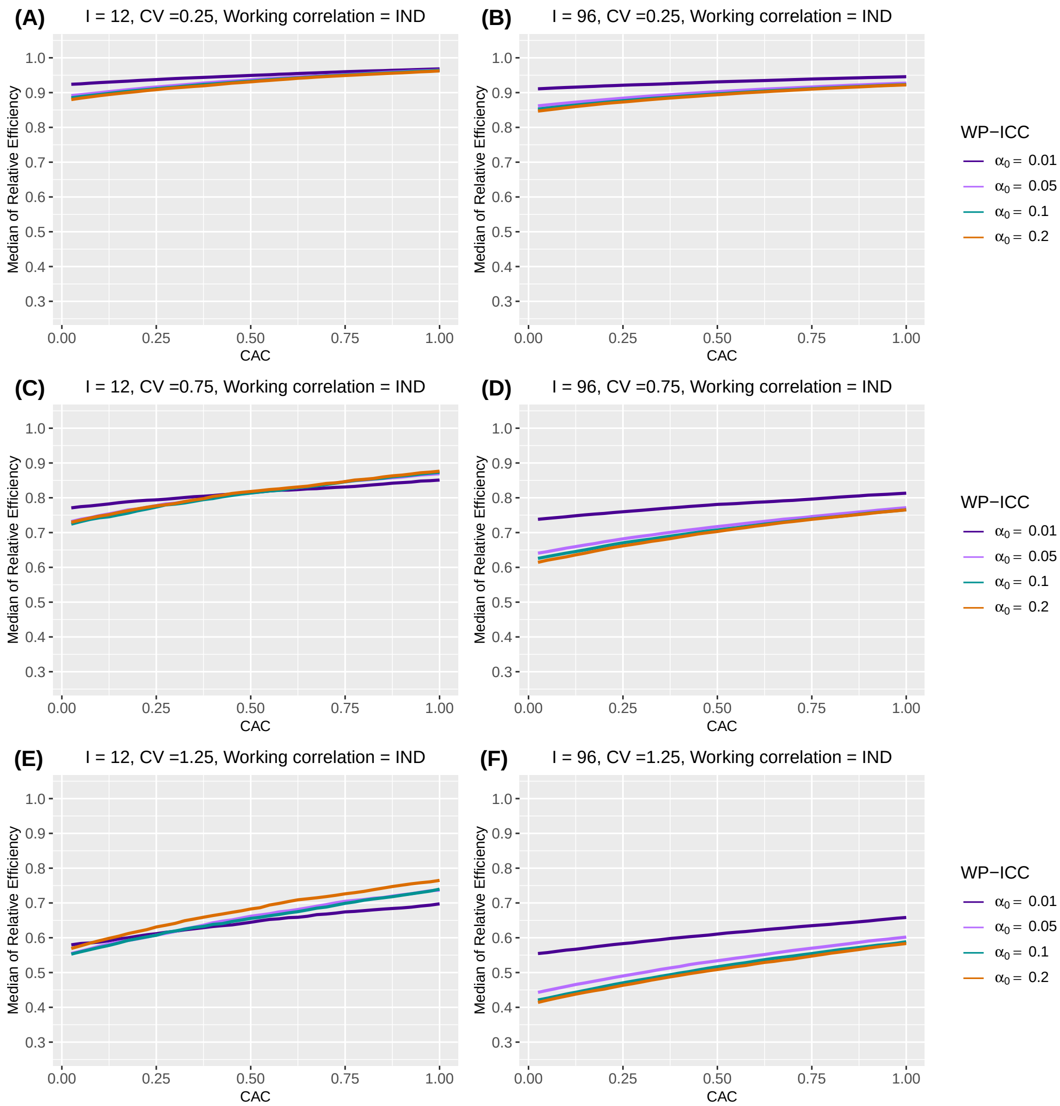
Web Figure 7 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



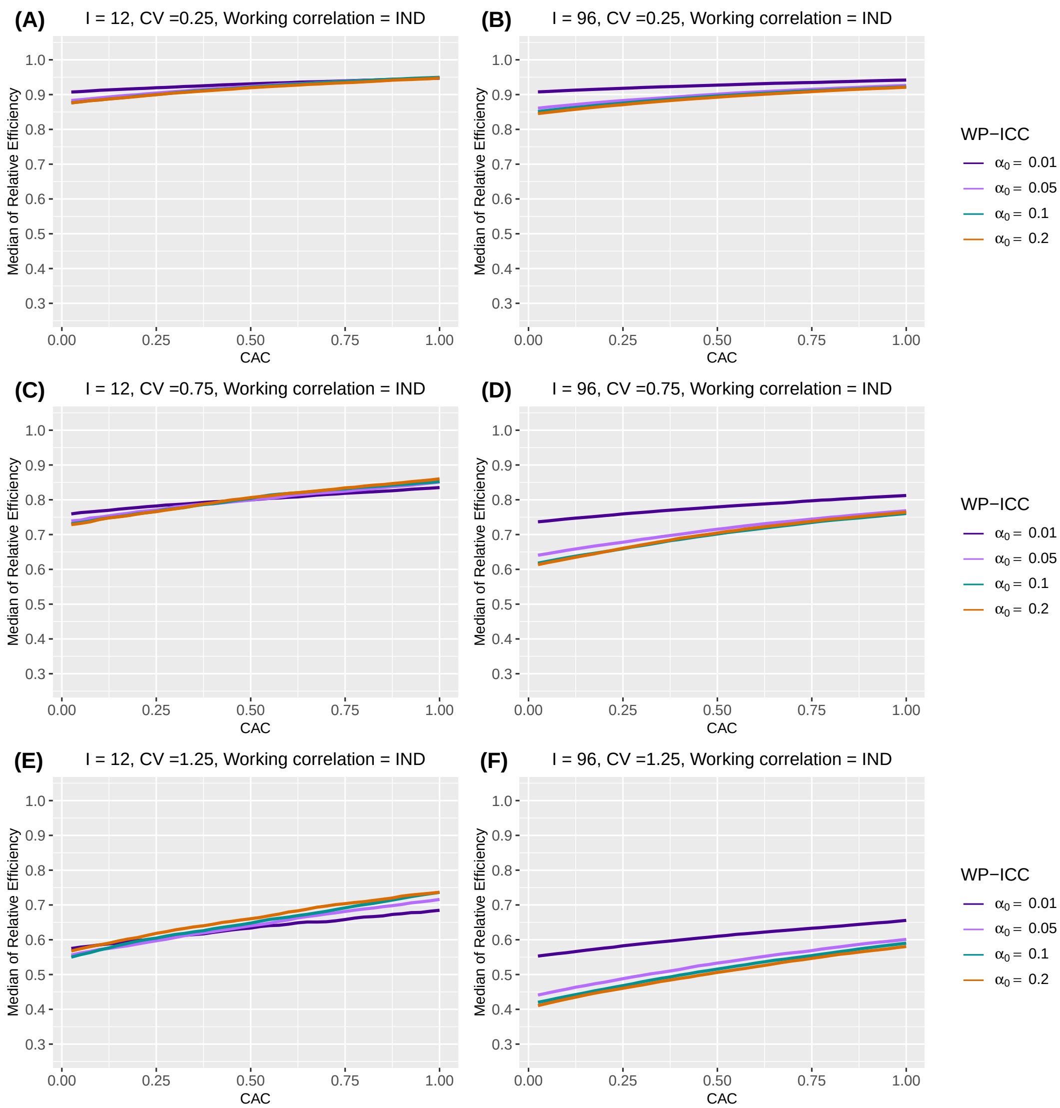
Web Figure 8 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 1: constant) is introduced.



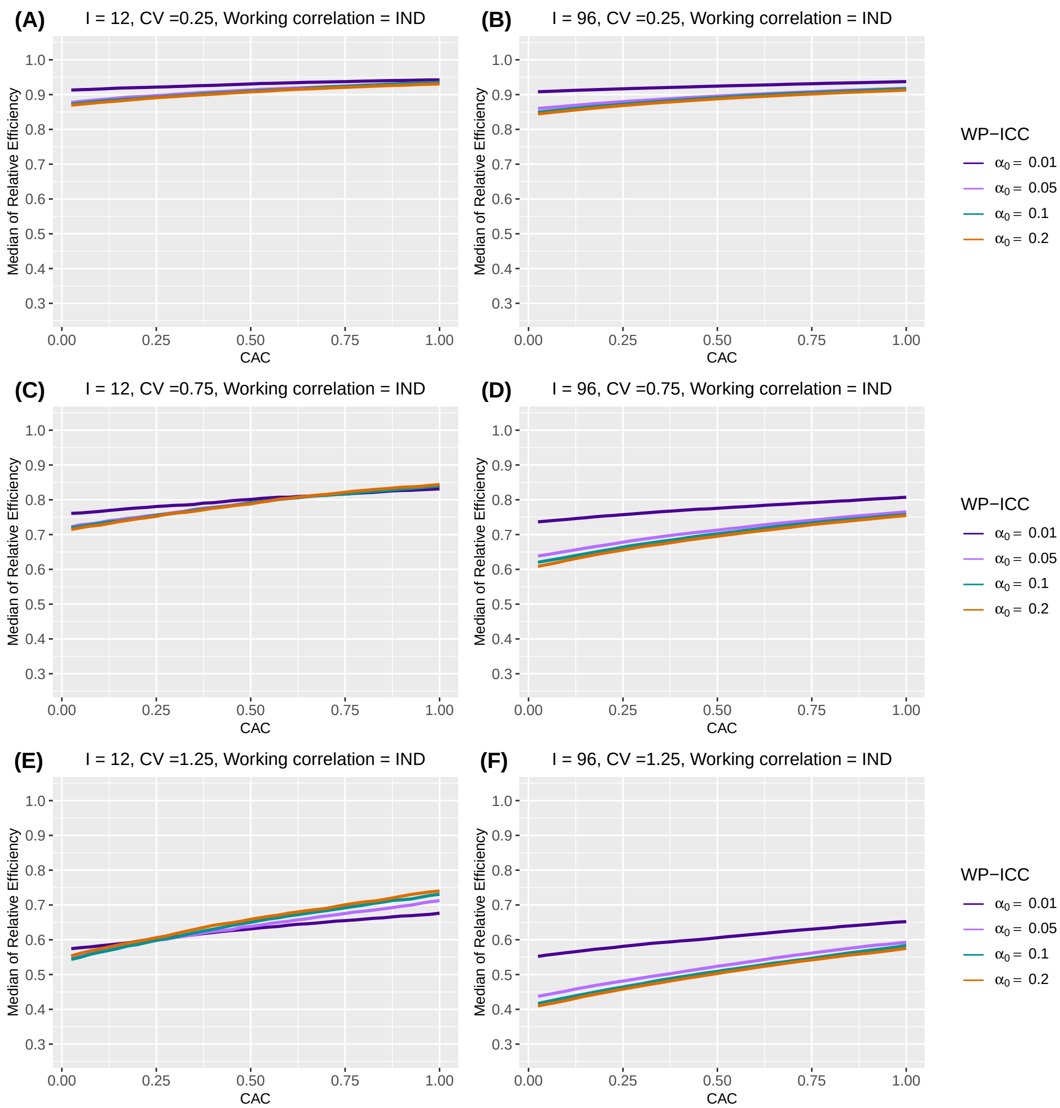
Web Figure 9 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



Web Figure 10 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



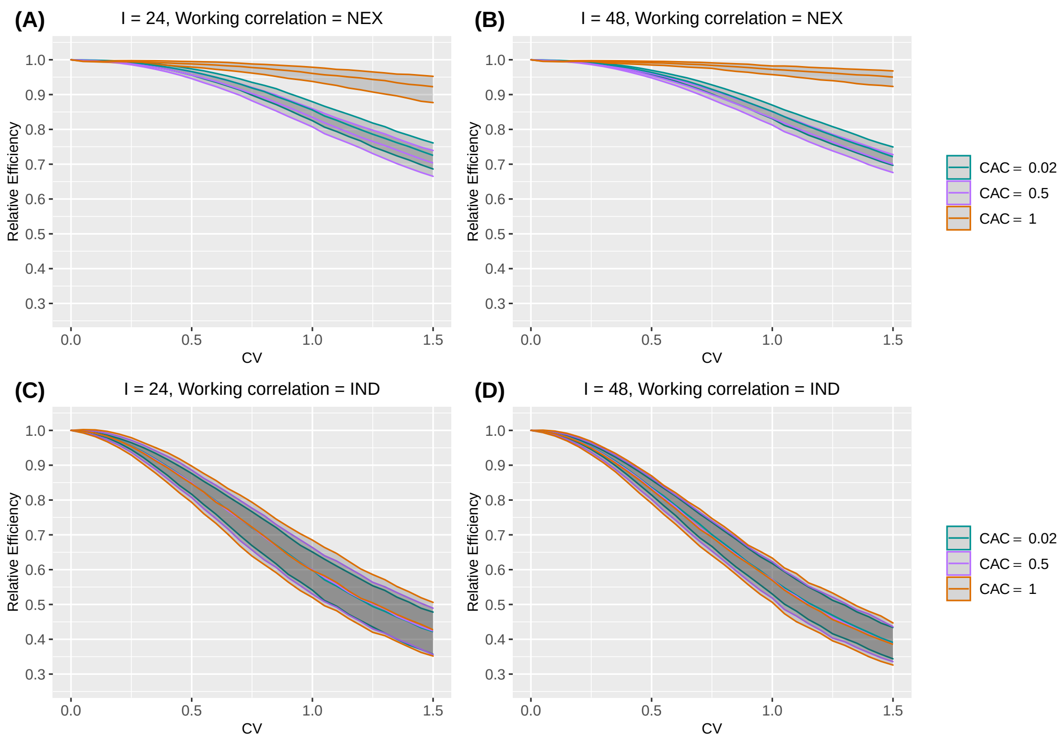
Web Figure 11 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Appendix C.3. Number of clusters

As mentioned in the first paragraph of Section 5.3 of the main article, the figure below shows the counterpart to Figure 2 in the main article when the number of clusters $I = 24$ and 48.

Web Figure 12 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 24$ and 48, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster variability in cluster-period sizes is introduced.



Web Appendix C.4. Number of periods

The following tables shows the counterparts of Table 2 in the main article when the number of clusters I is 12, 48 or 96.

Web Table 1 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters is $I = 12$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.989 (0.985, 0.993)	0.988 (0.971, 1.002)	0.967 (0.949, 0.979)	0.965 (0.949, 0.980)
		0.75	0.906 (0.876, 0.931)	0.896 (0.816, 0.955)	0.867 (0.788, 0.926)	0.873 (0.794, 0.931)
		1.25	0.760 (0.707, 0.807)	0.726 (0.606, 0.837)	0.699 (0.589, 0.808)	0.697 (0.584, 0.821)
	5	0.25	0.989 (0.985, 0.993)	0.987 (0.976, 0.997)	0.964 (0.952, 0.974)	0.958 (0.946, 0.970)
		0.75	0.907 (0.878, 0.933)	0.889 (0.832, 0.933)	0.872 (0.810, 0.914)	0.865 (0.805, 0.911)
		1.25	0.764 (0.713, 0.813)	0.711 (0.623, 0.793)	0.697 (0.611, 0.782)	0.692 (0.610, 0.776)
	13	0.25	0.989 (0.986, 0.992)	0.986 (0.982, 0.990)	0.973 (0.969, 0.977)	0.977 (0.972, 0.982)
		0.75	0.909 (0.882, 0.933)	0.892 (0.854, 0.921)	0.879 (0.842, 0.905)	0.880 (0.845, 0.910)
		1.25	0.767 (0.722, 0.815)	0.728 (0.667, 0.782)	0.712 (0.650, 0.772)	0.718 (0.665, 0.774)
IND	3	0.25	0.961 (0.948, 0.972)	0.970 (0.951, 0.988)	0.891 (0.871, 0.908)	0.889 (0.869, 0.908)
		0.75	0.755 (0.694, 0.806)	0.825 (0.749, 0.899)	0.749 (0.674, 0.818)	0.759 (0.677, 0.821)
		1.25	0.547 (0.474, 0.621)	0.666 (0.583, 0.769)	0.611 (0.529, 0.704)	0.604 (0.532, 0.707)
	5	0.25	0.962 (0.937, 0.983)	0.978 (0.952, 1.004)	0.934 (0.906, 0.960)	0.911 (0.881, 0.943)
		0.75	0.753 (0.686, 0.826)	0.847 (0.769, 0.920)	0.819 (0.740, 0.890)	0.791 (0.713, 0.863)
		1.25	0.548 (0.467, 0.636)	0.687 (0.577, 0.784)	0.660 (0.554, 0.757)	0.640 (0.554, 0.732)
	13	0.25	0.961 (0.928, 0.995)	0.989 (0.982, 0.997)	0.966 (0.955, 0.975)	0.977 (0.963, 0.991)
		0.75	0.759 (0.669, 0.838)	0.924 (0.895, 0.950)	0.906 (0.864, 0.933)	0.909 (0.876, 0.939)
		1.25	0.558 (0.457, 0.655)	0.812 (0.744, 0.863)	0.794 (0.725, 0.850)	0.794 (0.726, 0.849)

Web Table 2 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters is $I = 48$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.988 (0.985, 0.990)	0.986 (0.978, 0.993)	0.965 (0.956, 0.973)	0.962 (0.954, 0.970)
		0.75	0.898 (0.884, 0.911)	0.882 (0.847, 0.914)	0.865 (0.833, 0.895)	0.861 (0.829, 0.890)
		1.25	0.759 (0.731, 0.784)	0.733 (0.674, 0.781)	0.713 (0.660, 0.764)	0.713 (0.659, 0.760)
	5	0.25	0.988 (0.986, 0.990)	0.986 (0.979, 0.992)	0.960 (0.954, 0.967)	0.958 (0.951, 0.964)
		0.75	0.900 (0.886, 0.913)	0.888 (0.860, 0.913)	0.866 (0.837, 0.889)	0.864 (0.837, 0.889)
		1.25	0.763 (0.738, 0.786)	0.735 (0.685, 0.783)	0.714 (0.673, 0.758)	0.714 (0.665, 0.762)
	13	0.25	0.988 (0.986, 0.990)	0.986 (0.980, 0.990)	0.976 (0.971, 0.981)	0.976 (0.971, 0.982)
		0.75	0.902 (0.888, 0.915)	0.882 (0.858, 0.906)	0.875 (0.854, 0.898)	0.875 (0.850, 0.899)
		1.25	0.770 (0.745, 0.790)	0.725 (0.684, 0.764)	0.719 (0.678, 0.757)	0.721 (0.681, 0.757)
IND	3	0.25	0.952 (0.945, 0.959)	0.951 (0.939, 0.962)	0.874 (0.860, 0.884)	0.871 (0.859, 0.884)
		0.75	0.701 (0.665, 0.734)	0.725 (0.669, 0.778)	0.666 (0.619, 0.707)	0.662 (0.611, 0.711)
		1.25	0.469 (0.419, 0.514)	0.530 (0.461, 0.590)	0.481 (0.421, 0.538)	0.477 (0.415, 0.537)
	5	0.25	0.951 (0.937, 0.963)	0.965 (0.947, 0.982)	0.911 (0.896, 0.927)	0.899 (0.881, 0.915)
		0.75	0.694 (0.653, 0.740)	0.792 (0.740, 0.837)	0.741 (0.692, 0.789)	0.732 (0.688, 0.778)
		1.25	0.462 (0.403, 0.521)	0.608 (0.542, 0.676)	0.568 (0.497, 0.625)	0.557 (0.487, 0.620)
	13	0.25	0.949 (0.928, 0.966)	0.987 (0.964, 1.007)	0.974 (0.953, 0.994)	0.974 (0.954, 0.995)
		0.75	0.686 (0.634, 0.742)	0.903 (0.845, 0.958)	0.887 (0.834, 0.944)	0.885 (0.830, 0.937)
		1.25	0.449 (0.390, 0.519)	0.765 (0.693, 0.839)	0.761 (0.689, 0.841)	0.766 (0.694, 0.838)

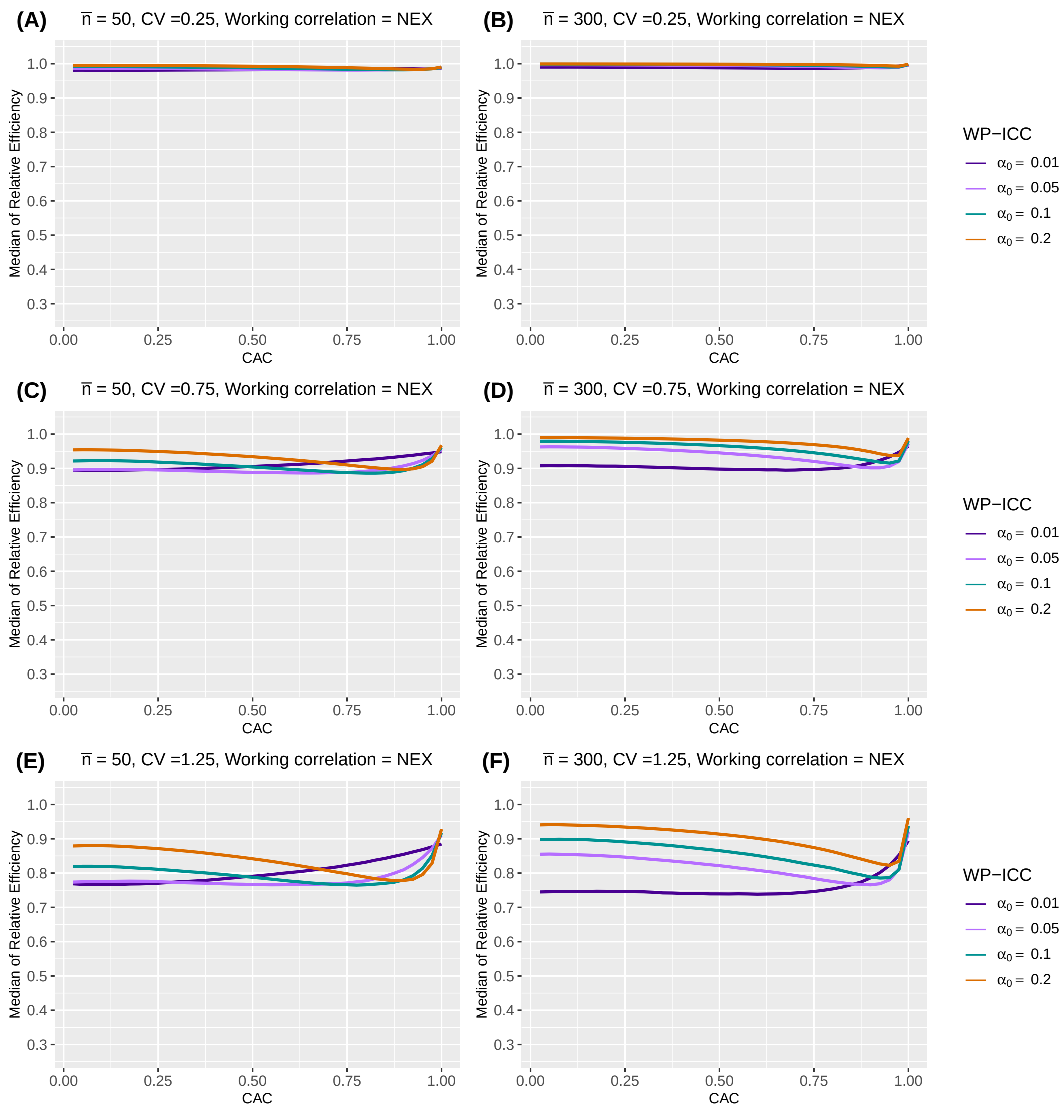
Web Table 3 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters is $I = 96$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.987 (0.986, 0.989)	0.985 (0.979, 0.991)	0.964 (0.958, 0.969)	0.962 (0.956, 0.968)
		0.75	0.896 (0.885, 0.906)	0.881 (0.858, 0.905)	0.863 (0.839, 0.886)	0.862 (0.837, 0.885)
		1.25	0.758 (0.739, 0.775)	0.725 (0.688, 0.761)	0.712 (0.677, 0.745)	0.708 (0.668, 0.742)
	5	0.25	0.988 (0.986, 0.989)	0.985 (0.980, 0.990)	0.960 (0.955, 0.965)	0.958 (0.954, 0.963)
		0.75	0.899 (0.889, 0.907)	0.886 (0.867, 0.906)	0.867 (0.845, 0.885)	0.864 (0.844, 0.882)
		1.25	0.762 (0.745, 0.780)	0.738 (0.709, 0.769)	0.721 (0.693, 0.751)	0.723 (0.690, 0.752)
	13	0.25	0.988 (0.987, 0.989)	0.986 (0.981, 0.990)	0.977 (0.973, 0.981)	0.977 (0.972, 0.981)
		0.75	0.901 (0.892, 0.910)	0.888 (0.870, 0.907)	0.882 (0.864, 0.899)	0.881 (0.863, 0.899)
		1.25	0.769 (0.752, 0.785)	0.740 (0.712, 0.769)	0.736 (0.709, 0.762)	0.736 (0.708, 0.763)
IND	3	0.25	0.951 (0.946, 0.956)	0.947 (0.938, 0.956)	0.870 (0.861, 0.879)	0.870 (0.860, 0.879)
		0.75	0.689 (0.662, 0.714)	0.706 (0.660, 0.743)	0.643 (0.604, 0.679)	0.641 (0.603, 0.681)
		1.25	0.450 (0.414, 0.488)	0.482 (0.430, 0.534)	0.441 (0.396, 0.486)	0.438 (0.389, 0.485)
	5	0.25	0.949 (0.940, 0.958)	0.963 (0.951, 0.975)	0.903 (0.891, 0.915)	0.896 (0.883, 0.907)
		0.75	0.681 (0.648, 0.715)	0.772 (0.735, 0.810)	0.720 (0.682, 0.755)	0.713 (0.675, 0.747)
		1.25	0.446 (0.401, 0.486)	0.573 (0.523, 0.627)	0.526 (0.483, 0.572)	0.526 (0.474, 0.577)
	13	0.25	0.947 (0.933, 0.959)	0.984 (0.967, 1.002)	0.974 (0.959, 0.992)	0.970 (0.952, 0.988)
		0.75	0.668 (0.631, 0.711)	0.890 (0.846, 0.939)	0.878 (0.835, 0.920)	0.871 (0.828, 0.921)
		1.25	0.435 (0.384, 0.481)	0.759 (0.701, 0.820)	0.745 (0.682, 0.811)	0.740 (0.685, 0.803)

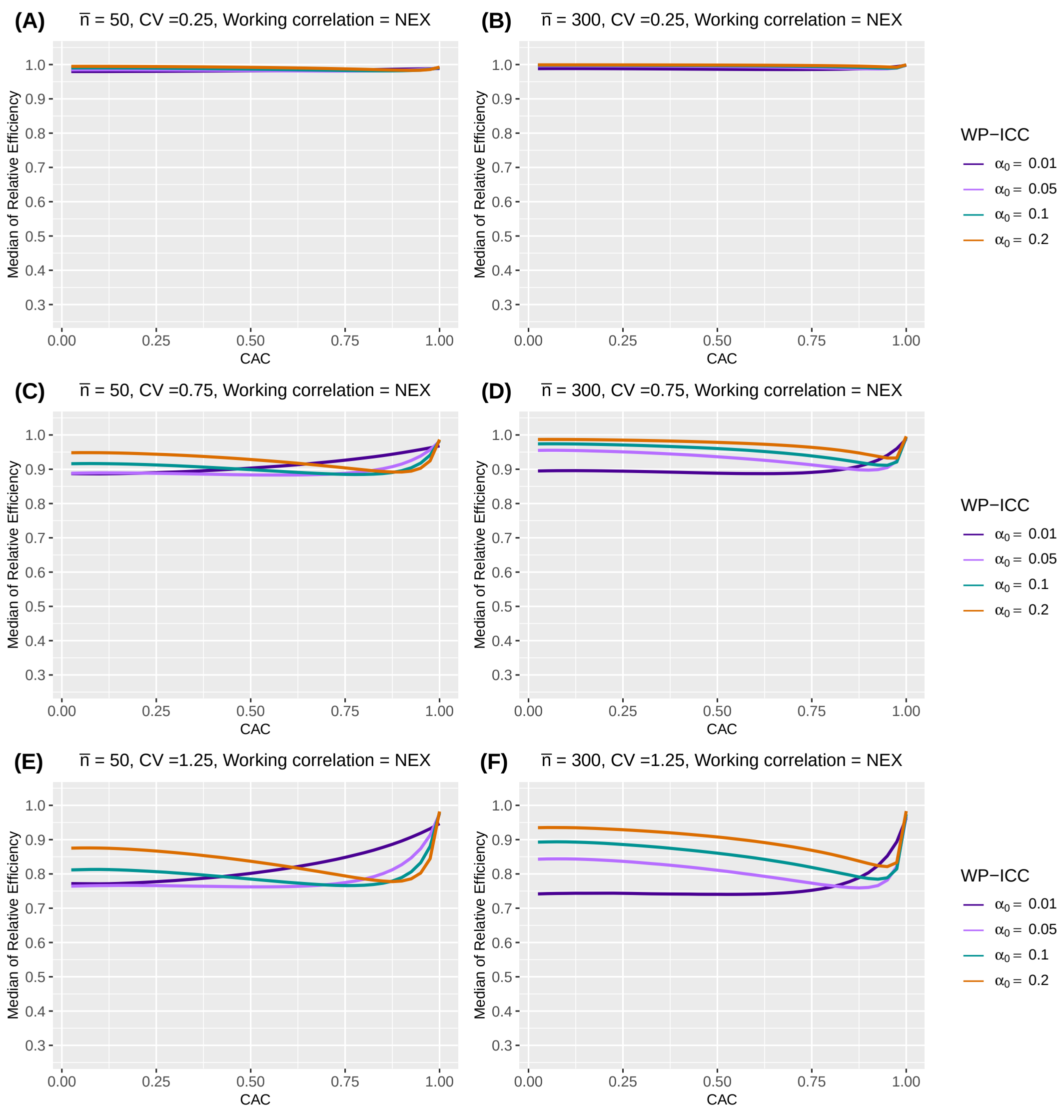
Web Appendix C.5. Cluster-period size

Figures that illustrate the impact of mean of cluster-period sizes on RE mentioned in section 5.5 of the main article are showed below.

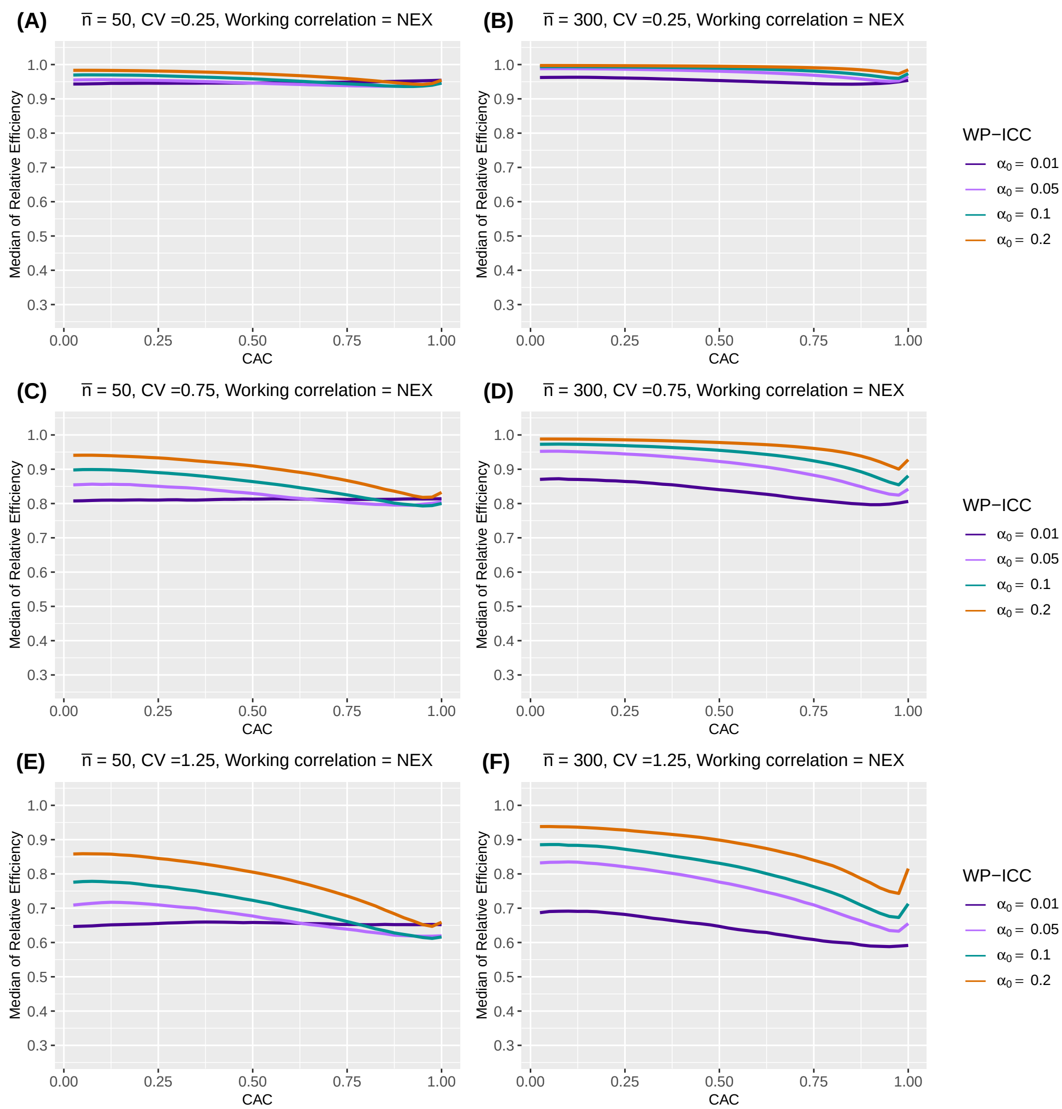
Web Figure 13 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance $\text{CV} \in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



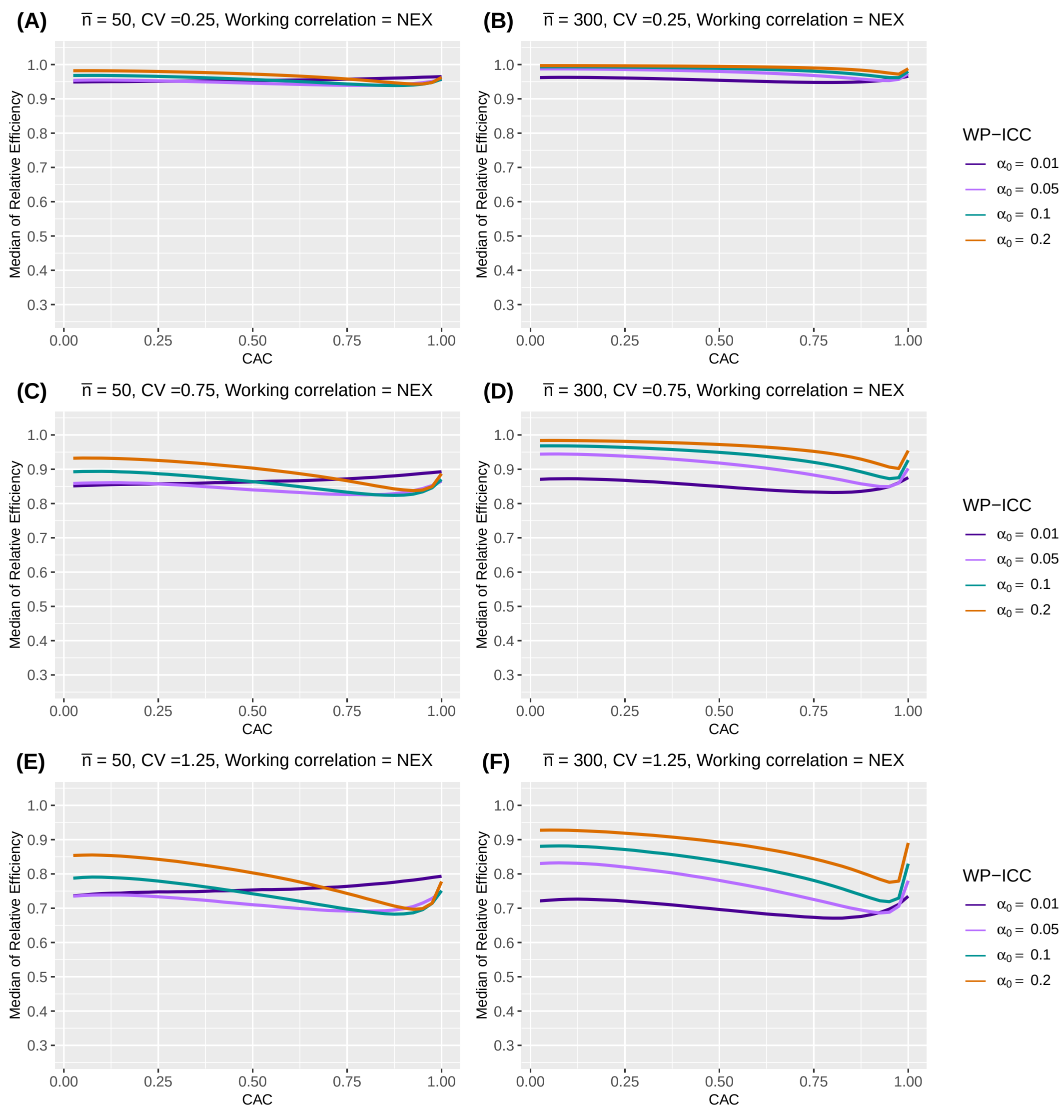
Web Figure 14 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



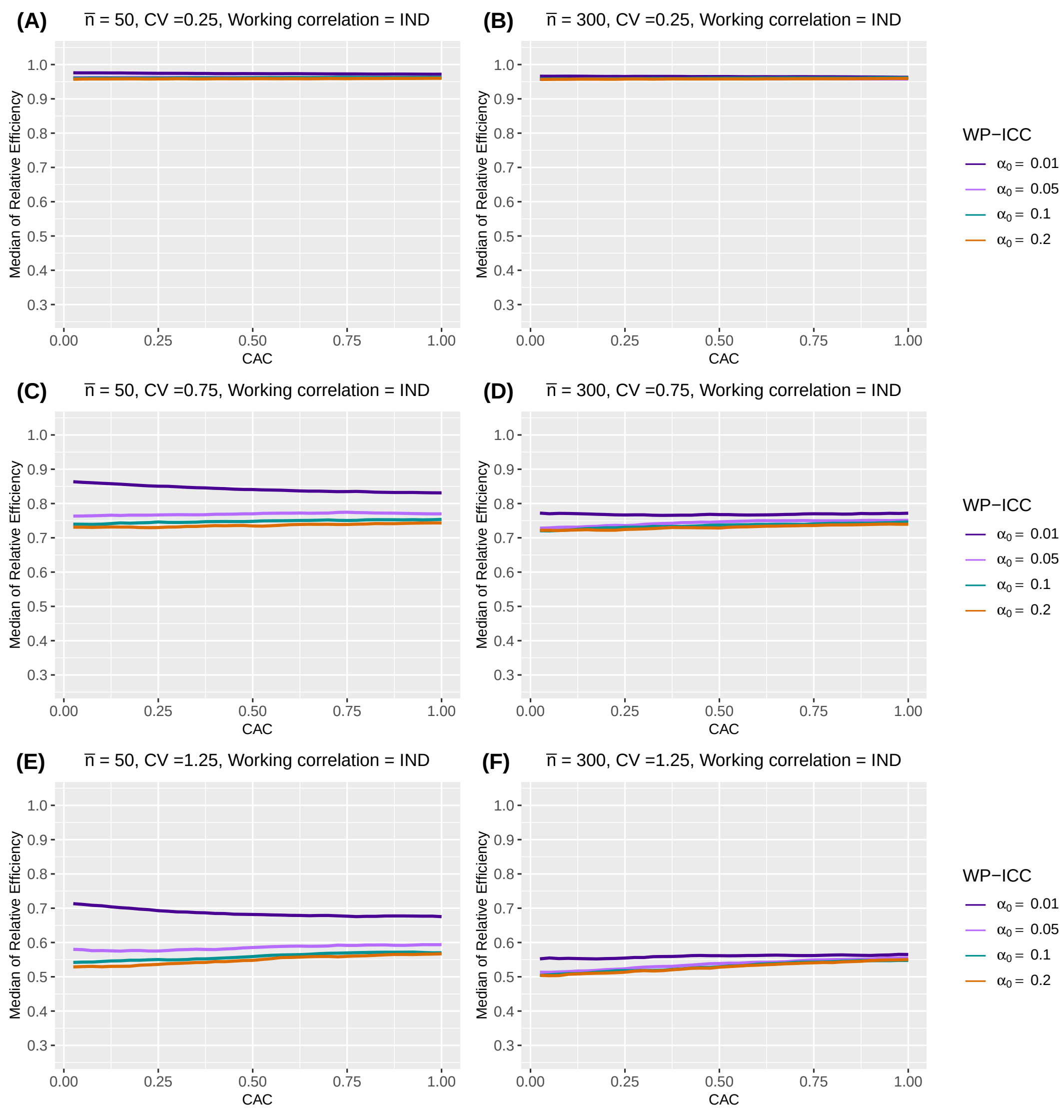
Web Figure 15 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



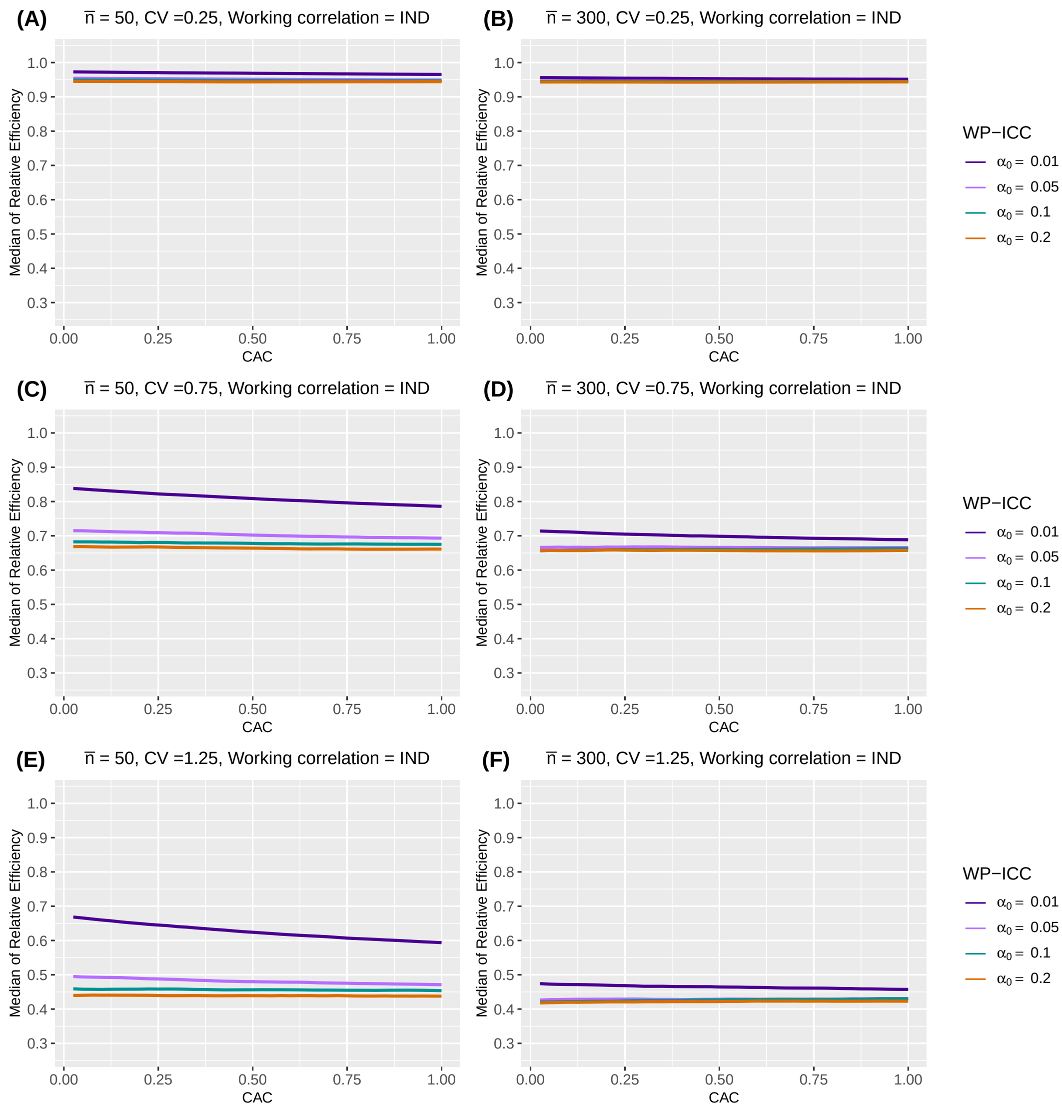
Web Figure 16 Median of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



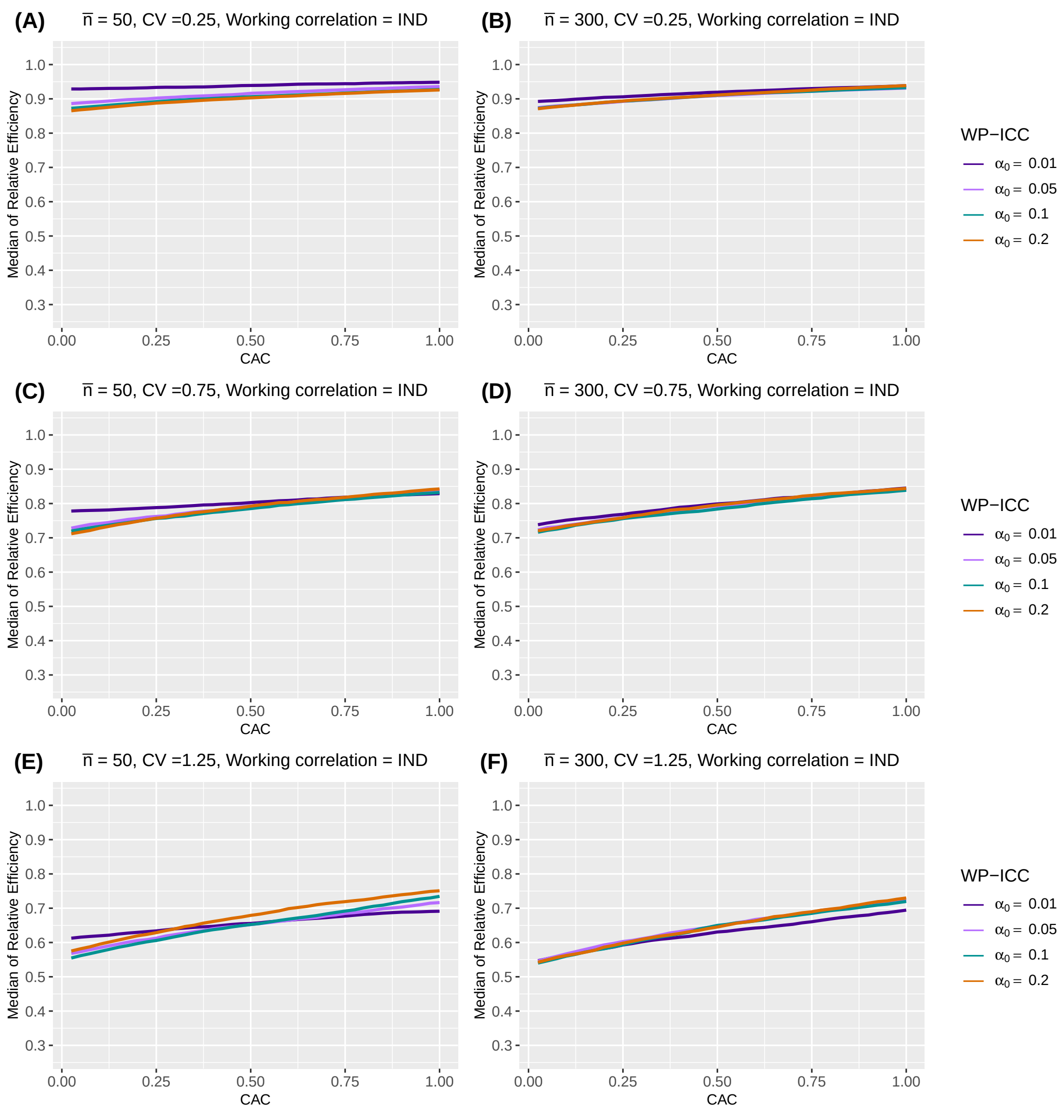
Web Figure 17 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



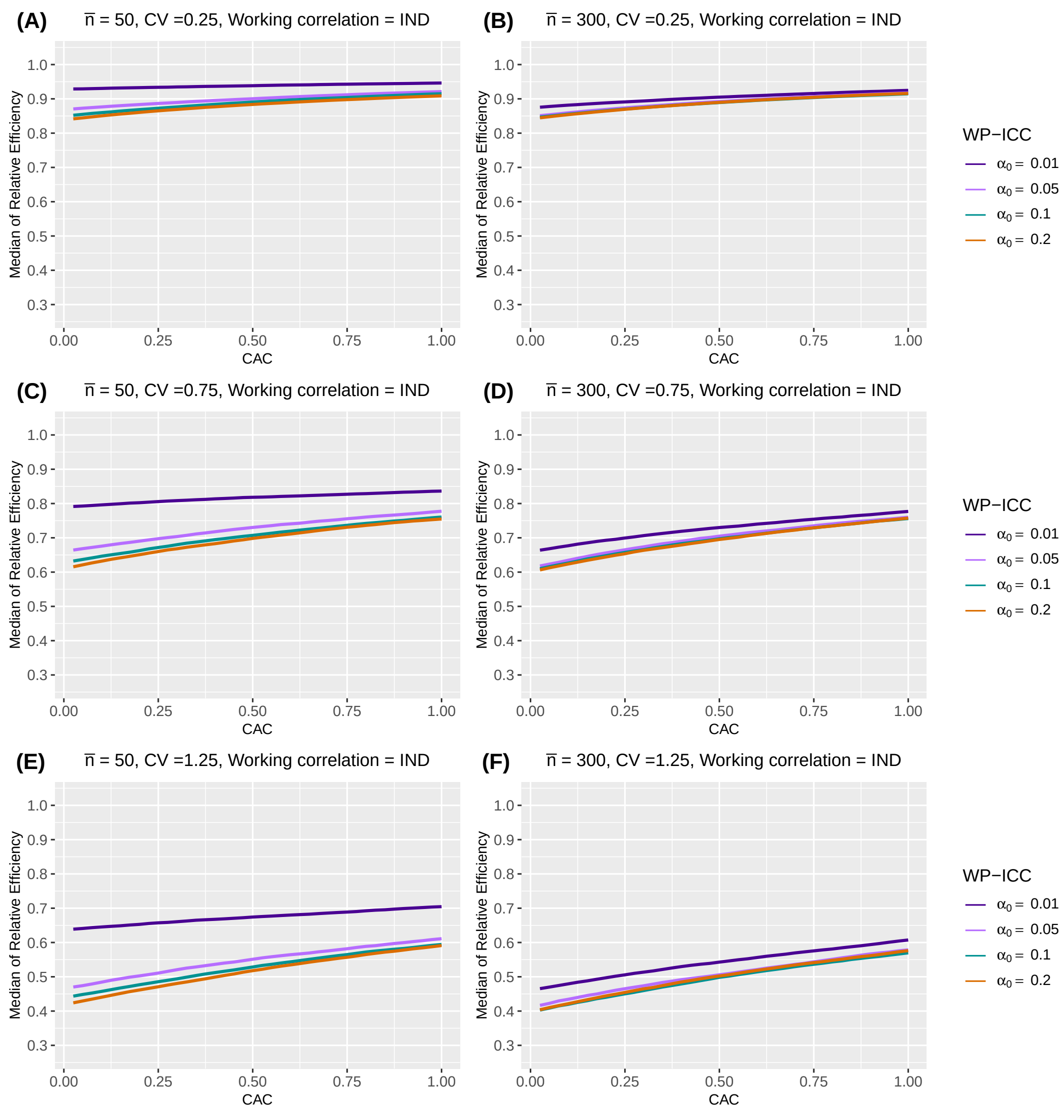
Web Figure 18 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



Web Figure 19 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Figure 20 Median of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Appendix C.6. Sensitivity to baseline prevalence, intervention effect and secular trend

As noted in Section 5.6 in the main article, tables below summarize the impact of treatment effect, baseline prevalence and secular trend of the outcomes on RE through a simple sensitivity analysis framework.

Web Table 4 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.995 (0.992, 0.997)	0.995 (0.991, 0.997)	0.994 (0.991, 0.998)
		0.3	0.994 (0.990, 0.997)	0.994 (0.989, 0.998)	0.994 (0.990, 0.998)
	0.75	0.1	0.976 (0.963, 0.985)	0.976 (0.960, 0.984)	0.974 (0.962, 0.986)
		0.3	0.978 (0.962, 0.988)	0.977 (0.958, 0.988)	0.976 (0.961, 0.988)
	1.25	0.1	0.936 (0.905, 0.954)	0.933 (0.901, 0.954)	0.935 (0.902, 0.957)
		0.3	0.943 (0.907, 0.965)	0.941 (0.903, 0.967)	0.942 (0.904, 0.967)
log(0.75)	0.25	0.1	0.993 (0.989, 0.998)	0.994 (0.988, 0.998)	0.993 (0.989, 0.998)
		0.3	0.993 (0.988, 0.998)	0.993 (0.988, 0.998)	0.993 (0.989, 0.998)
	0.75	0.1	0.979 (0.960, 0.992)	0.978 (0.957, 0.991)	0.978 (0.960, 0.992)
		0.3	0.979 (0.960, 0.993)	0.978 (0.957, 0.992)	0.978 (0.960, 0.993)
	1.25	0.1	0.950 (0.910, 0.976)	0.947 (0.903, 0.977)	0.951 (0.908, 0.980)
		0.3	0.952 (0.910, 0.978)	0.948 (0.903, 0.979)	0.953 (0.909, 0.981)

Web Table 5 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.988 (0.986, 0.991)	0.989 (0.986, 0.991)	0.989 (0.986, 0.991)
		0.3	0.988 (0.986, 0.991)	0.988 (0.986, 0.991)	0.988 (0.985, 0.991)
	0.75	0.1	0.905 (0.885, 0.922)	0.900 (0.882, 0.920)	0.902 (0.883, 0.919)
		0.3	0.903 (0.882, 0.920)	0.898 (0.879, 0.918)	0.900 (0.881, 0.917)
	1.25	0.1	0.768 (0.735, 0.801)	0.767 (0.731, 0.798)	0.769 (0.733, 0.803)
		0.3	0.765 (0.730, 0.798)	0.764 (0.727, 0.795)	0.765 (0.729, 0.799)
log(0.75)	0.25	0.1	0.988 (0.985, 0.991)	0.988 (0.985, 0.990)	0.988 (0.985, 0.990)
		0.3	0.988 (0.985, 0.991)	0.988 (0.985, 0.990)	0.988 (0.985, 0.990)
	0.75	0.1	0.901 (0.880, 0.919)	0.897 (0.878, 0.917)	0.898 (0.879, 0.916)
		0.3	0.901 (0.880, 0.919)	0.896 (0.878, 0.917)	0.898 (0.878, 0.916)
	1.25	0.1	0.763 (0.727, 0.795)	0.761 (0.725, 0.792)	0.763 (0.727, 0.796)
		0.3	0.763 (0.727, 0.795)	0.760 (0.725, 0.792)	0.763 (0.726, 0.796)

Web Table 6 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.954 (0.929, 0.979)	0.954 (0.925, 0.979)	0.954 (0.927, 0.980)
		0.3	0.954 (0.929, 0.979)	0.954 (0.925, 0.979)	0.955 (0.927, 0.979)
	0.75	0.1	0.723 (0.642, 0.793)	0.714 (0.640, 0.782)	0.721 (0.642, 0.786)
		0.3	0.722 (0.639, 0.794)	0.714 (0.639, 0.784)	0.724 (0.641, 0.785)
	1.25	0.1	0.503 (0.419, 0.586)	0.501 (0.421, 0.579)	0.503 (0.426, 0.591)
		0.3	0.504 (0.420, 0.586)	0.503 (0.421, 0.582)	0.506 (0.424, 0.592)
log(0.75)	0.25	0.1	0.954 (0.928, 0.979)	0.953 (0.926, 0.979)	0.954 (0.928, 0.979)
		0.3	0.954 (0.927, 0.978)	0.953 (0.926, 0.979)	0.954 (0.928, 0.979)
	0.75	0.1	0.719 (0.639, 0.795)	0.717 (0.638, 0.786)	0.723 (0.641, 0.787)
		0.3	0.717 (0.639, 0.794)	0.719 (0.639, 0.788)	0.722 (0.641, 0.788)
	1.25	0.1	0.505 (0.424, 0.586)	0.502 (0.424, 0.587)	0.506 (0.424, 0.593)
		0.3	0.503 (0.424, 0.588)	0.500 (0.425, 0.588)	0.508 (0.422, 0.590)

Web Table 7 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.954 (0.937, 0.971)	0.955 (0.935, 0.971)	0.954 (0.936, 0.972)
		0.3	0.954 (0.937, 0.972)	0.955 (0.935, 0.971)	0.954 (0.936, 0.972)
	0.75	0.1	0.723 (0.658, 0.775)	0.712 (0.654, 0.770)	0.718 (0.659, 0.771)
		0.3	0.722 (0.656, 0.776)	0.713 (0.653, 0.773)	0.718 (0.658, 0.770)
	1.25	0.1	0.501 (0.428, 0.565)	0.498 (0.428, 0.564)	0.501 (0.434, 0.576)
		0.3	0.502 (0.430, 0.564)	0.501 (0.432, 0.563)	0.501 (0.435, 0.576)
log(0.75)	0.25	0.1	0.954 (0.936, 0.972)	0.954 (0.935, 0.971)	0.953 (0.935, 0.972)
		0.3	0.954 (0.936, 0.971)	0.955 (0.935, 0.971)	0.953 (0.935, 0.972)
	0.75	0.1	0.720 (0.654, 0.778)	0.714 (0.654, 0.773)	0.719 (0.656, 0.772)
		0.3	0.720 (0.654, 0.778)	0.714 (0.654, 0.774)	0.718 (0.655, 0.771)
	1.25	0.1	0.503 (0.429, 0.564)	0.500 (0.432, 0.566)	0.501 (0.432, 0.573)
		0.3	0.502 (0.431, 0.563)	0.500 (0.430, 0.567)	0.502 (0.432, 0.572)

Web Table 8 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.963 (0.942, 0.984)	0.961 (0.941, 0.984)	0.963 (0.944, 0.984)
		0.3	0.956 (0.931, 0.980)	0.954 (0.930, 0.980)	0.958 (0.935, 0.981)
	0.75	0.1	0.860 (0.793, 0.917)	0.856 (0.796, 0.913)	0.857 (0.798, 0.920)
		0.3	0.837 (0.765, 0.903)	0.833 (0.764, 0.896)	0.837 (0.771, 0.908)
	1.25	0.1	0.695 (0.604, 0.785)	0.688 (0.601, 0.784)	0.700 (0.613, 0.791)
		0.3	0.659 (0.562, 0.753)	0.647 (0.554, 0.749)	0.666 (0.575, 0.764)
log(0.75)	0.25	0.1	0.950 (0.922, 0.977)	0.948 (0.921, 0.977)	0.951 (0.923, 0.977)
		0.3	0.949 (0.921, 0.977)	0.947 (0.919, 0.978)	0.950 (0.921, 0.976)
	0.75	0.1	0.816 (0.741, 0.890)	0.813 (0.740, 0.887)	0.814 (0.738, 0.889)
		0.3	0.813 (0.739, 0.888)	0.812 (0.737, 0.886)	0.811 (0.736, 0.887)
	1.25	0.1	0.625 (0.524, 0.724)	0.621 (0.522, 0.721)	0.629 (0.532, 0.733)
		0.3	0.622 (0.519, 0.719)	0.618 (0.518, 0.718)	0.625 (0.528, 0.729)

Web Table 9 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are NEX. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.960 (0.950, 0.969)	0.960 (0.950, 0.969)	0.960 (0.951, 0.969)
		0.3	0.959 (0.949, 0.969)	0.959 (0.949, 0.968)	0.959 (0.949, 0.968)
	0.75	0.1	0.868 (0.825, 0.905)	0.869 (0.828, 0.902)	0.868 (0.825, 0.904)
		0.3	0.863 (0.822, 0.902)	0.865 (0.823, 0.899)	0.864 (0.819, 0.900)
	1.25	0.1	0.712 (0.646, 0.772)	0.710 (0.645, 0.772)	0.715 (0.653, 0.776)
		0.3	0.708 (0.638, 0.768)	0.704 (0.639, 0.764)	0.710 (0.646, 0.770)
log(0.75)	0.25	0.1	0.959 (0.948, 0.968)	0.958 (0.948, 0.968)	0.958 (0.948, 0.967)
		0.3	0.958 (0.948, 0.968)	0.958 (0.948, 0.968)	0.958 (0.948, 0.967)
	0.75	0.1	0.860 (0.818, 0.900)	0.863 (0.820, 0.898)	0.862 (0.814, 0.898)
		0.3	0.860 (0.819, 0.899)	0.862 (0.820, 0.897)	0.861 (0.814, 0.897)
	1.25	0.1	0.704 (0.630, 0.762)	0.699 (0.636, 0.761)	0.706 (0.641, 0.767)
		0.3	0.704 (0.631, 0.762)	0.698 (0.635, 0.759)	0.706 (0.640, 0.767)

Web Table 10 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.929 (0.894, 0.960)	0.923 (0.893, 0.958)	0.928 (0.892, 0.966)
		0.3	0.928 (0.893, 0.961)	0.923 (0.892, 0.957)	0.927 (0.892, 0.966)
	0.75	0.1	0.807 (0.739, 0.893)	0.800 (0.730, 0.886)	0.811 (0.739, 0.893)
		0.3	0.806 (0.735, 0.890)	0.800 (0.730, 0.886)	0.811 (0.737, 0.894)
	1.25	0.1	0.657 (0.563, 0.758)	0.661 (0.574, 0.766)	0.671 (0.565, 0.767)
		0.3	0.658 (0.562, 0.761)	0.663 (0.573, 0.764)	0.668 (0.571, 0.769)
log(0.75)	0.25	0.1	0.928 (0.892, 0.961)	0.925 (0.891, 0.957)	0.927 (0.892, 0.968)
		0.3	0.928 (0.892, 0.960)	0.924 (0.891, 0.956)	0.927 (0.892, 0.967)
	0.75	0.1	0.805 (0.730, 0.889)	0.801 (0.728, 0.890)	0.809 (0.730, 0.894)
		0.3	0.806 (0.730, 0.889)	0.801 (0.729, 0.890)	0.810 (0.731, 0.894)
	1.25	0.1	0.657 (0.562, 0.762)	0.662 (0.575, 0.762)	0.665 (0.571, 0.767)
		0.3	0.657 (0.563, 0.764)	0.662 (0.575, 0.761)	0.664 (0.570, 0.765)

Web Table 11 Median and IQR (in parentheses) of relative efficiency when the true correlation model is NEX but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.907 (0.883, 0.927)	0.902 (0.881, 0.926)	0.904 (0.881, 0.930)
		0.3	0.906 (0.882, 0.927)	0.902 (0.882, 0.926)	0.904 (0.881, 0.930)
	0.75	0.1	0.761 (0.695, 0.819)	0.755 (0.693, 0.816)	0.765 (0.701, 0.823)
		0.3	0.760 (0.696, 0.820)	0.755 (0.693, 0.819)	0.764 (0.697, 0.823)
	1.25	0.1	0.589 (0.502, 0.675)	0.595 (0.516, 0.675)	0.600 (0.519, 0.680)
		0.3	0.590 (0.503, 0.673)	0.599 (0.515, 0.676)	0.597 (0.517, 0.683)
log(0.75)	0.25	0.1	0.906 (0.882, 0.927)	0.902 (0.881, 0.926)	0.904 (0.880, 0.930)
		0.3	0.906 (0.881, 0.927)	0.902 (0.881, 0.925)	0.905 (0.879, 0.930)
	0.75	0.1	0.758 (0.697, 0.819)	0.755 (0.693, 0.820)	0.764 (0.696, 0.823)
		0.3	0.758 (0.696, 0.821)	0.756 (0.694, 0.819)	0.764 (0.695, 0.824)
	1.25	0.1	0.594 (0.505, 0.672)	0.596 (0.512, 0.677)	0.595 (0.518, 0.682)
		0.3	0.594 (0.504, 0.674)	0.596 (0.511, 0.678)	0.596 (0.516, 0.680)

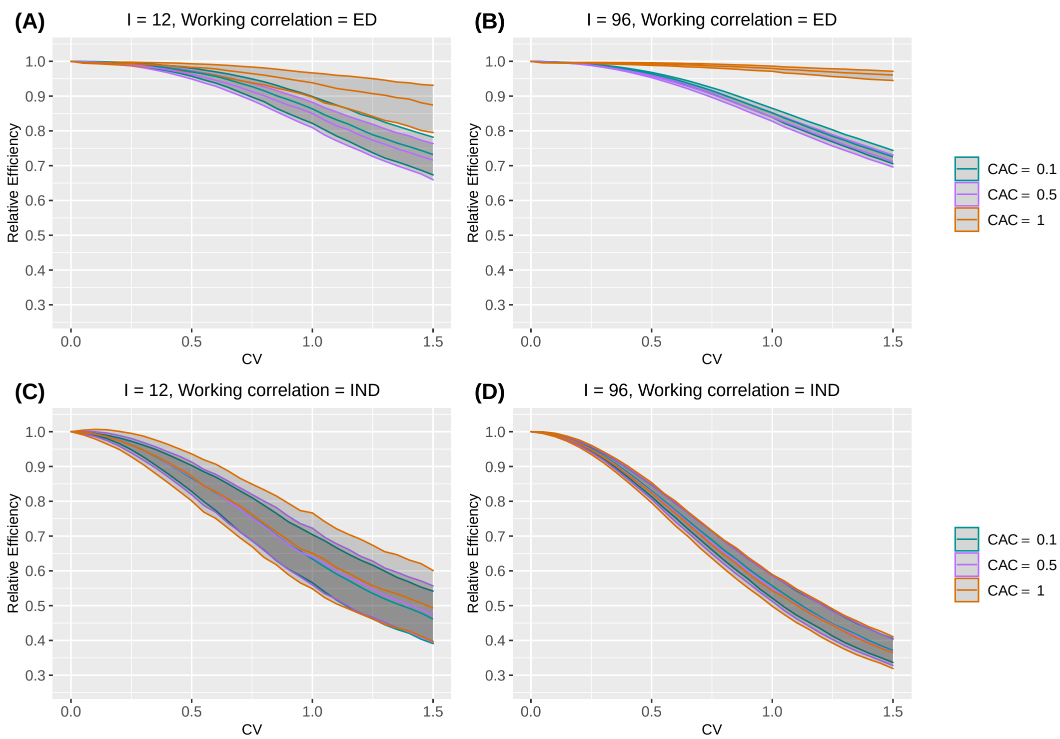
Web Appendix D. Supplementary simulation results under exponential decay true correlation structure

Simulation results under exponential decay true correlation model are parallelly showed in this section. As noted in Section 6 in the main article, all α_0/α_1 or α_1 expressions would be substituted by the decay rate ρ specified in the exponential decay correlation structure.

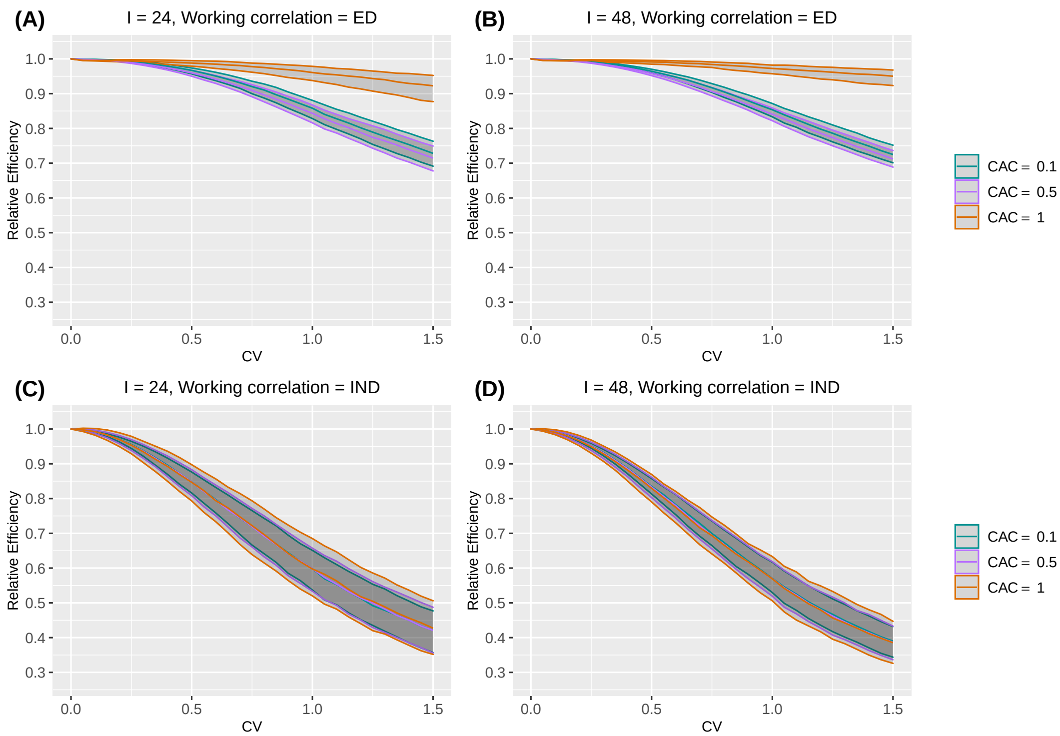
Web Appendix D.1. Cluster size variability and number of clusters

The following figures show the counterparts to all RE versus CV plots that illustrate the impact of cluster size variability and the number of clusters on RE, but under exponential decay true correlation structure.

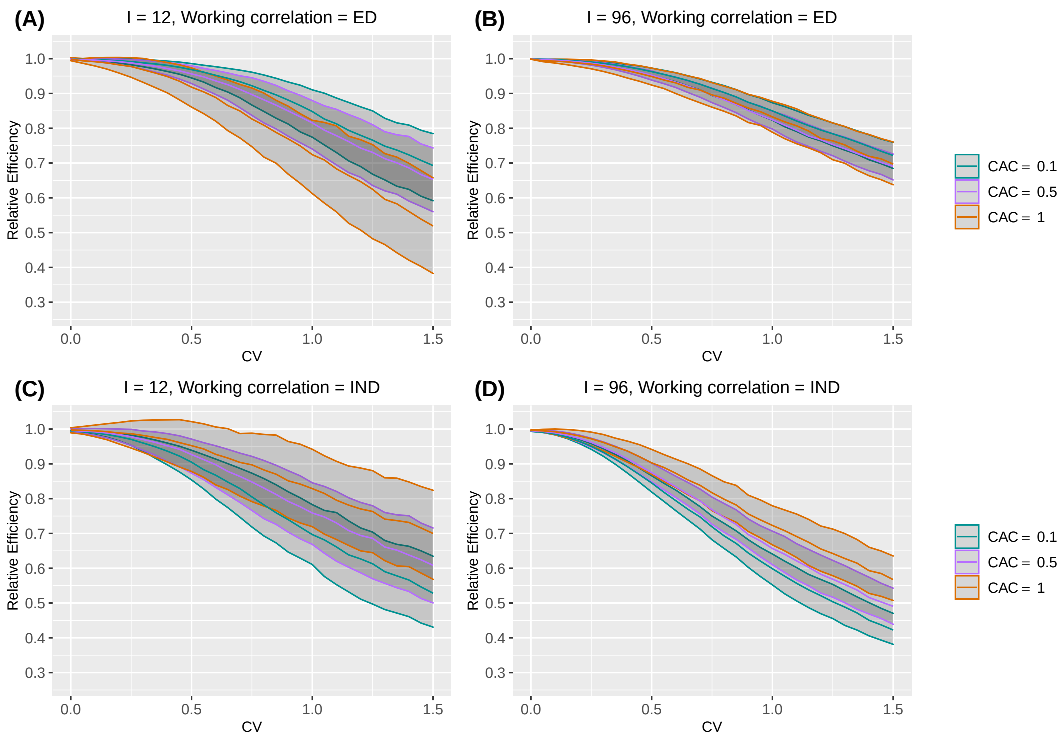
Web Figure 21 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster variability in cluster-period sizes is introduced.



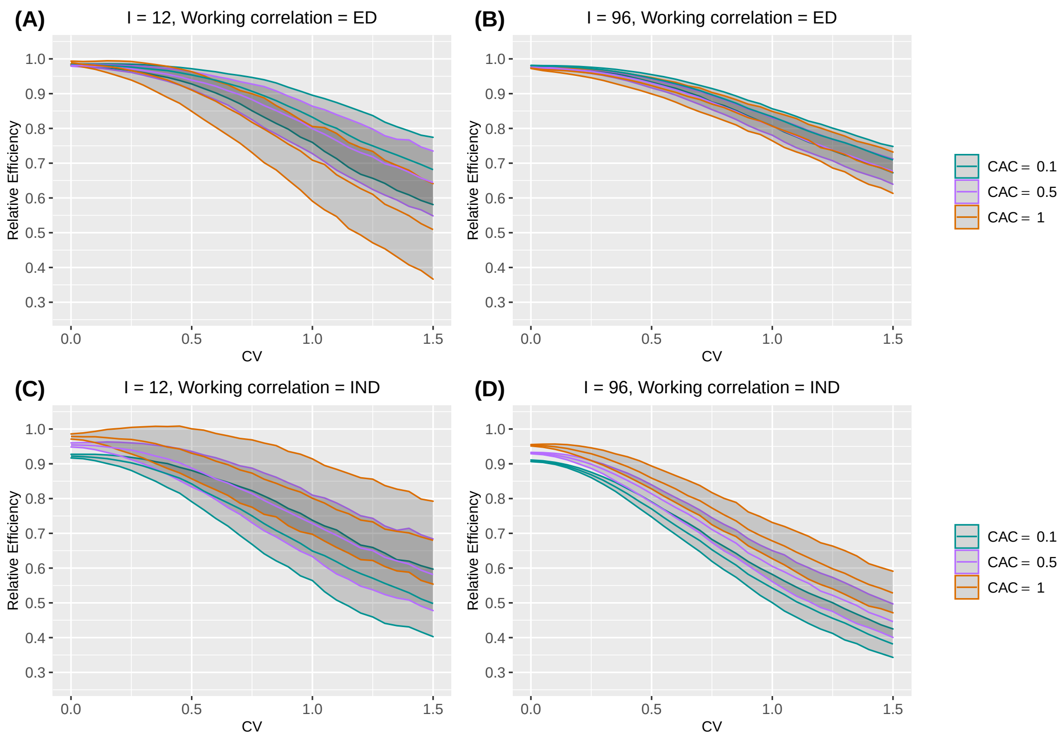
Web Figure 22 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 24$ and 48, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster variability in cluster-period sizes is introduced.



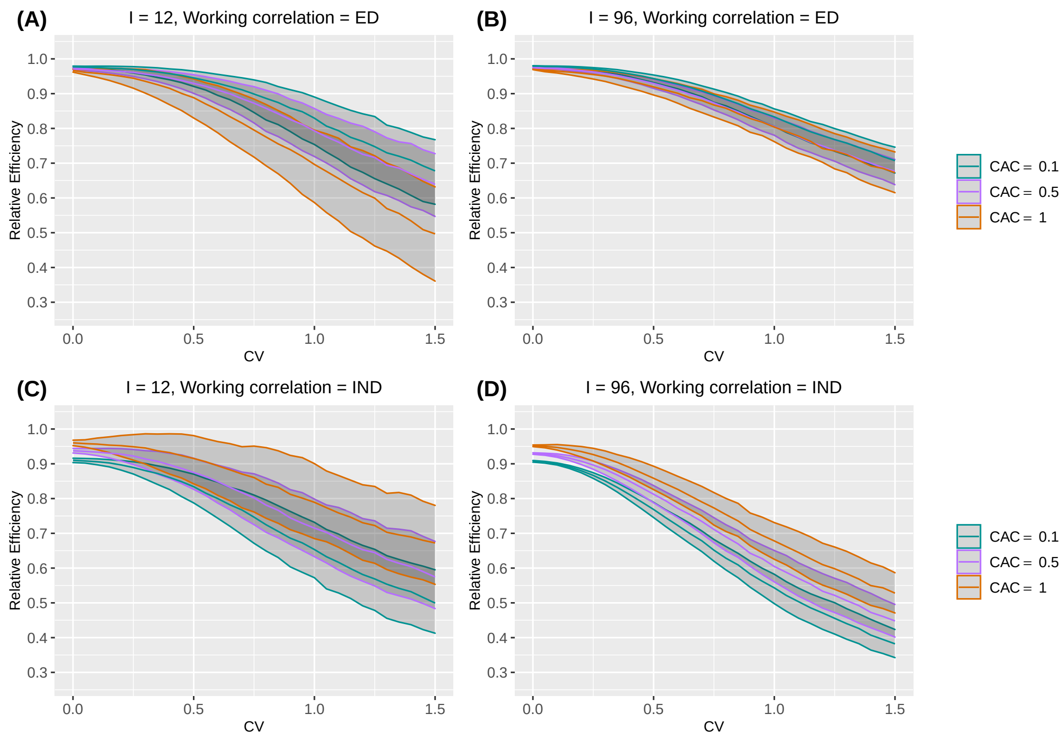
Web Figure 23 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 1: constant) is introduced.



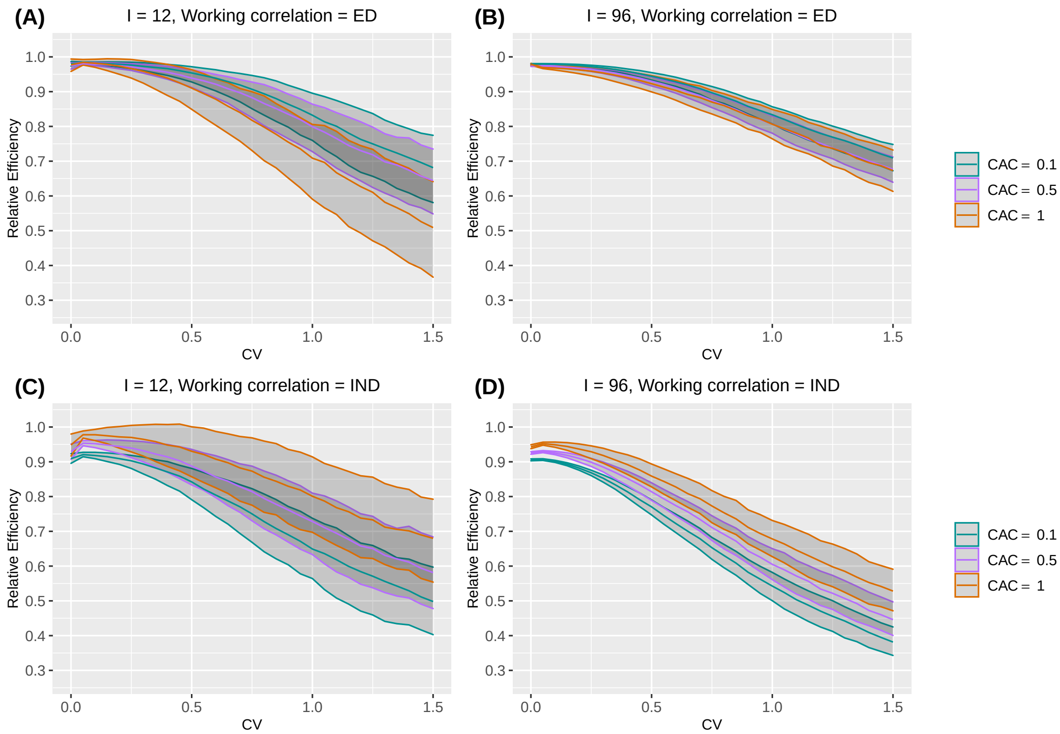
Web Figure 24 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



Web Figure 25 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



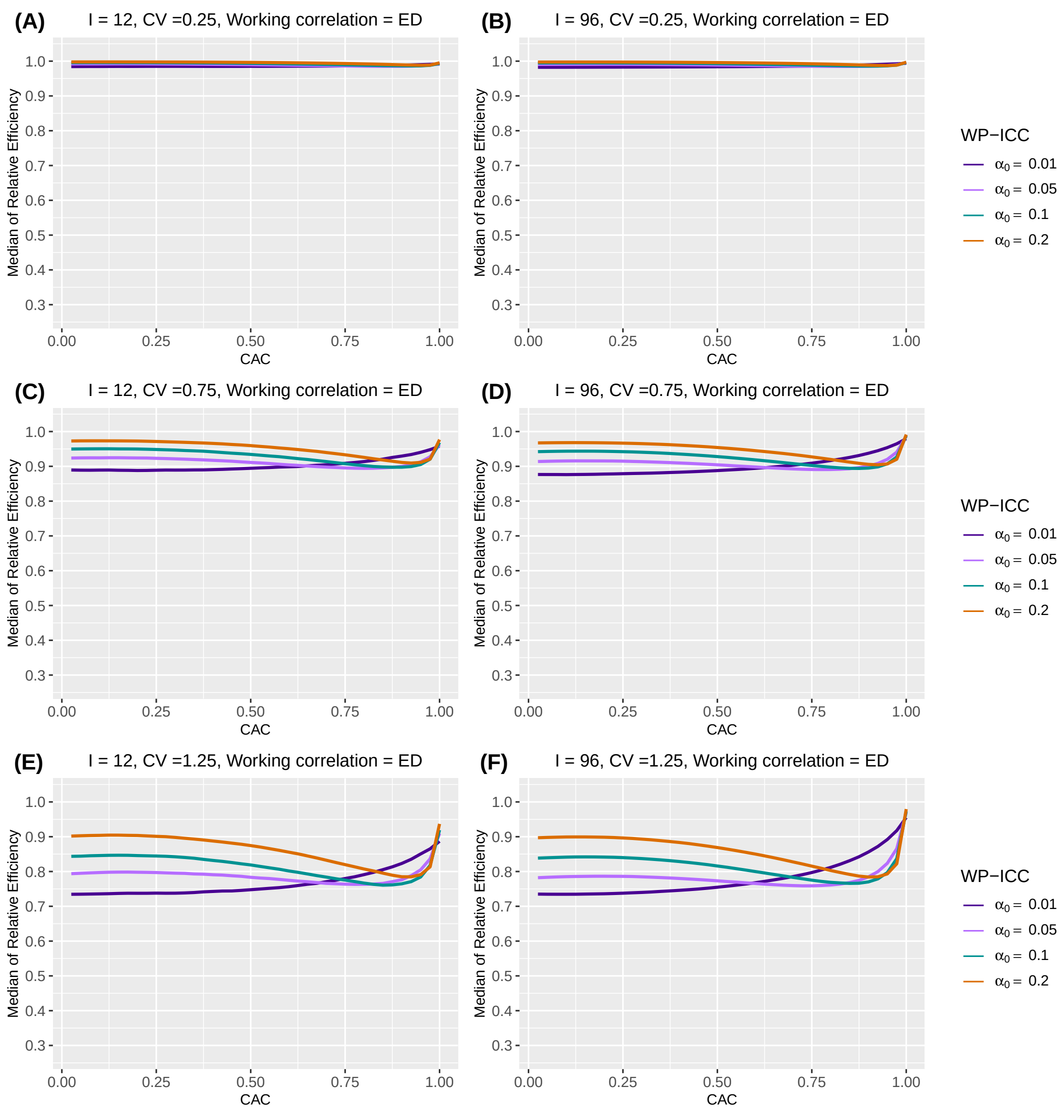
Web Figure 26 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



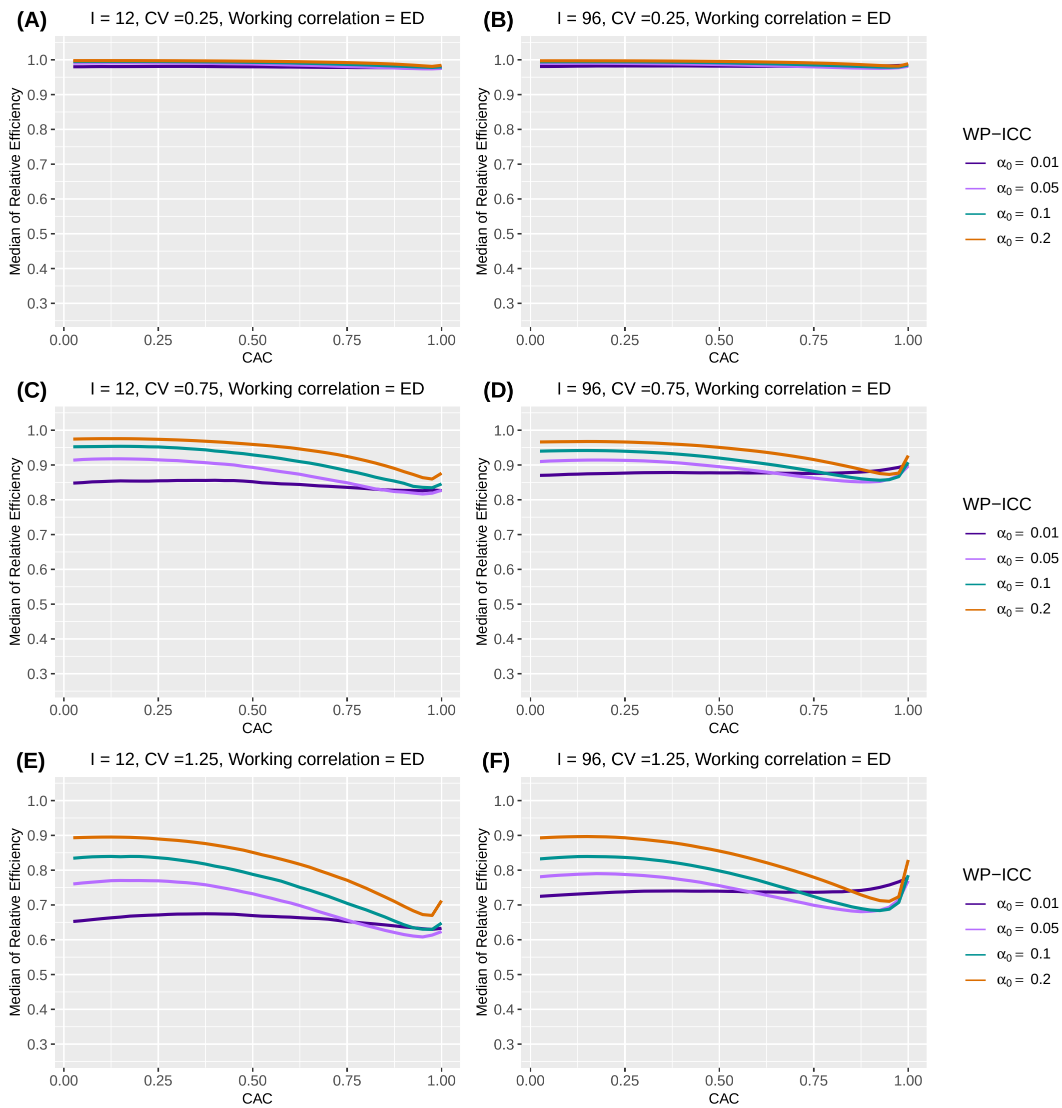
Web Appendix D.2. Intraclass correlation coefficients

Figures in this section show the counterparts to plots that illustrate the impact of ICC parameters on RE, but under exponential decay true correlation structure.

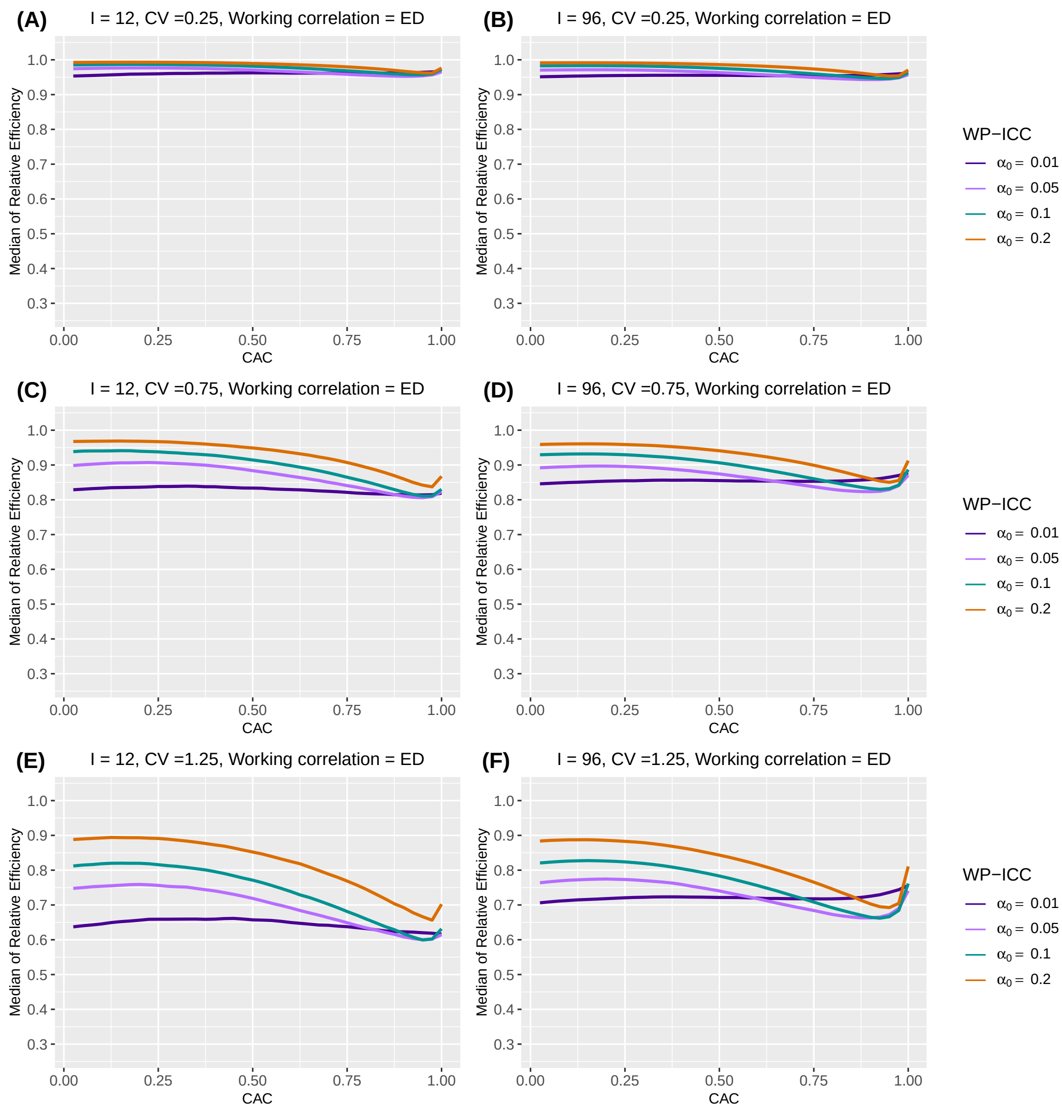
Web Figure 27 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



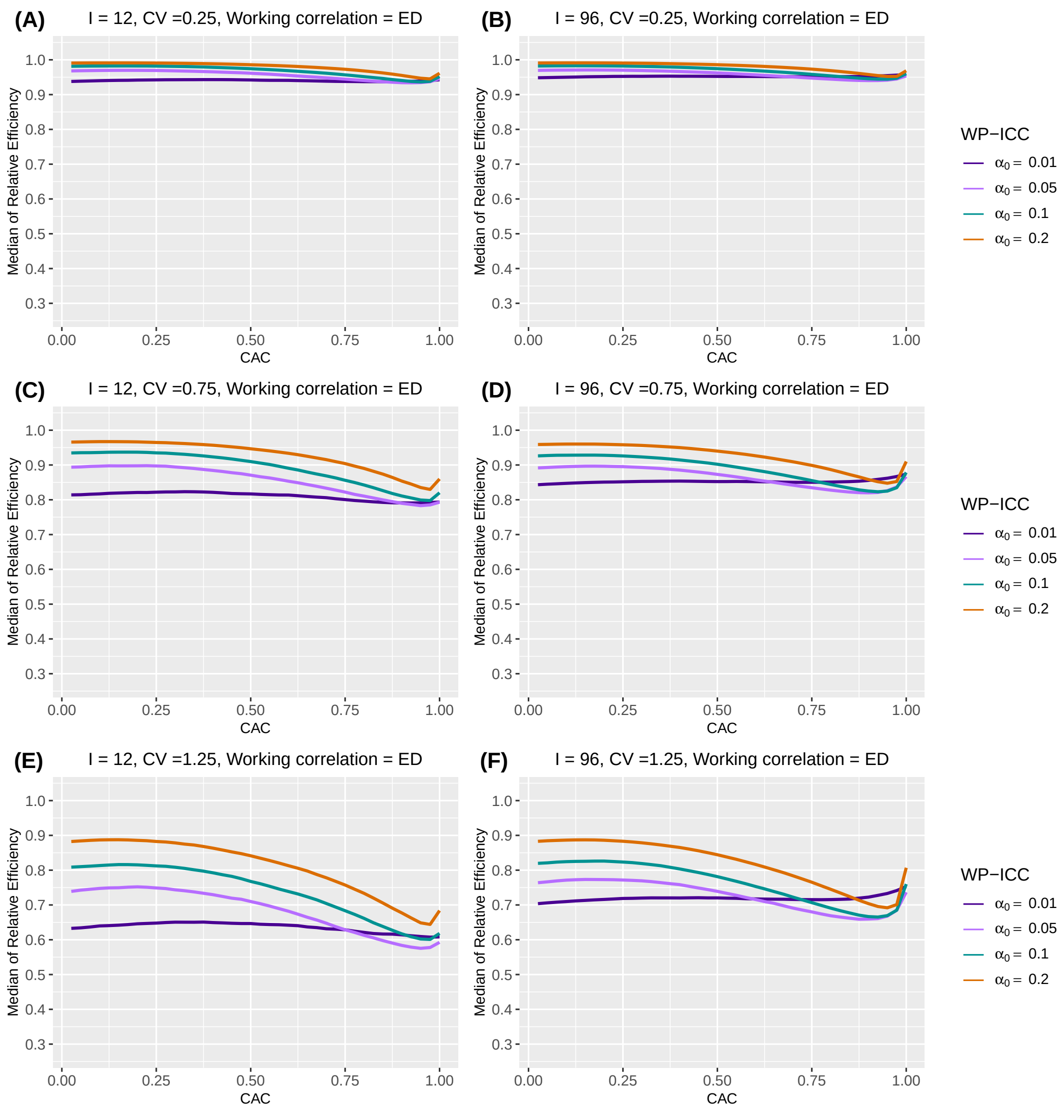
Web Figure 28 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 1: constant) is introduced.



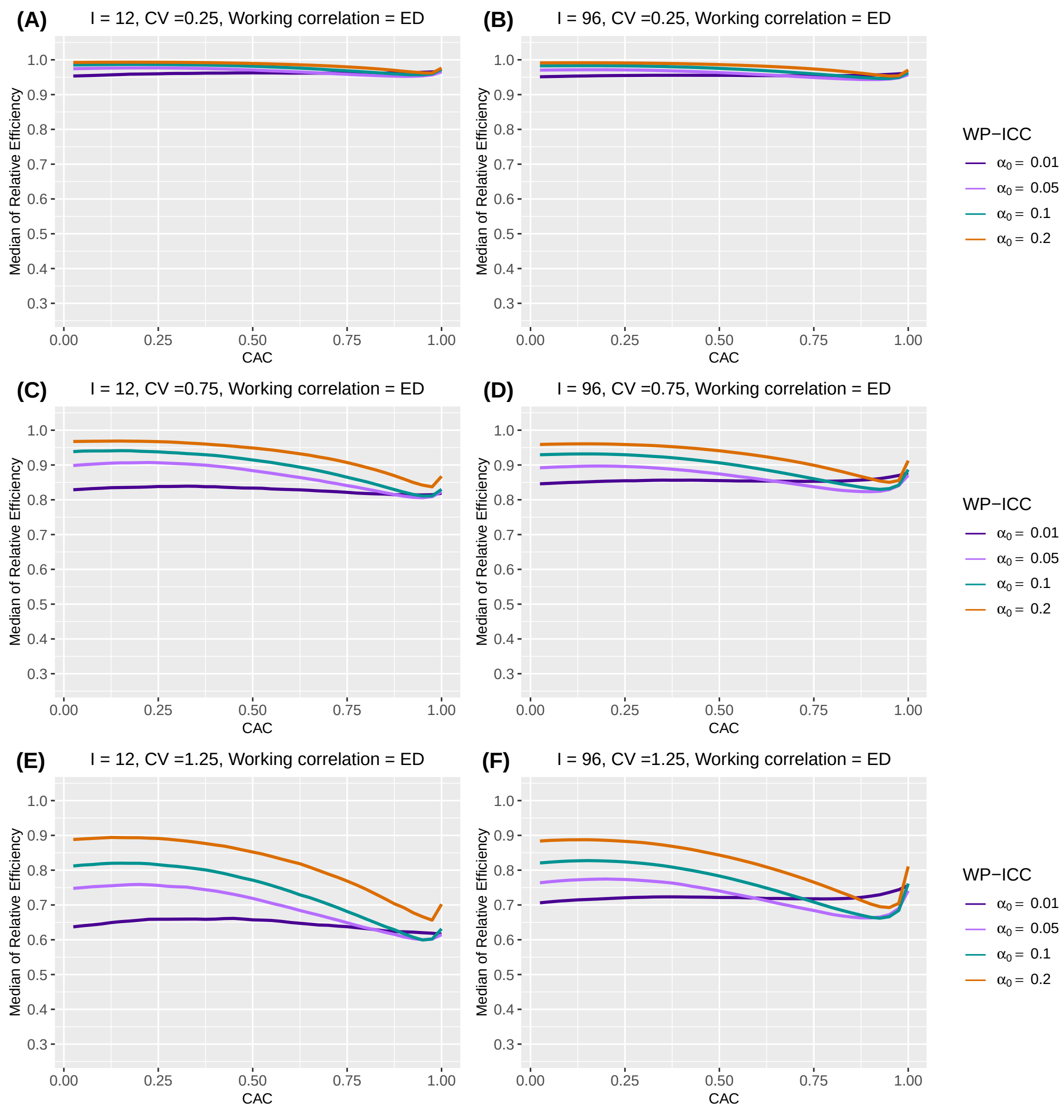
Web Figure 29 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



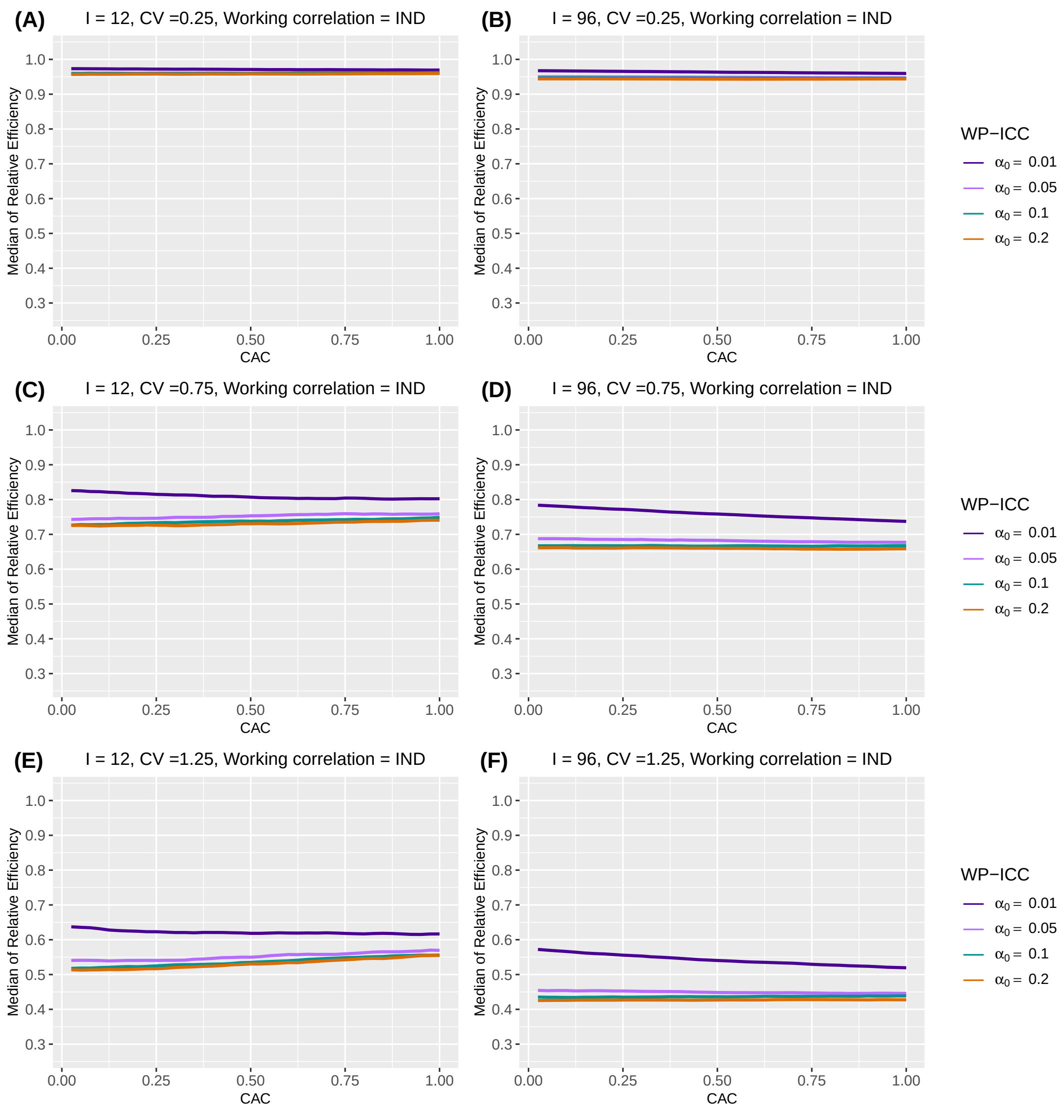
Web Figure 30 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



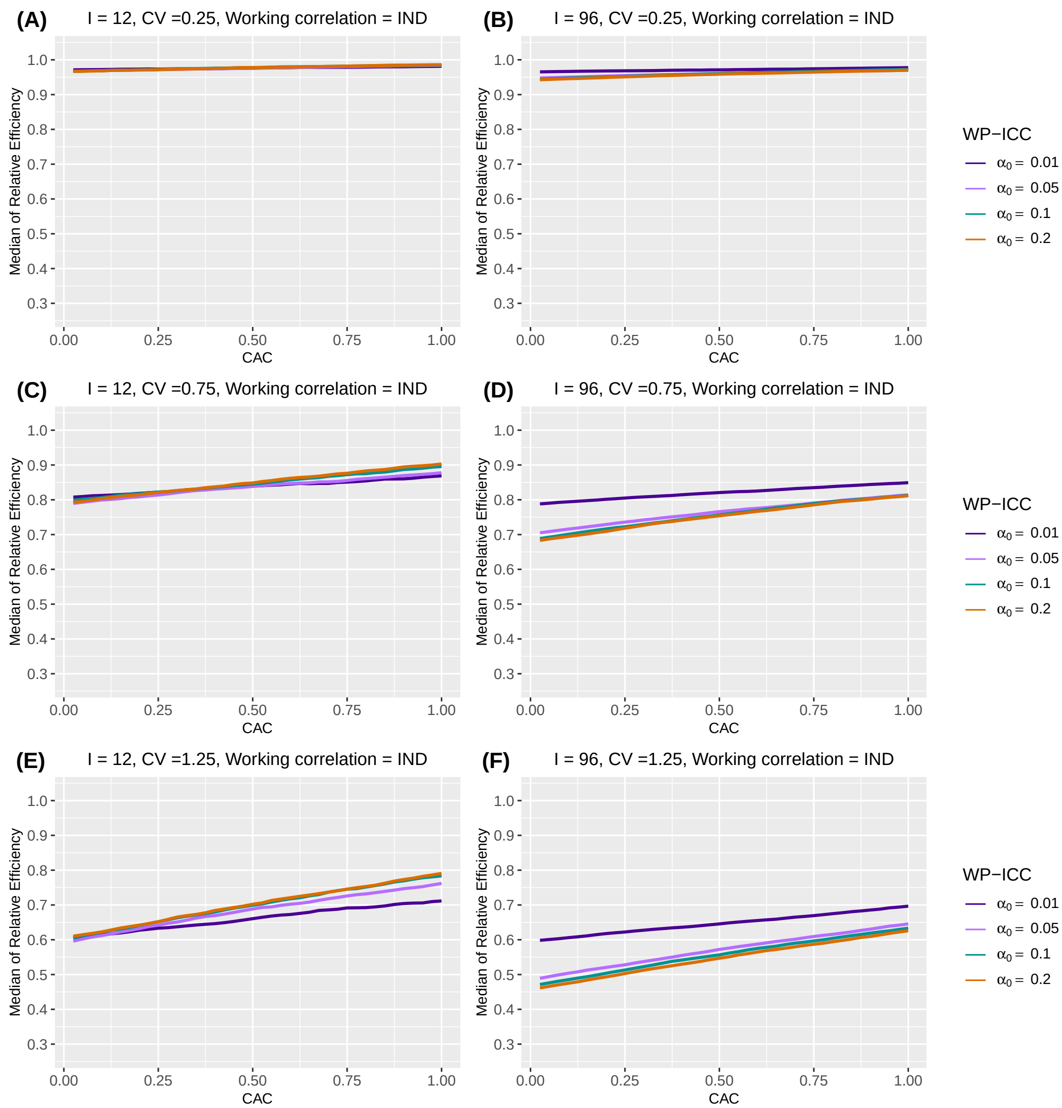
Web Figure 31 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



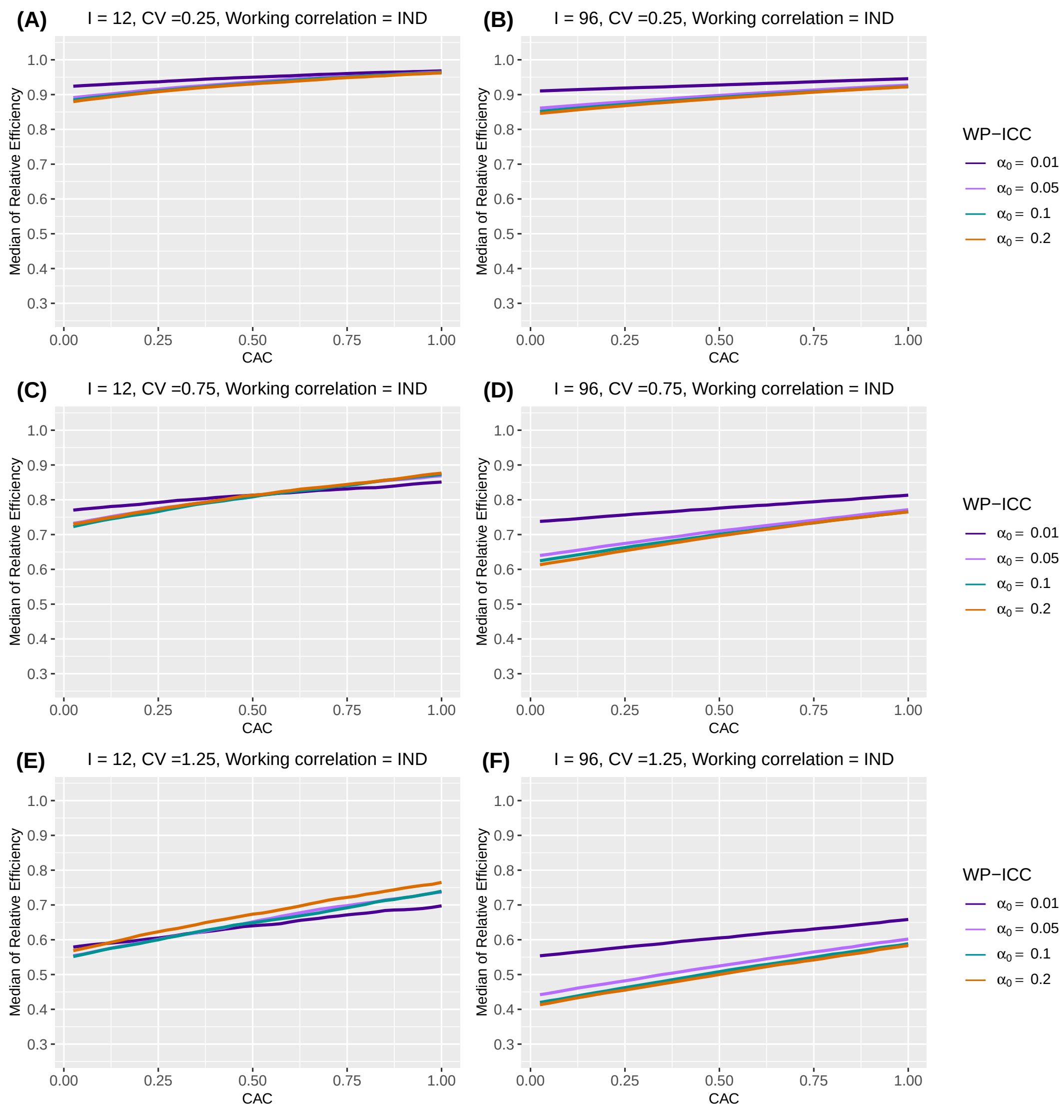
Web Figure 32 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



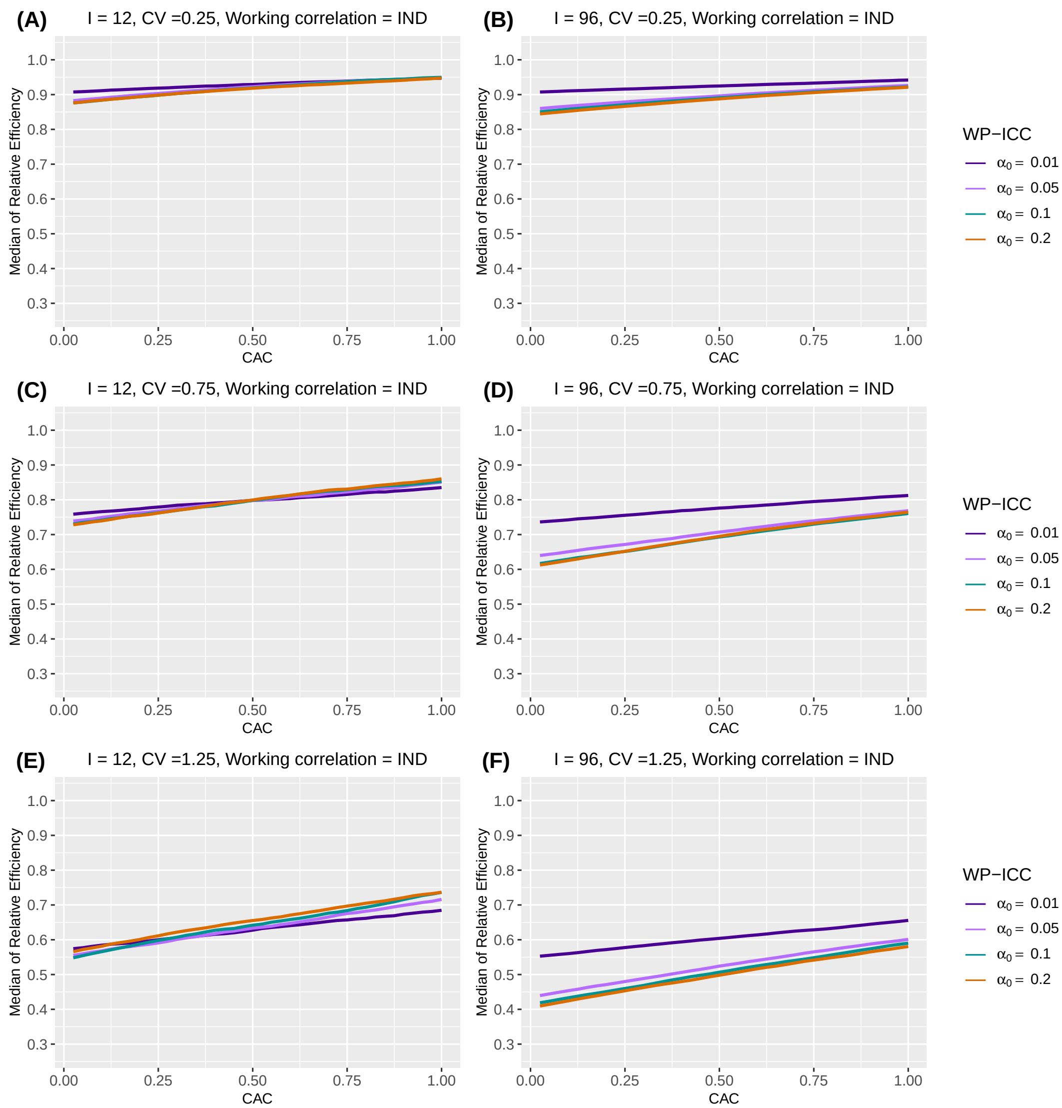
Web Figure 33 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 1: constant) is introduced.



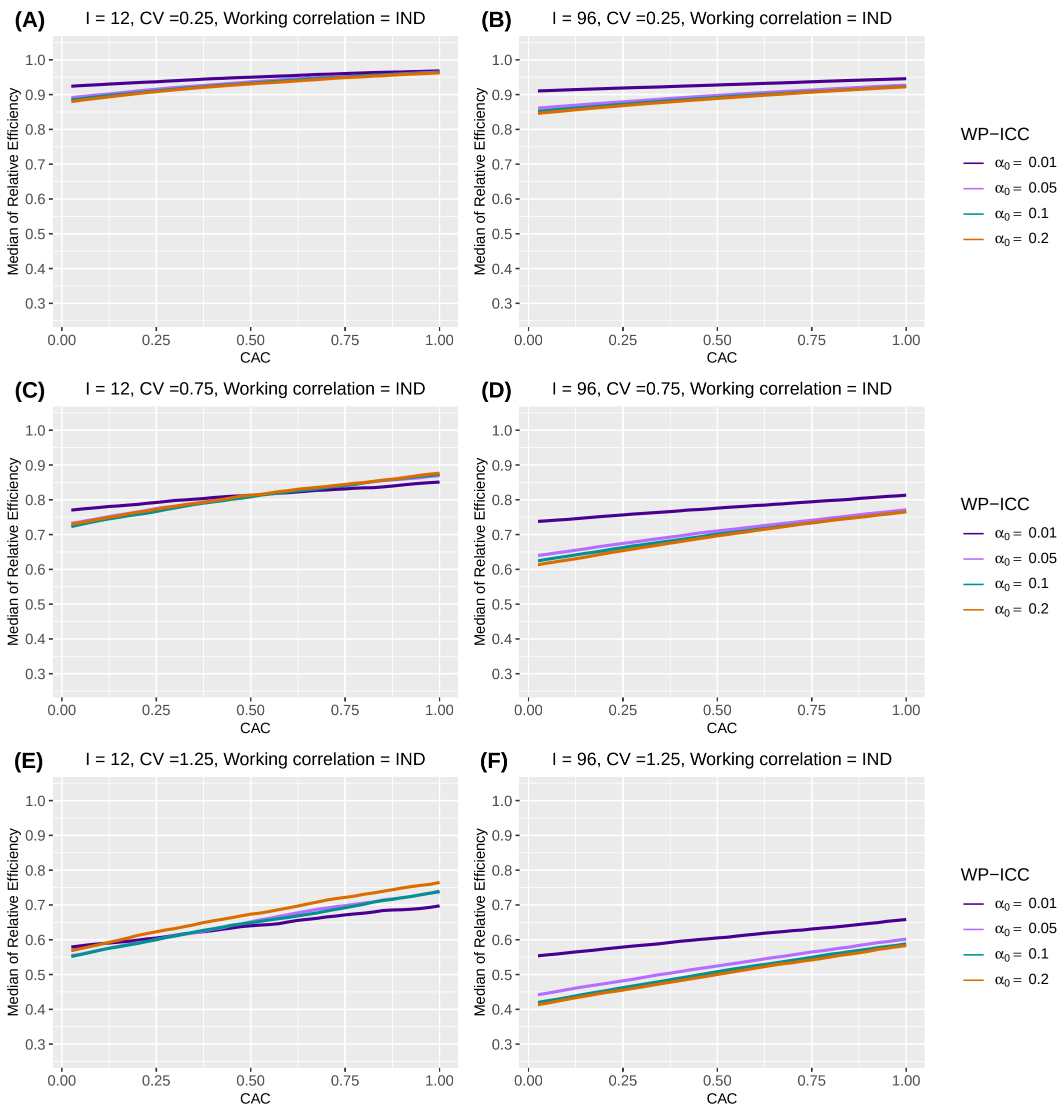
Web Figure 34 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 2: monotonically increasing) is introduced.



Web Figure 35 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 3: monotonically decreasing) is introduced.



Web Figure 36 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Appendix D.3. Number of periods

Tables in this section show the counterparts to tables that illustrate the impact of number of clusters on RE, but under exponential decay true correlation structure.

Web Table 12 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters is $I = 12$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.989 (0.984, 0.992)	0.986 (0.969, 1.001)	0.964 (0.947, 0.977)	0.964 (0.948, 0.979)
		0.75	0.902 (0.872, 0.928)	0.884 (0.803, 0.944)	0.856 (0.776, 0.915)	0.863 (0.782, 0.920)
		1.25	0.756 (0.704, 0.804)	0.704 (0.590, 0.817)	0.679 (0.571, 0.791)	0.684 (0.567, 0.800)
	5	0.25	0.990 (0.987, 0.992)	0.988 (0.978, 0.997)	0.970 (0.958, 0.98)	0.970 (0.959, 0.980)
		0.75	0.913 (0.888, 0.936)	0.897 (0.842, 0.940)	0.884 (0.825, 0.925)	0.881 (0.825, 0.925)
		1.25	0.773 (0.726, 0.819)	0.729 (0.648, 0.809)	0.720 (0.637, 0.803)	0.719 (0.635, 0.796)
	13	0.25	0.993 (0.990, 0.995)	0.991 (0.988, 0.994)	0.978 (0.974, 0.981)	0.978 (0.975, 0.981)
		0.75	0.933 (0.910, 0.952)	0.930 (0.903, 0.950)	0.917 (0.890, 0.936)	0.916 (0.890, 0.936)
		1.25	0.810 (0.769, 0.852)	0.811 (0.761, 0.852)	0.795 (0.744, 0.841)	0.795 (0.752, 0.840)
IND	3	0.25	0.961 (0.948, 0.972)	0.970 (0.951, 0.988)	0.891 (0.871, 0.908)	0.890 (0.870, 0.907)
		0.75	0.755 (0.694, 0.806)	0.825 (0.749, 0.899)	0.749 (0.674, 0.818)	0.755 (0.676, 0.823)
		1.25	0.547 (0.474, 0.621)	0.666 (0.583, 0.769)	0.611 (0.529, 0.704)	0.605 (0.531, 0.707)
	5	0.25	0.962 (0.939, 0.980)	0.977 (0.954, 0.998)	0.933 (0.907, 0.956)	0.936 (0.910, 0.959)
		0.75	0.752 (0.687, 0.819)	0.841 (0.766, 0.911)	0.813 (0.738, 0.882)	0.805 (0.725, 0.883)
		1.25	0.546 (0.466, 0.628)	0.678 (0.570, 0.771)	0.651 (0.546, 0.748)	0.645 (0.551, 0.747)
	13	0.25	0.961 (0.943, 0.974)	0.982 (0.976, 0.989)	0.950 (0.937, 0.961)	0.950 (0.937, 0.962)
		0.75	0.746 (0.677, 0.805)	0.873 (0.823, 0.909)	0.848 (0.786, 0.886)	0.847 (0.796, 0.889)
		1.25	0.530 (0.452, 0.610)	0.699 (0.621, 0.772)	0.681 (0.603, 0.749)	0.679 (0.597, 0.754)

Web Table 13 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters is $I = 24$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.987 (0.984, 0.990)	0.984 (0.972, 0.994)	0.962 (0.950, 0.973)	0.962 (0.951, 0.974)
		0.75	0.898 (0.876, 0.916)	0.877 (0.822, 0.921)	0.854 (0.806, 0.894)	0.855 (0.806, 0.897)
		1.25	0.758 (0.720, 0.793)	0.707 (0.630, 0.776)	0.696 (0.622, 0.769)	0.684 (0.605, 0.761)
	5	0.25	0.989 (0.987, 0.991)	0.987 (0.977, 0.996)	0.960 (0.950, 0.969)	0.960 (0.951, 0.969)
		0.75	0.909 (0.891, 0.926)	0.897 (0.855, 0.932)	0.873 (0.830, 0.908)	0.871 (0.825, 0.916)
		1.25	0.774 (0.743, 0.807)	0.738 (0.671, 0.801)	0.719 (0.654, 0.786)	0.725 (0.658, 0.794)
	13	0.25	0.992 (0.990, 0.994)	0.991 (0.987, 0.993)	0.979 (0.975, 0.982)	0.979 (0.976, 0.982)
		0.75	0.929 (0.912, 0.942)	0.921 (0.902, 0.938)	0.910 (0.890, 0.926)	0.912 (0.892, 0.930)
		1.25	0.810 (0.780, 0.838)	0.796 (0.760, 0.830)	0.788 (0.748, 0.821)	0.787 (0.750, 0.820)
IND	3	0.25	0.955 (0.945, 0.964)	0.958 (0.942, 0.972)	0.878 (0.863, 0.892)	0.881 (0.864, 0.894)
		0.75	0.721 (0.673, 0.763)	0.767 (0.696, 0.831)	0.693 (0.629, 0.756)	0.700 (0.642, 0.756)
		1.25	0.501 (0.440, 0.560)	0.591 (0.508, 0.659)	0.532 (0.464, 0.611)	0.525 (0.452, 0.605)
	5	0.25	0.954 (0.938, 0.970)	0.970 (0.946, 0.993)	0.896 (0.875, 0.914)	0.895 (0.873, 0.914)
		0.75	0.720 (0.661, 0.771)	0.812 (0.748, 0.878)	0.753 (0.687, 0.809)	0.748 (0.683, 0.811)
		1.25	0.499 (0.429, 0.561)	0.633 (0.545, 0.719)	0.584 (0.504, 0.663)	0.582 (0.493, 0.670)
	13	0.25	0.953 (0.939, 0.965)	0.981 (0.973, 0.988)	0.954 (0.944, 0.961)	0.955 (0.944, 0.962)
		0.75	0.709 (0.652, 0.757)	0.870 (0.831, 0.897)	0.843 (0.802, 0.874)	0.845 (0.803, 0.877)
		1.25	0.480 (0.419, 0.541)	0.695 (0.622, 0.752)	0.673 (0.598, 0.736)	0.675 (0.612, 0.738)

Web Table 14 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters is $I = 48$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.987 (0.985, 0.989)	0.984 (0.976, 0.992)	0.962 (0.954, 0.971)	0.961 (0.953, 0.969)
		0.75	0.895 (0.880, 0.908)	0.872 (0.837, 0.903)	0.855 (0.822, 0.885)	0.853 (0.821, 0.882)
		1.25	0.756 (0.727, 0.781)	0.715 (0.658, 0.761)	0.695 (0.644, 0.746)	0.698 (0.644, 0.742)
	5	0.25	0.989 (0.987, 0.990)	0.987 (0.980, 0.993)	0.962 (0.955, 0.969)	0.961 (0.954, 0.968)
		0.75	0.906 (0.893, 0.917)	0.896 (0.866, 0.922)	0.875 (0.845, 0.898)	0.874 (0.845, 0.899)
		1.25	0.774 (0.750, 0.795)	0.747 (0.699, 0.796)	0.731 (0.687, 0.778)	0.732 (0.683, 0.780)
	13	0.25	0.992 (0.991, 0.993)	0.991 (0.987, 0.995)	0.982 (0.978, 0.986)	0.982 (0.978, 0.987)
		0.75	0.926 (0.915, 0.936)	0.922 (0.902, 0.939)	0.914 (0.896, 0.933)	0.914 (0.896, 0.932)
		1.25	0.809 (0.788, 0.827)	0.807 (0.773, 0.839)	0.801 (0.766, 0.833)	0.802 (0.769, 0.831)
IND	3	0.25	0.952 (0.945, 0.959)	0.951 (0.939, 0.962)	0.874 (0.860, 0.884)	0.871 (0.859, 0.882)
		0.75	0.701 (0.665, 0.734)	0.725 (0.669, 0.778)	0.666 (0.619, 0.707)	0.661 (0.612, 0.711)
		1.25	0.469 (0.419, 0.514)	0.530 (0.461, 0.590)	0.481 (0.421, 0.538)	0.476 (0.417, 0.536)
	5	0.25	0.951 (0.938, 0.962)	0.965 (0.947, 0.980)	0.908 (0.894, 0.924)	0.908 (0.893, 0.922)
		0.75	0.694 (0.655, 0.737)	0.787 (0.734, 0.832)	0.737 (0.686, 0.781)	0.736 (0.690, 0.780)
		1.25	0.462 (0.407, 0.516)	0.597 (0.533, 0.666)	0.558 (0.494, 0.616)	0.551 (0.487, 0.615)
	13	0.25	0.949 (0.939, 0.959)	0.978 (0.967, 0.988)	0.961 (0.950, 0.971)	0.962 (0.951, 0.973)
		0.75	0.689 (0.646, 0.722)	0.853 (0.814, 0.885)	0.833 (0.794, 0.872)	0.836 (0.796, 0.870)
		1.25	0.452 (0.398, 0.499)	0.682 (0.619, 0.740)	0.672 (0.610, 0.726)	0.671 (0.609, 0.722)

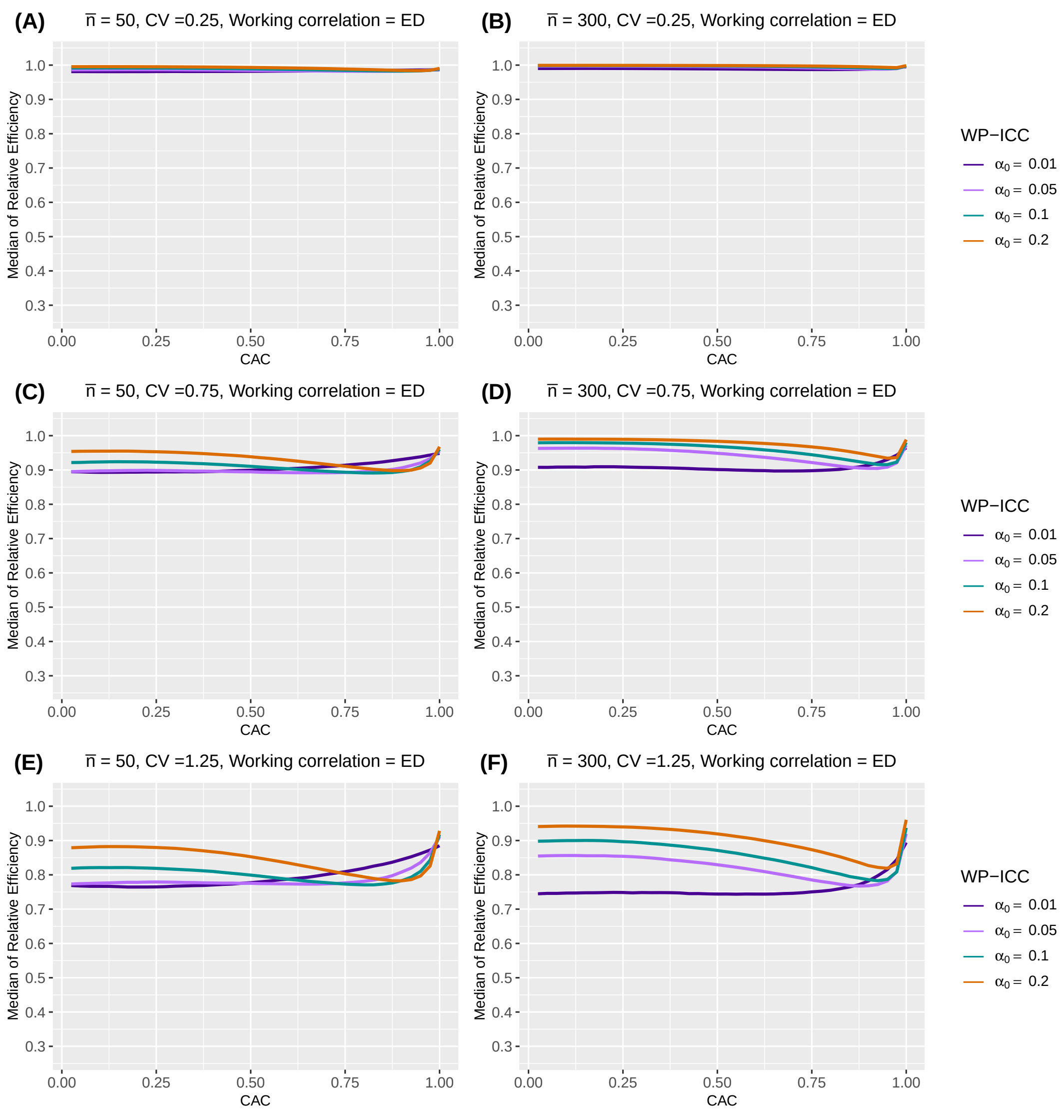
Web Table 15 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters is $I = 96$, WP-ICC α_0 is 0.05, and the CAC is 0.5.

Working correlation	J	CV	No within-cluster imbalance	Within-cluster imbalance pattern 1	Within-cluster imbalance pattern 2	Within-cluster imbalance pattern 4
NEX	3	0.25	0.987 (0.985, 0.988)	0.983 (0.978, 0.989)	0.961 (0.956, 0.967)	0.961 (0.955, 0.967)
		0.75	0.893 (0.882, 0.903)	0.871 (0.847, 0.895)	0.853 (0.829, 0.875)	0.854 (0.828, 0.875)
		1.25	0.755 (0.736, 0.772)	0.708 (0.671, 0.743)	0.695 (0.661, 0.727)	0.693 (0.652, 0.726)
	5	0.25	0.989 (0.988, 0.990)	0.986 (0.981, 0.992)	0.963 (0.958, 0.969)	0.963 (0.958, 0.968)
		0.75	0.905 (0.896, 0.913)	0.892 (0.874, 0.914)	0.876 (0.855, 0.896)	0.874 (0.852, 0.893)
		1.25	0.773 (0.756, 0.789)	0.753 (0.723, 0.785)	0.739 (0.707, 0.768)	0.740 (0.705, 0.771)
	13	0.25	0.992 (0.991, 0.993)	0.991 (0.986, 0.995)	0.985 (0.981, 0.990)	0.986 (0.981, 0.990)
		0.75	0.925 (0.917, 0.932)	0.924 (0.905, 0.940)	0.920 (0.901, 0.936)	0.920 (0.902, 0.937)
		1.25	0.807 (0.792, 0.823)	0.813 (0.786, 0.845)	0.812 (0.782, 0.838)	0.810 (0.781, 0.841)
IND	3	0.25	0.951 (0.946, 0.956)	0.947 (0.938, 0.956)	0.870 (0.861, 0.879)	0.870 (0.861, 0.878)
		0.75	0.689 (0.662, 0.714)	0.706 (0.660, 0.743)	0.643 (0.604, 0.679)	0.641 (0.603, 0.680)
		1.25	0.450 (0.414, 0.488)	0.482 (0.430, 0.534)	0.441 (0.396, 0.486)	0.438 (0.390, 0.485)
	5	0.25	0.949 (0.941, 0.957)	0.962 (0.950, 0.973)	0.898 (0.887, 0.910)	0.898 (0.886, 0.909)
		0.75	0.680 (0.649, 0.714)	0.767 (0.730, 0.804)	0.713 (0.676, 0.747)	0.710 (0.673, 0.743)
		1.25	0.445 (0.403, 0.484)	0.566 (0.515, 0.617)	0.516 (0.475, 0.564)	0.522 (0.470, 0.572)
	13	0.25	0.947 (0.940, 0.954)	0.975 (0.964, 0.986)	0.962 (0.952, 0.972)	0.962 (0.951, 0.973)
		0.75	0.673 (0.641, 0.700)	0.835 (0.802, 0.867)	0.823 (0.789, 0.852)	0.819 (0.786, 0.853)
		1.25	0.433 (0.394, 0.469)	0.668 (0.618, 0.715)	0.650 (0.599, 0.697)	0.650 (0.602, 0.698)

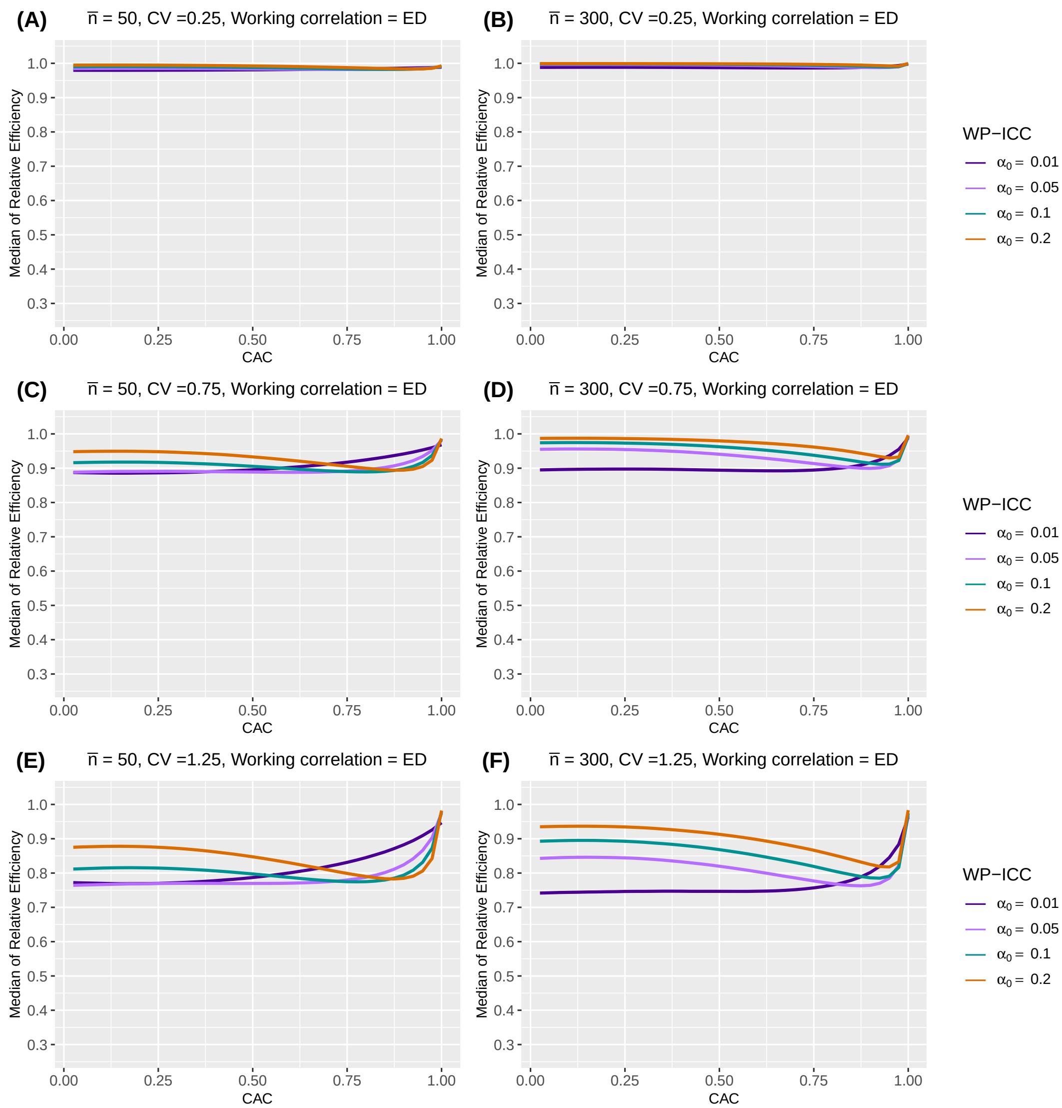
Web Appendix D.4. Cluster-period size

Figures in this section show the counterparts to plots that illustrate the impact of mean of cluster-period size on RE, but under exponential decay true correlation structure.

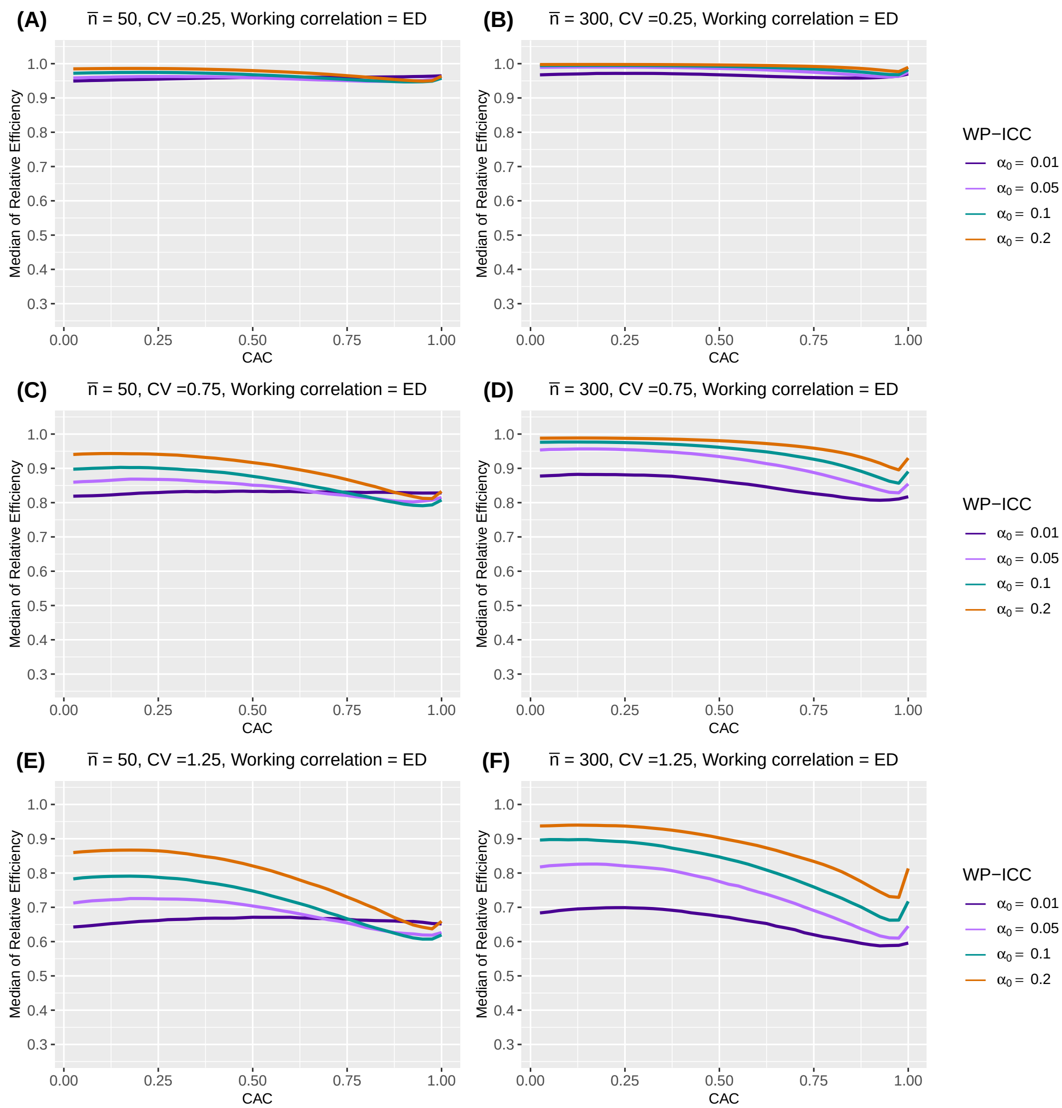
Web Figure 37 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



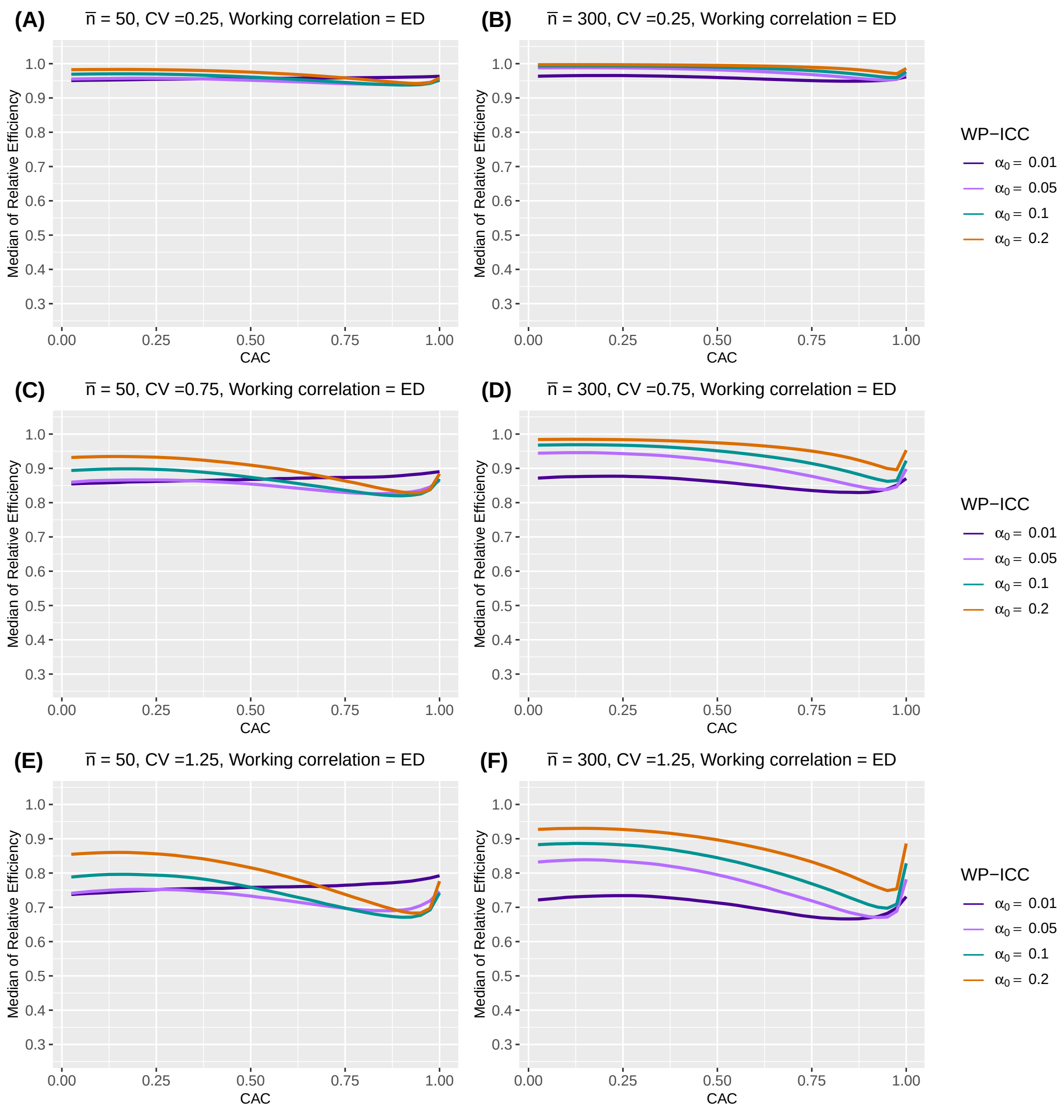
Web Figure 38 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



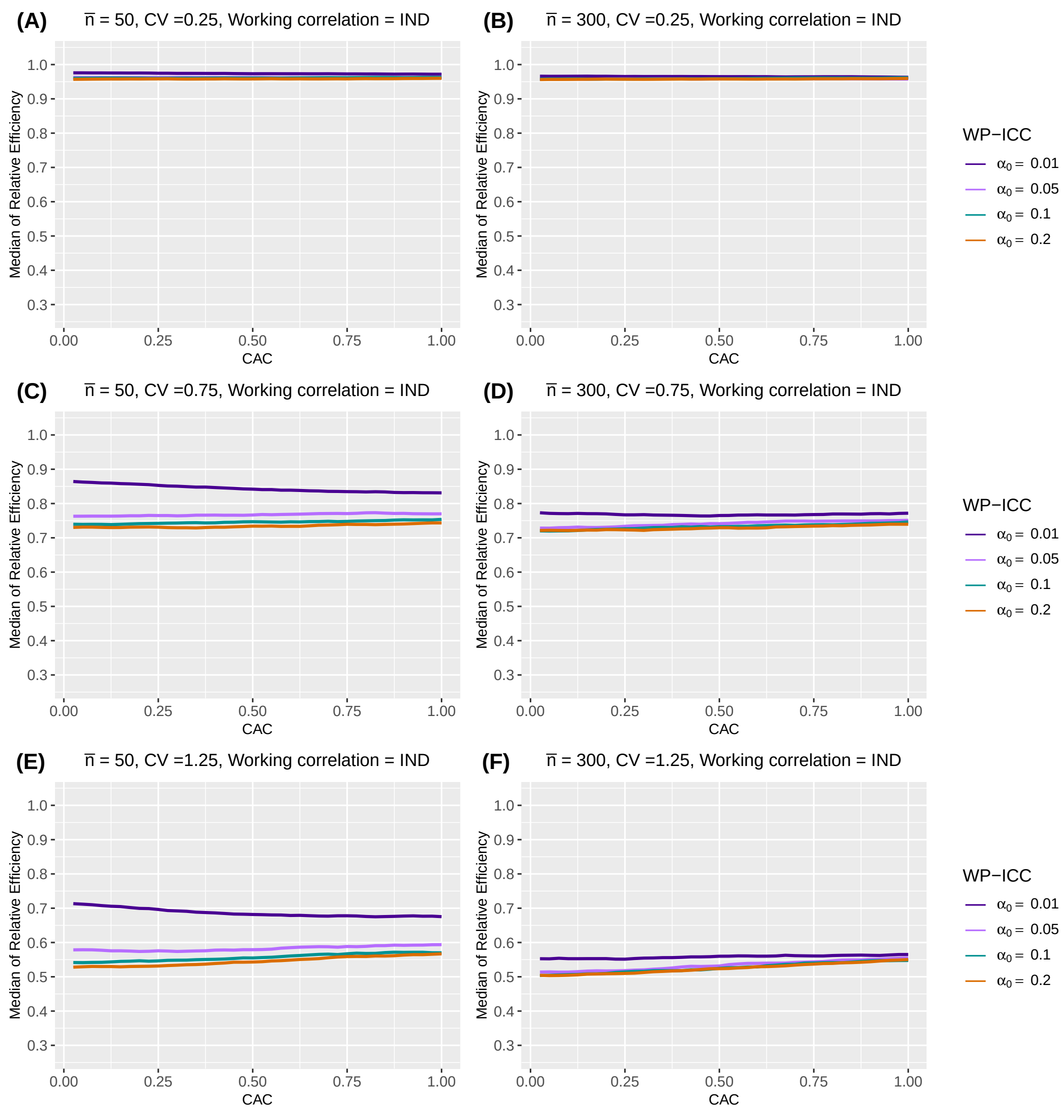
Web Figure 39 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



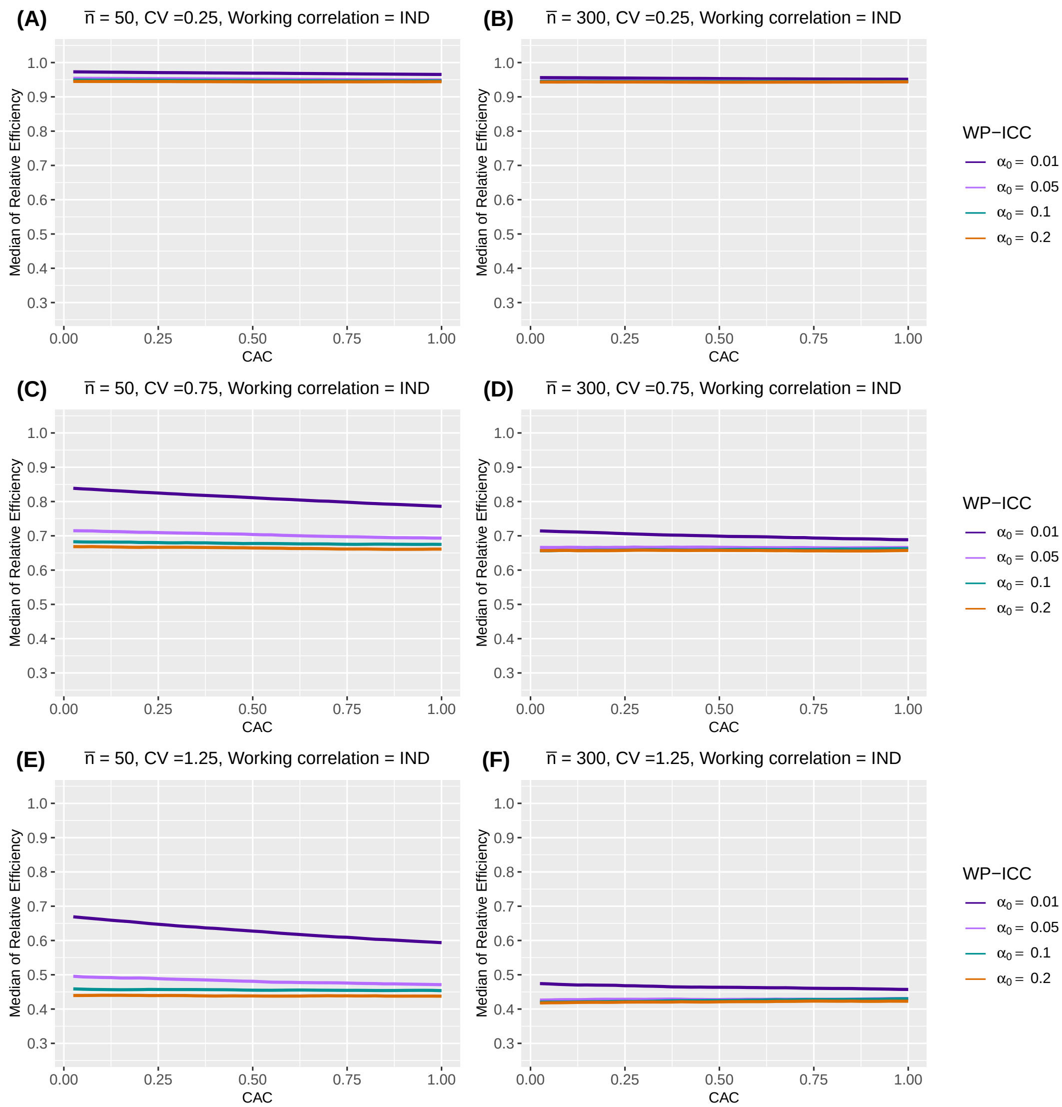
Web Figure 40 Median of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



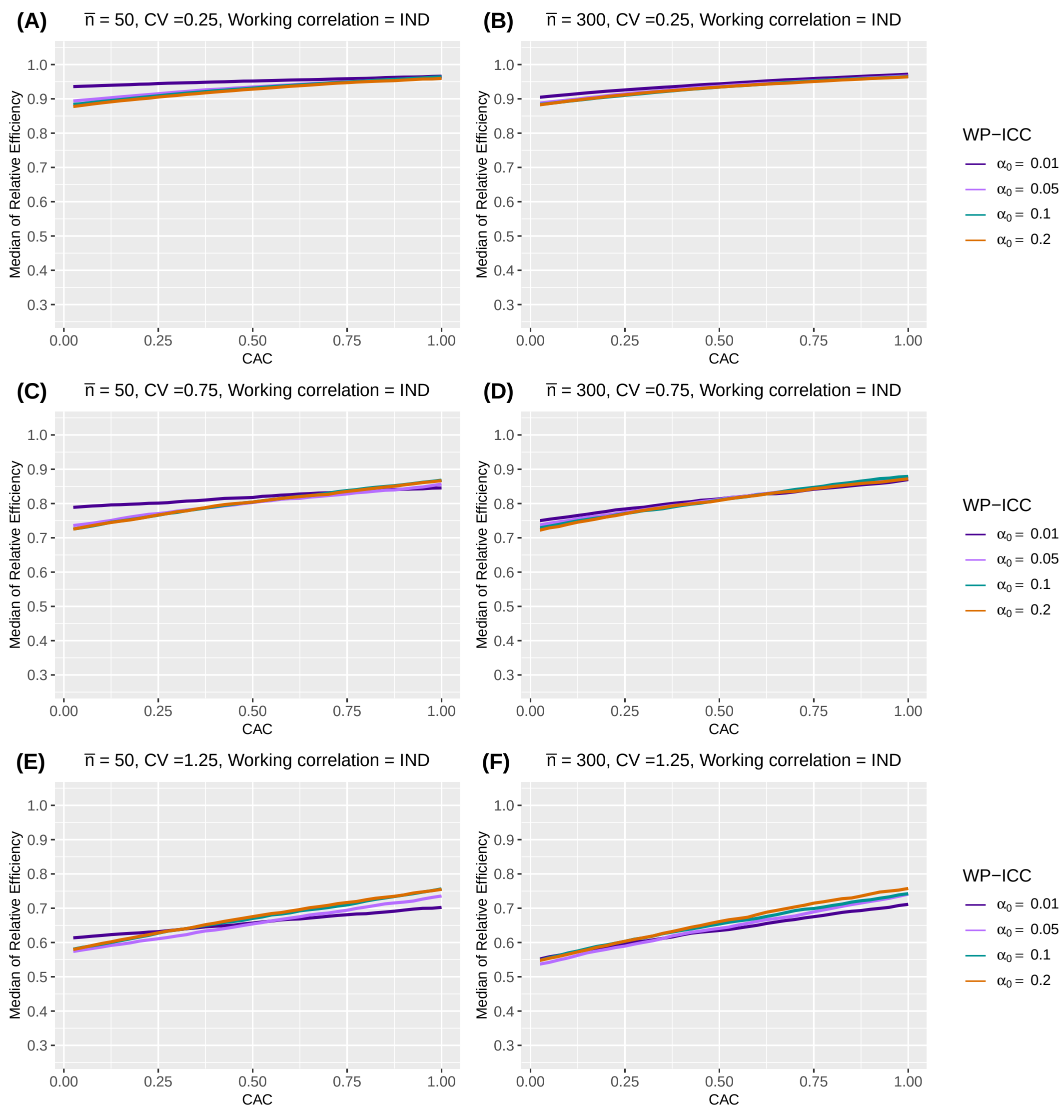
Web Figure 41 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



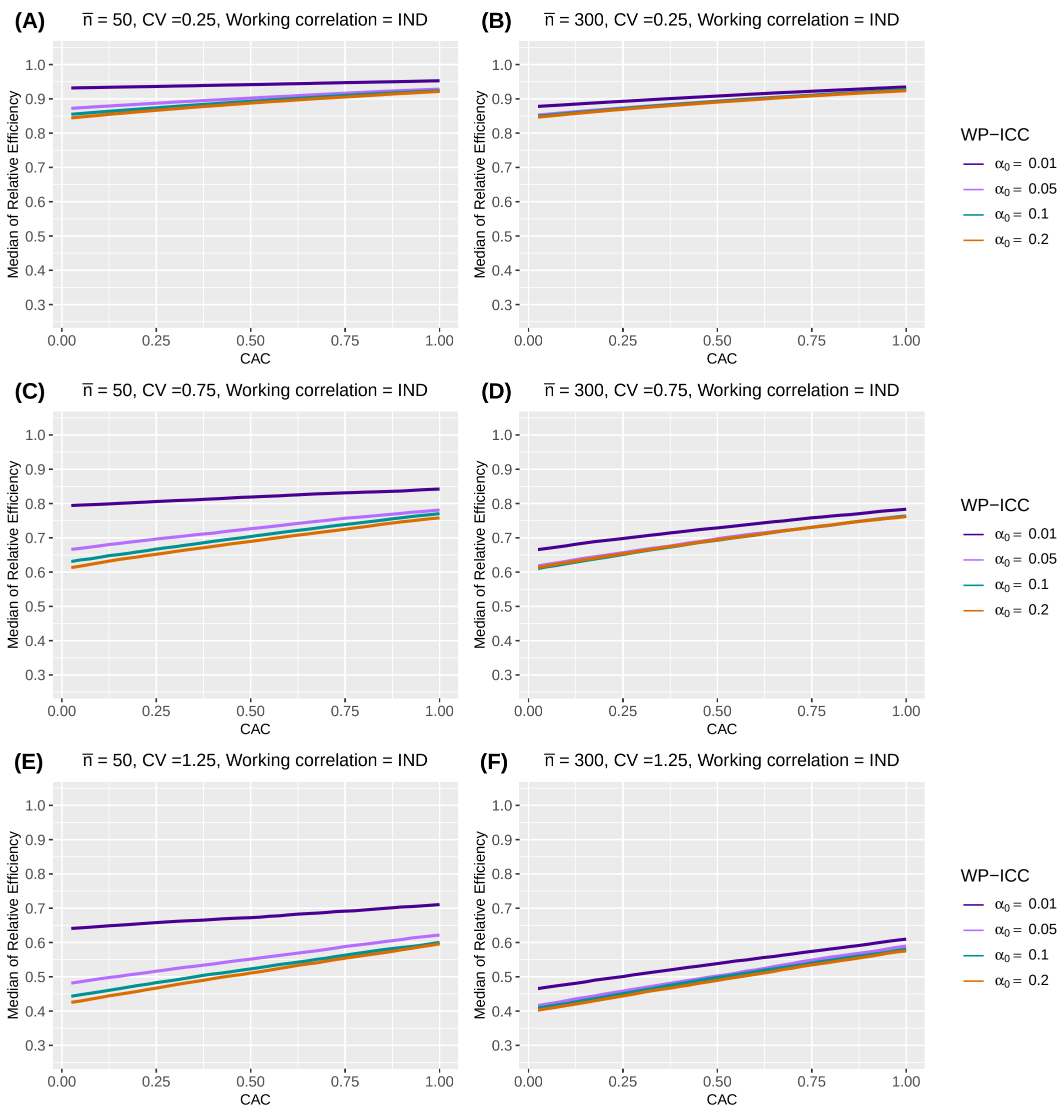
Web Figure 42 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. No within-cluster imbalance is introduced.



Web Figure 43 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 12$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Figure 44 Median of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 96$, number of periods $J = 5$, mean cluster-period sizes $\overline{n} \in \{50, 300\}$, the degree of between-cluster imbalance CV $\in \{0.25, 0.75, 1.25\}$. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.



Web Appendix D.5. Sensitivity to baseline prevalence, intervention effect and secular trend

Tables in this section show the counterparts to tables that illustrate the impact of baseline prevalence, intervention effect and secular trend of the outcomes on RE, but under exponential decay true correlation structure.

Web Table 16 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.987 (0.984, 0.989)	0.986 (0.984, 0.989)	0.987 (0.984, 0.989)
		0.3	0.985 (0.983, 0.988)	0.985 (0.982, 0.988)	0.985 (0.983, 0.988)
	0.75	0.1	0.908 (0.891, 0.924)	0.905 (0.888, 0.922)	0.907 (0.889, 0.921)
		0.3	0.902 (0.883, 0.918)	0.899 (0.879, 0.916)	0.901 (0.883, 0.917)
	1.25	0.1	0.790 (0.755, 0.822)	0.786 (0.754, 0.817)	0.792 (0.755, 0.822)
		0.3	0.780 (0.744, 0.814)	0.775 (0.741, 0.808)	0.784 (0.746, 0.815)
log(0.75)	0.25	0.1	0.984 (0.981, 0.987)	0.984 (0.981, 0.987)	0.984 (0.981, 0.987)
		0.3	0.984 (0.981, 0.987)	0.984 (0.981, 0.987)	0.984 (0.981, 0.987)
	0.75	0.1	0.897 (0.877, 0.914)	0.894 (0.875, 0.912)	0.895 (0.876, 0.912)
		0.3	0.897 (0.877, 0.914)	0.893 (0.873, 0.911)	0.894 (0.875, 0.911)
	1.25	0.1	0.771 (0.735, 0.806)	0.768 (0.733, 0.802)	0.774 (0.735, 0.806)
		0.3	0.771 (0.733, 0.805)	0.767 (0.732, 0.801)	0.773 (0.735, 0.805)

Web Table 17 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)
		0.3	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)
	0.75	0.1	0.910 (0.892, 0.926)	0.907 (0.889, 0.924)	0.908 (0.890, 0.925)
		0.3	0.909 (0.891, 0.926)	0.906 (0.887, 0.923)	0.907 (0.889, 0.924)
	1.25	0.1	0.776 (0.745, 0.809)	0.777 (0.743, 0.805)	0.777 (0.744, 0.808)
		0.3	0.774 (0.743, 0.807)	0.775 (0.741, 0.803)	0.776 (0.741, 0.806)
log(0.75)	0.25	0.1	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)
		0.3	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)	0.989 (0.987, 0.991)
	0.75	0.1	0.908 (0.890, 0.925)	0.905 (0.886, 0.923)	0.906 (0.888, 0.922)
		0.3	0.908 (0.890, 0.925)	0.905 (0.886, 0.923)	0.906 (0.888, 0.922)
	1.25	0.1	0.773 (0.741, 0.805)	0.774 (0.739, 0.802)	0.775 (0.740, 0.805)
		0.3	0.774 (0.741, 0.805)	0.773 (0.739, 0.802)	0.775 (0.740, 0.805)

Web Table 18 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.954 (0.931, 0.977)	0.954 (0.927, 0.977)	0.954 (0.929, 0.978)
		0.3	0.954 (0.931, 0.977)	0.954 (0.927, 0.977)	0.955 (0.929, 0.977)
	0.75	0.1	0.721 (0.647, 0.789)	0.714 (0.642, 0.778)	0.721 (0.647, 0.78)
		0.3	0.722 (0.642, 0.791)	0.714 (0.641, 0.779)	0.723 (0.645, 0.78)
	1.25	0.1	0.501 (0.421, 0.582)	0.502 (0.421, 0.576)	0.503 (0.427, 0.585)
		0.3	0.502 (0.422, 0.582)	0.503 (0.423, 0.578)	0.504 (0.427, 0.587)
log(0.75)	0.25	0.1	0.953 (0.930, 0.977)	0.953 (0.928, 0.977)	0.954 (0.930, 0.977)
		0.3	0.954 (0.929, 0.976)	0.953 (0.928, 0.977)	0.954 (0.930, 0.977)
	0.75	0.1	0.718 (0.641, 0.790)	0.716 (0.642, 0.783)	0.723 (0.645, 0.781)
		0.3	0.718 (0.641, 0.791)	0.718 (0.641, 0.784)	0.722 (0.644, 0.782)
	1.25	0.1	0.503 (0.427, 0.580)	0.501 (0.425, 0.582)	0.504 (0.426, 0.583)
		0.3	0.503 (0.426, 0.581)	0.500 (0.426, 0.582)	0.505 (0.425, 0.584)

Web Table 19 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. No within-cluster imbalance is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.954 (0.939, 0.970)	0.954 (0.937, 0.969)	0.954 (0.938, 0.969)
		0.3	0.954 (0.938, 0.970)	0.954 (0.937, 0.969)	0.954 (0.938, 0.970)
	0.75	0.1	0.721 (0.661, 0.771)	0.712 (0.655, 0.766)	0.717 (0.660, 0.764)
		0.3	0.720 (0.661, 0.771)	0.711 (0.655, 0.769)	0.716 (0.661, 0.767)
	1.25	0.1	0.499 (0.428, 0.561)	0.495 (0.430, 0.562)	0.500 (0.433, 0.570)
		0.3	0.499 (0.429, 0.561)	0.497 (0.431, 0.561)	0.500 (0.434, 0.570)
log(0.75)	0.25	0.1	0.954 (0.938, 0.969)	0.955 (0.937, 0.968)	0.954 (0.938, 0.969)
		0.3	0.954 (0.938, 0.969)	0.955 (0.937, 0.969)	0.954 (0.938, 0.969)
	0.75	0.1	0.719 (0.659, 0.773)	0.711 (0.657, 0.768)	0.716 (0.660, 0.768)
		0.3	0.718 (0.659, 0.773)	0.712 (0.656, 0.768)	0.717 (0.661, 0.768)
	1.25	0.1	0.499 (0.431, 0.561)	0.497 (0.430, 0.562)	0.499 (0.433, 0.567)
		0.3	0.498 (0.432, 0.560)	0.496 (0.432, 0.562)	0.499 (0.432, 0.567)

Web Table 20 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.943 (0.925, 0.959)	0.940 (0.923, 0.955)	0.942 (0.925, 0.959)
		0.3	0.934 (0.915, 0.953)	0.930 (0.912, 0.948)	0.935 (0.916, 0.952)
	0.75	0.1	0.818 (0.762, 0.872)	0.819 (0.769, 0.875)	0.813 (0.763, 0.865)
		0.3	0.802 (0.741, 0.858)	0.799 (0.743, 0.859)	0.797 (0.743, 0.852)
	1.25	0.1	0.637 (0.560, 0.715)	0.640 (0.562, 0.710)	0.638 (0.576, 0.725)
		0.3	0.611 (0.531, 0.692)	0.613 (0.532, 0.687)	0.618 (0.550, 0.699)
log(0.75)	0.25	0.1	0.926 (0.905, 0.945)	0.923 (0.904, 0.942)	0.924 (0.906, 0.943)
		0.3	0.924 (0.904, 0.943)	0.922 (0.902, 0.940)	0.922 (0.904, 0.942)
	0.75	0.1	0.787 (0.721, 0.843)	0.784 (0.726, 0.848)	0.777 (0.719, 0.835)
		0.3	0.784 (0.719, 0.841)	0.781 (0.725, 0.845)	0.774 (0.717, 0.833)
	1.25	0.1	0.593 (0.513, 0.672)	0.594 (0.518, 0.672)	0.597 (0.524, 0.679)
		0.3	0.590 (0.511, 0.668)	0.592 (0.515, 0.670)	0.594 (0.521, 0.675)

Web Table 21 Median and IQR (in parentheses) of relative efficiency when both the true correlation model and the working correlation model are ED. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.963 (0.953, 0.972)	0.961 (0.952, 0.969)	0.962 (0.952, 0.971)
		0.3	0.961 (0.950, 0.970)	0.959 (0.949, 0.967)	0.960 (0.950, 0.969)
	0.75	0.1	0.875 (0.832, 0.914)	0.878 (0.838, 0.913)	0.872 (0.826, 0.908)
		0.3	0.871 (0.828, 0.911)	0.873 (0.831, 0.911)	0.868 (0.823, 0.905)
	1.25	0.1	0.724 (0.658, 0.790)	0.730 (0.662, 0.790)	0.728 (0.668, 0.797)
		0.3	0.722 (0.649, 0.784)	0.723 (0.657, 0.785)	0.724 (0.662, 0.792)
log(0.75)	0.25	0.1	0.958 (0.948, 0.968)	0.957 (0.947, 0.966)	0.958 (0.947, 0.967)
		0.3	0.958 (0.947, 0.967)	0.956 (0.946, 0.965)	0.957 (0.947, 0.967)
	0.75	0.1	0.868 (0.824, 0.907)	0.870 (0.826, 0.908)	0.863 (0.818, 0.903)
		0.3	0.867 (0.824, 0.906)	0.869 (0.825, 0.908)	0.863 (0.818, 0.902)
	1.25	0.1	0.720 (0.644, 0.780)	0.720 (0.657, 0.782)	0.719 (0.657, 0.788)
		0.3	0.717 (0.644, 0.782)	0.719 (0.656, 0.781)	0.719 (0.655, 0.788)

Web Table 22 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 1. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline prevalence	Constant secular trend	Increasing secular trend	Decreasing secular trend
log(0.35)	0.25	0.1	0.920 (0.893, 0.947)	0.916 (0.886, 0.944)	0.920 (0.892, 0.949)
		0.3	0.917 (0.891, 0.944)	0.914 (0.884, 0.941)	0.917 (0.889, 0.947)
	0.75	0.1	0.799 (0.728, 0.874)	0.805 (0.725, 0.874)	0.790 (0.725, 0.864)
		0.3	0.797 (0.726, 0.870)	0.800 (0.722, 0.872)	0.788 (0.721, 0.862)
	1.25	0.1	0.649 (0.551, 0.744)	0.642 (0.550, 0.740)	0.650 (0.560, 0.742)
		0.3	0.646 (0.554, 0.744)	0.641 (0.553, 0.739)	0.652 (0.558, 0.743)
log(0.75)	0.25	0.1	0.914 (0.888, 0.942)	0.911 (0.881, 0.938)	0.914 (0.886, 0.944)
		0.3	0.914 (0.887, 0.941)	0.911 (0.881, 0.938)	0.913 (0.885, 0.943)
	0.75	0.1	0.794 (0.723, 0.865)	0.798 (0.721, 0.868)	0.787 (0.719, 0.862)
		0.3	0.794 (0.720, 0.864)	0.797 (0.721, 0.868)	0.787 (0.719, 0.861)
	1.25	0.1	0.642 (0.553, 0.736)	0.638 (0.557, 0.737)	0.647 (0.562, 0.741)
		0.3	0.643 (0.554, 0.735)	0.639 (0.558, 0.734)	0.648 (0.562, 0.740)

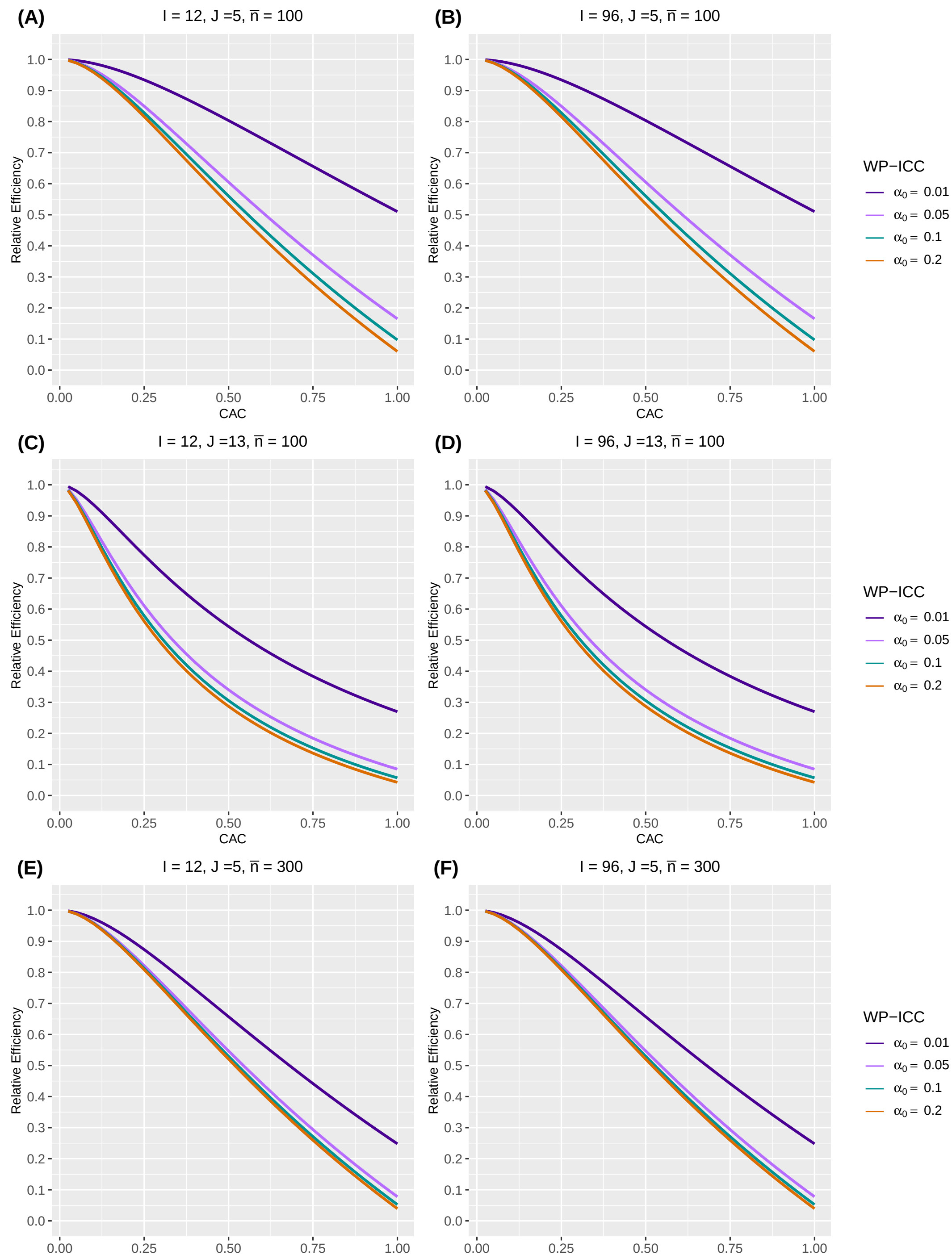
Web Table 23 Median and IQR (in parentheses) of relative efficiency when the true correlation model is ED but the working correlation model is IND. Parameter specifications: number of clusters $I = 24$, number of periods $J = 5$, mean cluster-period size $\overline{n} = 100$, WP-ICC α_0 is 0.05, CAC is 0.5. Within-cluster imbalance (pattern 4: randomly permuted) is introduced.

δ	CV	Baseline Prevalence	Constant Secular trend	Increasing Secular trend	Decreasing Secular trend
log(0.35)	0.25	0.1	0.900 (0.879, 0.918)	0.895 (0.873, 0.914)	0.899 (0.877, 0.920)
		0.3	0.897 (0.877, 0.916)	0.893 (0.872, 0.913)	0.897 (0.875, 0.918)
	0.75	0.1	0.755 (0.687, 0.812)	0.760 (0.687, 0.815)	0.746 (0.688, 0.806)
		0.3	0.753 (0.688, 0.809)	0.756 (0.687, 0.815)	0.744 (0.686, 0.805)
	1.25	0.1	0.581 (0.491, 0.668)	0.577 (0.500, 0.664)	0.585 (0.498, 0.665)
		0.3	0.584 (0.493, 0.668)	0.577 (0.503, 0.667)	0.586 (0.498, 0.666)
log(0.75)	0.25	0.1	0.895 (0.875, 0.913)	0.891 (0.870, 0.911)	0.894 (0.873, 0.915)
		0.3	0.895 (0.874, 0.913)	0.891 (0.870, 0.910)	0.894 (0.873, 0.915)
	0.75	0.1	0.752 (0.687, 0.806)	0.753 (0.686, 0.813)	0.744 (0.686, 0.805)
		0.3	0.750 (0.686, 0.806)	0.752 (0.689, 0.812)	0.742 (0.685, 0.805)
	1.25	0.1	0.586 (0.495, 0.662)	0.579 (0.507, 0.667)	0.587 (0.498, 0.669)
		0.3	0.586 (0.496, 0.662)	0.578 (0.506, 0.664)	0.587 (0.498, 0.668)

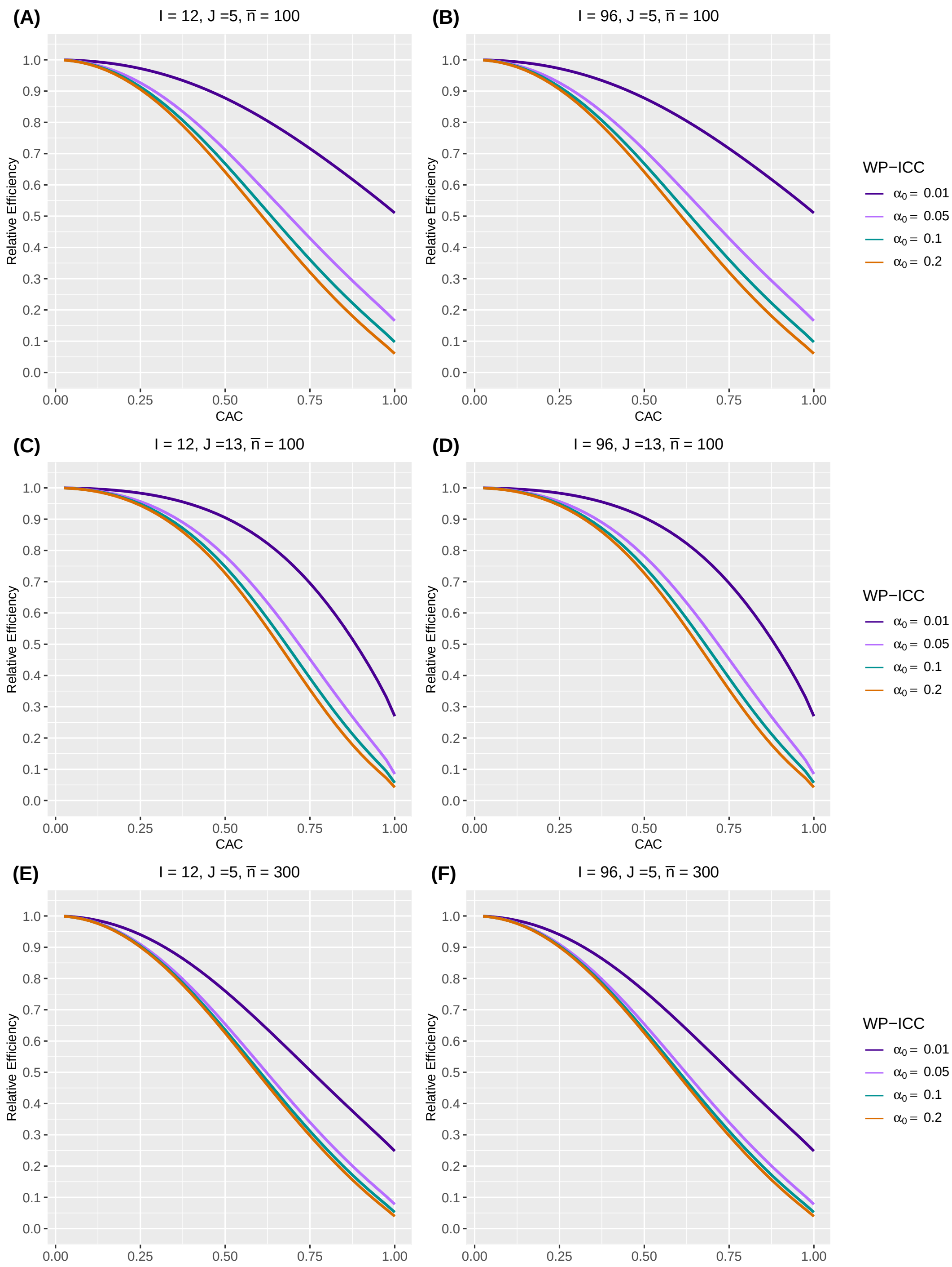
Web Appendix E. RE of GEE analysis under the true versus independence working correlation model

Figures in this section provide insight about the relative efficiency under the true versus independence working correlation model defined in Section 8 of the main article.

Web Figure 45 Median of newly defined relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$ and 13, and mean cluster-period sizes $\overline{n} = 100$ and 300.



Web Figure 46 Median of newly defined relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$ and 13, and mean cluster-period sizes $\overline{n} = 100$ and 300.



Web Appendix F. Compare simulation-based RE for binary outcomes and the reciprocal of the inflation factor derived for continuous outcomes

Figures in this section compare the relative efficiency in our simulations and the reciprocal of a conservative inflation factor $1 + \text{CV}^2$ as well as the relative efficiency given by the Taylor series expansion DE in Girling's paper (Girling, 2018) mentioned in Section 8 of the main article. CV denotes the coefficient of variation that measures the between-cluster imbalance.

The relative efficiency curves implied by the Taylor series expansion DE has the form follows.

$$RE = \frac{A\Psi\left(\frac{\lambda_0 \overline{n}\alpha_0}{1-\alpha_0}\right) + Bv\Psi\left(\frac{\lambda_1 \overline{n}\alpha_0}{1-\alpha_0}\right)}{A + Bv}$$

where

$$A = \frac{1}{12}\left(1 - \frac{2}{J(J-1)}\right)$$

$$B = \frac{1}{12}\left(1 - \frac{2}{J}\right)$$

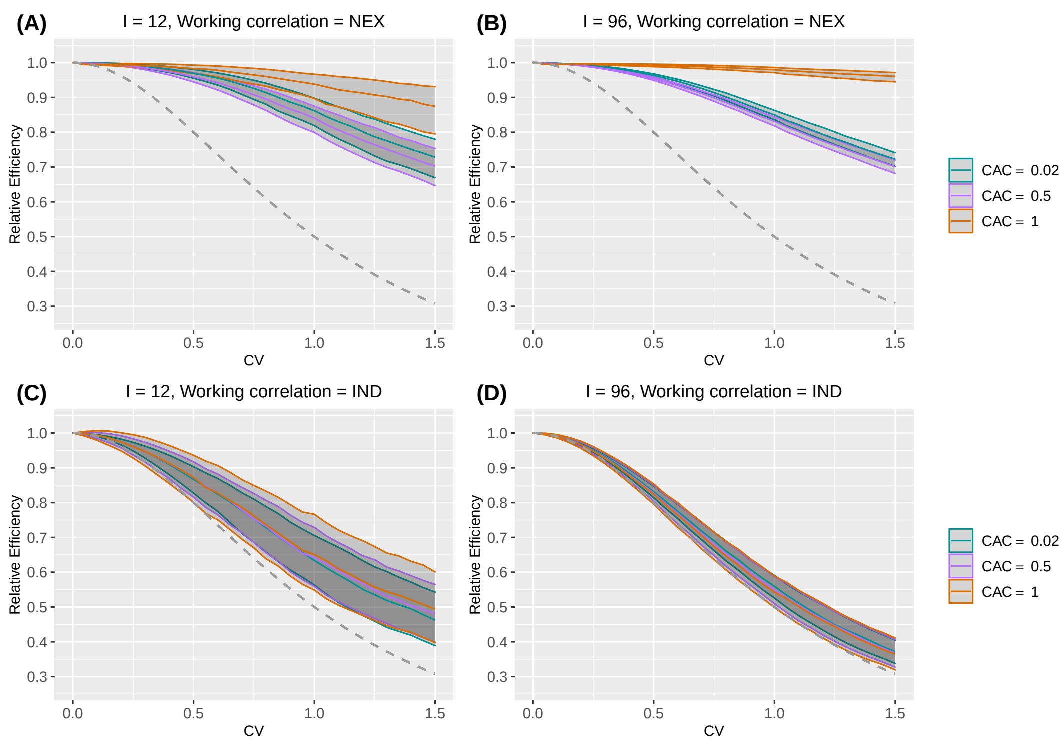
$$\lambda_0 = 1 - \text{CAC}$$

$$\lambda_1 = 1 + (J-1) \times \text{CAC}$$

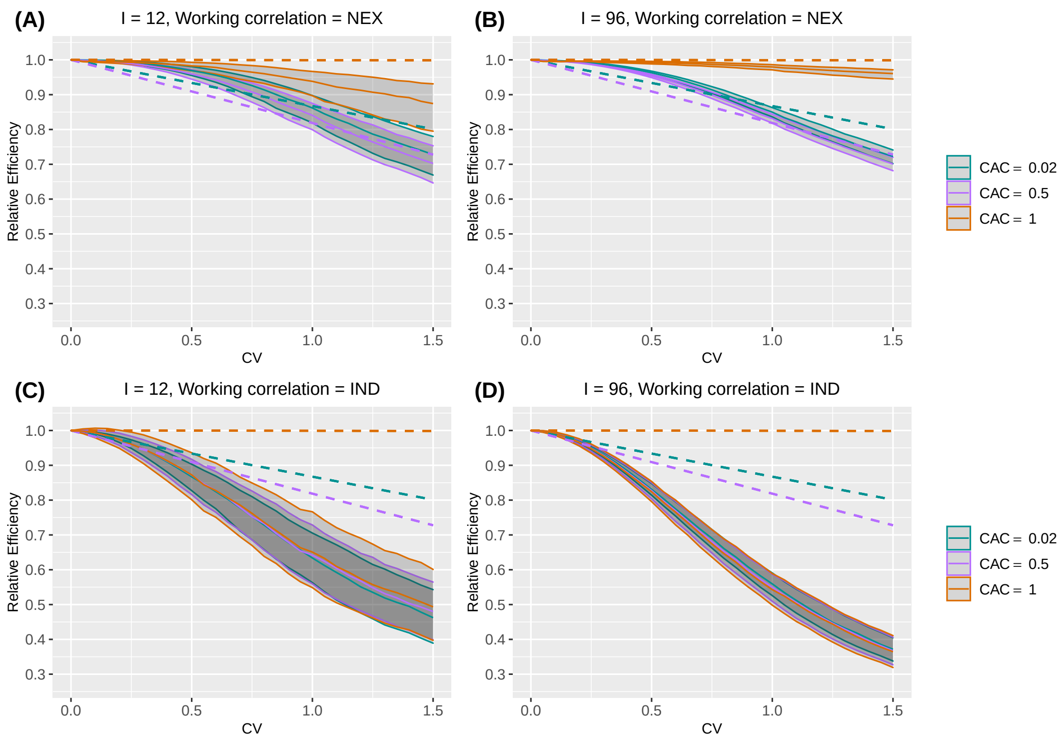
$$v = \frac{1 + \frac{\lambda_0 \overline{n}\alpha_0}{1-\alpha_0}}{1 + \frac{\lambda_1 \overline{n}\alpha_0}{1-\alpha_0}}$$

$$\Psi(\alpha) = 1 - \frac{\alpha \times \text{CV}^2}{(1+\alpha)^2}$$

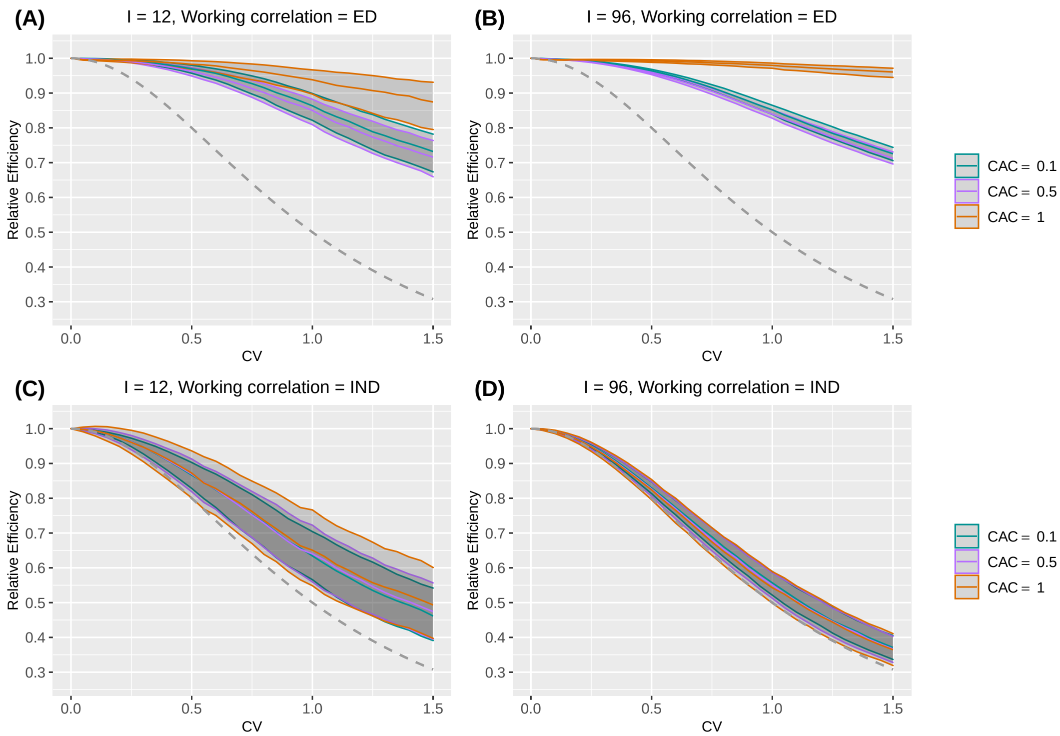
Web Figure 47 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster imbalance is introduced. The broken grey curve shows the reciprocal of the conservative inflation factor $1 + CV^2$.



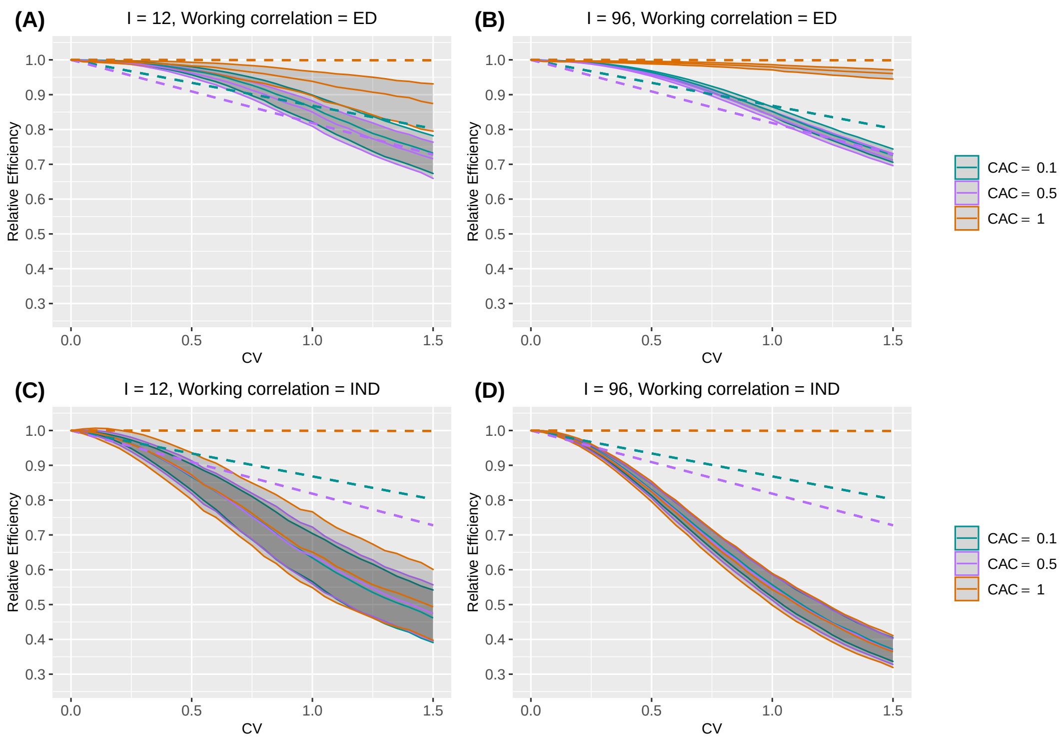
Web Figure 48 Median and IQR of relative efficiency when the true correlation model is NEX. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster imbalance is introduced. The broken lines show the computed Taylor series DE corresponding to different values of the cluster autocorrelation.



Web Figure 49 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster imbalance is introduced. The broken grey curve shows the reciprocal of the conservative inflation factor $1 + CV^2$.



Web Figure 50 Median and IQR of relative efficiency when the true correlation model is ED. Parameter specifications: number of clusters $I = 12$ and 96, number of periods $J = 5$, WP-ICC is fixed at $\alpha_0 = 0.05$. No within-cluster imbalance is introduced. The broken lines show the computed Taylor series DE corresponding to different values of the cluster autocorrelation.



References

Girling, A. J. (2018). Relative efficiency of unequal cluster sizes in stepped wedge and other trial designs under longitudinal or cross-sectional sampling. *Statistics in Medicine* **37,** 4652–4664.

Henderson, H. V. and Searle, S. R. (1981). On deriving the inverse of a sum of matrices. *Siam Review* **23,** 53–60.

Li, F., Forbes, A. B., Turner, E. L., and Preisser, J. S. (2019). Power and sample size requirements for gee analyses of cluster randomized crossover trials. *Statistics in Medicine* **38,** 636–649.

Li, F., Turner, E. L., and Preisser, J. S. (2018). Sample size determination for GEE analyses of stepped wedge cluster randomized trials. *Biometrics* **74,** 1450–1458.

Teerenstra, S., Lu, B., Preisser, J. S., Van Achterberg, T., and Borm, G. F. (2010). Sample size considerations for gee analyses of three-level cluster randomized trials. *Biometrics* **66,** 1230–1237.