Assessing the Statistical Power/Precision Of Multisite Trials for Estimating Parameters Of Cross-site ITT Impact Distributions

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Overview

Questions

- How does the number and size of sites influence the precision of a multi-site trial?
- What other factors influence this precision?
- How should this information be used to design trials?

Designs

- Multi-site individually-randomized trials
- Multi-site cluster-randomized trials

Parameters

- The cross-site mean effect size
- The cross-site effect size standard deviation
- The difference in mean effect sizes for two categories of sites (i.e. the coefficient for a binary site-level moderator)

Reference

- Bloom, H.S. and J. Spybrook (under review) "Assessing the Precision of Multisite Trials for Estimating Parameters of Cross-site Distributions of ITT Program Effects."

Effect Sizes and Precision

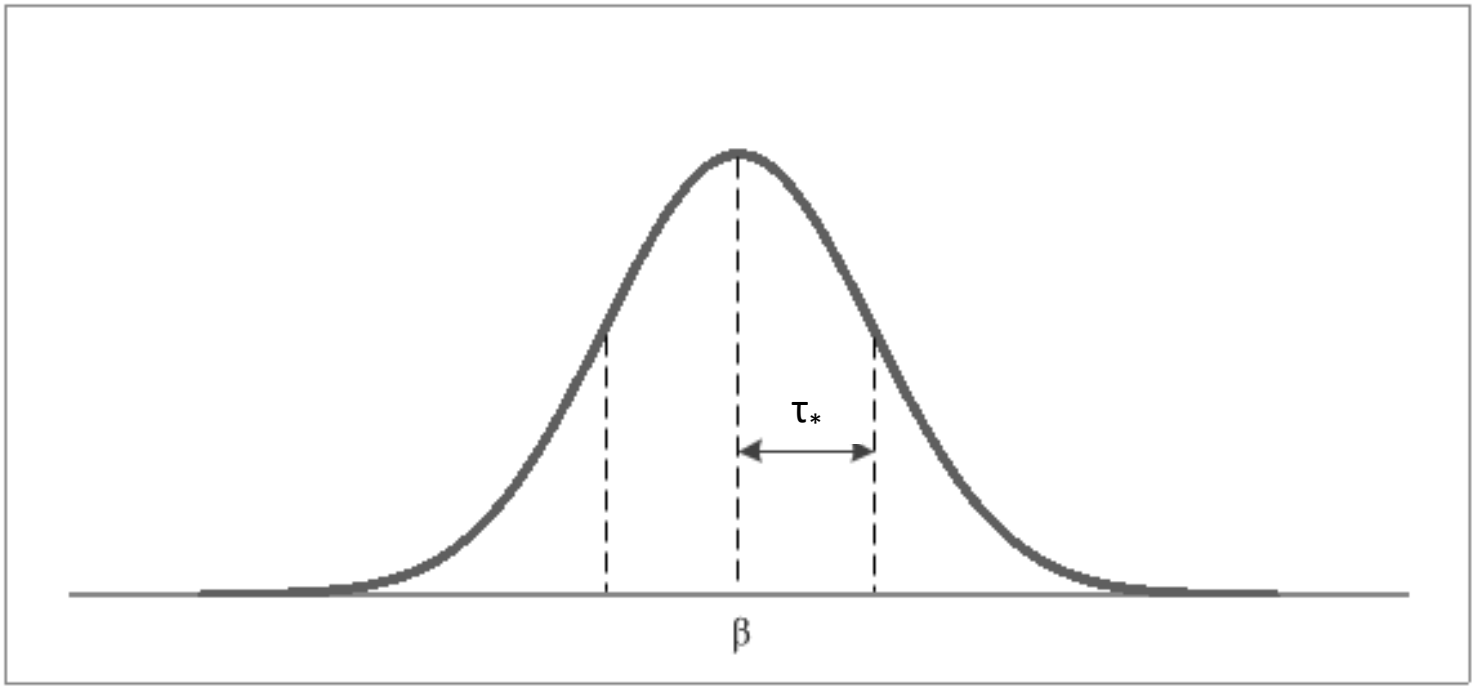
A Standardized Mean Difference Effect Size

$$Effect\ Size = \frac{Program\ Effect\ in\ Original\ Units}{Standard\ Deviation\ of\ Untreated\ Outcomes}$$

A Minimal Detectable Effect Size

The smallest program effect size that can be detected with 80 power at the 0.05 level of statistical significance.

A Cross-Site Distribution of Program Effect Sizes



β = The cross-site mean effect size
(*Associated design parameter*: Minimum detectable cross-site effect size, MDES)

τ_* = The cross-site standard deviation of effect sizes
(*Associated design parameter*: Minimum detectable effect-size standard deviation, MDESSD)

Prototypical Estimation Model for MDES and MDESSD: FIRC

Fixed Site-Specific Intercepts, Random Site-Specific Program Effects, a Single Level-One Covariate and a Single Level-One Residual Variance (for simplicity)

Level One: Individuals

$$Y_{ij} = \alpha_j + \beta_j \cdot T_{ij} + \theta \cdot X_{ij} + r_{ij}$$

$$r_{ij} \sim N(0, \sigma^2_{|x\alpha_j})$$

$$\sigma^2_{|x\alpha_j} = (1 - \rho_C)(1 - R^2_{C(within)})\sigma^2$$

Level Two: Sites

$$\alpha_j = \alpha_j$$

$$\beta_j = \beta + b_j$$

$$b_j \sim N(0, \tau^2_*)$$

MDES Computational Expression
(Individual Randomization)

$$MDES = M_{J-1}\sqrt{(\frac{1}{J})(\tau_*^2 + \frac{(1-\rho_C)(1-R^2_{C(within)})}{n\bar{T}(1-\bar{T})})}$$

where:

σ^2 is set equal to one.

M_{j-1} = a multiplier that rapidly approaches 2.8 as J increases (for a two-tail test at the 0.05 significance level with 80 percent power)

J = number of sites

n = number of individuals per site

$\bar{T}$ = proportion of individuals from each site randomized to treatment

τ_*= cross-site standard deviation of effect sizes

ρ_C = intra-class correlation for control group outcomes (i.e. the proportion of σ^2explained by site indicators)

$R^2_{C(within)}$ = the proportion of within-site outcome *variance* explained by our baseline covariate

Harmonic vs. Arithmetic Mean Site Sizes
(It Matters)

- You should always use the harmonic mean site sample size when assessing statistical precision.
- When site sample sizes vary, the harmonic mean will be smaller than the arithmetic mean – often by a lot.
- For example:
  - Welfare to work (site sample sizes range from 177 to 4,418)
    - Harmonic mean = 621
    - Arithmetic mean = 1,176
  - Head Start (site sample sizes range from 2 to 75)
    - Harmonic mean = 13
    - Arithmetic mean = 19

MDES for a Cross-Site Mean Effect Size

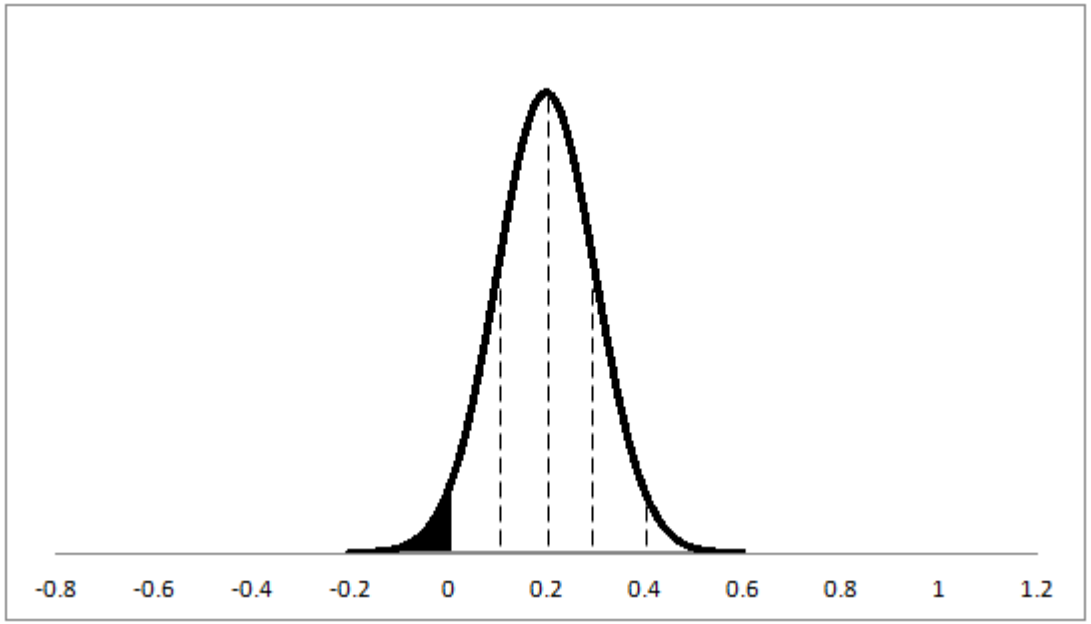
(Individual Randomization)

Individuals per site (n)	Sites					
	5	10	20	50	100	200
5	1.10	0.65	0.43	0.27	0.19	0.13
10	0.80	0.47	0.31	0.19	0.14	0.10
20	0.59	0.35	0.23	0.14	0.10	0.07
50	0.42	0.25	0.17	0.10	0.07	0.05
100	0.35	0.21	0.14	0.08	0.06	0.04
200	0.30	0.18	0.12	0.07	0.05	0.04
500	0.27	0.16	0.11	0.07	0.05	0.03

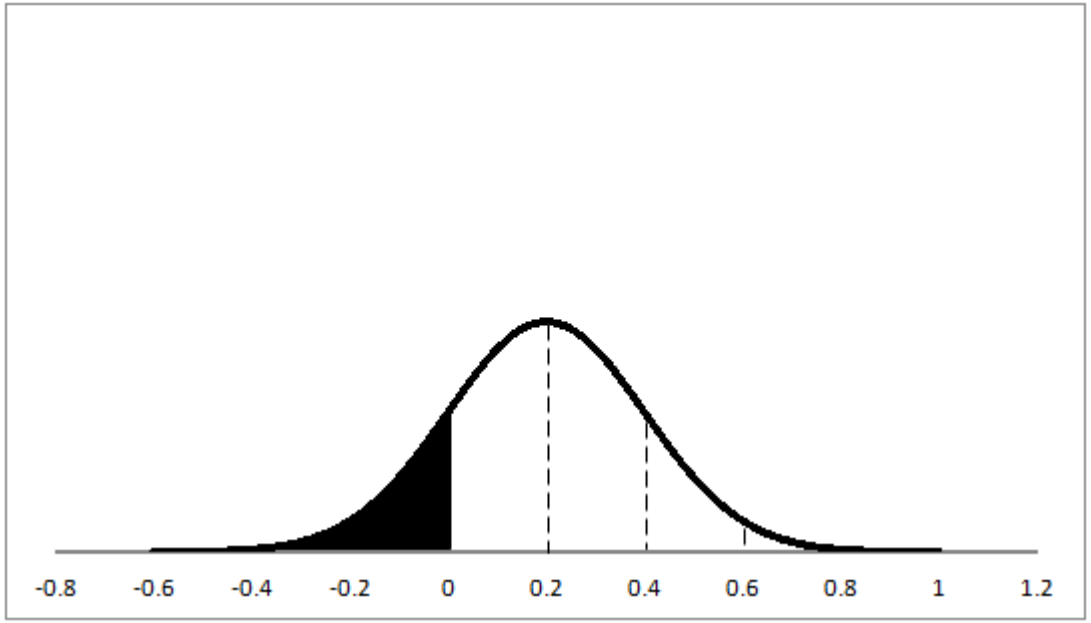
NOTES: Values in the table are for two-tail significance = 0.05, power = 80 percent, a single level-one baseline covariate, $\rho_C = 0.15$, $R^2_{C(within)} = 0.4$, $\bar{T} = 0.5$, constant n within sites, and $\tau_* = 0.15$.

Thinking About the Magnitude of Cross-Site Effect-Size Variation

"Moderate" Variation ($\tau_* = 0.1$)



"Substantial" Variation ($\tau_* = 0.2$)



MDESSD Computational Expression
(Individual Randomization)

$$MDESSD = \sqrt{\left(\frac{(1-\rho_C)\left(1-R^2_{C(within)}\right)}{n\bar{T}(1-\bar{T})}\right)\left(\frac{F_{0.05}}{F_{0.80}} - 1\right)}$$

where:

σ^2 set equal to one

n = number of individuals per site

$\bar{T}$ = proportion of individuals at each site randomized to treatment

ρ_C = intra-class correlation for control group outcomes (i.e. the proportion of σ^2 explained by our site indicators)

$R^2_{C(within)}$ = proportion of within-site outcome variance explained by our baseline covariate

$F_{0.05}$ = 0.05 critical value for an F statistic with J-1 numerator degrees of freedom and J(n – 2) – K denominator degrees of freedom (for J sites, n individuals per site and K level-one covariates)

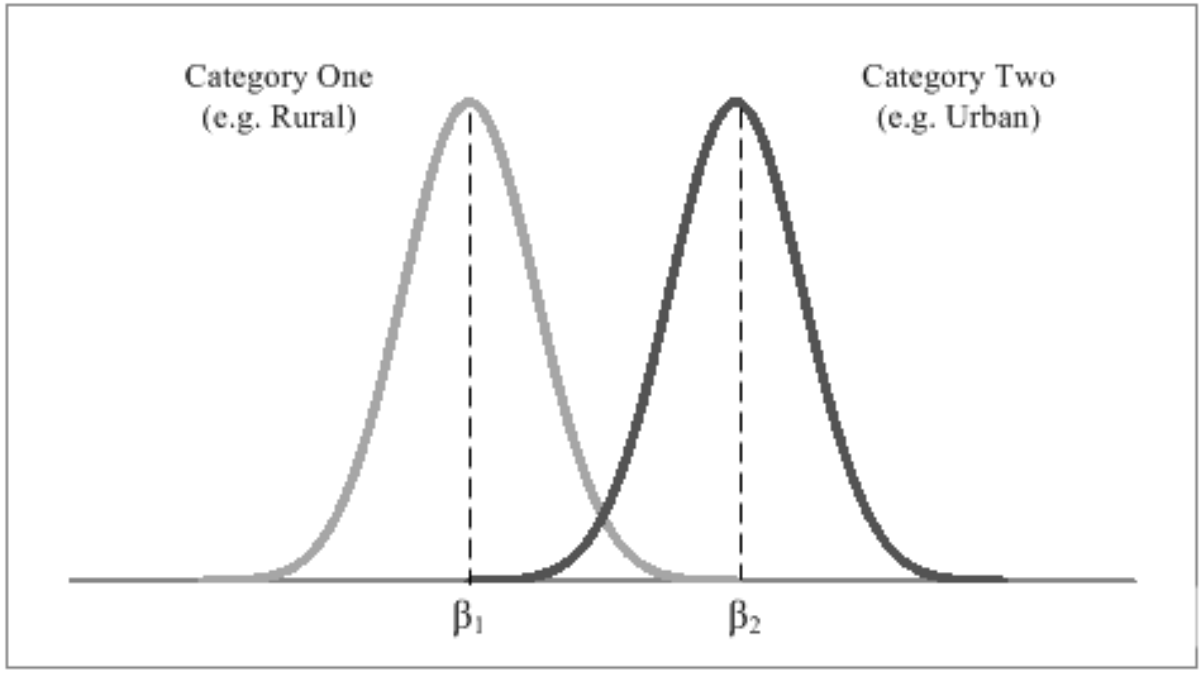
$F_{0.80}$ = the value that is below 80 percent of the distribution of possible values for an F statistic with J-1 numerator degrees of freedom and J(n – 2) – K denominator degrees of freedom

MDESSD for a Cross-Site Standard Deviation of Effect Sizes
(Individual Randomization)

	Number of Sites (J)					
Individuals/site (n)	**5**	**10**	**20**	**50**	**100**	**200**
5	1.65	1.07	0.78	0.57	0.45	0.37
10	1.05	0.70	0.52	0.38	0.30	0.35
20	0.72	0.48	0.36	0.26	0.21	0.17
50	0.45	0.30	0.22	0.16	0.13	0.11
100	0.31	0.21	0.16	0.11	0.09	0.08
200	0.22	0.15	0.11	0.08	0.07	0.05
500	0.14	0.09	0.07	0.05	0.03	0.03

NOTE: Values in the table are for significance = 0.05, power = 80 percent, a single level-one baseline covariate, $\rho_C = 0.15, R^2_{C(within)} = 0.4, \bar{T} = 0.5$ and constant *n* within sites.

Cross-Site Effect-Size Distributions For Two Categories of Sites



β_1 = Grand mean effect size for category #1
β_2 = Grand mean effect size for category #2

$\beta_2 - \beta_1$ = The moderator coefficient
(Associated design parameter: Minimum detectable effect size difference, MDESD)

Estimation Model for MDESD: FIRC with a Binary Site-Level Impact Predictor

Fixed Site-Specific Intercepts, Random Site-Specific Program Effects, a Single Level-One Covariate, A Single Level-One Residual (for simplicity) and a Single Binary Site-Level Impact Predictor

Level One: Individuals

$$Y_{ij} = \alpha_j + \beta_j \cdot T_{ij} + \theta \cdot X_{ij} + r_{ij}$$

$$r_{ij} \sim N(0, \sigma^2_{|x\alpha_j})$$

$$\sigma^2_{|x\alpha_j} = (1 - \rho_C)(1 - R^2_{C(within)})\sigma^2$$

- Level Two: Sites

$$\alpha_j = \alpha_j$$

$$B_j = \beta' + \Delta W_j + b'_j$$

$$b_j \sim N(0, \tau^2_{*|W})$$

$$where\ \tau^2_{*|W} = \left(1 - R^2_{\Delta}\right)\tau^2_*$$

More on MDESD

A Few noteworthy distinctions:

Max Possible Effect Size difference is a function of τ_*^2

$$\Delta_{\max} = \sqrt{\frac{\tau_*^2}{\pi(1-\pi)}}$$

Relationship between R_Δ^2 **and** Δ

$$\Delta = \sqrt{\frac{\tau_*^2 R_\Delta^2}{\pi(1-\pi)}} \quad \text{or} \quad R_\Delta^2 = \frac{\Delta^2 \pi(1-\pi)}{\tau_*^2}$$

where π **is the proportion of sites in a particular category.**

MDESD Power Calculations
(Individual Randomization)

Steps

1. Determine $\Delta_{\max}$, $\Delta_{\max} = \sqrt{\frac{\tau_*^2}{\pi(1-\pi)}}$

Note: For $\Delta_{\max}$, $R_\Delta^2 = 1$

2. Calculate Power for $\Delta_{\max}$ where $\Delta = \Delta_{\max}$, and

$$POWER = 1 - \Phi\left(t_{critical} - \frac{\Delta}{SE\left(\hat{\Delta}\right)} \right)$$

where

$$se\left(\hat{\Delta}_{*|R_W^2}\right) = \sqrt{\left(\frac{1}{\pi(1-\pi)J}\right) + \left((1-R_W^2)\tau_*^2 + \frac{(1-\rho_C)(1-R_{C(within)}^2)}{n\bar{T}(1-\bar{T})}\right)}$$

MDESD Power Calculations
(Individual Randomization)

Steps (Continued)

3. If power less than 0.80, there is no MDESD for that particular sample size combination

4. If power greater than 0.80, select $\Delta < \Delta_{\max}$

5. Calculate $R_{\Delta}^{2} = \dfrac{\Delta^{2}\pi(1-\pi)}{\tau_{*}^{2}}$

6. Calculate Power (see step 2)

7. Repeat steps 4-7 until power = 0.80

Please email jessaca.spybrook@wmich.edu for R code for MDESD power calculations.

MDESD for the Coefficient (Δ) On a Binary Site-Level Moderator
(Individual Randomization)

Individuals per site (n)	Sites 5	10	20	50	100	200
5	--	--	--	--	--	0.26 (0.72)
10	--	--	--	--	0.26 (0.72)	0.19 (0.39)
20	--	--	--	0.27 (0.78)	0.20 (0.43)	0.14 (0.21)
50	--	--	0.28 (0.84)	0.19 (0.39)	0.14 (0.21)	0.10 (0.11)
100	--	0.30 (0.96)	0.23 (0.56)	0.16 (0.27)	0.12 (0.15)	0.08 (0.07)
200	--	0.26 (0.72)	0.20 (0.43)	0.14 (0.21)	0.10 (0.11)	0.07 (0.05)
500	0.30 (0.96)	0.24 (0.61)	0.18 (0.35)	0.13 (0.18)	0.09 (0.09)	0.07 (0.05)

NOTE: Values in the table are for two-tail significance = 0.05, power = 80 percent, $\rho_C = 0.15$, $R^2_{C(within)} = 0.4$, $\bar{T} = 0.5$, $\tau_* = 0.15$ and π = 0.6. Values in the table represent the MDESD. Values in parentheses represent R^2_W.

Appendix

Extensions to Cluster Randomized Multisite Trials

Extension to Multi-site Cluster Randomized Trials

Trial Design:

- Students in schools (clusters), schools in districts (sites)
- Treatment assigned at the school (cluster) level

Additional Challenges:

- Three sample sizes
  - Number of individuals per cluster
  - Number of clusters per site
  - Number of sites

Parameters Examined:

- MDES, MDESSD, MDESD

MDES for a Cross-Site Mean Effect Size

(Cluster Randomization)

Clusters per site	Sites					
	4	6	8	10	12	20
4	0.43	0.29	0.23	0.20	0.18	0.13
6	0.37	0.25	0.20	0.17	0.15	0.11
8	0.34	0.23	0.18	0.16	0.14	0.10
10	0.32	0.21	0.17	0.15	0.13	0.10
12	0.30	0.20	0.16	0.14	0.13	0.09
20	0.27	0.18	0.15	0.13	0.11	0.08

NOTES. Values in the table are for two-tail significance = 0.05, power = 80 percent , no level-one covariates, a single level two covariate, $R^2_{CC} = 0.74, \bar{T} = 0.5, \rho_{CS} = 0.07, \rho_{CC} = 0.10$, constant n = 200 within each cluster, constant J clusters per site and $\tau_* = 0.10$.

MDESSD for a Cross-Site Standard Deviation of Effect Sizes
(Cluster Randomization)

Clusters per site	Sites					
	4	6	8	10	12	20
4	0.60	0.43	0.35	0.31	0.28	0.23
6	0.41	0.31	0.26	0.23	0.21	0.17
8	0.35	0.26	0.22	0.19	0.18	0.14
10	0.31	0.23	0.19	0.17	0.16	0.13
12	0.27	0.20	0.17	0.15	0.14	0.11
20	0.21	0.15	0.13	0.12	0.11	0.09

NOTES. Values in the table are for two-tail significance = 0.05, power = 80 percent , no level-one covariates, a single level two covariate, $R^2_{CC} = 0.74, \bar{T} = 0.5, \rho_{CS} = 0.07, \rho_{CC} = 0.10$, constant n = 200 within each cluster and constant J clusters per site and $\pi = 0.60$.

MDESD for the Coefficient On a Binary Site-Level Moderator
(Cluster Randomization)

Clusters per site	Sites					
	4	6	8	10	12	20
4	--	--	--	--	--	--
6	--	--	--	--	--	0.20 (0.92)
8	--	--	--	--	--	0.18 (0.77)
10	--	--	--	--	--	0.17 (0.68)
12	--	--	--	--	0.19 (0.90)	0.16 (0.61)
20	--	--	0.20 (0.94)	0.18 (0.82)	0.17 (0.72)	0.14 (0.49)

NOTES. Values in the table are for two-tail significance = 0.05, power = 80 percent, no level-one covariates, a single level two covariate, $R^2_{CC} = 0.74, \bar{T} = 0.5, \rho_{CS} = 0.07, \rho_{CC} = 0.10$, constant n = 200 within each cluster, constant J clusters per site and $\tau_* = 0.10$. Values in the table represent the MDESD. Values in parentheses represent R^2_W.

Conclusions

Challenges (Individual Randomization)

-MDES, total **number of sites** is key sample size

-MDESSD, total **number of individuals per site** is key sample size

-MDESD, total **number of sites** is key sample size (and there is a maximum possible value for the difference)

Challenges (Cluster Randomization)

-MDES, total **number of sites** is key sample size

-MDESSD, total **number of clusters per site** is key sample size

-MDESD, total **number of sites** is key sample size (and there is a maximum possible value for the difference)

- **Bottom line** = Power is a substantial limitation for this design