Power Analysis for Three Level Blocked Randomized Cost Effectiveness Trials

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Background:
Cost-effectiveness analysis (CEA) is a method of comparing alternative programs, policies, and practices with similar goals to determine which one has the largest effects relative to cost (Levin & Belfield, 2015). Randomized experiments that aim at estimating the cost-effective of the treatments are commonly referred to as randomized cost effectiveness trials (RCETs). Recently, with the resurgence of experiments in education, it is increasingly common for educational interventions to also include rigorous cost analysis components to support subsequent cross-study comparisons of the cost-effectiveness of alternative strategies for achieving target educational outcomes (Hollands et al., 2016).

When educational researchers design RCETs, it is important to conduct a priori power computations. The objective of this analysis is to ensure that the sample design offers a "good enough" chance (e.g., $power \geqslant 0.80$) to detect the cost-effectiveness of the treatment. Although CEA has been widely used in educational research, there is quite limited literature to guide educational researchers to design RCETs, which has some unique features, such as the nested nature of units of analysis in education trials. Recent work in health economics (Manju, Candel, & Berger, 2014, 2015) utilized two-level (e.g., students nested in schools) hierarchical linear models (HLMs) to resolve the potential nesting effects and provided formulas to calculate power for two-level RCETs. However, these approaches generally cannot be adopted by educational researchers easily for three reasons: (1) these methods assumed individual level cost information is available, while in education such information is usually missing, even though costs often do vary by individual (Levin & Belfield, 2015); (2) these models do not account for the influences of covariates, which are commonly available in education and can impact quite considerably the required sample sizes in education studies (e.g., Hedges & Hedberg, 2007); and (3) educational interventions often have more complicated nesting structure (e.g., students nested with classroom, and classrooms nested within schools). Although advancement was made for two-level designs by Li, Dong, and Maynard (2017), three-level designs have not been studied.

Purpose:
This study is designed to address these limitations of prior studies and contributes to CEA and power analysis literature by extending prior work to accommodate three-level models with various assumptions about covariates and alternative sample designs. Specifically, we develop new formulas for estimating the statistical power of three-level blocked RCETs with five alternative designs (see Table 1) varying by the level of treatment assignment and the level of the available cost information.

Research Design:
We will develop and implement new formulas for estimating the statistical power to RCETs with five alternative three-level block designs (see Table 1). In this proposal, we show the results for design 1 without covariates as an example. Consider a three-level blocked RCETs where leve-2 units (e.g., classes) are randomly assigned to treatment or control conditions within level-3 units (e.g., schools). When the level-1 information of effectiveness and cost is available, three-level HLMs could be used to estimate the mean differences of effectiveness measures and cost between the treatment and control groups. Specifically, let e_{ijk} represent the effectiveness measure (e.g.,

achievement) for individual i in level-2 unit j within level-3 unit k; c_{ijk} represents the cost for individual i in level-2 unit j within level-3 unit k; and T_{jk} is a treatment indicator variable. The three-level unconditional HLMs that do not include any covariates are

$$\begin{cases} e_{ijk} = \gamma_{000}^{e} + \gamma_{010}^{e} T_{jk} + u_{00k}^{e} + u_{01k}^{e} T_{jk} + r_{0jk}^{e} + \varepsilon_{ijk}^{e} \\ c_{ijk} = \gamma_{000}^{c} + \gamma_{010}^{c} T_{jk} + u_{00k}^{c} + u_{01k}^{c} T_{jk} + r_{0jk}^{c} + \varepsilon_{ijk}^{c} \end{cases}, \tag{1}$$

where the random effects at the second level and third level follow bivariate normal distribution, namely

$$\begin{pmatrix} u_{00k}^{e} \\ u_{00k}^{c} \end{pmatrix} \sim N\left(\begin{pmatrix} 0 \\ 0 \end{pmatrix}, \begin{pmatrix} \omega_{Ie}^{2} & \omega_{Iec} \\ \omega_{Iec} & \omega_{Ic}^{2} \end{pmatrix}\right), \begin{pmatrix} u_{01k}^{e} \\ u_{01k}^{c} \end{pmatrix} \sim N\left(\begin{pmatrix} 0 \\ 0 \end{pmatrix}, \begin{pmatrix} \omega_{Te}^{2} & \omega_{Tec} \\ \omega_{Tec} & \omega_{Tc}^{2} \end{pmatrix}\right), \begin{pmatrix} r_{0jk}^{e} \\ r_{0jk}^{c} \end{pmatrix} \sim N\left(\begin{pmatrix} 0 \\ 0 \end{pmatrix}, \begin{pmatrix} \tau_{e}^{2} & \tau_{ec} \\ \tau_{ec} & \tau_{c}^{2} \end{pmatrix}\right), \text{ and}$$
$$\begin{pmatrix} \varepsilon_{ijk}^{e} \\ \varepsilon_{ijk}^{c} \end{pmatrix} \sim N\left(\begin{pmatrix} 0 \\ 0 \end{pmatrix}, \begin{pmatrix} \sigma_{e}^{2} & \sigma_{ec} \\ \sigma_{ec} & \sigma_{c}^{2} \end{pmatrix}\right). \tag{2}$$

Let NMB_{ij} represent the net monetary benefit for individual i in level-2 unit j within level-3 unit k, we could reconstruct equation (1) as

$$NMB_{ij} = ke_{ijk} - c_{ijk} = \gamma_{000} + \gamma_{010} T_{jk} + u_{00k} + u_{01k}\, T_{jk} + r_{0jk} + \varepsilon_{ijk}, \tag{3}$$

where k is a positive constant that can be considered as the amount of society is willing to pay for a unit of effectiveness (Willan, 2001), $\gamma_{000} = k\gamma_{000}^{e} - \gamma_{000}^{c}$, $\gamma_{010} = \mathrm{k}\gamma_{010}^{e} - \gamma_{010}^{c}$, $u_{00k} = ku_{00k}^{e} - u_{00k}^{c}$, $u_{01k} = ku_{01k}^{c} - u_{01k}^{c}$, $r_{0jk} = kr_{0jk}^{e} - r_{0jk}^{c}$, $\varepsilon_{ijk} = k\varepsilon_{ijk}^{e} - \varepsilon_{ijk}^{c}$, $\mathrm{u_{00k}} \sim N(0, \omega_{I}^{2})$, $\mathrm{u_{01k}} \sim N(0, \omega_{T}^{2})$ $\mathrm{r_{0jk}} \sim N(0, \tau^{2})$, and $\varepsilon_{ijk} \sim N(0, \sigma^{2})$. The parameter of interest now is γ_{010}. When $\gamma_{010} > 0$, it indicates the treatment is cost-effective, when $\gamma_{010} < 0$, it indicates the treatment is not cost-effective.

Suppose there are K level-3 units, J level-2 units within each level-3 unit, and n level-1 units within each level-2 unit. The total number of level-1 units is nJK. Also, suppose within each level-3 unit, there are J_T units in the treatment group and J_C in the control condition. Let $p = \frac{J_T}{J}$ and define $\phi = \frac{1}{p(1-p)}$, then the variance of $\hat{\gamma}_{010}$ is

$$Var(\hat{\gamma}_{010}) = \frac{1}{nJK}(nJ\omega_{T}^{2} + n\phi\tau^{2} + \phi\sigma^{2}), \tag{4}$$

where $\omega_{T}^{2} = k^{2}\omega_{Te}^{2} - 2k\omega_{ec} + \omega_{Tc}^{2}$ $\tau_{T}^{2} = k^{2}\tau_{e}^{2} - 2k\tau_{ec} + \tau_{c}^{2}$ and $\sigma^{2} = k^{2}\sigma_{e}^{2} - 2k\sigma_{ec} + \sigma_{c}^{2}$. The unstandardized non-centrality parameter is

$$\lambda = \hat{\gamma}_{010}\sqrt{nJK}\sqrt{\frac{1}{(nJ\omega_{T}^{2} + n\phi\tau^{2} + \phi\sigma^{2})}}. \tag{5}$$

Let ω_{e}^{2} and ω_{c}^{2} represent the third level variance of effectiveness and cost, and $\sigma_{e} = \sigma_{e}^{2} + \tau_{e}^{2} + \omega_{e}^{2}$ and $\sigma_{c} = \sigma_{e}^{2} + \tau_{e}^{2} + \omega_{e}^{2}$ represent the total variance of effective measures and cost. We could use the σ_e or σ_c to standardize $\hat{\gamma}_{010}$ and to define effect size accordingly. Because one goal of this study is to compare the power for the same designs with or without taking cost variation into consideration, we will assume σ_c is equal to zero in some scenarios. Therefore, we define the standardized effect size as $\delta = \frac{\hat{\gamma}_{010}}{\sqrt{\sigma_e}}$. Assume the effectiveness measures are standardized with mean zero and standard deviation one (i.e., $\sigma_e = 1$), we can standardize the non-centrality parameter as

$$\lambda = \delta \sqrt{\frac{nJK}{k^2[(nJ\eta_e - \phi)\rho_3^e + \phi(n-1)\rho_2^e] + \sigma_c[(nJ\eta_c - \phi)\rho_3^c + \phi(n-1)\rho_2^c] + \phi(k^2 + \sigma_c) - 2k\sqrt{\sigma_c}(nJ\eta_{ec}r_3 + n\phi r_2 + \phi\rho_1)}}, \tag{6}$$

where $\rho_3^e = \frac{\omega_e^2}{\sigma_e}$ and $\rho_2^e = \frac{\tau_e^2}{\sigma_e}$ are the intra-class correlations (ICCs) of effectiveness data at the third and second levels; $\rho_3^c = \frac{\omega_c^2}{\sigma_c}$ and $\rho_2^c = \frac{\tau_c^2}{\sigma_c}$ are ICCs of cost data at the third and second levels; $\eta_e = \frac{\omega_{Te}^2}{\omega_e^2}$ and $\eta_c = \frac{\omega_{Tc}^2}{\omega_c^2}$ are the proportion of the treatment by block variance to the total variance at the third level (block) for effectiveness data and cost data; and $r_1 = \frac{\sigma_{ec}}{\sqrt{\sigma_e \sigma_c}}$ and $r_2 = \frac{\tau_{ec}}{\sqrt{\sigma_e \sigma_c}}$, are the correlations between cost and effectiveness at the first and second levels. Also, let ω_{ec} denote the covariance between effectiveness and cost at the third level, then $\eta_{ec} = \frac{\omega_{Tec}}{\omega_{ec}}$ is the proportion of the treatment by block covariance to the total covariance between cost and effectiveness at the third level and $r_3 = \frac{\omega_{ec}}{\sqrt{\sigma_e \sigma_c}}$ is the correlation between effectiveness and cost at the third level.

Results and Discussion:

In general, the power computation takes account of sample sizes (e.g., the number of students, classrooms, and schools), effect size, covariate effects, nesting effects (i.e., intra-class correlations for both cost and effectiveness data), and the correlations between cost and effectiveness at each level. We demonstrate how to design cluster RCETs with adequate power using the framework of PowerUp! (Dong & Maynard, 2013).

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Table 1. Four Designs of RCETs

	Level of Clustering	Level of Treatment Assignment	Level of Cost Information Available
Design 1	3	2	1
Design 2	3	2	2
Design 3	3	2	3
Design 4	3	1	1
Design 5	3	1	3