Optimal two-stage sampling for mean estimation in multilevel populations when cluster size is informative

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Outline

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2. Definitions of population means
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4. Unbiased estimation
5. Optimal and maximin design
6. Application
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Motivating example

Average alcohol consumption among adolescents:

- Adolescents clustered in schools
- Schools vary in # of enrolled students
- Adolescents' alcohol consumption can be related to school size (McNeely et al. [2002]; Resnick et al. [1997]; Thompson et al. [2006])

General framework:

- Two-level population
- Cluster size variation
- Informative cluster size (Nevalainen et al. [2014]; Seaman et al. [2014])

General framework

The outcome variable Y_{ij} is **quantitative**

$$y_{ij} = \beta_0 + u_j + \epsilon_{ij} \tag{1}$$

$$\epsilon_{ij} \sim N\left(0, \sigma_\epsilon^2\right),\ u_j \perp \epsilon_{ij}.$$

Informative cluster size $\left(N_j\right)$:

$$u_j = \gamma\left(N_j - \theta_N\right) + \upsilon_j, \tag{2}$$

$$\upsilon_j \sim N\left(0, \sigma_\upsilon^2\right),\ \upsilon_j \perp N_j$$

γ = Informativeness parameter
θ_N = Population mean of cluster size

Definitions of population means

1. The average of all individual outcomes:

   $\mu =$ *Expected outcome for an individual randomly sampled from the population ignoring cluster membership*

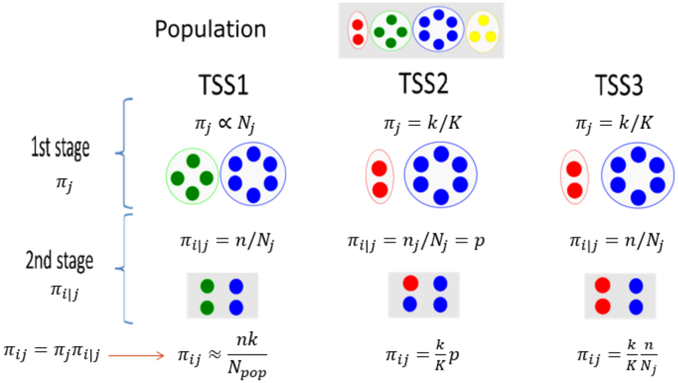
2. The average of all cluster-specific means:

   $\beta_0 =$ *Expected outcome for the average individual from the average cluster*

$$\mu = \beta_0 + \gamma \theta_N \tau_N^2 \tag{3}$$

$\gamma =$ Informativeness parameter
$\theta_N =$ Population mean of cluster size
$\tau_N =$ Population coefficient of variation of cluster size

Sampling from a multilevel population



- K = # of clusters in the population, k = # of clusters in the sample, N_j = cluster j size in the population, n or n_j = # of individuals sampled per cluster, N_{pop} = population size
- TSS1 requires prior knowledge of the whole cluster size distribution
- Sampling fraction is assumed to be negligible at each sampling stage

Unbiased estimation of μ

- Informative cluster size
  - SRS and TSS1: $\hat{\mu} = \sum_{j=1}^{k} \frac{\bar{y}_j}{k}$, $\bar{y}_j$ = cluster j mean, $k = \#$ of clusters
  - TSS2 and TSS3: $\hat{\mu} = \frac{\sum_{j=1}^{k} N_j \bar{y}_j}{\sum_{j=1}^{k} N_j} \Leftarrow$ <u>only asymptotically unbiased!</u>
  - For a given total sample size:

$$V_{SRS}(\hat{\mu}) \leq V_{TSS1}(\hat{\mu}) \leq V_{TSS2}(\hat{\mu}) \leq V_{TSS3}(\hat{\mu})$$

  TSS1 is the most efficient TSS for many cluster size distributions

- Non-informative cluster size (i.e. $\mu = \beta_0$)
  - SRS, TSS1, and TSS3: $\hat{\mu} = \sum_{j=1}^{k} \frac{\bar{y}_j}{k}$
  - TSS2: $\hat{\mu} = \frac{\sum_{j=1}^{k} V(\bar{y}_j)^{-1} \bar{y}_j}{\sum_{j=1}^{k} V \bar{y}_j)^{-1}}$, $V(\bar{y}_j)$ variance of cluster j mean
  - For a given total sample size:

$$V_{SRS}(\hat{\mu}) \leq V_{TSS1}(\hat{\mu}) = V_{TSS3}(\hat{\mu}) \leq V_{TSS2}(\hat{\mu})$$

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Optimal design (1/3)

- To maximize power and precision, is it better to sample more clusters or more individuals per cluster?

Optimal design (OD) = # of clusters (k) and # of individuals per cluster (n) that minimize $V(\hat{\mu})$ subject to $C = k\left(c_2 + c_1 n\right)$

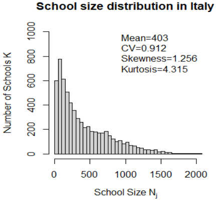
C = budget for sampling and measuring

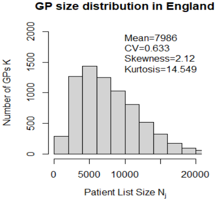
c_2 = (average) cost for sampling a cluster

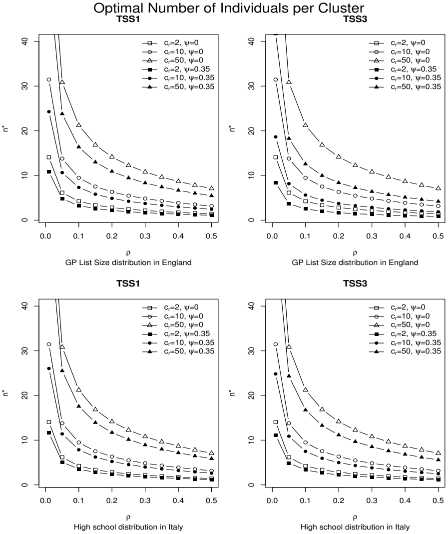
c_1 = (average) cost for sampling an individual from a sampled cluster

- OD maximizes power and precision for a fixed budget C, or minimizes the budget C for the required power or precision level

- Optimal # of clusters: $k^* = \dfrac{C}{c_1(c_r+n^*)}$
- $c_r = \dfrac{c_2}{c_1}$ cluster-to-individual cost ratio
- $\rho = \dfrac{\sigma_v^2}{\sigma_v^2+\sigma_\epsilon^2}$ intraclass correlation coefficient (ICC)
- $\psi = \dfrac{corr(u,N)^2}{1-corr(u,N)^2}$ cluster size informativeness





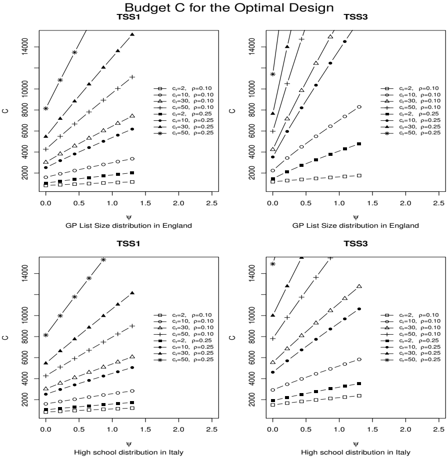


Optimal design (3/3)

- **Given the research budget** C, the OD is robust against misspecification of cluster size informativeness ψ

$$\frac{V(\hat{\mu})\text{ under OD for }\psi > 0}{V(\hat{\mu})\text{ under OD for }\psi = 0} \approx 1$$

- **Given the desired power level and effect size**, the required budget C for the OD is sensitive to misspecification of ψ
- Required C for TSS1 < Required C for TSS2 < Required C for TSS3



- c_r = cluster-to-individual cost ratio
- ψ = cluster size informativeness
- ρ = ICC

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Maximin design (1/2)

- **Local optimality problem:** OD depends on prior knowledge of ICC (ρ), cluster size informativeness (ψ), coefficient of variation (τ_N), skewness (ζ_N), and kurtosis (η_N) of the cluster size distribution

- A solution ⇒ **Maximin approach** (Van Breukelen and Candel [2018]):
  1. Define parameter space (e.g. $\rho \in [0, 0.10]$)
  2. Define design space (i.e. set of all candidate desgins (n, k))
  3. For each design (n, k), find those values of ρ, ψ, τ_N, ζ_N, and η_N that **minimize efficiency** $V(\hat{\mu})^{-1}$
  4. Choose n and k that **maximize** the **minimum efficiency** $V(\hat{\mu})^{-1}$ as determined in step 3

Maximin design (2/2)

- Maximin design = OD for the **worst-case scenario** of the unknown ICC (ρ), cluster size informativeness (ψ), coefficient of variation (τ_N), skewness (ζ_N), and kurtosis (η_N) of the cluster size distribution
  - Maximin TSS1 = Optimal TSS1 for the largest plausible values of ρ and ψ
  - Maximin TSS2 and TSS3 = Optimal TSS2/TSS3 for the largest plausible values of ρ, ψ, η_N, ζ_N, and τ_N if largest $\tau_N \leq 1$
    - ✓ If largest $\tau_N > 1$, the worst-case values for τ_N and ζ_N are obtained via a numerical evaluation (R function)
    - ✓ It depends on some approximations used to derive $V(\hat{\mu})$, which are accurate (bias $\leq 5\%$) only if $k \geq 20$ clusters are sampled ($k \geq 100$ if η_N and ζ_N are extreme) ⇒ sample 10% more clusters
- Advantages:
  - Simple to implement
  - By maximizing the minimum efficiency over the parameter space, it is **robust against misspecification of the unknown parameters**

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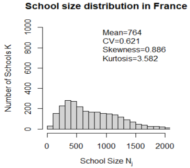
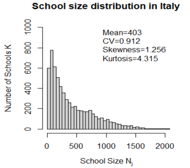
Application (1/3)

- Sample size calculation for cross-population comparions with TSS1:
  1. Optimal sample sizes
  2. **Optimal budget split** between populations

- Example: Comparison of the average alcohol consumption among adolescents in France and Italy
  $\Rightarrow H_0 : \mu_F = \mu_I$ versus $H_1 : \mu_F \neq \mu_I$





- Inputs for **R code**:
  - Coefficient of variation and skewness of cluster size distribution per country
  - Sampling costs per country
  - Largest realistic ICC: $\rho = 0.10$
  - Largest realistic cluster size informativeness: $\psi = 0.35$
  - Range for the ratio of the outcome SDs: $\frac{\sigma_{y,F}}{\sigma_{y,I}} \in \left[\frac{1}{3}, 3\right]$
  - Effect size $d = 0.5$, power $= 90\%$, and $\alpha = 0.05$

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Budget to detect $d = 0.5$, power level 90%, Type I error rate $\alpha = 0.05$

c_2 = cost for sampling a cluster

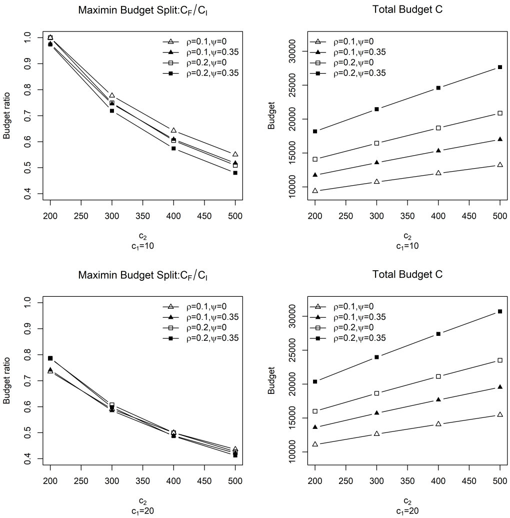
c_1 = cost for sampling an individual

ρ = ICC

ψ = cluster size informativeness

 : $c_1 = 10$ and $c_2 = 200$

 : $c_1 \in \{10, 20\}$ and $c_2 \in [200, 500]$



Application (3/3)

c_2 = cost for sampling a cluster

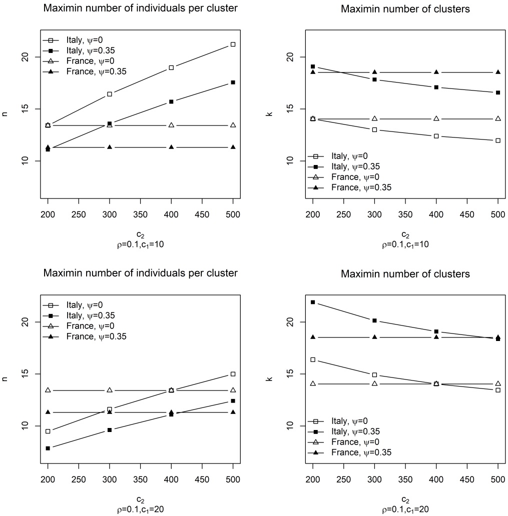
c_1 = cost for sampling an individual

ρ = ICC

ψ = cluster size informativeness

 $= \triangle$: $c_1 = 10$ and $c_2 = 200$

 $= \square$: $c_1 \in \{10, 20\}$ and $c_2 \in [200, 500]$



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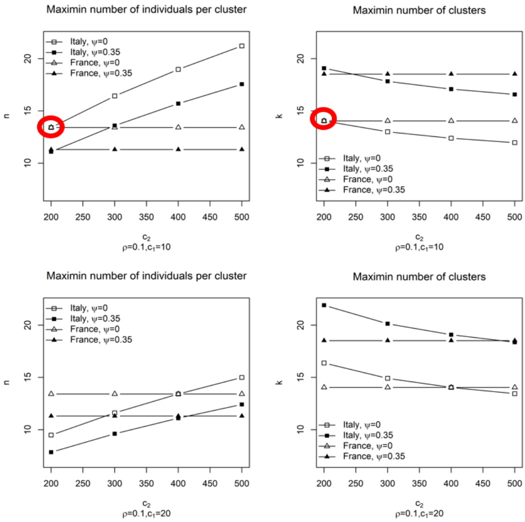

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Application (3/3)

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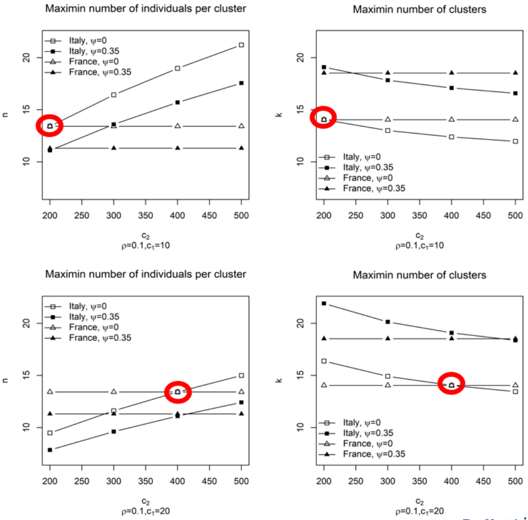
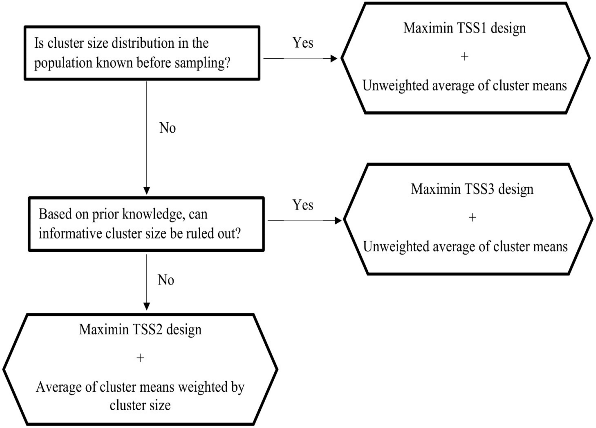


Guidelines



Parameter	**Range of plausible values**
ICC (ρ)	$[0, 0.10]$ in health and medical research (Adams et al. [2004]; Eldridge et al. [2004]) $[0, 0.25]$ in educational research (Hedges and Hedberg [2007]; Shackleton et al. [2016])
Informativeness parameter (ψ)	$[0, 0.35]$ wich corresponds to a correlation of $[-0.51, +0.51]$
CV of cluster size $\left(\tau_N\right)$	$[0, 1]$
Skewness of cluster size $\left(\zeta_N\right)$	$[0.5, 2]$
Kurtosis of cluster size $\left(\eta_N\right)$	$[3, 15]$

Future research

- Binary outcome variables
- Three-level populations
- Extension to non-linear effect of cluster size
- Multipurpose surveys

Thank you for your attention!

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Appendices

1. Sampling variances
   - Equations
   - Simulation study
2. Optimal design
   - Equations
   - Robustness against misspecification of ψ
3. Relative efficiency for a fixed total sample size
   - Equations
   - Figures
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   - Results
   - Equations
   - Figures
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6. Examples of real cluster size distributions
7. Model-based versus design-based inference

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Sampling variances

- SRS:

$$V(\hat{\mu}) = \frac{\sigma_y^2}{m}\left\{1 + \rho\psi\left[\tau_N\left(\zeta_N - \tau_N\right) + 1\right]\right\}$$

- TSS1:

$$V(\hat{\mu}) = \frac{\sigma_y^2}{nk}\left\{1 + \rho\left[(n-1) + n\psi\left(\tau_N\left(\zeta_N - \tau_N\right) + 1\right)\right]\right\}$$

- TSS2:

$$V(\hat{\mu}) \approx \frac{\sigma_y^2}{nk}\left\{1 + \rho\left[n\left(\left(\tau_N^2 + 1\right) + \psi\left(\tau_N^4 + \tau_N^2\left(\eta_N - 3\right) + 2\zeta_N\tau_N\left(1 - \tau_N^2\right) + 1\right)\right) - 1\right]\right\}$$

- TSS3:

$$V(\hat{\mu}) \approx \frac{\sigma_y^2}{nk}\left\{\tau_N^2 + 1 + \rho\left[\left(\tau_N^2 + 1\right)(n-1) + n\psi\left(\tau_N^4 + \tau_N^2\left(\eta_N - 3\right) + 2\zeta_N\tau_N\left(1 - \tau_N^2\right) + 1\right)\right]\right\}$$

- $V(\hat{\mu})$ for TSS2 and TSS3 are derived using the **delta method** (see subsection 2) and are based on a large k approximation (i.e. k such that $\frac{\tau_N^2}{k} \approx 0$, $\frac{k-1}{k} \approx 1$, and $\frac{k-3}{k-1} \approx 1$)

Simulation study

$V(\hat{\mu})$ for TSS2 and TSS3 are based on the delta method, so their accuracy were evaluated through a simulation study:

- Sampling $k = 20$ clusters guarantees nearly unbiased estimates of μ under TSS2 and TSS3
- Sampling $k = 20$ clusters guarantees fair accuracy (i.e. relative bias $\leq 5\%$) of $V(\hat{\mu})$ for TSS2 and TSS3 when $|corr(u, N)| \leq 0.75$, $\rho \leq 0.3$, and ζ_N and η_N are relatively close (say, ± 1.5) to those of the Normal distribution (i.e. $\zeta_N = 0$ and $\eta_N = 3$)
- For cluster size distributions with extreme skewness and kurtosis (e.g. $\zeta_N \geq 2$ and $\eta_N \geq 9$) at least $k = 100$ clusters must be sampled to achieve a reasonable accuracy (i.e. bias $\leq 6\%$) of $V(\hat{\mu})$, for $|corr(u, N)| \leq 0.5$ and $\rho \leq 0.3$
- These lower-bounds for k (i.e. 20 and 100) guarantee the corresponding accuracy level across different values for n (at least for $2 \leq n \leq 100$) (Shackleton et al. [2016]: in the ESPAD study $k \in [36, 531]$, median=123, and $\bar{n} \in [5.92, 119.62]$, median=20.74)

Optimal designs (1/2)

- SRS:

$$V\left(\hat{\mu}\right)^{*} = \frac{c_{srs}\sigma_y^2\left(1+\rho\psi\left[\tau_N\left(\zeta_N-\tau_N\right)+1\right]\right)}{C-c_0}$$

where c_{srs} is the average cost for sampling an individual directly from the population, and c_0 represents the extra-cost due to constructing the sampling frame for a SRS compared with the sampling frame for a TSS.

- TSS1: $n^{*} = \sqrt{c_r\left(\frac{1-\rho}{\rho}\right)\left(\frac{1}{1+\psi\left[\tau_N\left(\zeta_N-\tau_N\right)+1\right]}\right)}$

$$V\left(\hat{\mu}\right)^{*} = \frac{c_1\sigma_y^2\left(\sqrt{c_r\rho\left(1+\psi\left[\tau_N\left(\zeta_N-\tau_N\right)+1\right]\right)}+\sqrt{1-\rho}\right)^2}{C}$$

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Optimal designs (2/2)

- TSS2: $n^* = p^*\theta_N = \sqrt{c_r\left(\frac{1-\rho}{\rho}\right)\frac{1}{(\tau_N^2+1)+\psi\left[\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1\right]}}$

$$V(\hat{\mu})^* = \frac{c_1\sigma_y^2\left(\sqrt{c_r\rho\left[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)\right]}+\sqrt{1-\rho}\right)^2}{C}$$

- TSS3: $n^* = \sqrt{c_r\left(\frac{1-\rho}{\rho}\right)\frac{(\tau_N^2+1)}{(\tau_N^2+1)+\psi\left[\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1\right]}}$

$$V(\hat{\mu})^* = \frac{c_1\sigma_y^2\left(\sqrt{c_r\rho\left[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)\right]}+\sqrt{(1-\rho)(\tau_N^2+1)}\right)^2}{C}$$

- optimal number of clusters for any TSS: $k^* = \frac{C}{c_1(c_r+n^*)}$

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Robustness of the optimal design against misspecification of ψ

Assuming the general practice list size distribution in England, $\rho = 0.05$, $c_r = 10$, and $C/c_1 = 1000$

	TSS1		TSS2		TSS3	
	$\psi = 0$	$\psi = 1/3$	$\psi = 0$	$\psi = 1/3$	$\psi = 0$	$\psi = 1/3$
n^*	13.78	10.74	11.65	7.01	13.78	8.30
k^*	42.04	48.22	46.2	58.79	42.04	54.65
$Var(\hat{\mu})/\sigma_y^2$ if $\psi = 1/3$	0.00360	0.00354	0.00595	0.00559	0.00690	0.00647
$\frac{Var(\hat{\mu}\vert\psi = 1/3)}{Var(\hat{\mu}\vert\psi = 0)}$	0.983		0.939		0.938	
	$\psi = 0$	$\psi = 1$	$\psi = 0$	$\psi = 1$	$\psi = 0$	$\psi = 1$
n^*	13.78	8.04	11.65	4.65	13.78	5.50
k^*	42.04	55.44	46.2	68.27	42.04	64.52
$Var(\hat{\mu})/\sigma_y^2$ if $\psi = 1$	0.00514	0.00478	0.01129	0.00944	0.01276	0.01057
$\frac{Var(\hat{\mu}\vert\psi = 1)}{Var(\hat{\mu}\vert\psi = 0)}$	0.930		0.836		0.828	

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Relative efficiency for a fixed total sample size (1/4)

- $RE\,(TSS1\ vs\ SRS) = \dfrac{\left(1-corr(u_j,N_j)^2\right)+corr(u_j,N_j)^2\rho[\tau_N(\zeta_N-\tau_N)+1]}{\left(1-corr(u_j,N_j)^2\right)[1+(n-1)\rho]+corr(u_j,N_j)^2 n\rho[\tau_N(\zeta_N-\tau_N)+1]}$
- $RE\,(TSS2\ vs\ SRS) =$

$$\frac{\left(1-corr(u_j,N_j)^2\right)+corr(u_j,N_j)^2\rho[\tau_N(\zeta_N-\tau_N)+1]}{\left(1-corr(u_j,N_j)^2\right)\left[1+\left(\bar{n}\left(\frac{k\left(\tau_N^2+1\right)}{\tau_N^2+k}\right)-1\right)\rho\right]+corr(u_j,N_j)^2\bar{n}\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}$$

- $RE\,(TSS3\ vs\ SRS) =$

$$\frac{\left(1-corr(u_j,N_j)^2\right)+corr(u_j,N_j)^2\rho[\tau_N(\zeta_N-\tau_N)+1]}{\left(1-corr(u_j,N_j)^2\right)\left[\left(\frac{k\left(\tau_N^2+1\right)}{\tau_N^2+k}\right)(1+(n-1)\rho)\right]+corr(u_j,N_j)^2 n\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}$$

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Relative efficiency for a fixed total sample size (2/4)

- $RE\,(TSS2\ vs\ TSS1) =$

$$\frac{\left(1-corr(u_j,N_j)^2\right)[1+(n-1)\rho]+corr(u_j,N_j)^2 n\rho[(\tau_N(\zeta_N-\tau_N)+1)]}{\left(1-corr(u_j,N_j)^2\right)\left[1+\left(\bar{n}\left(\frac{k(\tau_N^2+1)}{\tau_N^2+k}\right)-1\right)\rho\right]+corr(u_j,N_j)^2\bar{n}\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}$$

- $RE\,(TSS3\ vs\ TSS1) =$

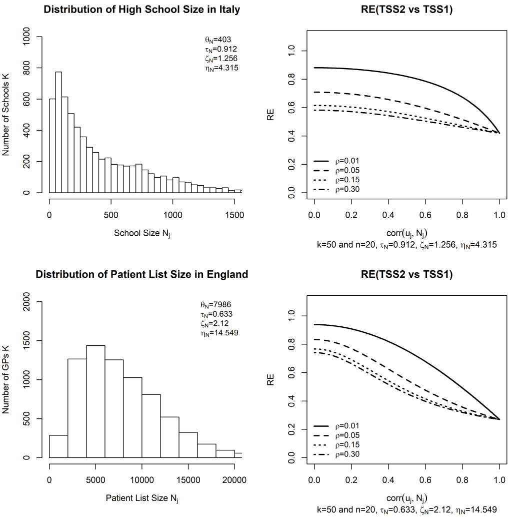
$$\frac{\left(1-corr(u_j,N_j)^2\right)[1+(n-1)\rho]+corr(u_j,N_j)^2 n\rho[\tau_N(\zeta_N-\tau_N)+1]}{\left(1-corr(u_j,N_j)^2\right)\left[\left(\frac{k(\tau_N^2+1)}{\tau_N^2+k}\right)(1+(n-1)\rho)\right]+corr(u_j,N_j)^2 n\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}$$

- $RE\,(TSS3\ vs\ TSS2) =$

$$\frac{\left(1-corr(u_j,N_j)^2\right)\left[1+\left(\bar{n}\left(\frac{k(\tau_N^2+1)}{\tau_N^2+k}\right)-1\right)\rho\right]+corr(u_j,N_j)^2\bar{n}\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}{\left(1-corr(u_j,N_j)^2\right)\left[\left(\frac{k(\tau_N^2+1)}{\tau_N^2+k}\right)(1+(n-1)\rho)\right]+corr(u_j,N_j)^2 n\rho\left[\left(\frac{k-1}{k}\right)^2\tau_N^2\left(\eta_N-\frac{k-3}{k-1}+\tau_N(\tau_N-2\zeta_N)\right)+2\left(\frac{k-1}{k}\right)\tau_N(\zeta_N-\tau_N)+1\right]}$$

- TSS1 is more efficient than TSS2 and TSS3 if one of the following conditions is met: the cluster size distribution is positively skewed with $\tau_N \in [0, \zeta_N]$, or is symmetric with $\tau_N \in [0, 1]$ and $k \in \left[1,\ \frac{(2-\tau_N^2)+\sqrt{2-\tau_N^2}}{(1-\tau_N^2)}\right]$, or is Normal.

Relative efficiency for a fixed total sample size (3/4)



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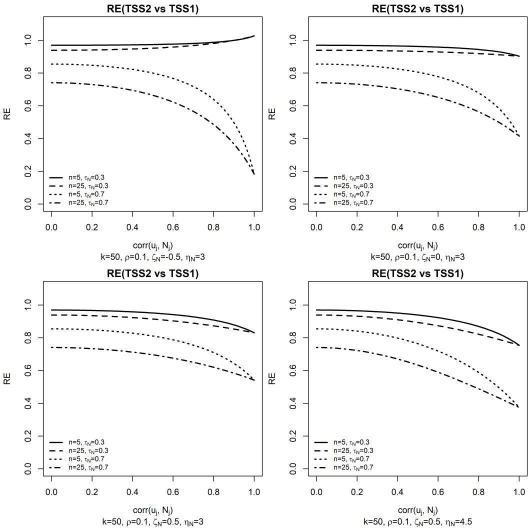
Relative efficiency for a fixed total sample size (4/4)



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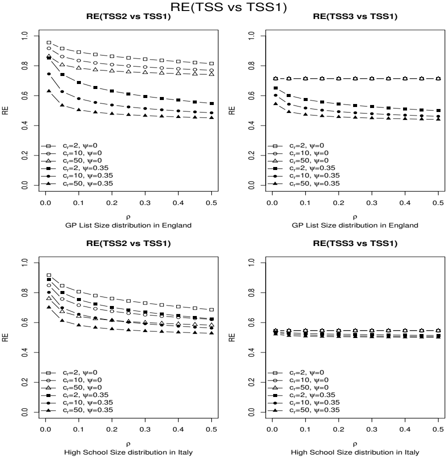
Relative efficiency for a given budget

- Relative efficiency of OD_1 versus OD_2: $RE = \frac{V_{OD_2}(\hat{\mu})}{V_{OD_1}(\hat{\mu})}$
- Informative cluster size
  - RE depends on cluster size distribution
  - TSS1 is the most efficient TSS for many cluster size distributions
  - TSS3 is always the least efficient TSS
- Non-informative cluster size
  - TSS1 and TSS3 are equally efficient and outperform TSS2



- c_r = cluster-to-individual cost ratio
- ψ = cluster size informativeness
- ρ = ICC

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Relative efficiency for a fixed budget (1/6)

- TSS1 vs SRS:

$$\frac{1+\rho\psi[\tau_N(\zeta_N-\tau_N)+1]}{\left(\sqrt{c_r\rho(1+\psi[\tau_N(\zeta_N-\tau_N)+1])}+\sqrt{1-\rho}\right)^2}\times\left(\frac{c_{srs}}{c_1}\right)\times\left(\frac{C}{C-c_0}\right)$$

which is ≤ 1 if $\zeta_N \geq \tau_N - \frac{1}{\tau_N} - \frac{1}{\tau_N\psi}$ and $\left(\frac{c_{srs}}{c_1}\right) = \left(\frac{C}{C-c_0}\right) = 1$

- TSS2 vs SRS:

$$\frac{1+\rho\psi[\tau_N(\zeta_N-\tau_N)+1]}{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{1-\rho}\right)^2}\times\left(\frac{c_{srs}}{c_1}\right)\times\left(\frac{C}{C-c_0}\right)$$

which is ≤ 1 if $\zeta_N \leq \tau_N - \frac{1}{\tau_N}$ or $\zeta_N \geq \tau_N + \frac{1}{\tau_N c_r} - \frac{1}{\tau_N}$ or $N_j \sim N\left(\theta_N, \sigma_N^2\right)$, and $\left(\frac{c_{srs}}{c_1}\right) = \left(\frac{C}{C-c_0}\right) = 1$

- TSS3 vs SRS:

$$\frac{1+\rho\psi[\tau_N(\zeta_N-\tau_N)+1]}{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{(1-\rho)(\tau_N^2+1)}\right)^2}\times\left(\frac{c_{srs}}{c_1}\right)\times\left(\frac{C}{C-c_0}\right)$$

which is ≤ 1 if $\zeta_N \leq \tau_N - \frac{1}{\tau_N}$ or $\zeta_N \geq \tau_N + \frac{1}{\tau_N c_r} - \frac{1}{\tau_N}$ or $N_j \sim N\left(\theta_N, \sigma_N^2\right)$, and $\left(\frac{c_{srs}}{c_1}\right) = \left(\frac{C}{C-c_0}\right) = 1$

Relative efficiency for a fixed budget (2/6)

- TSS2 vs TSS1:

$$\frac{\left(\sqrt{c_r\rho[1+\psi(\tau_N(\zeta_N-\tau_N)+1)]}+\sqrt{1-\rho}\right)^2}{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{1-\rho}\right)^2}$$

which is ≤ 1 if $\tau_N - \frac{1}{\tau_N} - \frac{1}{\tau_N\psi} \leq \zeta_N \leq \tau_N - \frac{1}{\tau_N}$ or $\zeta_N \geq \tau_N$ or $N_j \sim N\left(\theta_N, \sigma_N^2\right)$

- TSS3 vs TSS1:

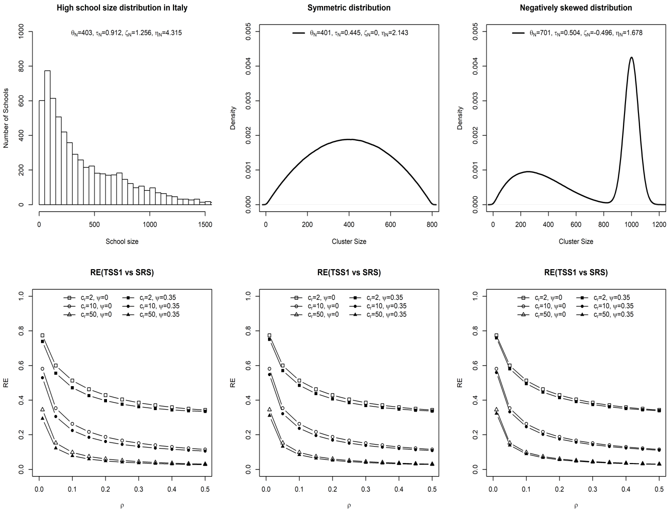
$$\frac{\left(\sqrt{c_r\rho[1+\psi(\tau_N(\zeta_N-\tau_N)+1)]}+\sqrt{1-\rho}\right)^2}{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{(1-\rho)(\tau_N^2+1)}\right)^2}$$

which is ≤ 1 if $\tau_N - \frac{1}{\tau_N} - \frac{1}{\tau_N\psi} \leq \zeta_N \leq \tau_N - \frac{1}{\tau_N}$ or $\zeta_N \geq \tau_N$ or $N_j \sim N\left(\theta_N, \sigma_N^2\right)$

- TSS3 vs TSS2:

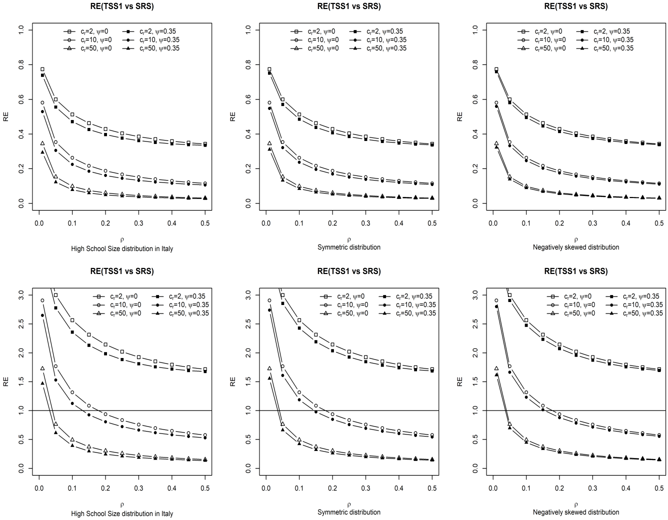
$$\frac{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{1-\rho}\right)^2}{\left(\sqrt{c_r\rho[\tau_N^2+1+\psi(\tau_N^4+\tau_N^2(\eta_N-3)+2\zeta_N\tau_N(1-\tau_N^2)+1)]}+\sqrt{(1-\rho)(\tau_N^2+1)}\right)^2} \leq 1$$

Relative efficiency for a fixed budget (3/6)



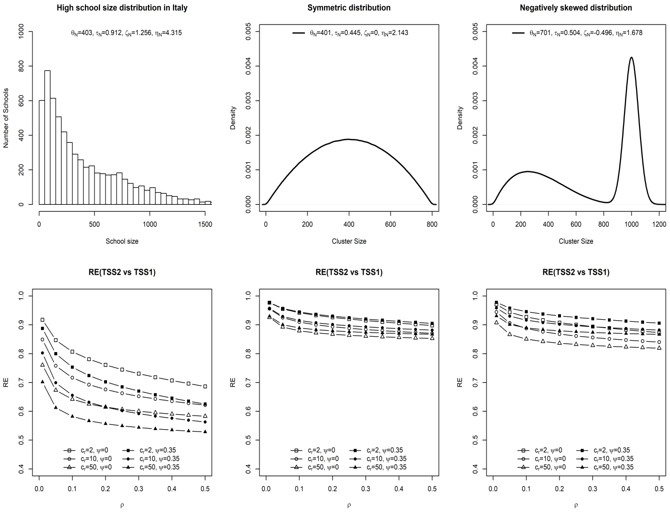
No extra costs for SRS

Relative efficiency for a fixed budget (4/6)



Extra costs for SRS: $c_{SRS} = 4c_1$ and $c_0 = 20\%$

Relative efficiency for a fixed budget (5/6)



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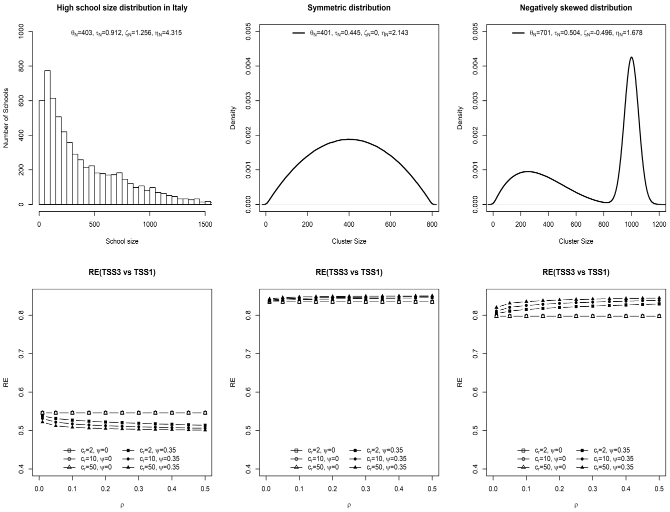
Relative efficiency for a fixed budget (6/6)



Sample size calculation for cross-population comparisons (1/3)

Example: $H_0 : \mu_F = \mu_I$ versus $H_1 : \mu_F \neq \mu_I$ where e.g.  and 

1. Specify sampling costs $\left(c_1, c_2\right)$ per population, largest realistic ρ and ψ values, smallest relevant standardized difference d, $\frac{\sigma_{y,F}}{\sigma_{y,I}} \in \left[\frac{1}{q}, q\right]$, power level and Type I error rate
2. Compute the maximum allowable $V\left(\hat{\mu}_F - \hat{\mu}_I\right)$ to guarantee the desired power
3. Compute the maximin n_F^{MD} and n_I^{MD}
4. Compute the maximin budget split $\frac{C_F}{C_I}$
5. Compute the total budget C by equating the maximum variance for the maximin design with $V\left(\hat{\mu}_F - \hat{\mu}_I\right)$ as computed in step 2
6. Compute the separate budget per population using C and $\frac{C_F}{C_I}$
7. Compute the maximin k_F^{MD} and k_I^{MD}

Sample size calculation for cross-population comparisons (2/3)

1. Specify $c_{1,F}$, $c_{1,I}$, $c_{2,F}$, $c_{2,I}$, $\rho\,(max)$, $\psi\,(max)$, $\min\left\{\mu_F - \mu_I\right\}$, $V_{max} \geq \sigma^2_{y,F} + \sigma^2_{y,I}$, $\frac{\sigma_{y,F}}{\sigma_{y,I}} \in \left[\frac{1}{q}, q\right]$, and $d = \sqrt{\frac{\mu_F - \mu_I}{V_{max}/2}}$
2. Compute $V\left(\hat{\mu}_F - \hat{\mu}_I\right) = \left(\frac{\mu_F - \mu_I}{z_{1-\frac{\alpha}{2}} + z_{1-\gamma}}\right)^2$
3. Compute the maximin n_F^{MD} and n_I^{MD} (see subsection 1)
4. Compute the maximin budget split $\frac{C_F}{C_I}$ using h and $\left[\frac{1}{q}, q\right]$ (see next slide)
5. Compute the total budget C by equating the maximum variance for the maximin design with $V\left(\hat{\mu}_F - \hat{\mu}_I\right)$ as computed in step 2
6. Using C from step 6 and $\frac{C_F}{C_I}$ from step 5, compute C_F and C_I
7. Compute the maximin k_F^{MD} and k_I^{MD} (see subsection 1)

Sample size calculation for cross-population comparisons (3/3)

$$h = \sqrt{\frac{g_F\left(\rho\left(max\right),\ \psi\left(max\right)\right)}{g_I\left(\rho\left(max\right),\ \psi\left(max\right)\right)}}$$

$$= \sqrt{\frac{c_{1,F}\left(\sqrt{c_{r,F}\rho_F\left(1+\psi_F\left[\tau_{N,F}\left(\zeta_{N,F}-\tau_{N,F}\right)+1\right]\right)}+\sqrt{1-\rho_F}\right)^2}{c_{1,I}\left(\sqrt{c_{r,I}\rho_I\left(1+\psi_I\left[\tau_{N,I}\left(\zeta_{N,I}-\tau_{N,I}\right)+1\right]\right)}+\sqrt{1-\rho_I}\right)^2}}$$

Relation of h to q	Maximin budget split	Maximum variance for MD
$\frac{1}{q} \leq h \leq q$	h^2	$\frac{g_I(\rho(max),\,\psi(max))V_{max}}{C} \times \left(1+h^2\right)$
$h > q$	hq	$\frac{g_I(\rho(max),\,\psi(max))V_{max}}{C} \times \frac{(hq+1)^2}{(q^2+1)}$
$h < \frac{1}{q}$	$\frac{h}{q}$	$\frac{g_I(\rho(max),\,\psi(max))V_{max}}{C} \times \frac{(h+q)^2}{(q^2+1)}$

Real cluster size distributions

Cluster Size distribution	θ_N	τ_N	ζ_N	η_N
GP List size distribution in England (Salt [2017])	7,986	0.633	2.12	14.549
High School size distribution in Italy (DGCASIS [2018])	403	0.912	1.256	4.315
High School size distribution in France (MENJVA [2015])	764	0.621	0.886	3.582
Lower Secondary School size distribution in Italy (DGCASIS [2018])	225	0.789	1.351	5.303
Lower Secondary School size distribution in France (MENJVA [2015])	493	0.387	0.63	5.47
Primary School size distribution in Italy (DGCASIS [2018])	171	0.761	1.451	5.740
Primary School size distribution in France (MENJVA [2015])	135	0.71	1.045	4.084

Model-based versus design-based inference (1/2)

Model-based approach:

- Y_{ij} is random
- Inference based on the stochastic model for Y_{ij}
- Advantage: It simplifies sample size planning and sampling schemes comparisons

Design-based approach:

- Y_{ij} is fixed but unknown. The inclusion indicator I_{ij} is random (i.e. $I_{ij} = 1$ with π_{ij}, and $I_{ij} = 0$ otherwise)
- Inference based on the distribution of I_{ij} over repeated sampling with a given sampling design
- Advantage: Robustness

In the considered setting, the two approaches yield almost the same results (if the model assumptions are met) (Innocenti et al. [2019]):

- same estimators of the population mean
- approximately the same relative efficiencies (i.e. for k sufficiently large)

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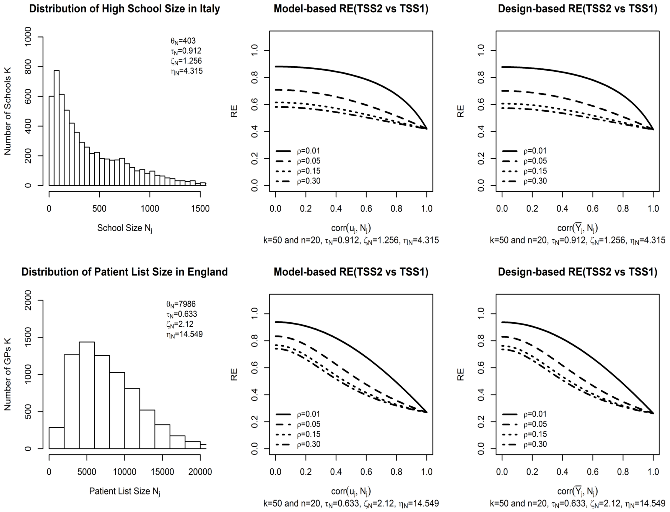
Model-based versus design-based inference (2/2)



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