

An Applied Researcher's Guide to ITT Effects from Multisite (blocked) Individually Randomized Trials: Estimands, Estimators, and Estimates

UC Berkeley Simons Institute – Feb, 2022

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Thanks to Mike Weiss for
making most of these slides for
an initial presentation

I'm planning an evaluation that will randomize 6,000 people within 20 sites. Can you write the part of the analysis plan that describes the estimation model for the overall average ITT effect?



ALEX

O.K.

Why don't
you do
your own
job?

**My collaborator, who
initiated this adventure** →



MIKE

Here's the estimator I have in mind:

$$Y = \sum_{j=1}^{20} \alpha_j * Site_j + \beta * T + \varepsilon$$

Where:

Y = Outcome of interest

$Site_j$ = Set to 1 if person was at site j and 0 otherwise

T = Set to 1 if person assigned to treatment and 0 otherwise

ε = error, assumed i.i.d. normal



ALEX

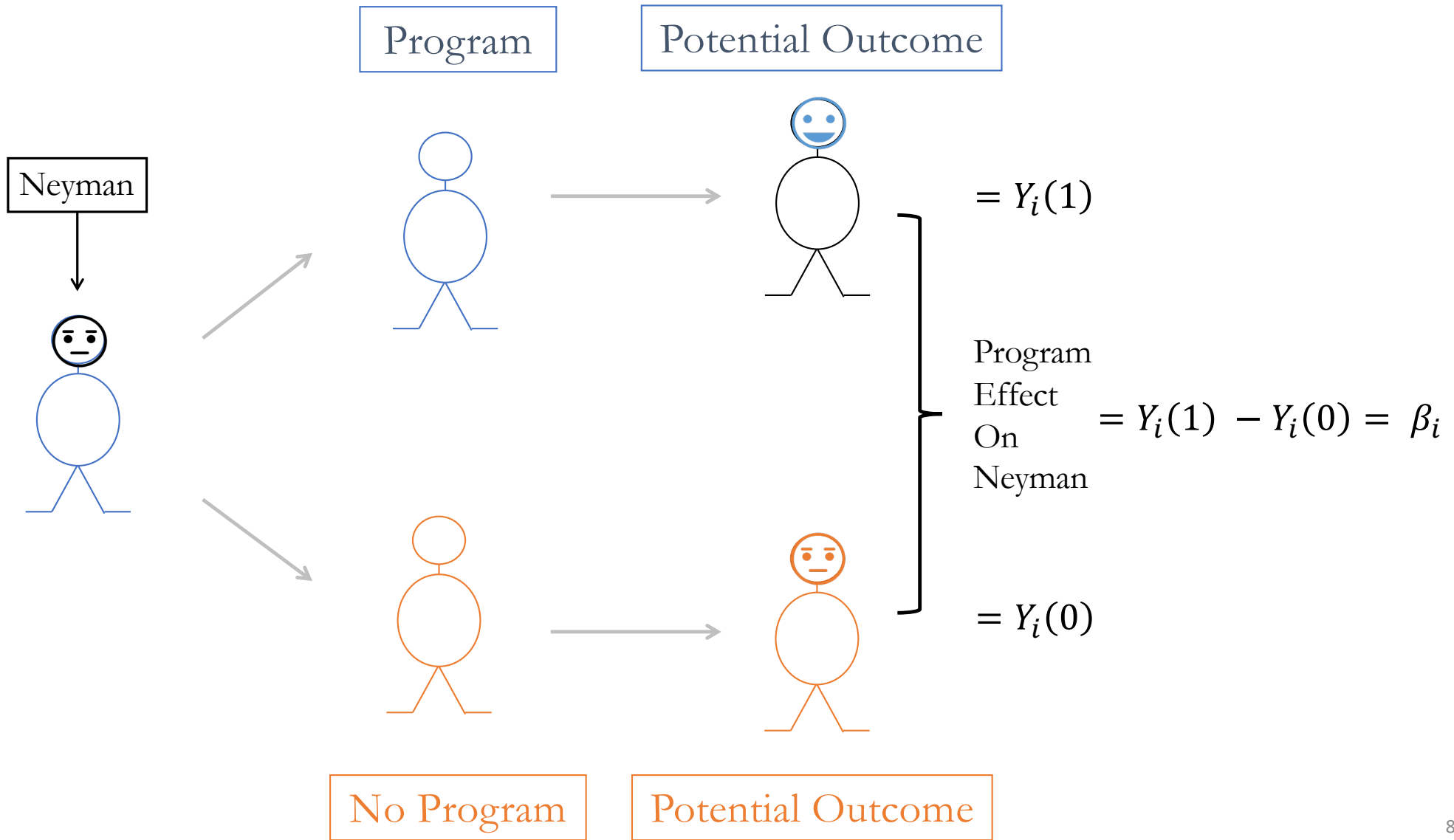


ESTIMAND

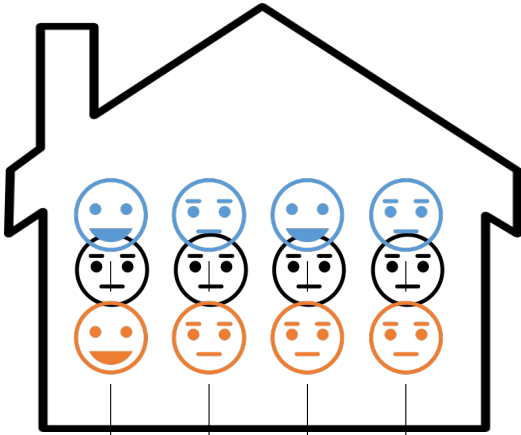
What is the target of inference?



Program Effect for a Person



Average Program Effect for a Group of People at a Site



$$N_j = \sum_{i=1}^{N_j} 1 = \text{# of persons at site } j$$

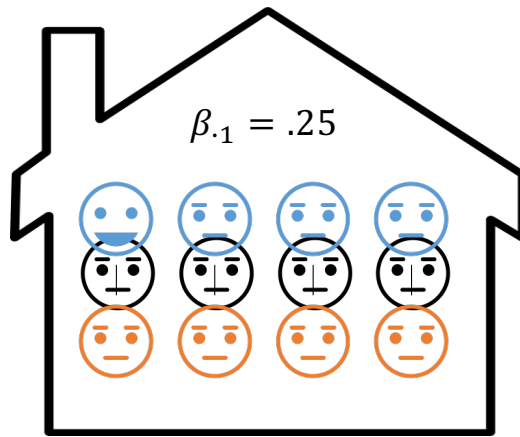
$$= \frac{1}{4} * 0 + \frac{1}{4} * 0 + \frac{1}{4} * 1 + \frac{1}{4} * 0 = 0.25$$

Where:

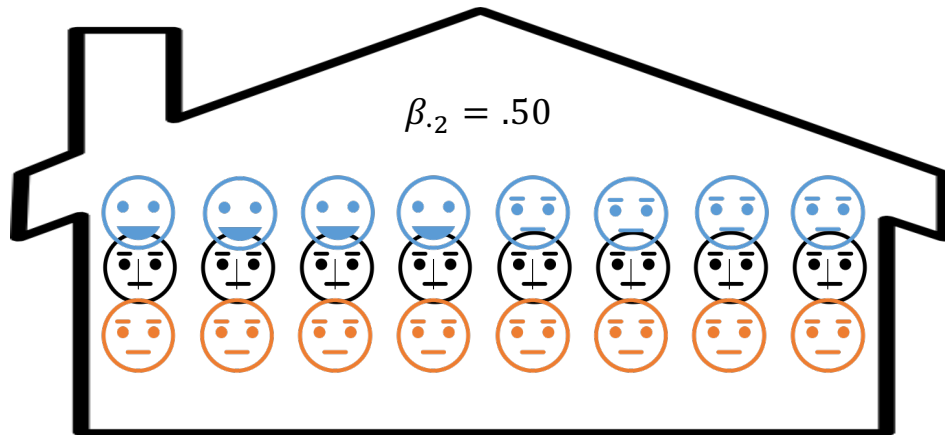
β_j = average ITT effect at site j

β_{ij} = ITT effect for person i at site j

Estimand: Effect for Average Person or Site?



$$\beta_{Person} = 0.416$$



$$\beta_{Site} = 0.375$$

Estimand: Effect for Average Person or Site?

Target Unit	Person	$\beta_{person} = \sum_{j=1}^J \frac{N_j}{N} \beta_{.j}$
	Site	$\beta_{site} = \sum_{j=1}^J \frac{1}{J} \beta_{.j}$

Where:

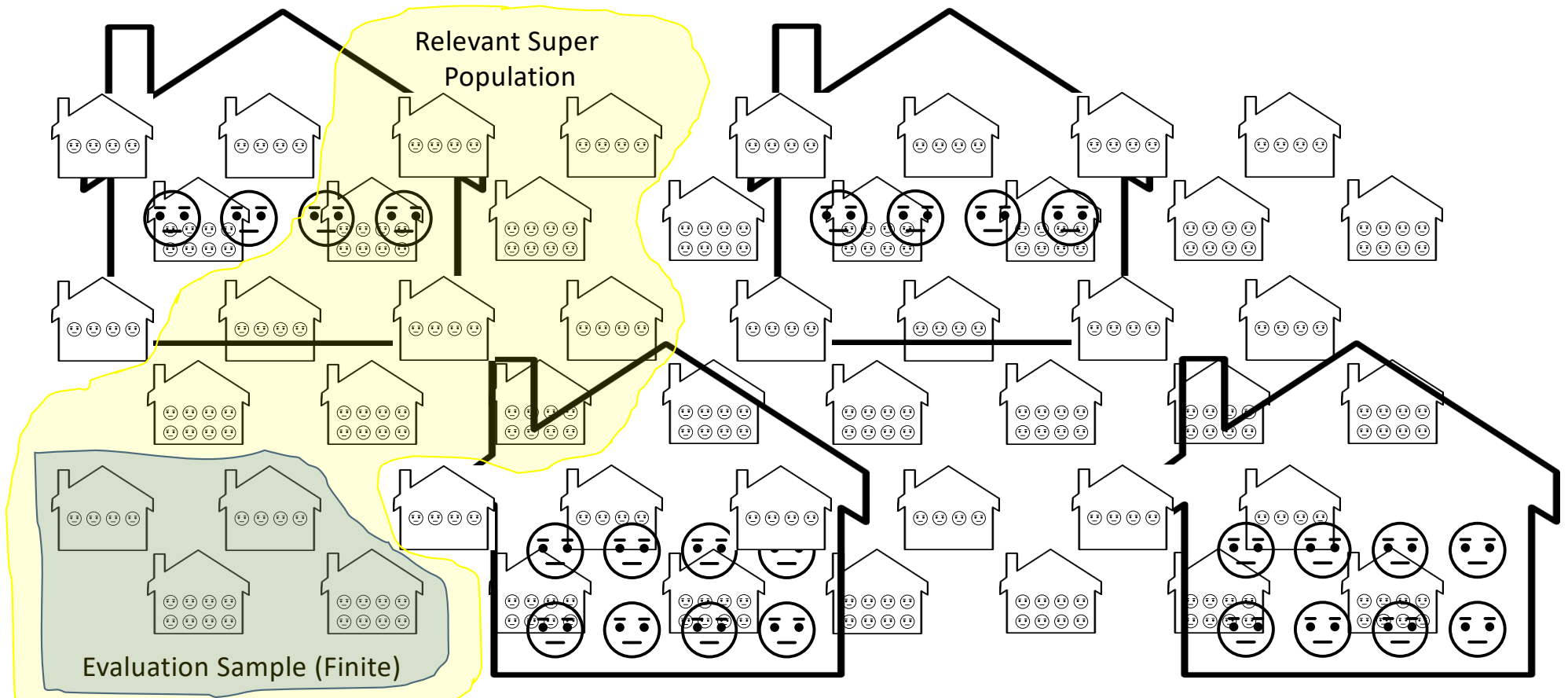
J = # of sites in study

N = total # of persons

N_j = # of persons at site j

$\beta_{.j}$ = average ITT effect at site j

Estimand: Effect for Finite or Super Population?

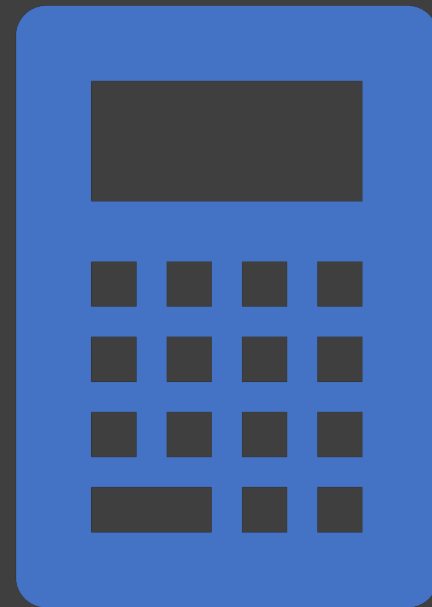


Four Estimands

		Target Population	
		Finite	Super
Target Unit	Person	$\beta_{FP-person} = \sum_{j=1}^J \frac{N_j}{N} \beta_{.j}$	$\beta_{SP-person} = \sum_{j=1}^{J^*} \frac{N_j^*}{N^*} \beta_{.j}^*$
	Site	$\beta_{FP-site} = \sum_{j=1}^J \frac{1}{J} \beta_{.j}$	$\beta_{SP-site} = \sum_{j=1}^{J^*} \frac{1}{J^*} \beta_{.j}^*$

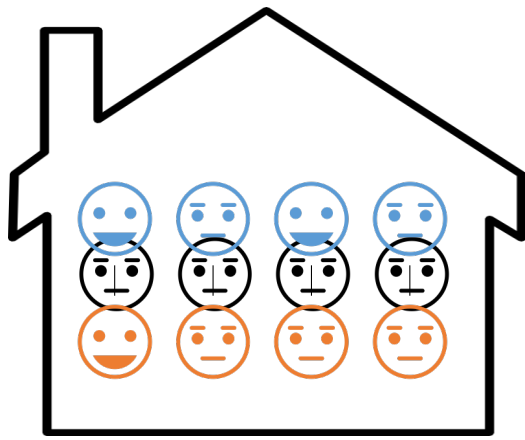
ESTIMATORS

Rule for calculating an
estimate based on
observed data



Convenient way to describe estimators of β

$$\hat{\beta} = \sum_{j=1}^J w_j * \hat{\beta}_{.j}$$



$\hat{\beta}_j = \text{estimated average ITT effect at site } j = 0.66$

(simple difference-in-means estimator)

Classes of Estimators

1. Design Based
2. Linear Regression
3. Multilevel Modeling

1. Design-based Estimators (4 of them)

Analytic Technical Assistance and Development

Statistical Theory for the RCT-YES Software: Design-Based Causal Inference for RCTs

Peter Z. Schochet

Mathematica Policy Research, Inc.

Second Edition: March 2016

 NATIONAL CENTER FOR
EDUCATION EVALUATION
AND REGIONAL ASSISTANCE
Institute of Education Sciences
U.S. Department of Education

Some nice features of design-based estimators

- Simple
- Clear connection to estimands
- Unbiased
- Specialized software designed for RCTs and for easy use

Design-based estimators of β

Name	Estimator	w_j	Estimand
Design Based – person $\hat{\beta}_{DB-person}$	$\sum_{j=1}^J \frac{N_j}{N} \hat{\beta}_{.j}$	$w_j \propto N_j$	β_{person}
Design Based – site $\hat{\beta}_{DB-site}$	$\sum_{j=1}^J \frac{1}{J} \hat{\beta}_{.j}$	$w_j \propto 1$	β_{site}


Where:

N_j = number of people at site j in sample

N = number of people in full sample

$\hat{\beta}_{.j}$ = *estimated* average ITT effect at site j (simple difference-in-means estimator)

J = number of sites in sample



2. Linear
Regression
(8 of them)

- See lots of text books

The linear regression estimators

	Our Name	Model	w_j	Estimand
Fixed Effects	(1) Fixed Effects (FE) $\hat{\beta}_{FE}$	$Y = \sum_{j=1}^J \alpha_j * Site_j + \beta * T + \varepsilon$	$w_j \propto N_j T_j (1 - T_j)$	$\beta_{FP-person}$
	(2) FE - heteroskedastic robust $\hat{\beta}_{FE-Het}$	"	"	$\beta_{FP-person}$
	(3) FE - cluster robust $\hat{\beta}_{FE-CR}$	"	"	$\beta_{SP-person}$
	(4) FE - club sandwich $\hat{\beta}_{Club}$	"	"	$\beta_{SP-person}$
Weighted Regression	(5) FE - person-weights $\hat{\beta}_{FE-weight-person}$	" $\omega_{ij}^{person} = T_{ij} \left(\frac{T_{..}}{T_{.j}} \right) + (1 - T_{ij}) \left(\frac{1 - T_{..}}{1 - T_{.j}} \right)$	$w_j \propto N_j$	$\beta_{FP-person}$
	(6) FE - site-weights $\hat{\beta}_{FE-weight-site}$	" $\omega_{ij}^{site} = \left[T_{ij} \left(\frac{T_{..}}{T_{.j}} \right) + (1 - T_{ij}) \left(\frac{1 - T_{..}}{1 - T_{.j}} \right) \right] \left[\frac{N}{N_j} \right]$	$w_j \propto 1$	$\beta_{FP-site}$
Fully Interacted	(7) FE - w/ interactions - person $\hat{\beta}_{FE-inter-person}$	$Y = \sum_{j=1}^J \alpha_j * Site_j + \sum_{j=1}^J \beta_j * Site_j * T + \varepsilon$	$w_j \propto N_j$	$\beta_{FP-person}$
	(8) FE - w/ interactions - site $\hat{\beta}_{FE-inter-site}$	"	$w_j \propto 1$	$\beta_{FP-site}$

3. Multilevel Models (3 of them)



Journal of Research on Educational Effectiveness

ISSN: 1934-5747 (Print) 1934-5739 (Online) Journal homepage: <http://www.tandfonline.com/loi/uree20>

Using Multisite Experiments to Study Cross-Site Variation in Treatment Effects: A Hybrid Approach With Fixed Intercepts and a Random Treatment Coefficient

Howard S. Bloom, Stephen W. Raudenbush, Michael J. Weiss & Kristin Porter

The multilevel model estimators

Our Name	Model	w_j	Estimand
1. Fixed Intercepts Random Treatment Coefficient (FIRC) $\hat{\beta}_{ML-FIRC}$	<p><u>Level 1:</u> $Y_{ij} = \alpha_{.j} + \beta_{.j}T_{ij} + e_{ij}$</p> <p><u>Level 2:</u> $\alpha_{.j} = \alpha_{.j}$ $\beta_{.j} = \beta + b_j$</p> <p>Where: $e_{ij} \sim N(0, \sigma^2)$ $b_j \sim N(0, \tau_b^2)$</p>	$w_j \propto \left[\hat{\tau} + \frac{\hat{\sigma}^2}{N_j T_{.j} (1 - T_{.j})} \right]^{-1}$	$\beta_{SP-site}$
2. Random Intercept Random Treatment Coefficient (RIRC) $\hat{\beta}_{ML-RIRC}$	<p><u>Level 1:</u> $Y_{ij} = \alpha_{.j} + \beta_{.j}T_{ij} + e_{ij}$</p> <p><u>Level 2:</u> $\alpha_{.j} = \alpha + a_j$ $\beta_{.j} = \beta + b_j$</p> <p>Where: $e_{ij} \sim N(0, \sigma^2)$ $\begin{pmatrix} a_j \\ b_j \end{pmatrix} \sim N \left[\begin{pmatrix} 0 \\ 0 \end{pmatrix}, \begin{pmatrix} \tau_a^2 & \tau_{ab} \\ \tau_{ab} & \tau_b^2 \end{pmatrix} \right]$</p>	Unsure	$\beta_{SP-site}$
2. Random intercept, constant coefficient ($\hat{\beta}_{ML-RICC}$)	<p><u>Level 1:</u> $Y_{ij} = \alpha_{.j} + \beta T_{ij} + e_{ij}$</p> <p><u>Level 2:</u> $\alpha_{.j} = \alpha + a_j$</p> <p>Where: $\alpha_j \sim N(0, \tau_\alpha^2)$</p>	Basically like fixed effects model	<i>Surprise!</i> $\beta_{FP-person}$

ESTIMATES



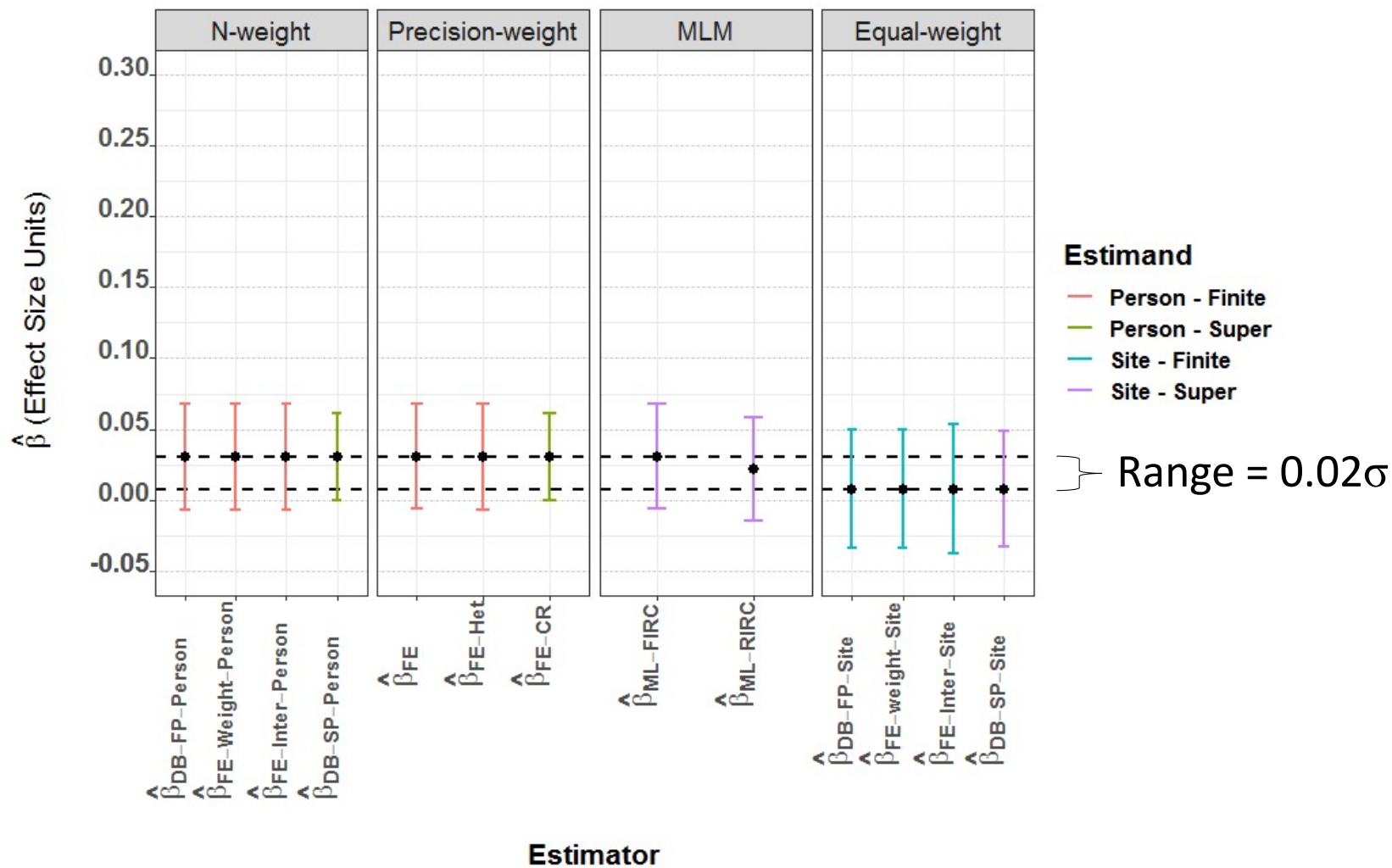
The 12 Studies

Early Childhood-Element. School	Middle School-High School	Post-secondary Education	Labor Market Programs
Head Start Impact Study mdrc	Enhanced Reading Opportunity mdrc	Learning Communities mdrc	Welfare-to-Work Programs mdrc
After School – Reading Program mdrc	Career Academies mdrc	Performance-based Scholarships mdrc	
After School – Math Program mdrc	Communities in Schools	Encouraging Summer Enrollment (1) mdrc	
	Early College H.S.	Encouraging Summer Enrollment (2) mdrc	

RQ1:

Does choice of estimand/estimator matter for the estimate of β ?

Learning Communities - Total Credits, 3 Sem

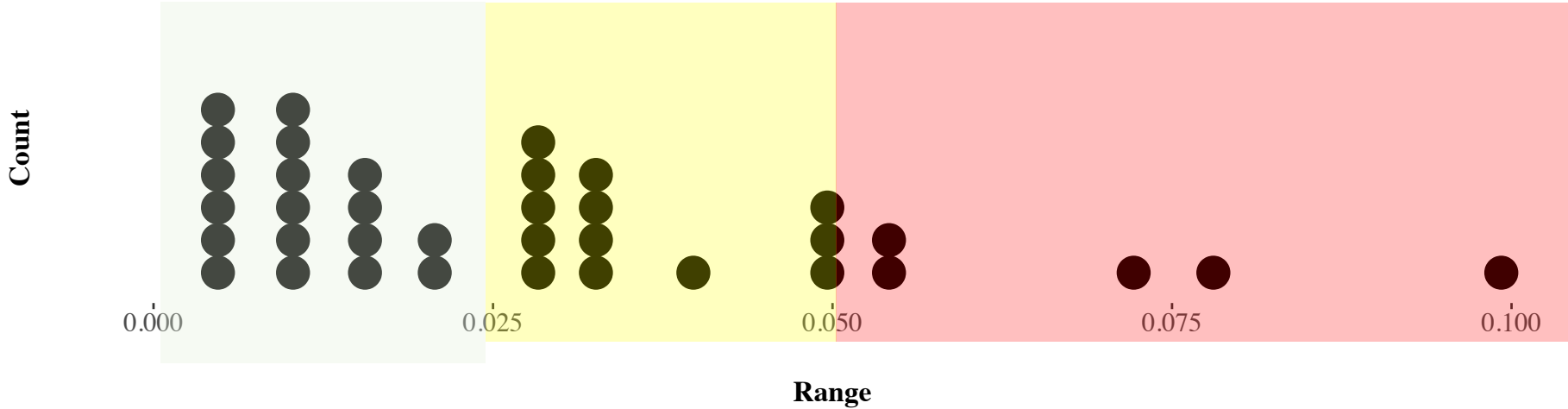


Meh

OK, I'm paying attention

Yikes?

Range of Estimates across all Estimands

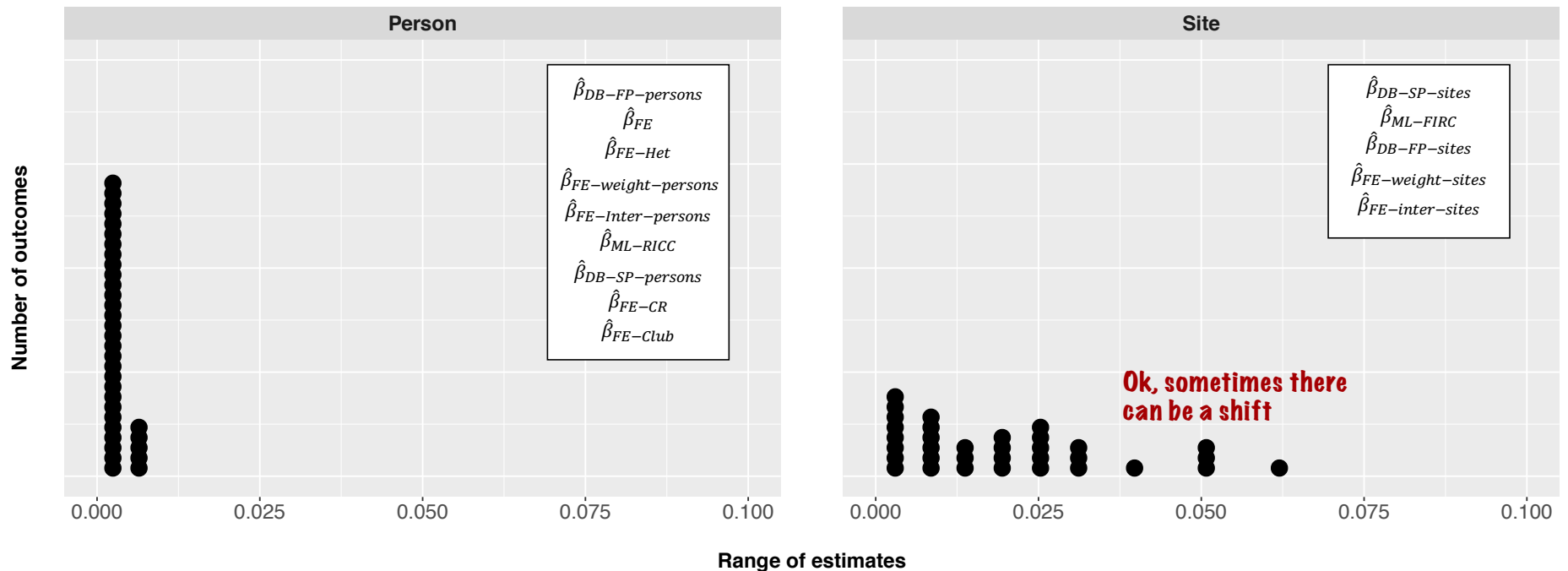


Impacts in effect size units.

Notes: Each dot represents a single outcome for a single study.

The x-axis is the range of point estimates ($\max[\hat{\beta}] - \min[\hat{\beta}]$) across all 14 estimators, in effect size units.

Group estimators by whether they are person or site targeting



The person weighting ones are all basically the same. Site, less so.

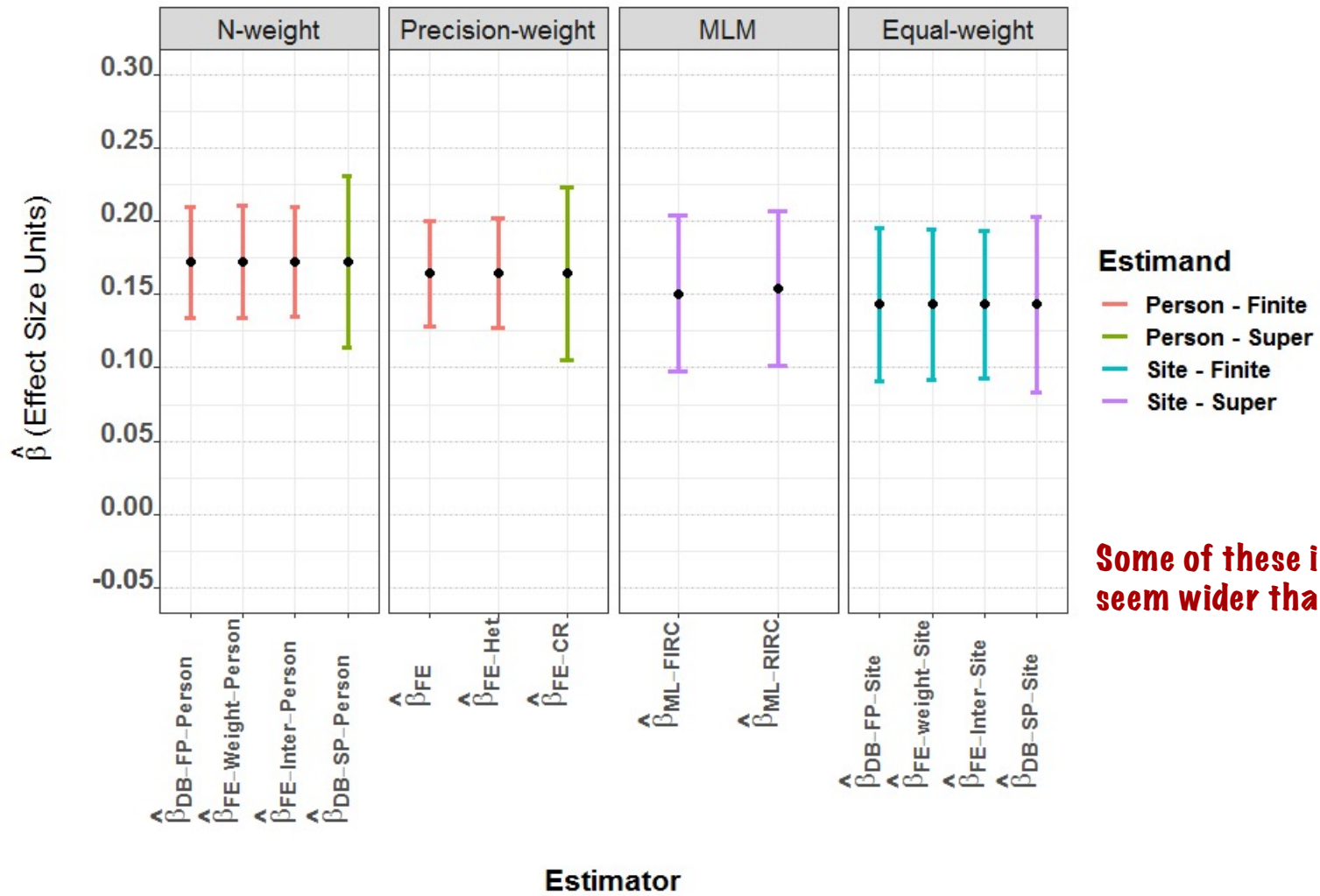
Notes: Each dot represents a single outcome for a single study.

The x-axis is the range of point estimates ($\max[\hat{\beta}] - \min[\hat{\beta}]$) across all 14 estimators, in effect size units.

RQ2:

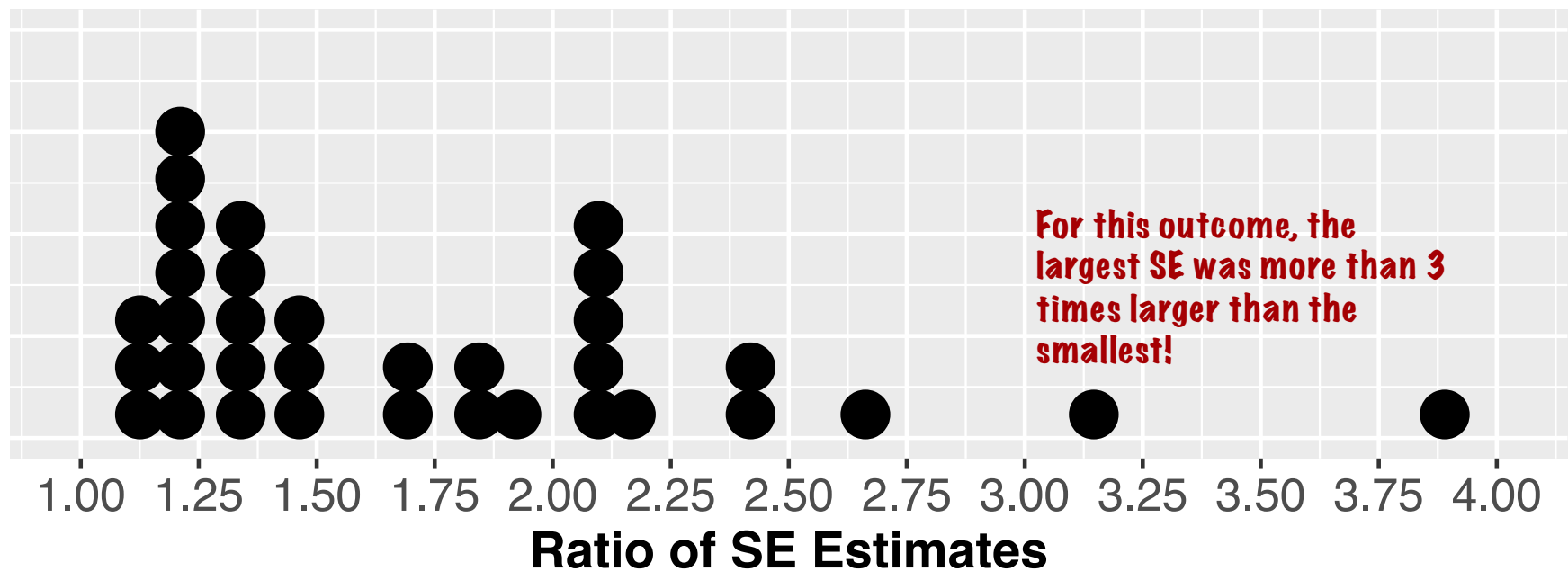
Does choice of estimand/estimator matter for the estimate of $SE(\hat{\beta})$?

Tennessee STAR - Reading Scores



Some of these intervals seem wider than others

The largest estimated SE can be a lot different than the smallest

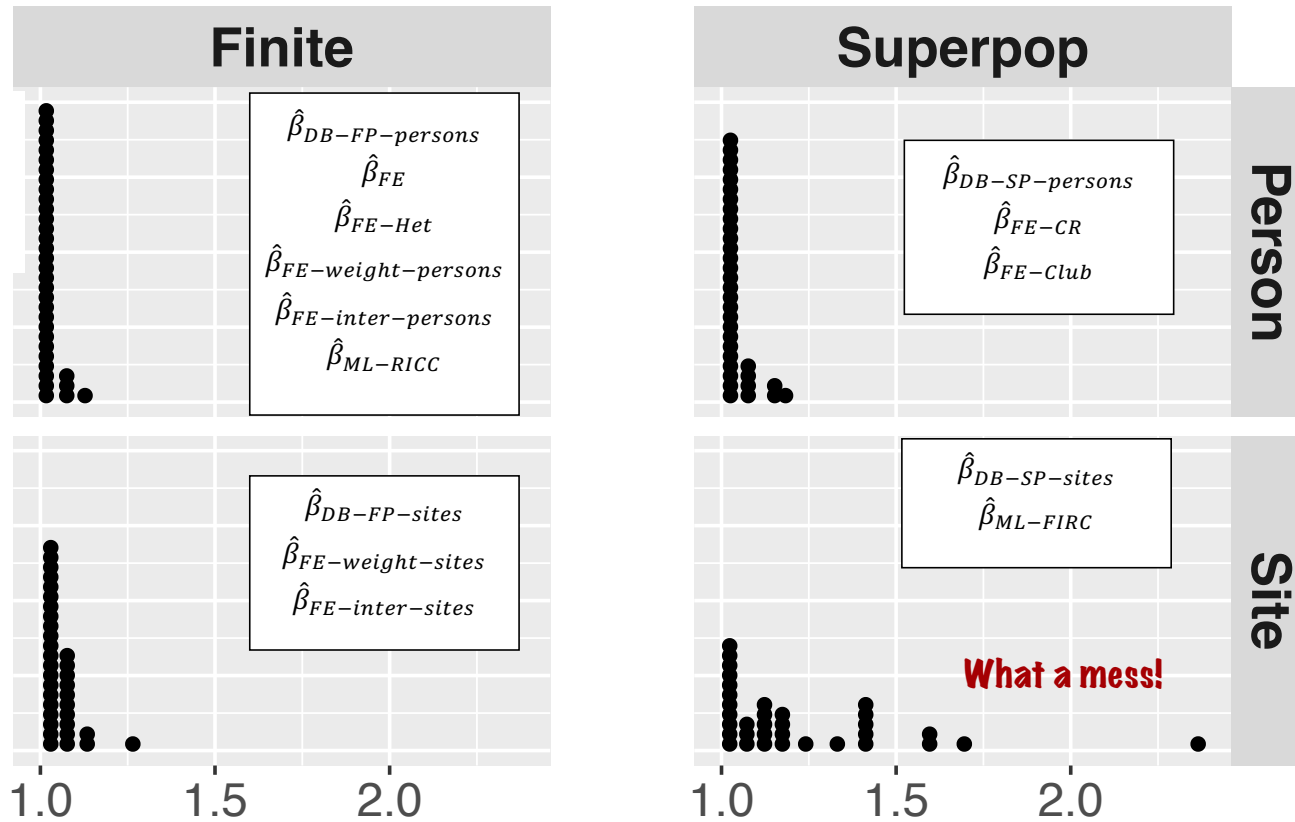


Notes: Each dot represents a single outcome for a single study.

The x-axis is the ratio of largest to smallest estimated SE ($\max[\widehat{SE}(\hat{\beta})]/\min[\widehat{SE}(\hat{\beta})]$) across all estimators, in effect size units.

Mostly no real difference, but can reach +20% or so, worst case

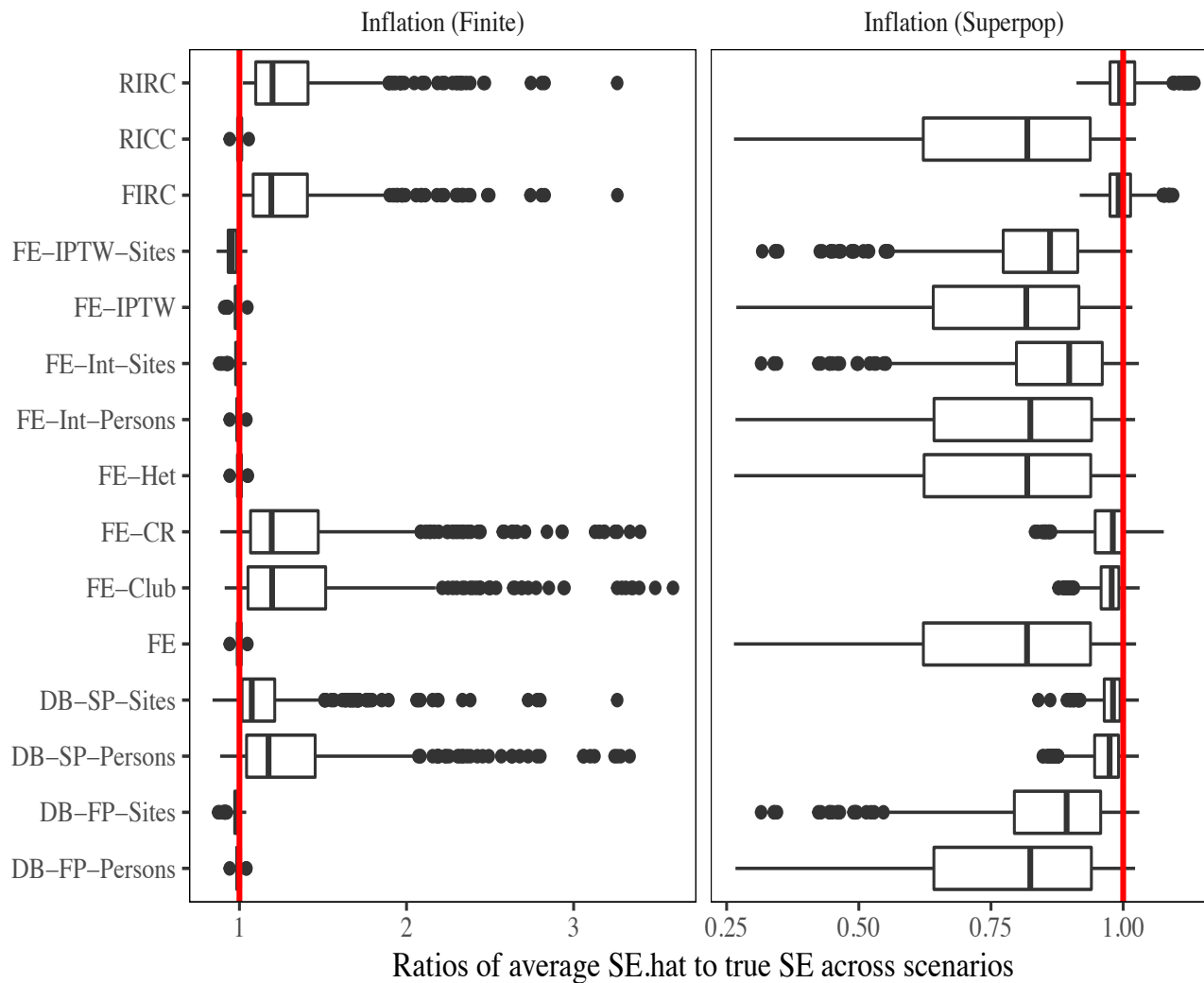
Number Outcome



Ratio of SE Estimates

Notes: Each dot represents a single outcome for a single study.

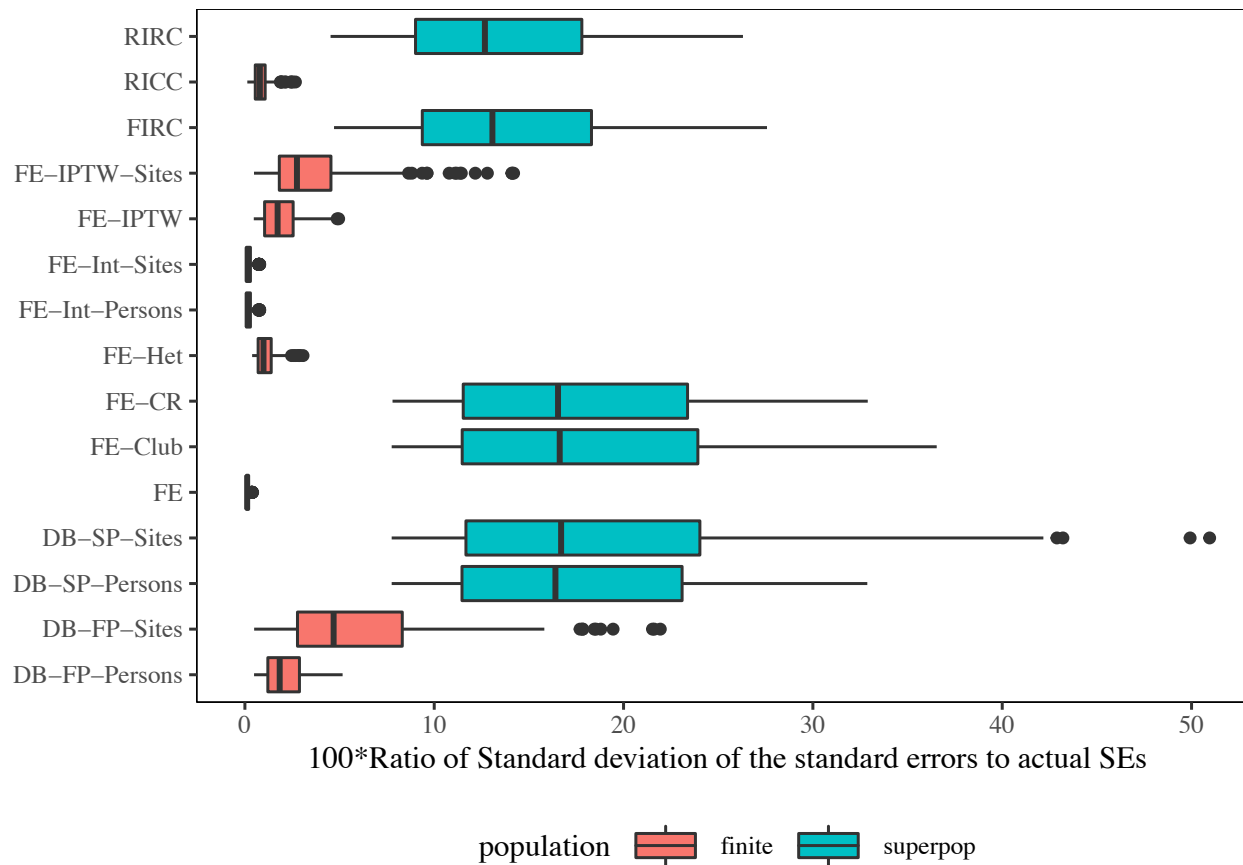
The x-axis is the ratio of largest to smallest estimated SE ($\max[\widehat{SE}(\hat{\beta})]/\min[\widehat{SE}(\hat{\beta})]$) across all 13 estimators, in effect size units.



Are the Standard Error estimates calibrated?

Generally yes, if you are in the right framework.

Boxplots show calibration across simulation scenarios



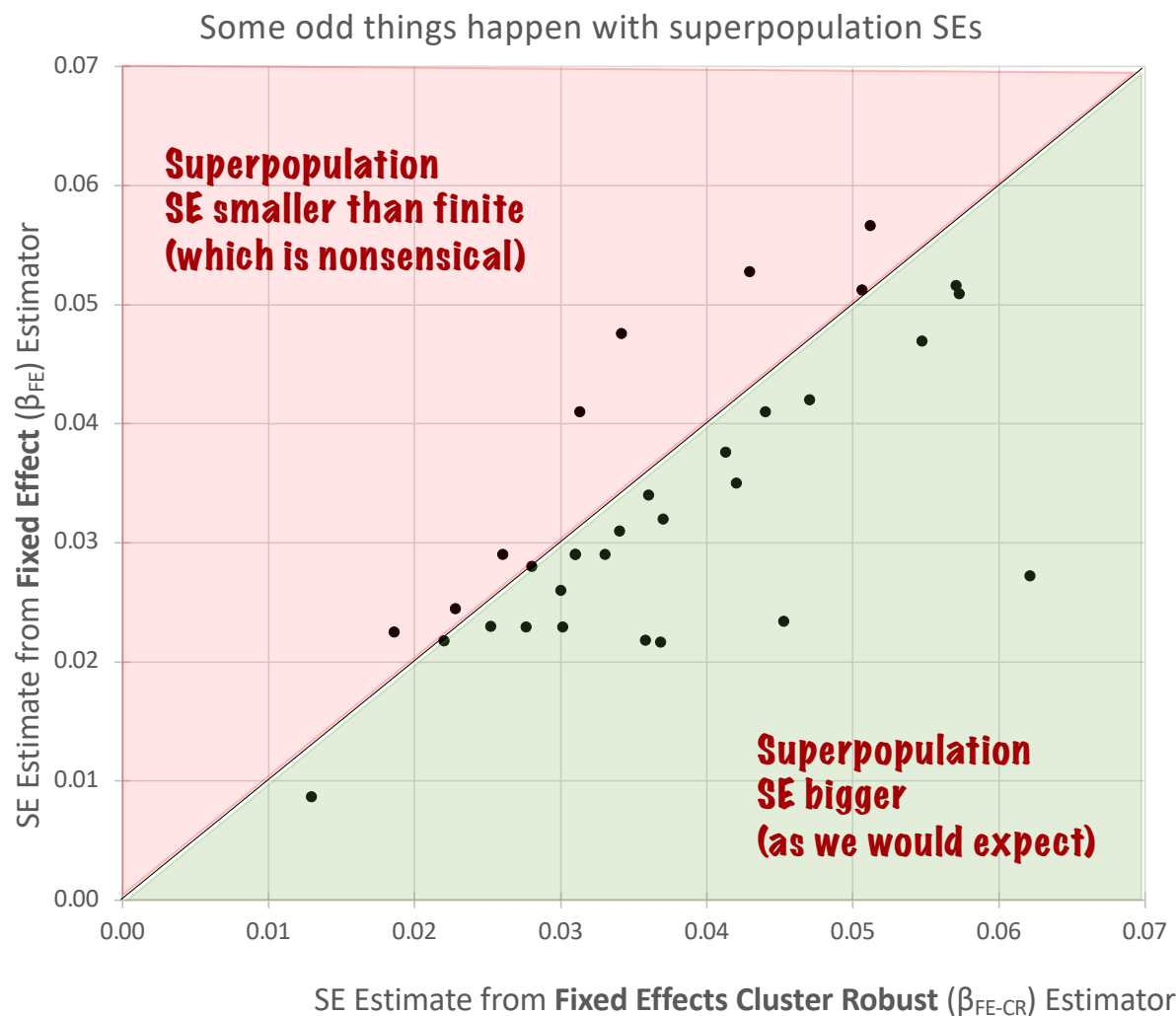
How well can we estimate our standard errors anyway?

For superpopulation, not well.

For finite, almost perfectly in some cases.

Site average estimation does have a price, as usual.

Superpopulation SE estimates vary a lot, causing trouble.

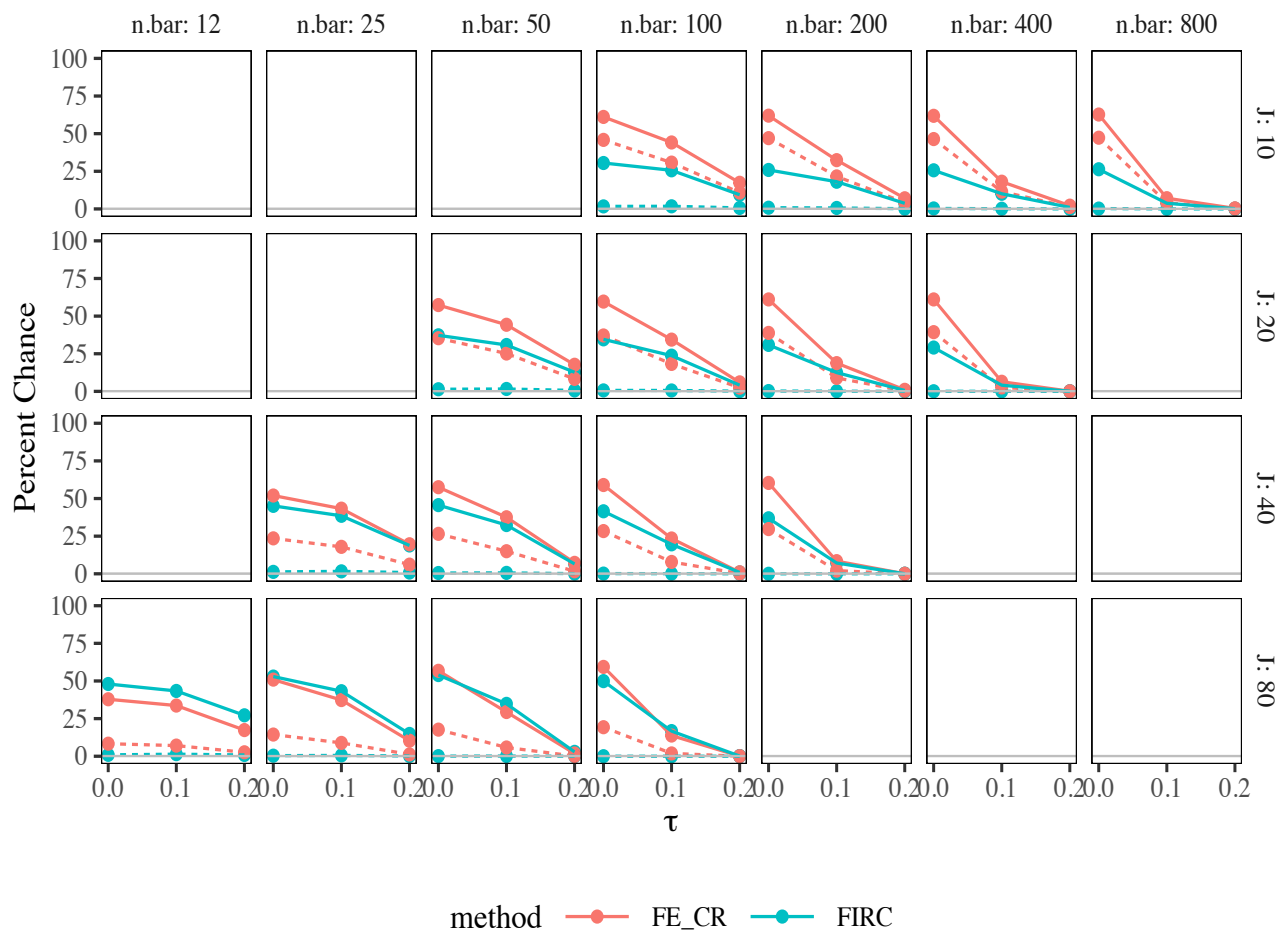


Cluster robust FE targets superpop person weighted. We expect the SEs to be LARGER than the finite person weighted

This often does not happen.

(The other superpopulation estimators suffer the same.)

Superpopulation estimated SEs are lower than finite estimated SEs quite often



From Simulation:

How often do we get a smaller finite population standard error than a superpopulation one?

Not infrequently.

Instability of all estimators at the superpopulation level in the face of cross site impact variation makes life difficult.

RQ3:

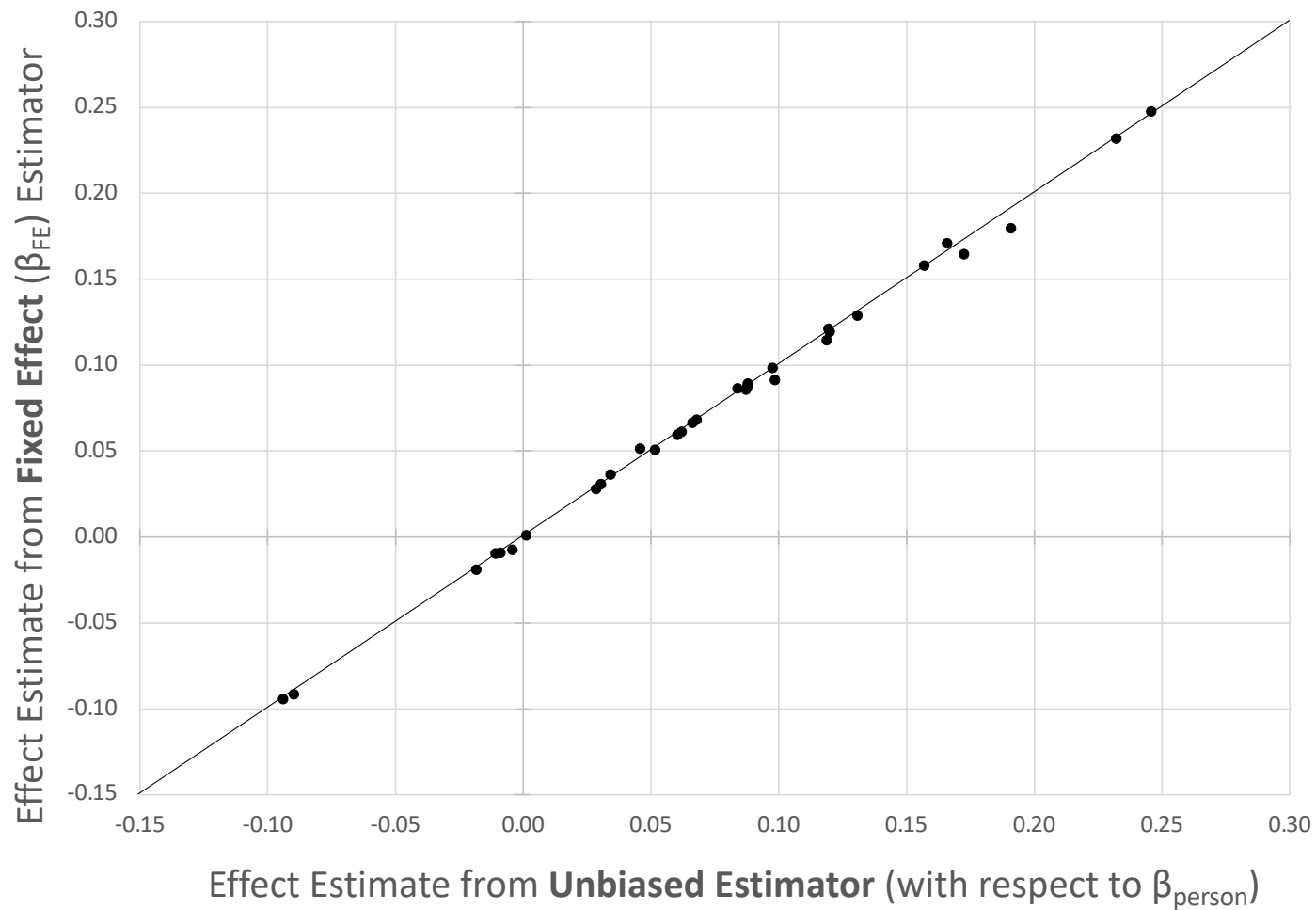
Bias Precision Trade-off?

RQ3: Bias Precision Trade-off?

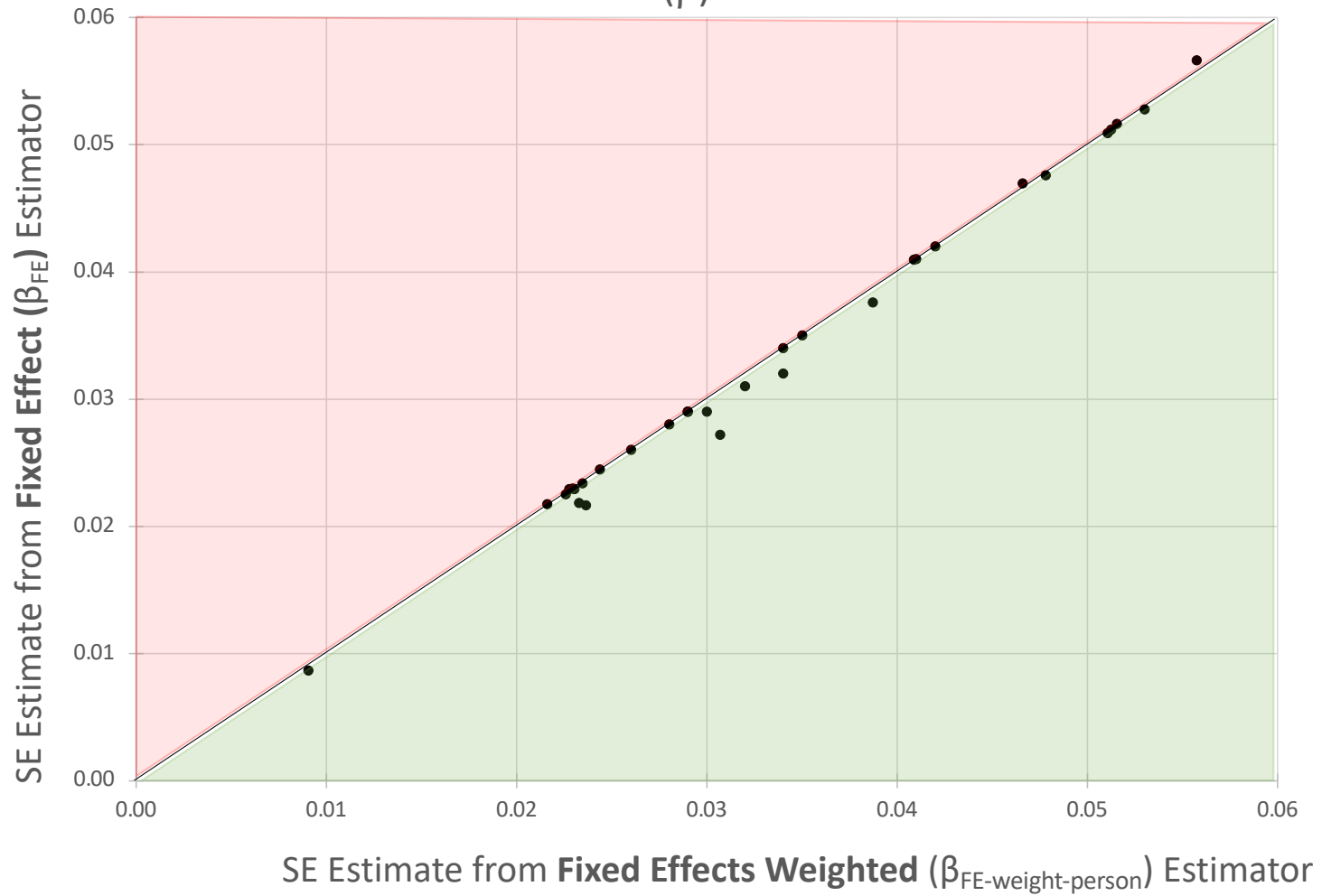
Part I: The estimand of $\beta_{FP-person}$

Unbiased vs. Fixed Effects models

Fixed Effects Estimator of β – Little Potential for Bias



Fixed Effects Estimator of $SE(\beta)$ - Limited Precision Gains

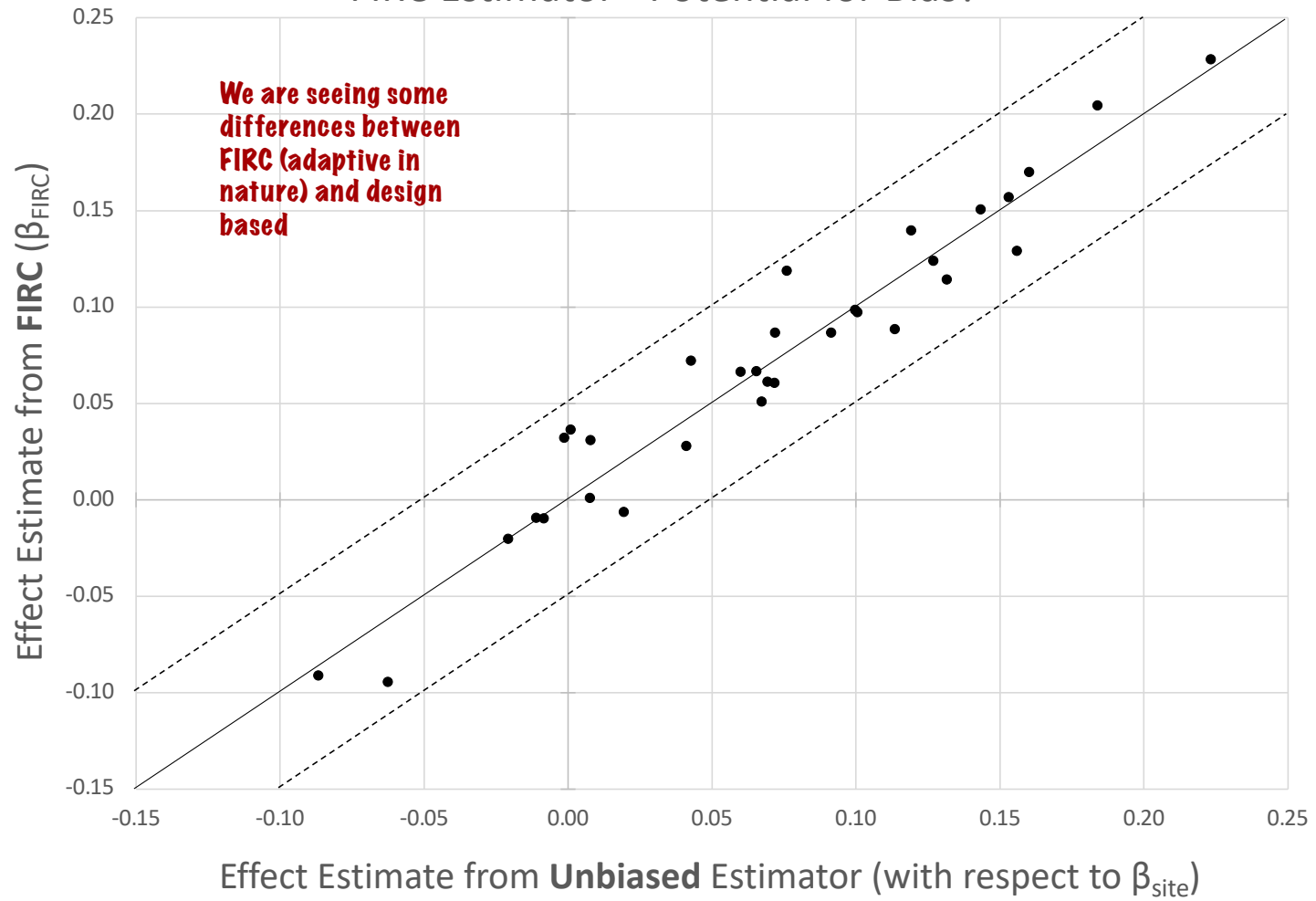


RQ3: Bias Precision Trade-off?

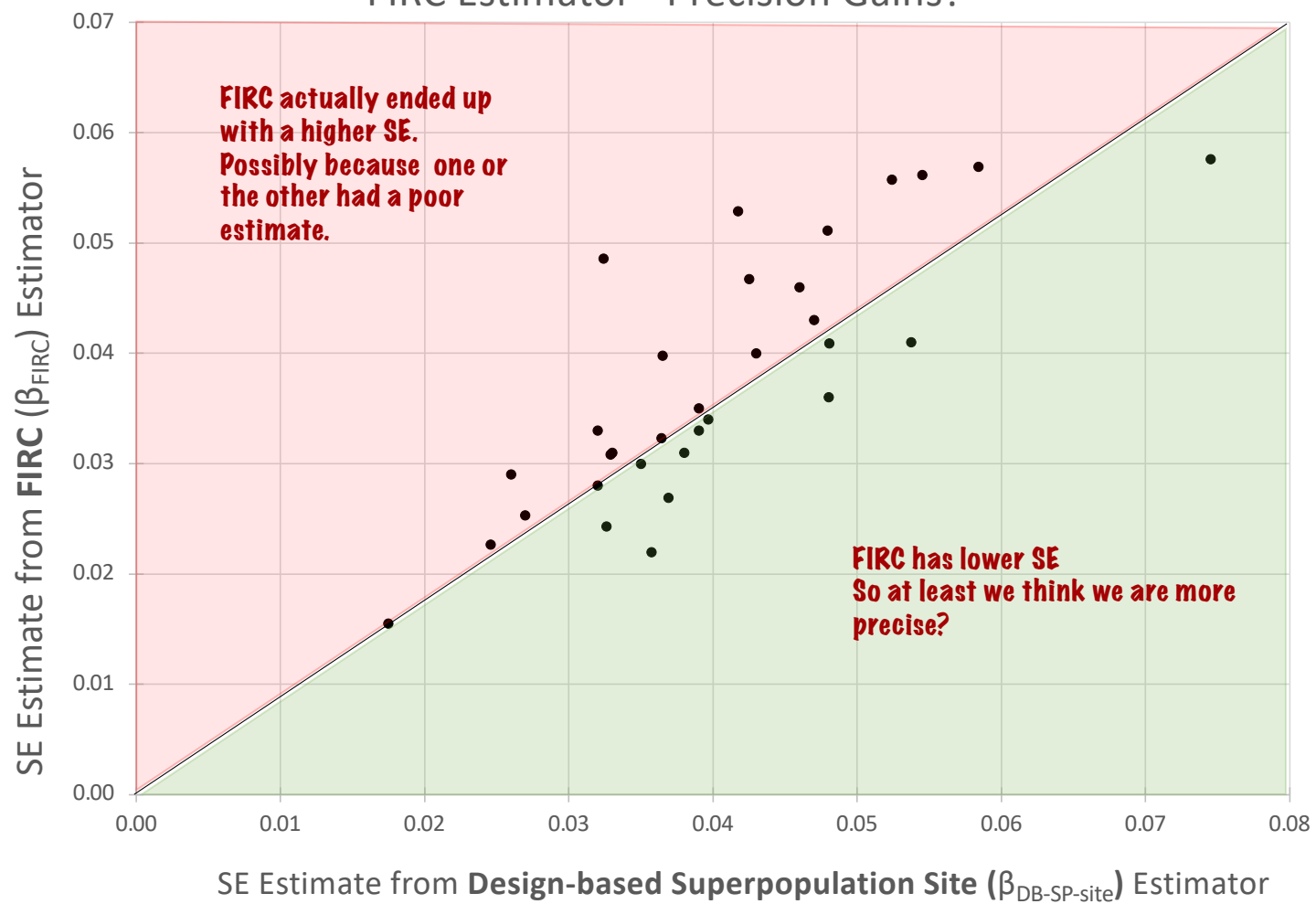
Part II: The estimand of $\beta_{SP-site}$

Unbiased Design Based vs. FIRC

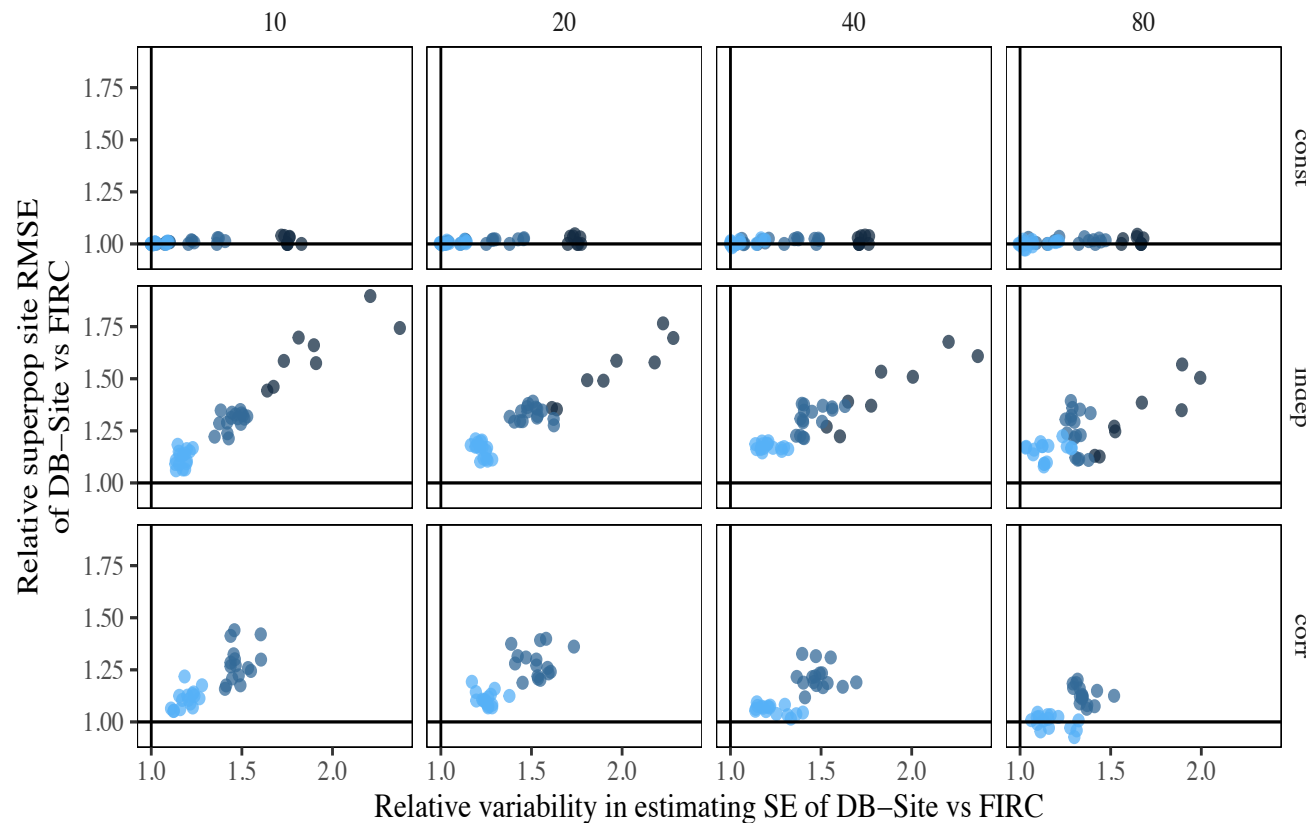
FIRC Estimator - Potential for Bias?



FIRC Estimator - Precision Gains?

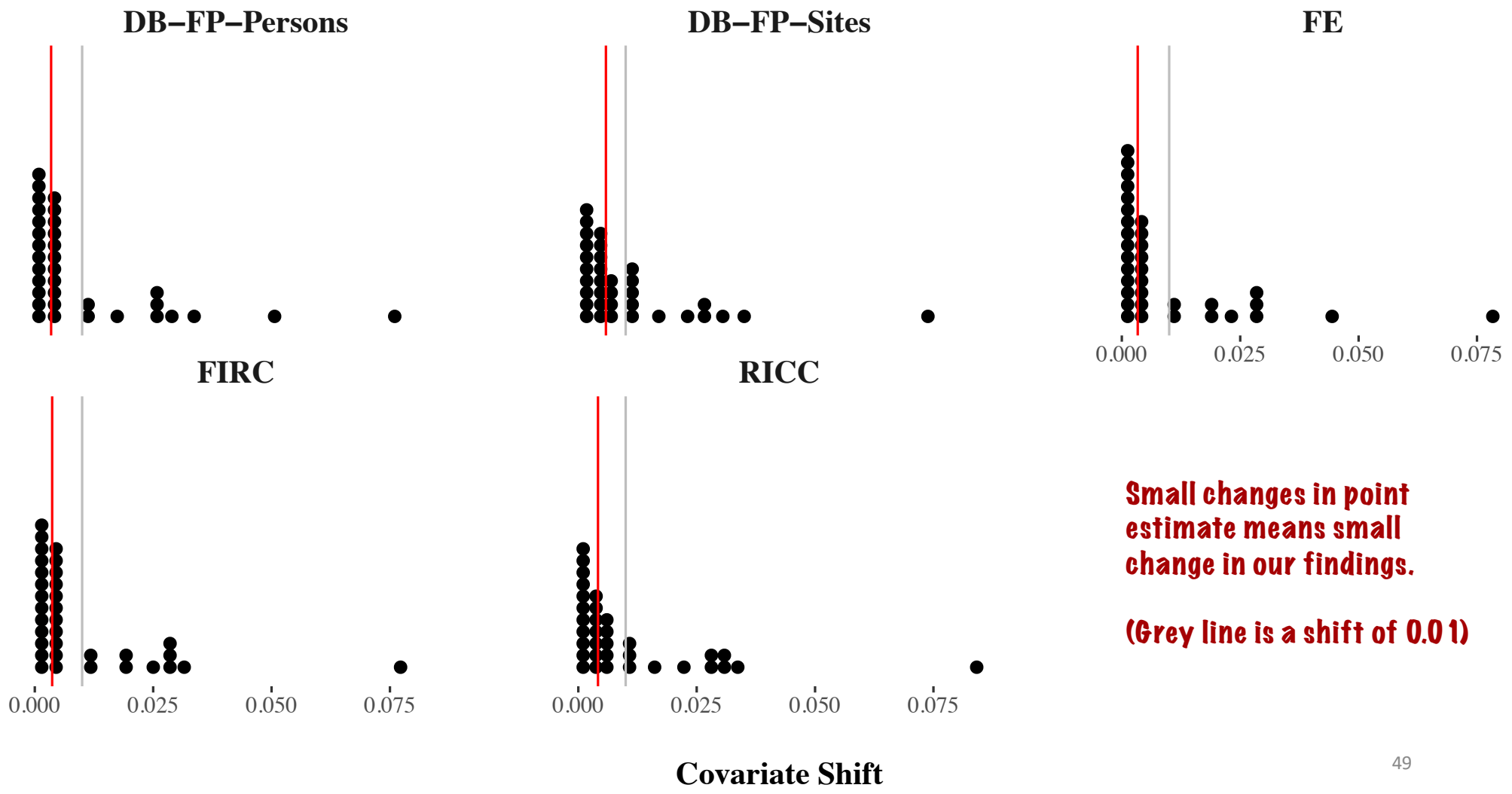


Simulation Commentary: Infinite Site is hard and the $\beta_{SP-person}$ is a troublemaker



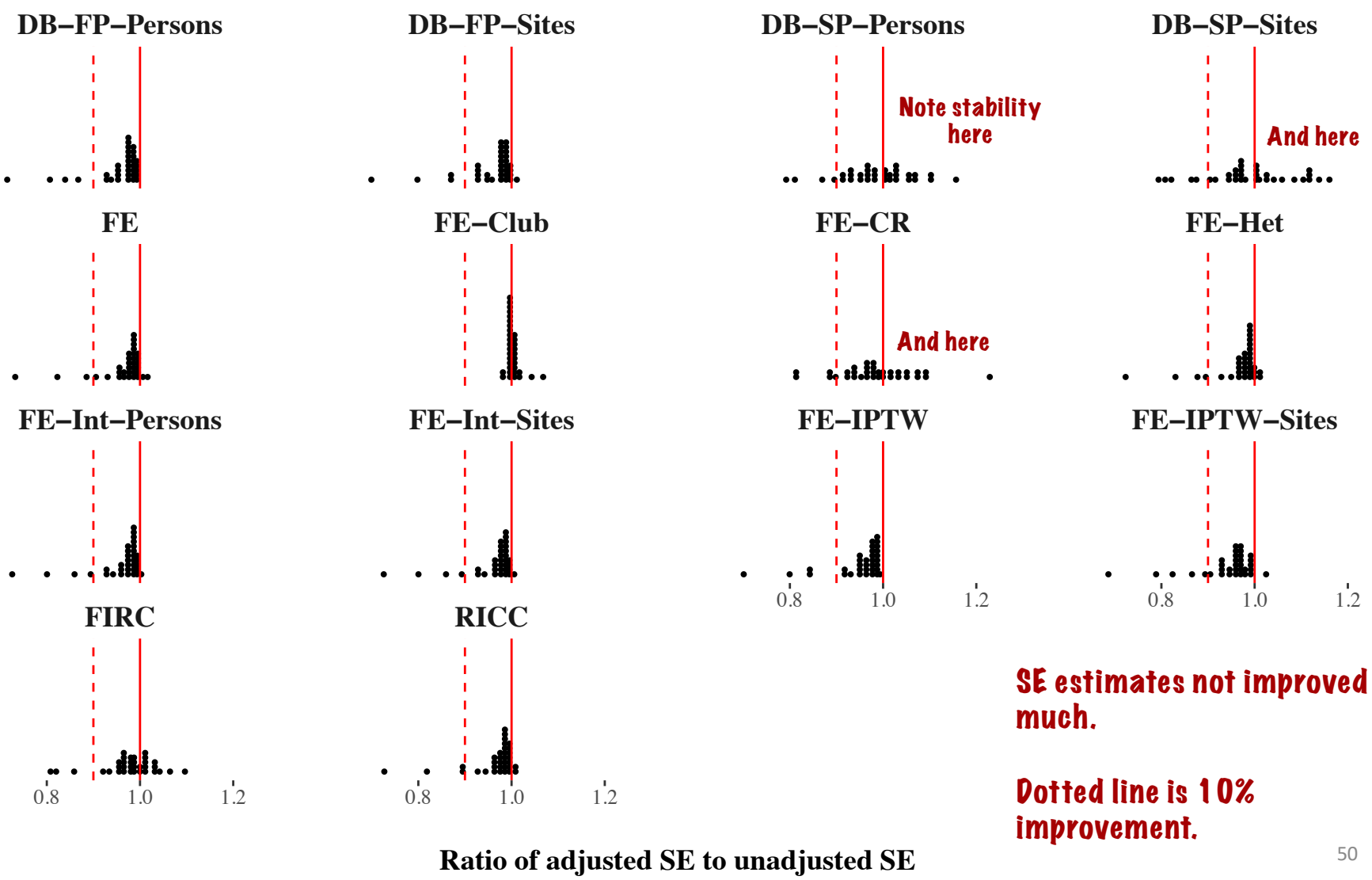
- FIRC is adaptive, clinging to fixed effect fairly strongly unless there is a large amount of cross site variation.
- That being said, FIRC is quite unstable.
- Unbiased approaches are even more unstable, however.
- Across of all simulation scenarios we consider, the RMSE of FIRC was higher than DB-SP-Site in only 2% of them.

And what about covariate adjustment?



Small changes in point estimate means small change in our findings.

(Grey line is a shift of 0.0 1)



Two additional resources with our paper

(3 papers for the price of one?)

A) Technical appendix gives overview of all estimators with some details and notes on their use

B) Multifactor simulation appendix explores estimator performance under hypothetical MLM DGP

The stand we take

- Estimand choice matters.
- β estimator choice matters for site-super estimand, otherwise not much
- $SE(\hat{\beta})$ estimator matters for site estimands, much less for person estimands
- The superpopulation site estimators differ the most, and are the most unstable (difficult).

Thank you

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Thanks to Mike Weiss for
making most of these slides for
an initial presentation 53