

# Robust Designs for Prospective Randomized Trials Surveying Sensitive Topics

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August 8, 2022

# Outline

- 1 Background and Motivation
- 2 Results
- 3 Power and Sample Size Analysis
- 4 Methods in Context

# A Cluster-Randomized Trial in Nairobi, Kenya

- Project emerged out of a **cluster-randomized trial** seeking to reduce sexual violence among adolescents in Nairobi, Kenya
  - 4,100 sixth grade girls
  - Randomized treatment among 94 schools
  - Two years of follow-up (2016 to 2018)

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  - Empowerment, situational awareness, verbal skills, self-defense

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  - Empowerment, situational awareness, verbal skills, self-defense
- **Outcome:** incidence of sexual violence in prior 12 months

# The Setting



# Measurement Tool

- Due to size of study, outcome and covariate data was **self-reported** via survey
- Several efforts made to improve data collection quality
  - Multiple languages
  - Colloquial phrasing
  - Privacy maintenance

10. Is it okay to use force and even injure anyone who is close to me if he is forcing me to have sex and will not listen to me (e.g. brother, boyfriend, father, cousin)? ☒ Yes ☐ No

11. Have you ever been forced against your will to have sex (penetration of your vagina, anus or mouth with a penis or another object)? ☒ YES ☐ NO

a. How many times? A. 1 Time B. 2 Times C. 3 Times D. 4 Times E. Never ☒ Other \_\_\_\_\_

b. Who has ever forced you to have sex (mark all that apply)? ☒ RELATIVE B. NEIGHBOUR C. STEPPATHER/FATHER D. BROTHER E. NEVER FORCED F. STRANGER G. TEACHER H. PASTOR I. GANGSTER J. POLICE K. DOCTOR L. FRIEND M. BOYFRIEND N. IMAM O. OTHER RELATIVE

c. Did you tell anyone about it? A. YES ☒ NO C. NEVER FORCED

d. If yes, where have you ever told (mark all that apply)? A. FRIEND B. NEIGHBOUR ☒ C. RELATIVE D. TEACHER E. PASTOR F. POLICE G. DOCTOR H. NEVER FORCED I. BOYFRIEND J. IMAM K. OTHER \_\_\_\_\_

12. In the last one year has anyone forced you against your will to have sex (penetration of your vagina, anus or mouth with a penis or another object)? A. YES ☒ NO

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13. What is the name of your apocynity village? Kajulu in Kisumu

14. What was the name of your first primary school? C.M.H.D.C. Primary

15. The first letter of your first (Christian/Muslim) name lies between? ☒ A-G ☒ H-N ☐ O-T ☐ U-Z

16. What is the third letter of your first (Christian/Muslim) name? T

# Motivating Questions

Results from the study showed **no protective effect** of treatment vs. standard of care

**Repeated critique:** misreporting error attenuated causal estimates toward zero

- Survey responses may be erroneous due to fear, shame, etc.
- Old canard: measurement error biases results toward the null



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## Research questions

- 1 With binary outcomes, how does non-differential\* misreporting error affect the difference-in-means causal estimator?
- 2 How can a researcher can power a study with misreporting?

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\*Meaning: misreporting behavior not influenced by the treatment itself

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# Running Example

Randomized controlled trial involving  $i = 1, \dots, 2n$  individuals.

Students randomized to receive either a **violence prevention program** (“intervention”) or an unrelated training (“control”).

Goal is to reduce students’ experience of violence.

Outcome is binary:

- “yes, I experienced violence in the prior 12 months” ( $= 1$ )
- “no, I didn’t experience violence in the prior 12 months” ( $= 0$ )

# Outcome Definitions

- Each individual  $i$  has two potential outcomes (Rubin, 1974)
  - $Y_i(1) \in \{0, 1\}$ , the outcome if treated
  - $Y_i(0) \in \{0, 1\}$ , the outcome if given the control

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- Denote as  $Y_i^{(t)} \in \{0, 1\}$  the realized, **true outcome** experienced by individual  $i$ . Follows definition

$$Y_i^{(t)} = W_i Y_i(1) + (1 - W_i) Y_i(0).$$

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- Denote as  $Y_i^{(r)} \in \{0, 1\}$ , as the **reported outcome** for individual  $i$ . In our setting, possible that  $Y_i^{(r)} \neq Y_i^{(t)}$ .

# Reporting Classes

Reporting class	$Y_i^{(r)}$ when $Y_i^{(t)} = 0$	$Y_i^{(r)}$ when $Y_i^{(t)} = 1$
True ( $T_i$ )	0	1
<del>False (<math>F_i</math>)</del>	<del>1</del>	<del>0</del>
Never ( $N_i$ )	0	0
Always ( $A_i$ )	1	1

Table 1: Reporting behavior for each of the four reporting classes.

Indicators  $T_i, N_i, A_i \in \{0, 1\}$  satisfy

$$T_i + N_i + A_i = 1$$

for all  $i \in 1, \dots, 2n$ .

# Response Classes

Response class	$Y_i(0)$	$Y_i(1)$
Decrease ( $D_i$ )	1	0
Increase ( $I_i$ )	0	1
Unsusceptible ( $U_i$ )	0	0
Predisposed ( $P_i$ )	1	1

Table 2: Potential outcomes for each of the four response classes.

See discussion in [Hernán and Robins \(2010\)](#).

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for all  $i \in 1, \dots, 2n$ .



# Joint Distribution of Class Types

	Y(0)	Y(1)	True- rep.	Always- rep.	Never- rep.	False- rep.	
<b>Decrease</b>	1	0	$\overline{TD}$	$\overline{AD}$	$\overline{ND}$	x	$\overline{D}$
<b>Increase</b>	0	1	$\overline{TI}$	$\overline{AI}$	$\overline{NI}$	x	$\overline{I}$
<b>Unsusceptible</b>	0	0	$\overline{TU}$	$\overline{AU}$	$\overline{NU}$	x	$\overline{U}$
<b>Predisposed</b>	1	1	$\overline{TP}$	$\overline{AP}$	$\overline{NP}$	x	$\overline{P}$
			$\overline{T}$	$\overline{A}$	$\overline{N}$		

Table 3: Population proportions across response and reporting classes.

# Bias Results (I)

- The  $2n$  RCT units are assumed sampled from a (large) super-population of  $N_{\text{sp}}$  units.  $n$  units treated,  $n$  units control
- We consider estimating the super-population treatment effect

$$\tau = \frac{1}{N_{\text{sp}}} \sum_{i=j}^{N_{\text{sp}}} Y_j(1) - Y_j(0)$$

using the difference-in-means estimator,

$$\hat{\tau} = \frac{1}{n} \sum_{i=1}^{2n} Y_i^{(r)} W_i - \frac{1}{n} \sum_{i=1}^{2n} Y_i^{(r)} (1 - W_i).$$

# Bias Results (II)

## Theorem (Bias of Difference-in-Means Estimator)

Define  $\tau_i = Y_i(1) - Y_i(0)$  for  $i = 1, 2, \dots, N_{sp}$ . The bias of the difference-in-means estimator in estimating  $\tau$  is given by

$$\begin{aligned}\text{Bias}(\hat{\tau}) &= -(\bar{A} + \bar{N})\tau - \text{cov}(A, \tau) - \text{cov}(N, \tau) \\ &= -\bar{NI} + \bar{ND} - \bar{AI} + \bar{AD}\end{aligned}$$

where  $\text{cov}(A, \tau)$  is the super-population covariance between  $A_i$  and  $\tau_i$ , and  $\text{cov}(N, \tau)$  defined analogously.

# Bias and Power Under Independence

As a direct corollary of Theorem 1, we see that if  $A_i, N_i \perp\!\!\!\perp \tau_i$ , then our estimate will be shrunk multiplicatively toward 0:

$$\frac{\mathbb{E}(\hat{\tau})}{\tau} = 1 - \bar{A} - \bar{N} \quad \text{under independence.}$$

## Theorem

*Suppose  $\tau \neq 0$  and, in the super-population,  $A_i, N_i \perp\!\!\!\perp \tau_i$ . Then, the detection power is a strictly decreasing function of  $\bar{A}$  and  $\bar{N}$ .*

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- No misreporting among Predisposed or Immune.
- In this case, Never-reporters would be more prevalent among those responsive to treatment  $\Rightarrow$  bigger bias!

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## Definition

Under sensitivity model indexed by  $\Gamma \geq 1$ , every subgroup proportion in the joint distribution table differs by a multiplicative factor no greater than  $\Gamma$  from the proportion under row-column independence. For example,

$$\frac{1}{\Gamma} \leq \frac{\overline{TD}}{\overline{T} \cdot \overline{D}} \leq \Gamma$$

with analogous bounds for other entries in joint incidence table.

# Joint Distribution of Class Types

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Table 4: Population proportions across response and reporting classes.

Sensitivity model bounds the **level of deviation** from row/column independence in this table.

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- User inputs (ideally derived from high-quality pilot study!)
  - Type I error bound  $\alpha$  and Type II error bound  $\beta$   
 $\Rightarrow$  power is  $1 - \beta$
  - Estimates of  $\pi_{\text{rep}} = (\bar{T}, \bar{A}, \bar{N})$  and  $\pi_{\text{res}} = (\bar{D}, \bar{I}, \bar{U}, \bar{P})$
  - Worst-case independence deviation  $\Gamma \geq 1$

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  - Estimates of  $\pi_{\text{rep}} = (\overline{T}, \overline{A}, \overline{N})$  and  $\pi_{\text{res}} = (\overline{D}, \overline{I}, \overline{U}, \overline{P})$
  - Worst-case independence deviation  $\Gamma \geq 1$
- Optimization variable  $\delta$  is population proportion table.  
Expected test statistic can be written as

$$t(\pi_{\text{rep}}, \pi_{\text{res}}, \delta, n)$$



# Worst-Case Sample-Size Calculations (III)

Obtain sample size by solving Optimization Problem 1:

$$\begin{aligned} \underset{n}{\operatorname{argmin}} \quad & \max_{\delta} \quad t(\pi_{\text{rep}}, \pi_{\text{res}}, \delta, n) \\ \text{subject to} \quad & \Phi(\Phi^{-1}(\alpha) - t(\pi_{\text{rep}}, \pi_{\text{res}}, \delta, n)) \geq 1 - \beta, \\ & \delta \mathbb{1}_3 = \pi_{\text{rep}}^{\top}, \\ & \delta^{\top} \mathbb{1}_4 = \pi_{\text{rep}}^{\top}, \\ & \frac{1}{\Gamma} \leq \delta / \left( \pi_{\text{res}}^{\top} \pi_{\text{rep}} \right) \leq \Gamma, \end{aligned} \tag{1}$$

where  $\Phi(\cdot)$  is the CDF of a standard normal, and  $\mathbb{1}_c$  is the length- $c$  vector containing all ones.

This is a quadratic fractional programming problem, and can be solved by **Dinkelbach's Method**.

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- [Baiocchi et al. \(2019\)](#) estimated the annual baseline rate of sexual violence in this population to be approximately 7%
- Expectation from a pilot study: 50% reduction in sexual violence (we now know effect is null)
- Under  $\alpha = 0.05$  and  $\beta = 0.2$ , a standard power analysis would estimate we would need to recruit 998 girls to be in our study

## Posited parameter values

- Never-reporters expected in study population
  - Girls may experience shame or confusion
  - Robust literature (Cook et al., 2011; Fisher and Cullen, 2000).

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$$0 \leq \overline{N} \leq 0.20 \quad \text{and} \quad 1 \leq \Gamma \leq 2$$

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Always-reporters and False-reporters are assumed absent.

- We consider a grid of possible values

$$0 \leq \bar{N} \leq 0.20 \quad \text{and} \quad 1 \leq \Gamma \leq 2$$

- Under the assumption that  $\bar{T} = 0$  (i.e., no one is harmed by the treatment), we have:

$$\bar{D} = 0.035, \quad \bar{P} = 0.035, \quad \text{and} \quad \bar{U} = 0.930$$



# Results

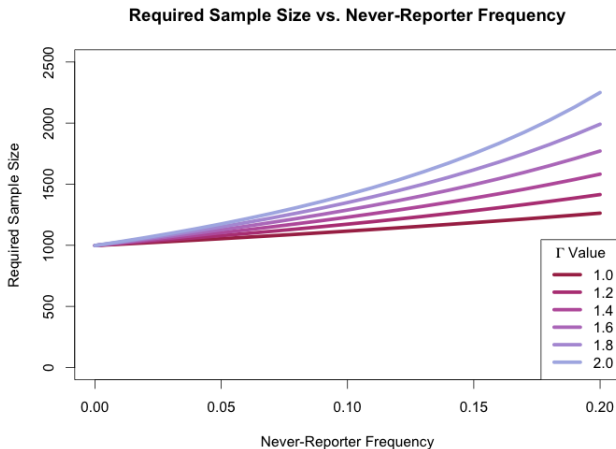


Figure 1: Required sample size by Never-reporter frequency.

# What's Going On?

- $\Gamma = 1$  : sample size grows slowly with Never-reporter frequency
- As  $\Gamma$  gets larger, the algorithm allows for more **adversarial** allocations of the population proportions
  - Allocates as much of the population as possible to the  $\overline{ND}$  subgroup, subject to  $\Gamma$  constraint
  - Yields the smallest possible causal estimate
- Hence, when  $\overline{N} = 20\%$  and  $\Gamma = 2$ , the required sample size is nearly twice as large as the case when  $\Gamma = 1$ .

# Wrap-Up

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- Key contributions
  - Demonstrate that **joint distribution** of reporting and response classes characterizes the bias (and variance) of the difference-in-means estimator
  - Proposed a method for practitioners to identify adequate **sample sizes** in presence of misreporting

# Wrap-Up

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- Key contributions
  - Demonstrate that **joint distribution** of reporting and response classes characterizes the bias (and variance) of the difference-in-means estimator
  - Proposed a method for practitioners to identify adequate **sample sizes** in presence of misreporting
- **Takeaways**
  - The time to think about misreporting error is the **design phase** of the experiment!
  - Some error can be mitigated through **careful design choices**.
  - Some may only be addressable by recruiting more participants

# Thanks!

Thanks to Mike & Rina!

arXiv: 2108.08944

Forthcoming in *AJE*.

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# Proof of Theorem 1

Proof.

Define

$$Y_i^{(r)}(1) = (1 - N_i)(1 - A_i)Y_i(1) + A_i,$$

$$Y_i^{(r)}(0) = (1 - N_i)(1 - A_i)Y_i(0) + A_i$$

s.t.  $Y_i^{(r)} = W_i Y_i^{(r)}(1) + (1 - W_i) Y_i^{(r)}(0)$ . Now, proceed as normal!

$$\begin{aligned} \mathbb{E}(\hat{\tau}) &= \mathbb{E}_R \left( \mathbb{E}_W \left( \frac{1}{n} \sum_{i=1}^{N_{\text{sp}}} Y_i^{(r)} W_i R_i - \frac{1}{n} \sum_{i=1}^{N_{\text{sp}}} Y_i^{(r)} (1 - W_i) R_i \mid \{R_i\}_{i=1}^{N_{\text{sp}}} \right) \right) \\ &= \mathbb{E}_R \left( \frac{1}{2n} \left( \sum_{i=1}^{N_{\text{sp}}} R_i (Y_i^{(r)}(1) - Y_i^{(r)}(0)) \right) \right) \\ &= \frac{1}{N_{\text{sp}}} \sum_{i=1}^{N_{\text{sp}}} (1 - N_i)(1 - A_i) \tau_i = \frac{1}{N_{\text{sp}}} \sum_{i=1}^{N_{\text{sp}}} (1 - N_i - A_i) \tau_i \\ &= (1 - \bar{N} - \bar{A}) \tau - \text{cov}(N, \tau) - \text{cov}(A, \tau). \end{aligned}$$





# Our Approach (I)

- Model each respondent as a member of a **reporting class**, defining how the individual reports realized outcomes
  - True-reporter
  - False-reporter
  - Never-reporter
  - Always-reporter
- Also invoke notion of a **response class** (Hernán and Robins, 2010), defining how each individual responds to the treatment
  - Decrease
  - Increase
  - Unsusceptible
  - Presdisposed

## Our Approach (II)

- Show the **joint distribution** of reporting classes and response classes exactly characterizes error terms for a causal estimate (but not just the marginal distribution of reporting classes).
- Propose a novel minimax procedure to obtain adequately powered experiments in presence of misreporting.

# Explanations for Misreporting Behavior

Why might someone be a Never-reporter?

- Fear of negative consequences if they report a violent event
- Feelings of shame for having been a victim of violence
- Confusion about what constitutes violence

How can researchers mitigate misreporting in the design phase?

- Use a technique like “randomized response,” which offers a higher degree of anonymity
- Word questions to avoid triggers of shame
- Use detailed descriptive scenarios; use specific physical and verbal acts; or use common slang terms more familiar to students to clarify what constitutes “violence”

# Sampling Mechanism

- Suppose our  $2n$  units for the RCT are sampled from a very large super-population of  $N_{\text{sp}}$  units
- Once sampled,  $n$  units selected via simple random sample to receive the intervention
- Target of estimation is the super-population treatment effect,

$$\tau = \frac{1}{N_{\text{sp}}} \sum_{i=1}^{N_{\text{sp}}} Y_i(1) - Y_i(0).$$

- $R_i \in \{0, 1\}$ : indicator of being sampled into RCT.  
 $W_i \in \{0, 1\}$ : treatment indicator. Assume  $R_i \perp\!\!\!\perp W_j$ .
- Expectation of any estimator  $\phi$  is defined as

$$\mathbb{E}(\phi) = E_R \left( E_W \left( \phi \mid \{R_i\}_{i=1}^{N_{\text{sp}}} \right) \right).$$

# Response Classes

Define binary indicators  $D_i, I_i, U_i, P_i \in \{0, 1\}$  reflecting whether each individual  $i$  falls into the Decrease, Increase, Unsusceptible, or Predisposed response classes.

Every individual belongs to exactly one class, so

$$D_i + I_i + U_i + P_i = 1 \text{ for all } i.$$

# Reporting Classes

- Assume there are no False-reporters
- Define two fixed, binary constants,

$$N_i = \begin{cases} 1 & \text{if } i \text{ is a Never-reporter} \\ 0 & \text{otherwise} \end{cases}$$

$$A_i = \begin{cases} 1 & \text{if } i \text{ is an Always-reporter} \\ 0 & \text{otherwise} \end{cases}$$

where  $N_i + A_i \leq 1$  and  $N_i = A_i = 0$  signifies that someone is a True-reporter

- We can express the reported outcome as

$$Y_i^{(r)} = (1 - N_i)(1 - A_i) (W_i Y_i(1) + (1 - W_i) Y_i(0)) + A_i.$$

# Worst-Case Sample-Size Calculations (II)

Define optimization variables:

$$\delta = \begin{pmatrix} \overline{TD} & \overline{AD} & \overline{ND} \\ \overline{TI} & \overline{AI} & \overline{NI} \\ \overline{TU} & \overline{AU} & \overline{NU} \\ \overline{TP} & \overline{AP} & \overline{NP} \end{pmatrix}.$$

Expected test statistic can be written as a function of  $\delta$  and  $\pi = (\pi_{\text{rep}}, \pi_{\text{res}})$ :

$$t(\pi, \delta) = n \cdot \frac{\mu_1(\pi, \delta) - \mu_0(\pi, \delta)}{\mu_1(\pi, \delta) \cdot (1 - \mu_1(\pi, \delta)) + \mu_0(\pi, \delta) \cdot (1 - \mu_0(\pi, \delta))}.$$

where

$$\begin{aligned} \mu_1(\pi, \delta) &= \overline{I} + \overline{P} - \overline{NI} - \overline{NP} + \overline{AD} + \overline{AU}, \\ \mu_0(\pi, \delta) &= \overline{D} + \overline{P} - \overline{ND} - \overline{NP} + \overline{AI} + \overline{AU}. \end{aligned}$$