Inference for Two-stage Experiments under Covariate-Adaptive Randomization

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Introduction

Randomized controlled trials (RCTs) ubiquitous throughout sciences Interference widely encountered in experimental designs

Medication: Miguel and Kremer (2004)

Information treatment: Duflo and Saez (2003)

Cash transfer: Haushofer and Shapiro (2016)

Key challenges related to interference:

Naive estimators bias primary effects:

Example: mean difference of vaccinated vs unvaccinated

Primary effects: total impact of vaccination (defined later)

Emergence of additional parameters of interest, such as spillover effects

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Two-stage randomized experiments:

Common approach to address interference

Stage 1: Assigns treatment intensity at cluster level

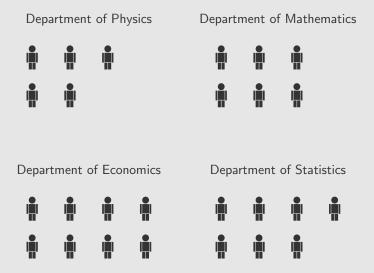
Stage 2: Assigns individual-level treatment within clusters

Example: Duflo and Saez (2003)

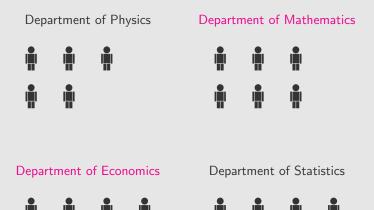
Stage 1: Assign 50% or 0% treatment intensity to departments

Stage 2: Assign \$20 to treated individuals in "50% departments"

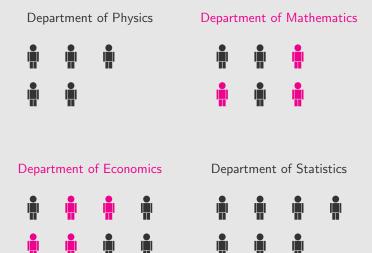
Example from Duflo and Saez (2003)



Example from Duflo and Saez (2003)



Example from Duflo and Saez (2003)



Covariate-adaptive randomization especially common in two-stage experiments:

Stratification: Hidrobo et al. (2016); Banerjee et al. (2021); Rogers and Feller (2018); Foos and de Rooij (2017); Muralidharan and Sundararaman (2015)

Matching: Duflo and Saez (2003); Beuermann et al. (2015); Ichino and Schündeln (2012); Kinnan et al. (2020); Malani et al. (2021)

Why use covariates? To improve efficiency

Applicable to both stages

Two-stage with matched pairs:

Pair clusters

Assign 50% treatment to one cluster, 0% to the other

Pair units within the 50% cluster

Randomly assign one unit to treatment within each pair

Goals for today:

Assess validity of common inference methods from the literature

Propose asymptotically exact inference methods

Optimize covariate use in two-stage designs

Introduce ex post covariate adjustment

LITERATURE

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Two-stage experiments (ignoring covariates)
     Basse and Feller (2018); Basse et al. (2019); Imai et al. (2021)
Partial population experiments
    Tortarolo et al. (2023); Baird et al. (2018); Hirano and Hahn (2010)
Split-plot design
    Zhao and Ding (2022); Shi et al. (2022)
Cluster randomized trials
     Bugni et al. (2022); Bai et al. (2022)
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Setup and Notation

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SETUP AND NOTATION

Index clusters (e.g. departments) by $g=1,\ldots,G$ and units in gth cluster by $i=1,\ldots,N_g$. Some notation:

 $Y_{i,g} = \text{observed outcome of unit } i \text{ in cluster } g \text{ (e.g. participation)}$

 $Z_{i,g} = \text{treatment assignment of unit } i \text{ in cluster } g \text{ (e.g. $20 check)}$

 $H_g =$ fraction of treated units in cluster g

 $N_g = \text{size of cluster } g$

Binary treatment:

Intensity levels: $H_g \in \{0, \pi_2\}$, Treatment status: $Z_{i,g} \in \{0, 1\}$

Stage 1:

 π_1 fraction of clusters with $H_g=\pi_2,$ remainder with $H_g=0$

Stage 2:

Within each treated cluster, π_2 fraction of units with $Z_{i,g}=1$, others with $Z_{i,g}=0$

Potential outcomes:

$$Y_{i,g}(z,h) \in \{Y_{i,g}(1,\pi_2), Y_{i,g}(0,\pi_2), Y_{i,g}(0,0)\}$$

where

z: Unit-level treatment status, $z \in \{0, 1\}$.

h: Fraction of treated units in the cluster, $h \in \{0, \pi_2\}$.

Observed outcome: $Y_{i,g} = Y_{i,g}(Z_{i,g}, H_g)$.

Equally-weighted cluster-level average treatment effect:

$$\begin{split} \gamma^P &:= E\left[\frac{1}{N_g}\sum_{i=1}^{N_g}\left(Y_{i,g}(1,\pi_2) - Y_{i,g}(0,0)\right)\right] \quad \text{(primary effect)} \\ \gamma^S &:= E\left[\frac{1}{N_g}\sum_{i=1}^{N_g}\left(Y_{i,g}(0,\pi_2) - Y_{i,g}(0,0)\right)\right] \quad \text{(spillover effect)} \end{split}$$

⇒ clusters are of interest.

Size-weighted cluster-level average treatment effect:

$$\begin{split} \theta^P &:= E\left[\frac{1}{E[N_g]} \sum_{i=1}^{N_g} \left(Y_{i,g}(1,\pi_2) - Y_{i,g}(0,0)\right)\right] \quad \text{(primary effect)} \\ \theta^S &:= E\left[\frac{1}{E[N_g]} \sum_{i=1}^{N_g} \left(Y_{i,g}(0,\pi_2) - Y_{i,g}(0,0)\right)\right] \quad \text{(spillover effect)} \end{split}$$

⇒ units in the clusters are of interest.

Weighted primary and spillover effects:

Definition

Primary effect:

$$\theta_{\omega}^{P} := E \left[\omega_{g} \left(\frac{1}{N_{g}} \sum_{i=1}^{N_{g}} Y_{i,g}(1, \pi_{2}) - Y_{i,g}(0, 0) \right) \right]$$

Spillover effect:

$$\theta_{\omega}^{S} := E\left[\omega_{g}\left(\frac{1}{N_{g}}\sum_{i=1}^{N_{g}}Y_{i,g}(0,\pi_{2}) - Y_{i,g}(0,0)\right)\right]$$

Equally-weighted: $\omega_g = 1$; Size-weighted: $\omega_g = N_g/E[N_g]$.

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ESTIMATION AND INFERENCE

OLS based on the model:

$$Y_{i,g} = \alpha + \beta_1 Z_{i,g} + \beta_2 L_{i,g} + \epsilon_{i,g} ,$$

where $L_{i,g} = I\{H_g = \pi_2\}(1 - Z_{i,g}).$

1. Standard OLS yields size-weighted estimators $\hat{\theta}^P$ and $\hat{\theta}^S$:

$$\hat{\theta}^P \xrightarrow{p} \theta^P, \quad \hat{\theta}^S \xrightarrow{p} \theta^S.$$

2. OLS weighted by $1/N_g$ yields equally-weighted estimators $\tilde{\theta}^P$ and $\tilde{\theta}^S$:

$$\tilde{\theta}^P \xrightarrow{p} \gamma^P, \quad \tilde{\theta}^S \xrightarrow{p} \gamma^S$$
.

Example:

$$\hat{\theta}^P = \frac{1}{N_1} \sum_{1 \le g \le G} I\{H_g = \pi_2\} N_g \bar{Y}_g^1 - \frac{1}{N_0} \sum_{1 \le g \le G} I\{H_g = 0\} N_g \bar{Y}_g^1$$

where

 $N_1 = \#$ units in treated clusters

 $N_0=\#$ units in control clusters

 $\bar{Y}_g^1 = \begin{cases} \text{average outcome of treated units,} & \text{if cluster } g \text{ is treated} \\ \text{average outcome of control units,} & \text{if cluster } g \text{ is control} \end{cases}$

Caveats for regression-based estimators:

Is everyone observed in each cluster?

OLS inconsistent for θ^P and θ^S if only a subset of units observed

Use weighted OLS: $\frac{\text{cluster size}}{\text{sample size}}$ to recover θ^P and θ^S

Strata fixed effects:

Avoid adding strata fixed effects in the regression

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Preview:

No covariates used in design:

"OLS + Cluster robust standard errors" is asymptotically exact

Covariates used for stratification or matching:

Requires further asymptotic analysis

Notation: C_g (cluster-level), $X_{i,g}$ (individual-level)

Stage 1:

n strata, each of size k (clusters grouped by $S_g = S(C_g, N_g)$).

Denote the *j*-th stratum by λ_j ; example:

$$\{1,\ldots,G\} \xrightarrow{\text{sort by } S_g} \{\underbrace{10,2,5}_{\lambda_1},\underbrace{21,99,6}_{\lambda_2},\ldots,\underbrace{47,23,11}_{\lambda_n}\}$$

Stage 2: similar to Stage 1.

Assumption 1

Strata satisfy:

$$\frac{1}{n} \sum_{1 < j < n} \max_{i, k \in \lambda_j} ||S_i - S_k||^2 \stackrel{P}{\to} 0.$$

Theorem 1

Under previous assumptions + regularity + sampling procedure conditions, as $G \to \infty$,

$$\sqrt{G} \left(\hat{\theta}^P - \theta^P \right) \xrightarrow{d} \mathcal{N}(0, V(1)) ,$$

$$\sqrt{G} \left(\hat{\theta}^S - \theta^S \right) \xrightarrow{d} \mathcal{N}(0, V(0)) ,$$

where

$$V(z) = \frac{1}{\pi_1} \operatorname{Var}[\tilde{Y}_g(z, \pi_2)] + \frac{1}{1 - \pi_1} \operatorname{Var}[\tilde{Y}_g(0, 0)] - \pi_1 (1 - \pi_1) E\left[\left(\frac{1}{\pi_1} E[\tilde{Y}_g(z, \pi_2) \mid S_g] + \frac{1}{1 - \pi_1} E[\tilde{Y}_g(0, 0) \mid S_g] \right)^2 \right],$$

with $ilde{Y}_g(z,\pi_2)$ as the demeaned, size-weighted average outcomes.

$$V(z) = \underbrace{\frac{1}{\pi_1} \operatorname{Var}[\tilde{Y}_g(z, \pi_2)] + \frac{1}{1 - \pi_1} \operatorname{Var}[\tilde{Y}_g(0, 0)]}_{:=V_1(z)} - \underbrace{\pi_1(1 - \pi_1) E\left[\left(\frac{1}{\pi_1} E[\tilde{Y}_g(z, \pi_2) \mid S_g] + \frac{1}{1 - \pi_1} E[\tilde{Y}_g(0, 0) \mid S_g]\right)^2\right]}_{:=V_2(z)}$$

Cluster-robust variance estimator:

Generally conservative: $\hat{V}_{CR}(z) \xrightarrow{p} V_1(z) \geq V(z)$.

Consistent if design is not covariate-adaptive, or S_g is irrelevant.

$$V(z) = \underbrace{\frac{1}{\pi_1} \operatorname{Var}[\tilde{Y}_g(z, \pi_2)] + \frac{1}{1 - \pi_1} \operatorname{Var}[\tilde{Y}_g(0, 0)]}_{:=V_1(z)} - \underbrace{\pi_1(1 - \pi_1) E\left[\left(\frac{1}{\pi_1} E[\tilde{Y}_g(z, \pi_2) \mid S_g] + \frac{1}{1 - \pi_1} E[\tilde{Y}_g(0, 0) \mid S_g]\right)^2\right]}_{:=V_2(z)}$$

Efficiency gain from the first-stage stratification/matching

$$V(z) = \underbrace{\frac{1}{\pi_1} \operatorname{Var}[\tilde{Y}_g(z, \pi_2)] + \frac{1}{1 - \pi_1} \operatorname{Var}[\tilde{Y}_g(0, 0)]}_{:=V_1(z)} - \underbrace{\pi_1(1 - \pi_1) E\left[\left(\frac{1}{\pi_1} E[\tilde{Y}_g(z, \pi_2) \mid S_g] + \frac{1}{1 - \pi_1} E[\tilde{Y}_g(0, 0) \mid S_g]\right)^2\right]}_{:=V_2(z)}$$

Efficiency gain from second-stage stratification/matching $\tilde{Y}_g(1,\pi_2)$: average of treated units (depends on $Z_{i,g}$)

$$V(z) = \underbrace{\frac{1}{\pi_1} \operatorname{Var}[\tilde{Y}_g(z, \pi_2)] + \frac{1}{1 - \pi_1} \operatorname{Var}[\tilde{Y}_g(0, 0)]}_{V_1(z)} - \underbrace{\pi_1(1 - \pi_1)E\left[\left(\frac{1}{\pi_1} E[\tilde{Y}_g(z, \pi_2) \mid S_g] + \frac{1}{1 - \pi_1} E[\tilde{Y}_g(0, 0) \mid S_g]\right)^2\right]}_{V_2(z)}$$

Relative importance:

First stage: O(1) vs. Second stage: O(1/N)

N: smallest cluster size

How to estimate V(z) consistently?

First, define

$$\tilde{Y}_{g}^{z} = \frac{N_{g}}{\frac{1}{G} \sum_{1 \leq g \leq G} N_{g}} \left(\bar{Y}_{g}^{z} - \frac{\frac{1}{G_{g}} \sum_{1 \leq j \leq G} \bar{Y}_{j}^{z} I\{H_{g} = H_{j}\} N_{j}}{\frac{1}{G} \sum_{1 \leq j \leq G} N_{j}} \right) ,$$

 \tilde{Y}^z_g acts like a "feasible" version of $\tilde{Y}_g(z,h)$:

$$H_g = h \Longrightarrow \tilde{Y}_g^z \approx \tilde{Y}_g(z, h).$$

Using this, "easy" to estimate quantities like $E[\tilde{Y}_g^2(z,h)]$ using, e.g.,

$$\frac{1}{G\pi_1} \sum_{1 \le g \le G} (\tilde{Y}_g^z)^2 I\{H_g = h\} \ .$$

"Harder" to estimate quantities like $E\left[E[\tilde{Y}_g(z,h)\mid S_g]^2\right]$

Problem: Need two independent copies of $\tilde{Y}_g(z,h)$ within a stratum.

matched pair: observe one $\tilde{Y}_g(0,0)$ and one $\tilde{Y}_g(z,\pi_2).$

Solution: Look at nearby pairs.

Strategy works if we assume adjacent pairs are suitably close:

Assumption 2

Strata satisfy:

$$\frac{1}{n} \sum_{1 \le j \le \lfloor n/2 \rfloor} \max_{i \in \lambda_{2j-1}, k \in \lambda_{2j}} ||S_i - S_k||^2 \xrightarrow{P} 0.$$

For matched-pair design, this strategy leads to following estimator:

$$\hat{V}(z) = \hat{\tau}_G^2 - \frac{1}{2}\hat{\lambda}_G^2$$

where

$$\hat{\tau}_{G}^{2} = \frac{2}{G} \sum_{j \leq G/2} \left(\sum_{i \in \lambda_{j}} \tilde{Y}_{i}^{z} I\{H_{i} = \pi_{2}\} - \sum_{i \in \lambda_{j}} \tilde{Y}_{i}^{z} I\{H_{i} = 0\} \right)^{2}$$

$$\hat{\lambda}_{G}^{2} = \frac{4}{G} \sum_{j \leq G/4} \left(\sum_{i \in \lambda_{2j-1}} \tilde{Y}_{i}^{z} I\{H_{i} = \pi_{2}\} - \sum_{i \in \lambda_{2j-1}} \tilde{Y}_{i}^{z} I\{H_{i} = 0\} \right)$$

$$\times \left(\sum_{i \in \lambda_{2j}} \tilde{Y}_{i}^{z} I\{H_{i} = \pi_{2}\} - \sum_{i \in \lambda_{2j}} \tilde{Y}_{i}^{z} I\{H_{i} = 0\} \right)$$

Summary:

Estimation:

OLS from the model:

$$Y_{i,g} = \alpha + \beta_1 Z_{i,g} + \beta_2 L_{i,g} + \epsilon_{i,g},$$

weighted by ω_g/N_g for θ^P_ω , θ^S_ω .

Inference: Adjusted t-test using variance estimator $\hat{V}(z).$

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Optimality

Covariate-Adjustment

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OPTIMALITY

Optimal matching via index function in the first-stage:

Theorem 2

$$V(z) \text{ is minimized when } S_g = E\left[\frac{\tilde{Y}_g(z,\pi_2)}{\pi_1} + \frac{\tilde{Y}_g(0,0)}{1-\pi_1} \mid C_g,N_g\right].$$

Design Recommendations:

- 1. Match on N_g (predictive of scale $\tilde{Y}_g(z,h)$).
- Match on sums of individual covariates, not averages (e.g., Duflo and Saez (2003), match on the number of participants, not participation rate).
- 3. Use estimated index function (with large pilot).

OPTIMALITY (CONT.)

Under homogeneous covariance:

Theorem 3

V(z) is minimized by stratifying/matching on

$$E[Y_{i,g}(z,\pi_2) \mid (X_{i,g} : 1 \le i \le N_g)].$$

Design Recommendation:

For highly correlated outcomes within clusters, match on both individual and neighbors' covariates.

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COVARIATE ADJUSTMENT

Match/stratify on a small set of covariates; adjust for additional covariates ex-post.

Linear adjustment improves efficiency:

Adjusted outcomes: $Y_{i,g}-(\psi_g-\bar{\psi}_G)'\hat{\beta}^P/N_g$ (for primary effects), where ψ_g is the sum of covariates in the g-th cluster. Estimate $\hat{\beta}^P$:

- 1. Compute pairwise differences for $\tilde{Y}_g^1 \bar{N}_G$ and ψ_g in each stratum.
- 2. Run linear regression on the pairwise differences.

COVARIATE ADJUSTMENT (CONT.)

Taking the covariate-adjusted estimator $\hat{\theta}^{P,adj}$ as an example:

$$\sqrt{G}\left(\hat{\theta}^{P,adj} - \theta^P\right) \xrightarrow{d} \mathcal{N}(0, V(1) - \kappa^2)$$
,

Efficiency improvement with

$$E\left[\operatorname{Cov}\left[\frac{1}{\pi_1}\tilde{Y}_g(1,\pi_2) + \frac{1}{1-\pi_1}\tilde{Y}_g(0,0), \psi_g \mid S_g\right]\right] \neq 0.$$

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SIMULATION

First-stage	Parameter	Complete Rand.	Stratification	Matched-pair
Complete David	θ^P	1.0000	0.9803	0.9560
Complete Rand.	θ^S	1.0000	0.9921	0.9596
Stratification	θ^P	0.8227	0.7601	0.7672
	θ^S	0.8244	0.7790	0.7693
Matched-pair	θ^P	0.0921	0.0873	0.0755
Matcheu-pair	θ^S	0.0997	0.0930	0.0757

Table: Ratio of MSE under various designs

First-stage Parame		Complete Rand.	Stratification	Matched-pair
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	θ^S	0.0997	0.0930	0.0757

Table: Ratio of MSE under various designs

Inference Method	Effect	S C	S S	S MP	MP C	MP S	MP MP
Adjusted	Primary	0.048	0.045	0.058	0.052	0.057	0.051
t-test	Spillover	0.049	0.046	0.061	0.047	0.058	0.050
OLS robust	Primary	0.184	0.194	0.156	0.062	0.086	0.049
(standard t -test)	Spillover	0.184	0.167	0.159	0.077	0.048	0.048
OLS cluster (clustered t -test)	Primary Spillover	0.000	0.000	0.000	0.000	0.000	0.000
OLS with strata	Primary	0.209	0.196	0.179	0.100	0.106	0.077
fixed effects (robust)	Spillover	0.201	0.184	0.177	0.113	0.100	0.075
OLS with strata	Primary	0.028	0.027	0.029	0.068	0.085	0.071
fixed effects (clustered)	Spillover	0.036	0.027	0.026	0.064	0.062	0.069

Table: Rejection probabilities under $\beta_1=\beta_2=0$

Inference Method	Effect	S C	S S	S MP	MP C	MP S	MP MP
$\begin{array}{c} Adjusted \\ t\text{-test} \end{array}$	Primary	0.048	0.045	0.058	0.052	0.057	0.051
	Spillover	0.049	0.046	0.061	0.047	0.058	0.050
OLS robust (standard t -test)	Primary	0.184	0.194	0.156	0.062	0.086	0.049
	Spillover	0.184	0.167	0.159	0.077	0.048	0.048
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Table: Rejection probabilities under $\beta_1=\beta_2=0$

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EMPIRICAL APPLICATION

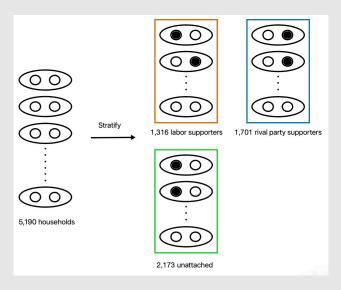
Foos and de Rooij (2017): randomized experiments in UK on social influence within households

Two-stage randomization: households, then individuals

Treatment: telephone encouragement to vote (PCC election)

Outcome: turnout rates

EMPIRICAL APPLICATION (CONT.)



EMPIRICAL APPLICATION (CONT.)

	adjusted t -test	OLS robust	OLS cluster	OLS fe robust	OLS fe cluster
Primary	3.0488	3.0488	3.0488	2.9971	2.9971
	$\pm~2.2149$	$\pm\ 2.0526$	$\pm~2.2386$	$\pm~2.0337$	$\pm\ 2.2159$
Spillover	4.5930	4.5930	4.5930	4.5413	4.5413
	$\pm~2.2500$	$\pm~2.0885$	$\pm~2.2715$	$\pm\ 2.0719$	± 2.2509

Table: Point estimates and confidence intervals

OLS robust: narrower intervals (invalid).

OLS cluster: wider intervals (conservative).

OLS fe: different point estimates.

Conclusion

Two-stage experiments are common

Often involve covariate-adaptive randomization

Developed methods for inference for matching/stratification

Provided guidance for efficient experimental designs

Proposed covariate-adjusted estimator

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DEFINITIONS

For
$$(z,h) \in \{(1,\pi_2), (0,\pi_2), (0,0)\}$$
,

$$\tilde{Y}_g(z,h) = \frac{N_g}{E[N_g]} \left(\bar{Y}_g(z,h) - \frac{E[\bar{Y}_g(z,h)N_g]}{E[N_g]} \right) \ , \label{eq:Yg}$$

where

$$\bar{Y}_g(1, \pi_2) = \frac{1}{M_g^1} \sum_{i \in \mathcal{M}_g} Y_{i,g}(1, \pi_2) Z_{i,g}(\pi_2)$$

$$\bar{Y}_g(0, \pi_2) = \frac{1}{M_g^0} \sum_{i \in \mathcal{M}_g} Y_{i,g}(0, \pi_2) (1 - Z_{i,g}(\pi_2))$$

$$\bar{Y}_g(0, 0) = \frac{1}{M_g} \sum_{i \in \mathcal{M}_g} Y_{i,g}(0, 0) .$$

ASSUMPTIONS

Homogeneous partial interference

Potential outcomes satisfy:

$$\begin{split} Y_{i,g}(\mathbf{z},n) &= Y_{i,g}(\mathbf{z}',n) \text{ w.p.1 if } z_{i,g} = z'_{i,g} \\ \text{and } \sum_{1 \leq j \leq n} z_{j,g} &= \sum_{1 \leq j \leq n} z'_{j,g} \text{ for any } 1 \leq i \leq n, 1 \leq g \leq G \ . \end{split}$$

where \mathbf{z} and \mathbf{z}' are any realized vectors of assignment, and $z_{i,g}, z'_{i,g}$ are the corresponding individual treatment indicators for i-th unit in g-th cluster.

Assumptions (cont.)

Sampling procedure

The distribution satisfies:

- (a) $\{(\mathcal{M}_g, C_g, N_g, X_g, (\bar{Y}_g(z, h), z \in \{0, 1\}, h \in \{0, \pi_2\})) : 1 \leq g \leq G\}$ is an i.i.d. sequence of random variables.
- (c) $P\{|\mathcal{M}_g| \ge 2\} = 1 \text{ and } E[N_g^2] < \infty.$
- (d) For some constant $C<\infty$, $P\left\{E[Y_{i,g}^2(z,h)\mid N_g,C_g,X_g]\leq C \text{ for all } 1\leq i\leq N_g\right\}=1 \text{ for all } z\in\{0,1\} \text{ and } h\in\mathcal{H} \text{ and } 1\leq g\leq G.$
- (e) $\mathcal{M}_g \perp ((Y_{i,g}(z,h):z\in\{0,1\},h\in\mathcal{H}):1\leq i\leq N_g)\mid C_g,N_g,X_g$ for all $1\leq g\leq G.$
- (f) For all $z \in \{0,1\}, h \in \mathcal{H}$ and $1 \leq g \leq G$,

$$E\left[\frac{1}{M_g}\sum_{i\in\mathcal{M}_g}Y_{i,g}(z,h)\mid N_g\right]=E\left[\frac{1}{N_g}\sum_{1\leq i\leq N_g}Y_{i,g}(z,h)\mid N_g\right]\text{w.p.1}\;.$$

Assumptions (cont.)

To formalize the second-stage assignment:

$$Z_{i,g} = \sum_{h \in \mathcal{H}} Z_{i,g}(h) I\{H_g = h\} \text{ for } 1 \leq i \leq N_g \ .$$

Second-stage assignment

The treatment assignment mechanism for the second-stage satisfies:

- (a) $(((Z_{i,g}(h):h\in\mathcal{H}):1\leq i\leq N_g):1\leq g\leq G)\perp H^{(G)}$,
- (b) $W^{(G)} \perp (((Z_{i,g}(h):h\in\mathcal{H}):1\leq i\leq N_g):1\leq g\leq G)\mid (B_g:1\leq g\leq G),$
- (c) For all $1 \le g \le G$, $E[Z_{i,g}(h) \mid B_g] = \frac{1}{M_g} \sum_{i \in \mathcal{M}_g} Z_{i,g}(h) = h + o_P(1).$

Assumptions (cont.)

Lipschitz condition

The distribution of data is such that

- (a) $E[\bar{Y}_g^r(z,h)N_g^\ell|S_g=s]$ is Lipschitz in s for $(z,h)\in\{(0,0),(0,\pi_2),(1,\pi_2)\}$ and $r,\ell\in\{0,1,2\}.$
- (b) For some $C<\infty$, $P\{E[N_g^2|S_g]\leq C\}=1$

VARIANCE ESTIMATOR

For $z \in \{0,1\}$,

$$\hat{V}(z) = \frac{1}{\pi_1} \hat{\mathbb{V}}_{1,n}^z(\pi_2) + \frac{1}{1-\pi_1} \hat{\mathbb{V}}_{1,n}^z(0) + \hat{\mathbb{V}}_{2,n}^z(\pi_2,\pi_2) + \hat{\mathbb{V}}_{2,n}^z(0,0) - 2\hat{\mathbb{V}}_{2,n}^z(\pi_2,0)$$

with

$$\begin{split} \hat{\mathbb{V}}_{1,n}^z(h) &= \hat{\mathbb{E}}\left[\operatorname{Var}\left[\tilde{Y}_g(z,h) \mid S_g\right]\right] := \left(\hat{\sigma}_n^z(h)\right)^2 - \left(\hat{\rho}_n^z(h,h) - \left(\hat{\Gamma}_n^z(h)\right)^2\right) \\ \hat{\mathbb{V}}_{2,n}^z(h,h') &= \operatorname{Cov}\left[E\left[\tilde{Y}_g(z,h) \mid S_g\right], E\left[\tilde{Y}_g(z,h') \mid S_g\right]\right] := \hat{\rho}_n^z(h,h') - \hat{\Gamma}_n^z(h)\hat{\Gamma}_n^z(h') \end{split}$$

where

$$\begin{split} \hat{\rho}_{n}^{z}(h,h) &:= \frac{2}{n} \sum_{1 \leq j \leq \lfloor n/2 \rfloor} \frac{1}{k^{2}(h)} \Big(\sum_{i \in \lambda_{2j-1}} \tilde{Y}_{i}^{z} I\{H_{i} = h\} \Big) \Big(\sum_{i \in \lambda_{2j}} \tilde{Y}_{i}^{z} I\{H_{i} = h\} \Big) \\ \hat{\rho}_{n}^{z}(\pi_{2},0) &:= \frac{1}{n} \sum_{1 \leq j \leq n} \frac{1}{l(k-l)} \Big(\sum_{i \in \lambda_{j}} \tilde{Y}_{i}^{z} I\{H_{i} = \pi_{2}\} \Big) \Big(\sum_{i \in \lambda_{j}} \tilde{Y}_{i}^{z} I\{H_{i} = 0\} \Big) \\ \Big(\hat{\sigma}_{n}^{z}(h) \Big)^{2} &:= \frac{1}{nk(h)} \sum_{1 \leq g \leq G} (\tilde{Y}_{g}^{z} - \hat{\Gamma}_{n}^{z}(h))^{2} I\{H_{g} = h\} \; . \end{split}$$

Variance Estimator (cont.)

For
$$z \in \{0, 1\}$$
,

$$\tilde{Y}_g^z = \frac{N_g}{\frac{1}{G} \sum_{1 \leq g \leq G} N_g} \left(\bar{Y}_g^z - \frac{\frac{1}{Gg} \sum_{1 \leq j \leq G} \bar{Y}_j^z I\{H_g = H_j\} N_j}{\frac{1}{G} \sum_{1 \leq j \leq G} N_j} \right) \; . \label{eq:Yg}$$

where $G_g = \sum_{1 \leq j \leq G} I\{H_g = H_j\}.$

$$\begin{split} \hat{\rho}_n^z(h,h) &\xrightarrow{P} E[E[\bar{Y}_g(z,h) \mid S_g]^2] \\ \hat{\rho}_n^z(\pi_2,0) &\xrightarrow{P} E[E[\bar{Y}_g(z,\pi_2) \mid S_g]E[\bar{Y}_g(z,0) \mid S_g]] \\ \left(\hat{\sigma}_n^z(h)\right)^2 &\xrightarrow{p} \mathrm{Var}[\tilde{Y}_{i,g}(z,h)] \; . \end{split}$$

Assumptions for Optimality Results

Assumption 3

For
$$z \in \{0,1\}$$
, $1 \le i \ne j \le N_g$,
$$\operatorname{Cov}\left[Y_{i,g}(z,\pi_2), Y_{j,g}(z,\pi_2) \mid (X_{i,g}: 1 \le i \le N_g)\right]$$
$$= \operatorname{Cov}\left[Y_{i,g}(z,\pi_2), Y_{j,g}(z,\pi_2)\right] \ .$$

COVARIATE ADJUSTMENT

Linear adjustment for primary effects:

$$\hat{\theta}^{P,adj} = \frac{1}{N_1} \sum_{1 \le g \le G} I\{H_g = \pi_2\} (N_g \bar{Y}_g^1 - (\psi_g - \bar{\psi}_G)' \hat{\beta}^P)$$

$$- \frac{1}{N_0} \sum_{1 \le g \le G} I\{H_g = 0\} (N_g \bar{Y}_g^1 - (\psi_g - \bar{\psi}_G)' \hat{\beta}^P) ,$$

where $\hat{\beta}^P$ is obtained from the linear regression of $\hat{\mu}_{1,j}-\hat{\mu}_{0,j}$ on a constant and $\hat{\psi}_{1,j}-\hat{\psi}_{0,j}$.

$$\hat{\mu}_{1,j} = \frac{1}{k\pi_1} \sum_{g \in \lambda_j} \tilde{Y}_g^1 \bar{N}_g I\{H_g = \pi_2\}$$

$$\hat{\psi}_{1,j} = \frac{1}{k\pi_1} \sum_{g \in \lambda_j} \psi_g I\{H_g = \pi_2\} .$$

COVARIATE ADJUSTMENT (CONT.)

Under reasonable assumptions, as $G \to \infty$,

$$\sqrt{G}(\hat{\theta}^{P,adj} - \theta^P) \stackrel{d}{\to} N(0, V^*(1))$$
,

where

$$V^*(1) = V(1) - \frac{1}{\pi_1(1 - \pi_1)} \frac{1}{E[N_g]^2} E[\text{Var}[\psi_g' \beta^P \mid S_g]] ,$$

with

$$\beta^{P} = \pi_{1}(1 - \pi_{1})(E[\operatorname{Var}[\psi_{g} \mid S_{g}]])^{-1}E\left[\operatorname{Cov}\left[\frac{\tilde{Y}_{g}(1, \pi_{2})}{\pi_{1}} + \frac{\tilde{Y}_{g}(0, 0)}{1 - \pi_{1}}, \psi_{g} \mid S_{g}\right]\right]E[N_{g}].$$