# <span id="page-0-0"></span>Design and Analysis of Two-Stage Randomized Experiments

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- Causal inference revolution over the last three decades
- $\bullet$  The first half of this revolution  $\rightsquigarrow$  no interference between units

- In social sciences, interference is the rule rather than the exception
- How should we account for spillover effects?

• Experimental design solution:

two-stage randomized experiments (Hudgens and Halloran, 2008)

### Empirical Motivation: Indian Health Insurance Experiment

- 150 million people worldwide face financial catastrophe due to health spending  $\rightsquigarrow$  1/3 live in India
- In 2008, Indian government introduced the national health insurance program (RSBY) to cover about 60 million poorest families
- The government wants to expand the RSBY to 500 million Indians
- What are financial and health impacts of this expansion?
- Do beneficiaries have spillover effects on non-beneficiaries?
- We conduct an RCT to evaluate the impact of expanding RSBY in the State of Karnakata

# Study Design

- Sample: 10,879 households in 435 villages
- Experimental conditions:
	- <sup>A</sup> Opportunity to enroll in RSBY essentially for free
	- B No intervention
- **o** Time line:
	- **1** September 2013 February 2014: Baseline survey
	- 2 April May 2015: Enrollment
	- **3** September 2016 January 2017: Endline survey
- Two stage randomization:



### Causal Inference and Interference between Units

**1** Causal inference without interference between units

- Potential outcomes:  $Y_i(1)$  and  $Y_i(0)$
- Observed outcome:  $Y_i = Y_i(D_i)$
- Causal effect:  $Y_i(1) Y_i(0)$

2 Causal inference with interference between units

- Potential outcomes:  $Y_i(d_1, d_2, \ldots, d_N)$
- Observed outcome:  $Y_i = Y_i(D_1, D_2, \ldots, D_N)$
- **Causal effects:** 
	- Direct effect =  $Y_i(D_i = 1, D_{-i} = d) Y_i(D_i = 0, D_{-i} = d)$
	- Spillover effect =  $Y_i(D_i = d, D_{-i} = d) Y_i(D_i = d, D_{-i} = d')$

Fundamental problem of causal inference  $\rightsquigarrow$  only one potential outcome is observed

# What Happens if We Ignore Interference?

- Completely randomized experiment
	- Total of  $N$  units with  $N_1$  treated units

$$
\bullet \ \Pr(D_i=1)=N_1/N \text{ for all } i
$$

• Difference-in-means estimator is unbiased for the average direct effect:

$$
\frac{1}{N} \sum_{i=1}^{N} \sum_{\mathbf{d}_{-i}} \left\{ Y_i(D_i = 1, \mathbf{D}_{-i} = \mathbf{d}_{-i}) \underbrace{\mathbb{P}(\mathbf{D}_{-i} = \mathbf{d}_{-i} | D_i = 1)}_{1/(N-1)} - Y_i(D_i = 0, \mathbf{D}_{-i} = \mathbf{d}_{-i}) \underbrace{\mathbb{P}(\mathbf{D}_{-i} = \mathbf{d}_{-i} | D_i = 0)}_{1/(N-1)} \right\}
$$

Bernoulli randomization (or large sample) simplifies the expression

$$
\frac{1}{N2^{N-1}}\sum_{i=1}^N\sum_{\mathbf{d}_{-i}}\{Y_i(D_i=1,\mathbf{D}_{-i}=\mathbf{d}_{-i})-Y_i(D_i=0,\mathbf{D}_{-i}=\mathbf{d}_{-i})\}
$$

• Cannot estimate spillover effects

### What about Cluster Randomized Experiment?

#### • Setup:

- Total of  $J$  clusters with  $J_1$  treated clusters
- Total of N units:  $n_i$  units in cluster j
- Complete randomization of treatment across clusters
- All units are treated in a treated cluster
- No unit is treated in a control cluster
- Partial interference assumption:
	- No interference across clusters
	- Interference within a cluster is allowed

Difference-in-means estimator is unbiased for the average total effect:

$$
\frac{1}{N}\sum_{j=1}^J\sum_{i=1}^{n_j}\{Y_{ij}(D_{1j}=1,D_{2j}=1,\ldots,D_{n_jj}=1)\\-\gamma_{ij}(D_{1j}=0,D_{2j}=0,\ldots,D_{n_jj}=0)\}
$$

• Cannot estimate spillover effects

#### Two-stage Randomized Experiments

- Individuals (households):  $i = 1, 2, \ldots, N$
- Blocks (villages):  $j = 1, 2, \ldots, J$
- Size of block  $j$ :  $\emph{n}_{j}$  where  $\emph{N}=\sum_{j=1}^{J}$   $\emph{n}_{j}$
- $\bullet$  Binary treatment assignment mechanism:  $A_i \in \{0, 1\}$
- **•** Binary encouragement to receive treatment:  $Z_{ii} \in \{0, 1\}$
- Binary treatment indicator:  $D_{ij} \in \{0, 1\}$
- $\bullet$  Observed outcome:  $Y_{ii}$
- Partial interference assumption: No interference across blocks
	- Potential treatment and outcome:  $D_{ij}(z_i)$  and  $Y_{ij}(z_i)$
	- Observed treatment and outcome:  $D_{ii} = D_{ii}(\mathbf{Z}_i)$  and  $Y_{ii} = Y_{ii}(\mathbf{Z}_i)$
- Number of potential values reduced from  $2^N$  to  $2^{n_j}$

#### Intention-to-Treat Analysis: Causal Quantities of Interest

• Average outcome under the treatment  $Z_{ii} = z$  and the assignment mechanism  $A_i = a$ :

$$
\overline{Y}_{ij}(z,a) = \sum_{\mathbf{z}_{-i,j}} Y_{ij}(Z_{ij}=z,\mathbf{Z}_{-i,j}=\mathbf{z}_{-i,j})\mathbb{P}_a(\mathbf{Z}_{-i,j}=\mathbf{z}_{-i,j} | Z_{ij}=z)
$$

• Average direct effect of encouragement on outcome:

$$
\mathsf{ADE}^{\mathsf{Y}}(a) = \frac{1}{N} \sum_{j=1}^{J} \sum_{i=1}^{n_j} \{ \overline{Y}_{ij}(1, a) - \overline{Y}_{ij}(0, a) \}
$$

• Average spillover effect of encouragement on outcome:

$$
\mathsf{ASE}^{\mathsf{Y}}(z) = \frac{1}{N} \sum_{j=1}^{J} \sum_{i=1}^{n_j} \{ \overline{Y}_{ij}(z,1) - \overline{Y}_{ij}(z,0) \}
$$

**• Horvitz-Thompson estimator for unbiased estimation** 

#### Effect Decomposition

• Average total effect of encouragement on outcome:

$$
ATE^{Y} = \frac{1}{N} \sum_{j=1}^{J} \sum_{i=1}^{n_j} \{ \overline{Y}_{ij}(1,1) - \overline{Y}_{ij}(0,0) \}
$$

• Total effect  $=$  Direct effect  $+$  Spillover effect:

$$
ATE^{Y} = ADE^{Y}(1) + ASE^{Y}(0) = ADE^{Y}(0) + ASE^{Y}(1)
$$

• In a two-stage RCT, we have an unbiased estimator,

$$
\mathbb{E}\left[\frac{\sum_{j=1}^{J} \mathbf{1}\{A_j = a\} \frac{n_j}{N} \frac{\sum_{i=1}^{n_j} Y_{ij} \mathbf{1}\{Z_{ij} = z\}}{\sum_{j=1}^{J} \mathbf{1}\{A_j = a\}}}{\frac{1}{J} \sum_{j=1}^{J} \mathbf{1}\{A_j = a\}}\right] = \frac{1}{N} \sum_{j=1}^{J} \sum_{i=1}^{n_j} \overline{Y}_{ij}(z, a)
$$

Halloran and Struchiner (1995), Sobel (2006), Hudgens and Halloran (2008)

#### When Should We Use Two-stage Randomization?

- Do we care about spillover effects?
	- Yes  $\rightsquigarrow$  two-stage randomization
	- No
		- **a** Interested in direct effects  $\rightsquigarrow$  individual randomization
		- **•** Interested in total effects  $\rightsquigarrow$  cluster randomization
- Do we lose statistical power if there is no spillover effect? variance of the difference-in-means estimator:

$$
\frac{1-\rho}{J^2}\left\{\mathbb{V}(Y_{ij}(1))\sum_{s=0}^1\frac{J_s}{n\rho_s}+\mathbb{V}(Y_{ij}(0))\sum_{s=0}^1\frac{J_s}{n(1-\rho_s)}\right\}-\frac{1-\rho}{Jn}\mathbb{V}(Y_{ij}(1)-Y_{ij}(0))
$$

- large intracluster correlation coefficient  $\rightsquigarrow$  more efficient
- large variation in  $p_a \rightarrow$  less efficient
- trade-off between detection of spillover effects and statistical efficiency

#### Complier Average Direct Effect

- Goal: Estimate the treatment effect rather than the ITT effect
- Use randomized encouragement as an instrument
	- **1** Monotonicity:  $D_{ii}(Z_{ii} = 1) > D_{ii}(Z_{ii} = 0)$  $\bullet$  Exclusion restriction:  $\ Y_{ij}(z_{ij},d_{ij})=Y_{ij}(z'_{ij},d_{ij})$  for any  $z_{ij}$  and  $z'_{ij}$
- **•** Generalization to the case with spillover effects **1** Monotonicity:  $D_{ii}(1, z_{-i,i}) > D_{ii}(0, z_{-i,i})$  for any  $z_{-i,i}$  $\bullet$  Exclusion restriction:  $\mathsf{Y}_{ij}(\mathsf{z}_j,\mathsf{d}_j)=\mathsf{Y}_{ij}(\mathsf{z}'_j,\mathsf{d}_j)$  for any  $\mathsf{z}_j$  and  $\mathsf{z}'_j$
- $\bullet$  Compliers:  $C_{ii}(z_{-i,j}) = 1\{D_{ii}(1, z_{-i,j}) = 1, D_{ii}(0, z_{-i,j}) = 0\}$
- Complier average direct effect of encouragement  $(CADE(z, a))$ :

$$
\frac{\sum_{j=1}^J\sum_{i=1}^{n_j}\left\{Y_{ij}(1,\mathbf{z}_{-i,j})-Y_{ij}(0,\mathbf{z}_{-i,j})\right\}C_{ij}(\mathbf{z}_{-i,j})\mathbb{P}_a(\mathbf{Z}_{-i,j}=\mathbf{z}_{-i,j} | Z_{ij}=z)}{\sum_{j=1}^J\sum_{i=1}^{n_j}C_{ij}(\mathbf{z}_{-i,j})\mathbb{P}_a(\mathbf{Z}_{-i,j}=\mathbf{z}_{-i,j} | Z_{ij}=z)}
$$

• We propose a consistent estimator of the CADE

### Key Identification Assumption

**•** Two causal mechanisms:

- $Z_{ii}$  affects  $Y_{ii}$  through  $D_{ii}$
- $Z_{ii}$  affects  $Y_{ii}$  through  $D_{-i,i}$
- $\bullet$  Idea: if  $Z_{ii}$  does not affect  $D_{ii}$ , it should not affect  $Y_{ii}$  through  $\mathbf{D}_{-i,i}$

# Assumption (Restricted Interference for Noncompliers)

If a unit has  $D_{ij}(1, \mathsf{z}_{-i,j}) = D_{ij}(0, \mathsf{z}_{-i,j}) = d$  for any given  $\mathsf{z}_{-i,j}$ , it must also satisfy  $Y_{ii}(d, D_{-i,j}(Z_{ii} = 1, z_{-i,j})) = Y_{ii}(d, D_{-i,j}(Z_{ii} = 0, z_{-i,j}))$ 

# Scenario I: No Spillover Effect of the Treatment Receipt on the Outcome

 $Y_{ij}(d_{ij}, \mathbf{d}_{-i,j}) = Y_{ij}(d_{ij}, \mathbf{d}'_{-i,j})$ 



# Scenario II: No Spillover Effect of the Treatment Assignment on the Treatment Receipt

 $D_{ij}(z_{ij}, \mathbf{z}_{-i,j}) = D_{ij}(z_{ij}, \mathbf{z}_{-i,j}^{\prime})$  (Kang and Imbens, 2016)



# Scenario III: Limited Spillover Effect of the Treatment Assignment on the Treatment Receipt

If 
$$
D_{ij}(1, \mathbf{z}_{-i,j}) = D_{ij}(0, \mathbf{z}_{-i,j})
$$
 for any given  $\mathbf{z}_{-i,j}$ ,  
then  $D_{i'j}(1, \mathbf{z}_{-i,j}) = D_{i'j}(0, \mathbf{z}_{-i,j})$  for all  $i' \neq i$ 



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#### Identification and Consistent Estimation

**1** Identification: monotonicity, exclusion restriction, restricted interference for noncompliers

$$
\lim_{n_j \to \infty} \text{CADE}(z, a) = \lim_{n_j \to \infty} \frac{\text{ADE}^Y(a)}{\text{ADE}^D(a)}
$$

<sup>2</sup> Consistent estimation: additional restriction on interference (e.g., Savje et al.)

$$
\frac{\widehat{ADE}^Y(a)}{\widehat{ADE}^D(a)} \xrightarrow{\rho} \lim_{n_j \to \infty, J \to \infty} CADE(z, a)
$$

#### Randomization Inference

• Variance is difficult to characterize

Assumption (**Stratified Interference** (Hudgens and Halloran. 2008))

$$
Y_{ij}(z_{ij}, \mathbf{z}_{-i,j}) = Y_{ij}(z_{ij}, \mathbf{z}_{-i,j}')
$$
 and 
$$
D_{ij}(z_{ij}, \mathbf{z}_{-i,j}) = D_{ij}(z_{ij}, \mathbf{z}_{-i,j}')
$$
 if 
$$
\sum_{i'=1}^{n_j} z_{ij} = \sum_{i=1}^{n_j} z_{ij}'
$$

Under stratified interference, our estimand simplifies to,

$$
\text{CADE}(a) \n= \frac{\sum_{j=1}^{J} \sum_{i=1}^{n_j} \{Y_{ij}(1,a) - Y_{ij}(0,a)\} \mathbf{1} \{D_{ij}(1,a) = 1, D_{ij}(0,a) = 0\}}{\sum_{j=1}^{J} \sum_{i=1}^{n_j} \mathbf{1} \{D_{ij}(1,a) = 1, D_{ij}(0,a) = 0\}}
$$

- Compliers:  $C_{ii} = \mathbf{1} \{ D_{ii}(1, a) = 1, D_{ii}(0, a) = 0 \}$
- Consistent estimation possible without additional restriction
- We propose an approximate asymptotic variance estimator

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#### Connection to the Two-stage Least Squares Estimator

• The model:

$$
Y_{ij} = \sum_{a=0}^{1} \alpha_a \mathbf{1} \{ A_j = a \} + \sum_{a=0}^{1} \beta_a D_{ij} \mathbf{1} \{ A_j = a \} + \epsilon_{ij}
$$
  

$$
D_{ij} = \sum_{a=0}^{1} \gamma_a \mathbf{1} \{ A_j = a \} + \sum_{a=0}^{1} \delta_a Z_{ij} \mathbf{1} \{ A_j = a \} + \eta_{ij}
$$

• Weighted two-stage least squares estimator:

$$
w_{ij} = \frac{1}{\Pr(A_j)\Pr(Z_{ij} | A_j)}
$$

- Transforming the outcome and treatment: multiplying them by  $n_j J/N$
- Randomization-based variance is equal to the weighted average of cluster-robust HC2  $\left(1-\frac{J_a}{J}\right)$  and individual-robust HC2 variances  $\left(\frac{J_a}{J}\right)$

#### Results: Indian Health Insurance Experiment

A household is more likely to enroll in RSBY if a large number of households are given the opportunity



Households will have greater hospitalization expenditure if few households are given the opportunity



# <span id="page-20-0"></span>Concluding Remarks

- In social science research,
	- **1** people interact with each other  $\rightsquigarrow$  interference
	- 2 people don't follow instructions  $\rightsquigarrow$  noncompliance
- Two-stage randomized controlled trials:
	- **1** randomize assignment mechanisms across clusters
	- <sup>2</sup> randomize treatment assignment within each cluster
- Spillover effects as causal quantities of interest
- **Qur contributions:** 
	- **1** Identification condition for complier average direct effects
	- Consistent estimator for CADE and its variance
	- <sup>3</sup> Connections to regression and instrumental variables
	- <sup>4</sup> Application to the India health insurance experiment
	- **5** Implementation as part of R package experiment

Send comments and suggestions to Imai@Harvard.Edu Other research at <https://imai.fas.harvard.edu>

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