Estimating Estimands with Estimators

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egap EVIDENCE IN GOVERNANCE AND POLITICS

Key points

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Estimands and estimators and averages

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Causal effects that differ by groups or covariates

Causal effects when we do not control the dose

Key points

Key points about estimation I

- A causal effect, τ_i, is a comparison of unobserved potential outcomes for each unit i: examples $\tau_i = Y_i(T_i = 1) - Y_i(T_i = 0)$ or $\tau_i = \frac{Y_i(T_i=1)}{Y_i(T_i=0)}$.
- To learn about τ_i, we can treat τ_i as an **estimand** or target quantity to be estimated (discussed here) or as a target quantity to be hypothesized about (session on hypothesis testing).
- Many focus on the **average treatment effect (ATE)**, $\bar{\tau} = \sum_{i=1}^{n} \tau_i$, in part, because it allows for easy **estimation**.

Key points about estimation II

- The key to estimation for causal inference is to choose an estimand that helps you learn about your theoretical or policy question. So, one could use the ATE but other common estimands include the ITT, LATE/CACE, ATT, or ATE for some subgroup (or even a different of causal effects between groups).
- An **estimator** is a recipe for calculating a guess about the value of an estimand. For example, the difference of observed means for m treated units is one estimator of $\bar{\tau}$: $\hat{\bar{\tau}} = \frac{\sum_{i=1}^{n}(T_i Y_i)}{m} - \frac{\sum_{i=1}^{n}((1-T_i)Y_i)}{(n-m)}$.

Key points about estimation III

- The **standard error** of an estimator in a randomized experiment summarizes how the estimates would vary if the experiment were repeated.
- We use the **standard error** to produce **confidence intervals** and **p-values**: so that we can begin with an estimator and end at a hypothesis test.
- Different randomizations will produce different values of the same estimator targeting the same estimand. A **standard error** summarizes this variability in an estimator.
- A $100(1-\alpha)\%$ **confidence interval** is a collection of hypotheses that cannot be rejected at the α level. We tend to report confidence intervals containing hypotheses about values of our estimand and use our estimator as a test statistic.

Key points about estimation IV

- Estimators should:
  - avoid systematic error in their guessing of the estimand (be unbiased);
  - vary little in their guesses from experiment to experiment (be precise or efficient); and
  - perhaps ideally converge to the estimand as they use more and more information (be consistent).

Key points about estimation V

- **Analyze as you randomize** in the context of estimation means that (1) our standard errors should measure variability from randomization and (2) our estimators should target estimands defined in terms of potential outcomes.
- We do not **control for** background covariates when we analyze data from randomized experiments. But covariates can make our estimation more **precise**. This is called **covariance adjustment** (or covariate adjustment). **Covariance adjustment** in randomized experiments differs from controlling for in observational studies.

Review

Review: Causal effects

Review: Causal inference refers to a comparison of unobserved, fixed, potential outcomes.

For example:

- the potential, or possible, outcome for unit i when assigned to treatment, $T_i = 1$ is $Y_i(T_i = 1)$.
- the potential, or possible, outcome for unit i when assigned to control, $T_i = 0$ is $Y_i(T_i = 0)$.

Treatment assignment, T_i, has a causal effect on unit i, that we call τ_i, if $Y_i(T_i = 1) - Y_i(T_i = 0) \neq 0$ or $Y_i(T_i = 1) \neq Y_i(T_i = 0)$.

Estimands and estimators and averages

How can we learn about causal effects from observed data?

1. Recall: we can **test hypotheses** about the pair of potential outcomes $\{Y_i(T_i = 1), Y_i(T_i = 0)\}$.
2. We can **define estimands** in terms of $\{Y_i(T_i = 1), Y_i(T_i = 0)\}$ or τ_i, **develop estimators** for those estimands, and then calculate values and standard errors for those estimators.

A common estimand and estimator: The average treatment effect and the difference of means

Say we are interested in the ATE, or $\bar{\tau} = \sum_{i=1}^{n} \tau_i$. What is a good estimator?

Two candidates:

1. The difference of means: $\hat{\bar{\tau}} = \frac{\sum_{i=1}^{n}(T_i Y_i)}{m} - \frac{\sum_{i=1}^{n}((1-T_i)Y_i)}{n-m}$.
2. A difference of means after top-coding the highest Y_i observation (a kind of “winsorized” mean to prevent extreme values from exerting too much influence over our estimator — to increase *precision*).

How would we know which estimator is best for our particular research design?

Let’s simulate!

Simulation Step 1: create some data with a known ATE

Notice that we need to *know* the potential outcomes and the treatment assignment in order to learn whether our proposed estimator does a good job.

Z	y0	y1
0	0	10
0	0	30
0	0	200
0	1	91
1	1	11
1	3	23
0	4	34
0	5	45
1	190	280
1	200	220

```
The true ATE is 54
```

In reality, we would observe only one of the potential outcomes.

Note that each unit has its own treatment effect.

First make fake data

The table in the previous slide was generated in R with:

```r
# We have ten units
N <- 10
#  y0 is potential outcome to control
y0 <- c(0, 0, 0, 1, 1, 3, 4, 5, 190, 200)
# Each unit has its own treatment effect
tau <- c(10, 30, 200, 90, 10, 20, 30, 40, 90, 20)
# y1 is potential outcome to treatment
y1 <- y0 + tau
# Two blocks, a and b
block <- c("a", "a", "a", "a", "a", "a", "b", "b", "b", "b")
# Z is treatment assignment (Z instead of T in the code)
Z <- c(0, 0, 0, 0, 1, 1, 0, 0, 1, 1)
# Y is observed outcomes
Y <- Z * y1 + (1 - Z) * y0
# The data
dat <- data.frame(Z = Z, y0 = y0, y1 = y1, tau = tau, b = block, Y = Y)
set.seed(12345)
```

Using DeclareDesign

DeclareDesign represents research designs in a few steps shown below:

```
# take just the potential outcomes under treatment and control from our
# fake data
small_dat <- dat[, c("y0", "y1")]

# DeclareDesign first asks you to declare your population
pop <- declare_population(small_dat)
N <- nrow(small_dat)

# 5 units assigned to treatment; default is simple random assignment with
# probability 0.5
trt_assign <- declare_assignment(Z = conduct_ra(N = N, m = 2), legacy = FALSE)

# observed Y is y1 if Z=1 and y0 if Z=0
pot_out <- declare_potential_outcomes(Y ~ Z * y1 + (1 - Z) * y0)

# specify outcome and assignment variables
reveal <- declare_reveal(Y, Z)

# the basic research design object includes these four objects
base_design <- pop + trt_assign + pot_out + reveal
```

Using DeclareDesign: make fake data

DeclareDesign renames `y0` and `y1` by default to `Y_Z_0` and `Y_Z_1`:

```r
## A simulation is one random assignment of treatment
sim_dat1 <- draw_data(base_design)

## Simulated data (just the first 6 lines)
head(sim_dat1)
```

```
  y0  y1 Z Y_Z_0 Y_Z_1  Y
1  0  10 0     0    10  0
2  0  30 1     0    30 30
3  0 200 0     0   200  0
4  1  91 0     1    91  1
5  1  11 0     1    11  1
6  3  23 1     3    23 23
```

Using DeclareDesign: define estimand and estimators

No output here. Just define functions and estimators and one estimand.

```r
## The estimand
estimandATE <- declare_inquiry(ATE = mean(Y_Z_1 - Y_Z_0))

## The first estimator is difference-in-means
diff_means <- declare_estimator(Y ~ Z,
  inquiry = estimandATE,
  model = lm_robust, se_type = "classical", label = "Diff-Means/OLS"
)
```

Using DeclareDesign: define estimand and estimators

```r
## The second estimator is top-coded difference-in-means
diff_means_topcoded_fn <- function(data) {
  data$rankY <- rank(data$Y)
  ## Code the maximum value of Y as the second to maximum value of Y
  data$newY <- with(
    data,
    ifelse(rankY == max(rankY), Y[rankY == (max(rankY) - 1)], Y)
  )
  obj <- lm_robust(newY ~ Z, data = data, se_type = "classical")
  res <- tidy(obj) %>% filter(term == "Z")
  return(res)
}
diff_means_topcoded <- declare_estimator(
  handler = label_estimator(diff_means_topcoded_fn),
  inquiry = estimandATE, label = "Top-coded Diff Means"
)
```

Using DeclareDesign: define estimand and estimators

Here we show how the DD estimators work using our simulated data.

```
## Demonstrate that the estimand works:
estimandATE(sim_dat1)
```

```
  inquiry estimand
1     ATE       54
```

```
## Demonstrate that the estimators estimate
## Estimator 1 (difference in means)
diff_means(sim_dat1)[-c(1, 2, 10, 11)]
```

```
  estimate std.error statistic p.value conf.low conf.high df
1   -23.62     66.18    -0.357  0.7304   -176.2       129  8
```

```
## Estimator 2 (top-coded difference in means)
diff_means_topcoded(sim_dat1)[-c(1, 2, 10, 11)]
```

```
  estimate std.error statistic p.value conf.low conf.high df
1   -22.37     64.44   -0.3472  0.7374     -171     126.2  8
```

Then simulate with one randomization

Recall the true ATE:

```
trueATE <- with(sim_dat1, mean(y1 - y0))
with(sim_dat1, mean(Y_Z_1 - Y_Z_0))
```

```
[1] 54
```

In one experiment (one simulation of the data) here are the simple estimates:

```
## Two ways to calculate the difference of means estimator
est_diff_means_1 <- with(sim_dat1, mean(Y[Z == 1]) - mean(Y[Z == 0]))
est_diff_means_2 <- coef(lm_robust(Y ~ Z,
  data = sim_dat1,
  se = "classical"
))[["Z"]]
c(est_diff_means_1, est_diff_means_2)
```

```
[1] -23.62 -23.62
```

Then simulate with one randomization

In one experiment (one simulation of the data) here are the estimates after top-coding:

```r
## Two ways to calculate the topcoded difference of means estimator
sim_dat1$rankY <- rank(sim_dat1$Y)
sim_dat1$Y_tc <- with(sim_dat1, ifelse(rankY == max(rankY),
  Y[rankY == (max(rankY) - 1)], Y
))
est_topcoded_1 <- with(sim_dat1, mean(Y_tc[Z == 1]) - mean(Y_tc[Z == 0]))
est_topcoded_2 <- coef(lm_robust(Y_tc ~ Z,
  data = sim_dat1,
  se = "classical"
))[["Z"]]
c(est_topcoded_1, est_topcoded_2)
```

```
[1] -22.38 -22.37
```

Then simulate a different randomization and estimate the ATE with the same estimators

Now calculate your estimate with the same estimators using a **different** randomization. Notice that the answers differ. The estimators are estimating the *same estimand* but now they have a different randomization to work with.

```r
# do another random assignment of the treatment in DeclareDesign
# this produces a new simulated dataset with a different random assignment
sim_dat2 <- draw_data(base_design)
# the first estimator (difference in means)
coef(lm_robust(Y ~ Z, data = sim_dat2, se = "classical"))[["Z"]]
```

```
[1] -18
```

```r
# the second estimator (top-coded difference in means)
sim_dat2$rankY <- rank(sim_dat2$Y)
sim_dat2$Y_tc <- with(sim_dat2, ifelse(rankY == max(rankY),
  Y[rankY == (max(rankY) - 1)], Y
))
coef(lm_robust(Y_tc ~ Z, data = sim_dat2, se = "classical"))[["Z"]]
```

```
[1] -16.75
```

How do our estimators behave in general for this design?

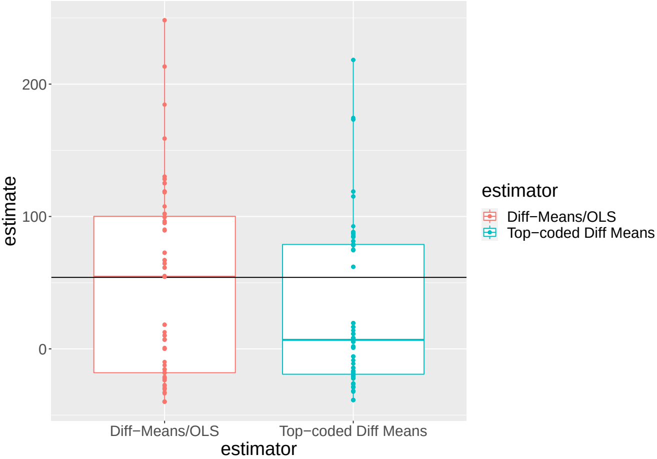
Our estimates vary across randomizations. Do our two estimators vary in the same ways?

```r
## Combine into one DeclareDesign design object
## This has the base design, estimand, then our two estimators
design_plus_ests <- base_design + estimandATE + diff_means +
  diff_means_topcoded
## Run 100 simulations (reassignments of treatment) and
## apply the two estimators (diff_means and diff_means_topcoded)
diagnosis1 <- diagnose_design(design_plus_ests,
  bootstrap_sims = 0, sims = 100
)
sims1 <- get_simulations(diagnosis1)
head(sims1[, -c(1:6)])
```

```
  estimate std.error statistic p.value conf.low conf.high df outcome
1   -18.00     66.23  -0.27179  0.7927   -170.7     134.7  8       Y
2   -16.75     64.49  -0.25974  0.8016   -165.5     132.0  8    newY
3     0.75     67.98   0.01103  0.9915   -156.0     157.5  8       Y
4     2.00     66.29   0.03017  0.9767   -150.9     154.9  8    newY
5    54.50     75.93   0.71775  0.4933   -120.6     229.6  8       Y
6   -19.14     75.85  -0.25237  0.8092   -204.7     166.5  6    newY
```

How do our estimators behave in general for this design?

Our estimates vary across randomizations. Do our two estimators vary in the same ways? How should we interpret this plot?



Which estimator is closer to the truth?

One way to choose among estimators is to choose the one that is **close to the truth** whenever we use it — regardless of the specific randomization.

An “unbiased” estimator is one for which **average of the estimates across repeated designs** is the same as the truth (or $E_R(\hat{\bar{\tau}}) = \bar{\tau}$). An unbiased estimator has “no systematic error” but doesn’t guarantee closeness to the truth.

Another measure of closeness is **root mean squared error** (RMSE) which records squared distances between the truth and the individual estimates.

Which estimator is better? (One is closer to the truth on average (RMSE) and is more precise. The other has no systematic error — is unbiased.)

Estimator	Bias	RMSE	SD Estimate	Power
Diff-Means/OLS	-5.70	71.23	71.36	0.09
Top-coded Diff Means	-25.50	64.86	59.94	0.03

Unbiased and biased estimators

Summary:

- We have a *choice* of both estimands and estimators
- A good estimator performs well regardless of the particular randomization of a given design. And *performs well* can mean "unbiased" and/or "low mse" (or "consistent" — which means increasingly close to the truth as the sample size increases).
- We can learn about how a given estimator performs in a given study using simulation.

Block randomization

Block-randomized experiments are a collection of mini-experiments

What is the **ATE** estimand in a block-randomized experiment?

If we think of the unit-level ATE as: $(1/N)\sum_{i=1}^{N} y_{i,1} - y_{i,0}$ then we could re-express this equivalently using the ATE in block j is ATE_j as follows:

$$ATE = \frac{1}{J}\sum_{j=1}^{J}\sum_{i=1}^{N_j}\frac{y_{i,1} - y_{i,0}}{N_j} = \sum_{j=1}^{J}\frac{N_j}{N}ATE_j$$

And it would be natural to *estimate* this quantity by plugging in what we can calculate: $\widehat{ATE} = \displaystyle\sum_{j=1}^{J}\frac{N_j}{N}\widehat{ATE}_j$

Block-randomized experiments are a collection of mini-experiments

And we could *define* the standard error of the estimator by also just averaging the within-block standard errors (if our blocks are large enough):

$$SE(\widehat{ATE}) = \sqrt{\sum_{j=1}^{J}(\frac{N_j}{N})^2 SE^2(\widehat{ATE}_j)}$$

Estimating the ATE in block-randomized experiments

One approach to estimation simply replaces ATE_j with $\widehat{ATE}$ above:

```
with(dat, table(b, Z))
```

```
   Z
b   0 1
  a 4 2
  b 2 2
```

We have 6 units in block a, 2 of which are assigned to treatment, and 4 units in block b, 2 of which are assignment to treatment.

Estimating the ATE in block-randomized experiments

One approach to estimation simply replaces ATE_j with $\widehat{ATE}$ above:

```
datb <- dat %>%
  group_by(b) %>%
  summarize(
    nb = n(), pb = mean(Z), estateb = mean(Y[Z == 1]) - mean(Y[Z == 0]),
    ateb = mean(y1 - y0), .groups = "drop"
  )
datb
```

```
# A tibble: 2 x 5
  b        nb    pb estateb  ateb
  <chr> <int> <dbl>   <dbl> <dbl>
1 a         6 0.333    16.8    60
2 b         4 0.5     246.     45
```

```
## True ate by block:
with(dat, mean(y1 - y0))
```

```
[1] 54
```

```
## This is another way to calculate the true ate
with(datb, sum(ateb * (nb / sum(nb))))
```

```
[1] 54
```

Estimating the ATE in block-randomized experiments

One approach is to estimate the overall ATE using block-size weights:

```
## Showing that difference_in_means uses the blocksize weight.
e1 <- difference_in_means(Y ~ Z, blocks = b, data = dat)
e2 <- with(datb, sum(estateb * (nb / sum(nb))))
c(coef(e1)[["Z"]], e2)
```

```
[1] 108.2 108.2
```

Estimating the ATE in block-randomized experiments

Notice that this is **not** the same as either of the following:

```r
## Ignoring blocks
e3 <- lm(Y ~ Z, data = dat)
coef(e3)[["Z"]]
```

```
[1] 131.8
```

```r
## With block fixed effects
e4 <- lm(Y ~ Z + block, data = dat)
coef(e4)[["Z"]]
```

```
[1] 114.8
```

How do they differ? (The first ignores the blocks. The second uses a different set of weights that are created by use of “fixed effects” or “indicator” or “dummy” variables.)

Which estimator should we use?

We now have three estimators each with a different estimate (imagining they all target the same estimand):

```
c(coef(e1)[["Z"]], coef(e3)[["Z"]], coef(e4)[["Z"]])
```

```
[1] 108.2 131.8 114.8
```

Which estimator should we use for this design? We can set up a DeclareDesign simulation to figure this out.

```
## declare a new base design that includes the block indicator b
base_design_blocks <-
  # declare the population
  declare_population(dat[, c("b", "y0", "y1")]) +
  # tell DD that b indicates block and to assign 2 treated units in each block
  declare_assignment(
    Z = conduct_ra(N = N, m = 2, blocks = b),
    Z_cond_prob =
      obtain_condition_probabilities(assignment = Z, m = 2)
  ) +
  # relationship of potential outcomes to observed outcome
  declare_potential_outcomes(Y ~ Z * y1 + (1 - Z) * y0) +
  # observed outcome and treatment assignment
  declare_reveal(Y, Z)
```

Which estimator should we use?

```r
# the estimand is the average treatment effect
estimandATEb <- declare_inquiry(ATE = mean(Y_Z_1 - Y_Z_0))

# three different estimators
est1 <- declare_estimator(Y ~ Z,
  inquiry = estimandATEb, model = lm_robust,
  label = "Ignores Blocks"
)
est2 <- declare_estimator(Y ~ Z,
  inquiry = estimandATEb, model = difference_in_means, blocks = b,
  label = "DiM: Block-Size Weights"
)
est3 <- declare_estimator(Y ~ Z,
  inquiry = estimandATEb, model = lm_robust,
  weights = (Z / Z_cond_prob) + ((1 - Z) / (Z_cond_prob)),
  label = "LM: Block Size Weights"
)
```

Which estimator should we use?

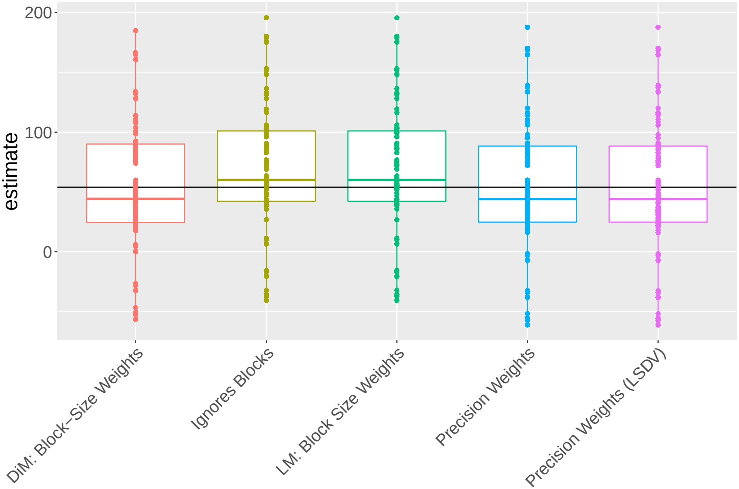
```
# two more estimators
est4 <- declare_estimator(Y ~ Z,
  inquiry = estimandATEb,
  model = lm_robust, fixed_effects = ~b, label = "Precision Weights"
)
est5 <- declare_estimator(Y ~ Z + b,
  inquiry = estimandATEb,
  model = lm_robust, label = "Precision Weights (LSDV)"
)

## new design object has the base design, the estimand, and five estimators
design_blocks <- base_design_blocks + estimandATEb +
  est1 + est2 + est3 + est4 + est5
```

Then we will run 10,000 simulations (reassign treatment 10,000 times) and summarize the estimates produced by each of these five estimators.

Which estimator should we use?

How should we interpret this plot?



Which estimator is closer to the truth?

Which estimator works better on this design and these data?

Estimator	Bias	RMSE	SD Est	Power	Coverage
DiM: Block-Size Weights	-0.63	53.08	53.11	0.22	0.77
Ignores Blocks	14.48	55.23	53.33	0.10	0.97
LM: Block Size Weights	14.48	55.23	53.33	0.10	0.97
Precision Weights	-1.02	55.39	55.40	0.11	0.92
Precision Weights (LSDV)	-1.02	55.39	55.40	0.11	0.92

Notice that the coverage is not always at 95% in all cases. We used 10,000 simulations so simulation error is around $\pm 2\sqrt{p(1-p)/10000}$ or, say, for coverage calculated as .93, a different simulation could have easily produced 0.9249 or 0.9351 (or would rarely have produced coverage numbers outside that range just by chance).

Cluster randomization

In cluster-randomized experiments, units are randomized as a group (cluster) to treatment I

- **Example 1:** an intervention is randomized across neighborhoods, so **all** households in a neighborhood will be assigned to the same treatment condition, but different neighborhoods will be assigned different treatment conditions.
- **Example 2:** an intervention is randomized across people and each person is measured four times after treatment, so our data contain four rows per person.
- **Not An Example 1:** Neighborhoods are chosen for the study. Within each neighborhood about half of the people are assigned to treatment and half to control. (What kind of study is this? It is not a cluster-randomized study.)

In cluster-randomized experiments, units are randomized as a group (cluster) to treatment II

- **Not an Example 2:** an intervention is randomized to some neighborhoods and not to others, the outcomes include measurements of neighborhood-level trust in government and total land area in the neighborhood devoted to gardens. (Sometimes a cluster randomized experiment can be turned into a simple randomized experiment. Or may contain more than one possible approach to analysis and interpretation.)

How might the distribution of test statistics and estimators differ from an experiment where individual units (not clusters) are randomized?

Estimating the ATE in cluster-randomized experiments

Bias problems in cluster-randomized experiments:

- When clusters are the same size, the usual difference-in-means estimator is unbiased.
- But be careful when clusters have different numbers of units or you have very few clusters because then treatment effects may be correlated with cluster size.
- When cluster size is related to potential outcomes, the usual difference-in-means estimator is biased. https://declaredesign.org/blog/bias-cluster-randomized-trials.html

Estimating the SE for the ATE in cluster-randomized experiments I

- **Misleading statistical inferences:** The default SE will generally underestimate precision in such designs and thus produce tests with false positive rates that are too high (or equivalently confidence intervals coverage rates that are too low).
- The "cluster robust standard errors" implemented in common software work well **when the number of clusters is large** (like more than 50 in some simulation studies).
- The default cluster-appropriate standard errors in `lm_robust` (the `CR2` SEs) work better than the common approach in Stata (as of this writing).

Estimating the SE for the ATE in cluster-randomized experiments II

- The wild bootstrap helps control error rates but gives up statistical power much more than perhaps necessary in a cluster randomized study where direct randomization inference is possible.
- When in doubt, one can produce *p*-values by direct simulation (direct randomization inference) to see if they agree with one of the cluster robust approaches.

Overall, it is worth simulating to study the performance of your estimators, tests, and confidence intervals if you have any worries or doubts.

An example of estimation

Imagine we had data from 10 clusters with either 100 people (for 2 clusters) or 10 people per cluster (for 8 clusters). The total size of the data is 280.

```
# A tibble: 6 x 6
# Groups:   clus_id [2]
  clus_id indiv Y_Z_0 Y_Z_1     Z     Y
  <chr>   <chr> <dbl> <dbl> <int> <dbl>
1 01      010    4.51  4.61     0  4.51
2 01      035    4.63  4.73     0  4.63
3 01      068    4.76  4.86     0  4.76
4 03      205    3.13  4.13     1  4.13
5 03      206    2.41  3.41     1  3.41
6 03      208    2.95  3.95     1  3.95
```

An example of estimation

Which estimator should we use? Which test should we use? On what basis should we choose among these approaches?

```
lmc1 <- lm_robust(Y ~ Z, data = dat1)
lmc2 <- lm_robust(Y ~ Z, clusters = clus_id, data = dat1)
lmc3 <- lm_robust(Y ~ Z + cl_sizeF, clusters = clus_id, data = dat1)
tidy(lmc1)[2, ]
```

```
  term estimate std.error statistic p.value conf.low conf.high  df outcome
2    Z   0.3024    0.1207     2.504 0.01284  0.06471    0.5401 278       Y
```

```
tidy(lmc2)[2, ]
```

```
  term estimate std.error statistic p.value conf.low conf.high    df outcome
2    Z   0.3024     1.079    0.2804   0.796   -2.969     3.574 3.282       Y
```

```
tidy(lmc3)[2, ]
```

```
  term estimate std.error statistic p.value conf.low conf.high    df outcome
2    Z   0.3024     0.306    0.9882  0.4386   -1.194     1.799 1.769       Y
```

Use simulation to assess estimators and tests

If you look at the code for the slides you will see that we simulate the design 5000 times, each time calculating an estimate and confidence interval for different estimators of the ATE.

What should we learn from this table? (Coverage? `sd_estimate` versus `mean_se`).

Table 1: Estimator and Test Performance in 5000 simulations of the cluster randomized design for different estimators and confidence intervals

estimator	coverage	sd_estimate	mean_se
Y~Z, CR2	0.57	1.07	0.73
Y~Z, cl_size fe, CR2	0.75	0.35	0.30
Y~Z, HC2	0.57	1.07	0.13
Y~Z, IID	0.57	1.07	0.12
Y~Z, weight=clus_size, CR2	0.57	1.22	0.85
Y~Z*I(cl_size-mean(cl_size)), CR2	0.75	1.60	0.06
Y~Z+cl_sizeF, CR2	0.75	0.35	0.30

Use simulation to assess estimators and tests

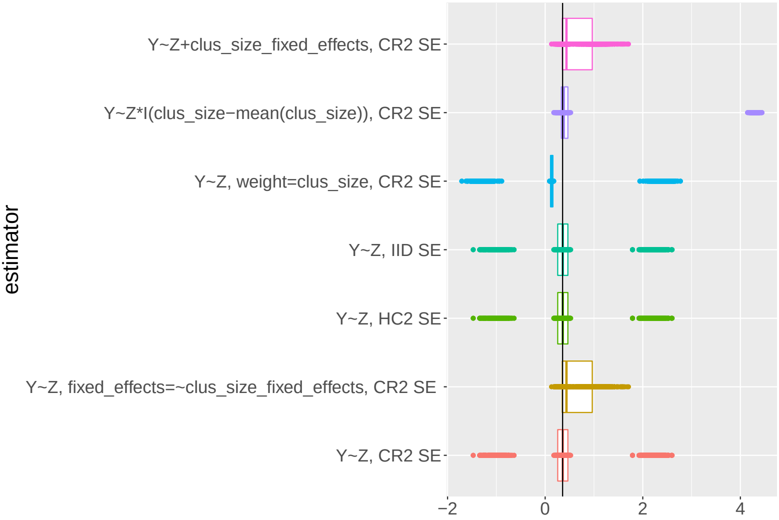
What should we learn from this table? (Bias? Closeness to truth?)

Table 2: Estimator and Test Performance in 5000 simulations of the cluster randomized design for different estimators and confidence intervals

estimator	bias	rmse
Y~Z, CR2	0.112	1.078
Y~Z, cl_size fe, CR2	0.279	0.451
Y~Z, HC2	0.112	1.078
Y~Z, IID	0.112	1.078
Y~Z, weight=clus_size, CR2	-0.038	1.223
Y~Z*I(cl_size-mean(cl_size)), CR2	0.815	1.792
Y~Z+cl_sizeF, CR2	0.279	0.451

Use simulation to assess estimators and tests

How should we interpret this plot?



Summary of estimation and testing in cluster-randomized trials

- Cluster randomized trials pose special problems for standard approaches to estimation and testing.
- If randomization is at the cluster level, then uncertainty arises from the cluster level randomization.
- If we have enough clusters, then one of the “cluster robust” standard errors can help us produce confidence intervals with correct coverage. **Cluster robust standard errors require many clusters**.
- If cluster size (or characteristic) is related to effect size, then we can have bias (and we need to adjust somehow).

Binary outcomes

Binary outcomes: Set up our data for simulation in DeclareDesign

```r
# population size
N <- 20
# declare the population
thepop_bin <- declare_population(
  N = N, x1 = draw_binary(prob = .5, N = N),
  x2 = rnorm(N)
)
# declare the potential outcomes
thepo_bin <- declare_potential_outcomes(Y ~ rbinom(
  n = N, size = 1,
  prob = 0.5 + 0.05 * Z + x1 * .05
))
# two possible targets: difference in means or difference in log-odds
thetarget_ate <- declare_inquiry(ate = mean(Y_Z_1 - Y_Z_0))
thetarget_logodds <- declare_inquiry(
  logodds = log(mean(Y_Z_1) / (1 - mean(Y_Z_1))) -
    log(mean(Y_Z_0) / (1 - mean(Y_Z_0)))
)
```

Binary outcomes: Set up our data for simulation in DeclareDesign

```r
# declare how treatment is assigned
# m units are assigned to levels of treatment Z
theassign_bin <- declare_assignment(Z = conduct_ra(N = N, m = floor(N / 3)))
# declare what outcome values are revealed for possible values of Z
thereveal_bin <- declare_reveal(Y, Z)
# pull this all together: population, potential outcomes, assignment,
# outcome values connected to Z
des_bin <- thepop_bin + thepo_bin + theassign_bin + thereveal_bin
# then make one draw (randomize treatment once)
set.seed(12345)
dat2 <- draw_data(des_bin)
```

Binary outcomes: Estimands I

How would we interpret the following true quantities or estimands? Y_Z_1, Y_Z_0 are potential outcomes, Y is observed, x1, x2 are covariates, Z is treatment assignment. Here N=20.

```
## Look at the first 6 observations only:
head(dat2[, -7])
```

```
  ID x1      x2 Y_Z_0 Y_Z_1 Z
1 01  1 -0.1162     0     1 0
2 02  1  1.8173     0     1 1
3 03  1  0.3706     0     1 0
4 04  1  0.5202     1     1 0
5 05  0 -0.7505     1     0 1
6 06  0  0.8169     0     1 0
```

Binary outcomes: Estimands II

How would we interpret the following true quantities or estimands? (`Y_Z_1`, `Y_Z_0` are potential outcomes, `Y` is observed, `x1`, `x2` are covariates, `Z` is treatment assignment. Here N=20.

```
ate_bin <- with(dat2, mean(Y_Z_1 - Y_Z_0))
bary1 <- mean(dat2$Y_Z_1)
bary0 <- mean(dat2$Y_Z_0)
diff_log_odds_bin <- with(
  dat2,
  log(bary1 / (1 - bary1)) - log(bary0 / (1 - bary0))
)
c(
  bary1 = bary1, bary0 = bary0, true_ate = ate_bin,
  true_diff_log_odds = diff_log_odds_bin
)
```

```
             bary1              bary0           true_ate true_diff_log_odds
              0.55               0.55               0.00               0.00
```

Binary outcomes: Estimands III

Do you want to estimate the difference in log-odds?

$$\delta = \log \frac{\bar{y}_1}{1 - \bar{y}_1} - \log \frac{\bar{y}_0}{1 - \bar{y}_0} \tag{1}$$

Or the difference in proportions?

$$\bar{\tau} = \bar{y}_1 - \bar{y}_0 \tag{2}$$

Recall that $\bar{y}_1$ is the *proportion* of $y_1 = 1$ in the data.

Freedman (2008b) shows us that the logit coefficient estimator is a biased estimator of the difference in log-odds estimand. He also shows an unbiased estimator of that estimand.

We know that the difference of proportions in the sample should be an unbiased estimator of the difference of proportions.

An example of estimation I

How should we interpret the following estimates? (What does the difference of means estimator require in terms of assumptions? What does the logistic regression estimator require in terms of assumptions?)

```r
lmbin1 <- lm_robust(Y ~ Z, data = dat2)
glmbin1 <- glm(Y ~ Z, data = dat2, family = binomial(link = "logit"))

tidy(lmbin1)[2, ]
```

```
  term estimate std.error statistic p.value conf.low conf.high df outcome
2    Z  -0.4048    0.2159    -1.875 0.07716  -0.8584   0.04884 18       Y
```

```r
tidy(glmbin1)[2, ]
```

```
# A tibble: 1 x 5
  term  estimate std.error statistic p.value
  <chr>    <dbl>     <dbl>     <dbl>   <dbl>
1 Z        -1.90      1.22     -1.55   0.120
```

An example of estimation II

What about with covariates? Why use covariates?

```
lmbin2 <- lm_robust(Y ~ Z + x1, data = dat2)
glmbin2 <- glm(Y ~ Z + x1, data = dat2, family = binomial(link = "logit"))

tidy(lmbin2)[2, ]
```

```
  term estimate std.error statistic p.value conf.low conf.high df outcome
2    Z  -0.4058    0.2179    -1.862 0.07996  -0.8656   0.05398 17       Y
```

```
tidy(glmbin2)[2, ]
```

```
# A tibble: 1 x 5
  term  estimate std.error statistic p.value
  <chr>    <dbl>     <dbl>     <dbl>   <dbl>
1 Z        -1.90      1.22     -1.55   0.120
```

An example of estimation III

Let's compare our estimates

```
c(
  dim = coef(lmbin1)[["Z"]],
  dim_x1 = coef(lmbin2)[["Z"]],
  glm = coef(glmbin1)[["Z"]],
  glm_x1 = coef(glmbin2)[["Z"]]
)
```

```
    dim  dim_x1     glm  glm_x1
-0.4048 -0.4058 -1.8971 -1.9025
```

An example of estimation: The Freedman plugin estimators I

No covariate:

```r
freedman_plugin_estfn1 <- function(data) {
  glmbin <- glm(Y ~ Z, data = dat2, family = binomial(link = "logit"))
  preddat <- data.frame(Z = rep(c(0, 1), nrow(dat2)))
  preddat$yhat <- predict(glmbin, newdata = preddat, type = "response")
  bary1 <- mean(preddat$yhat[preddat$Z == 1])
  bary0 <- mean(preddat$yhat[preddat$Z == 0])
  diff_log_odds <- log(bary1 / (1 - bary1)) - log(bary0 / (1 - bary0))
  return(data.frame(estimate = diff_log_odds))
}
```

An example of estimation: The Freedman plugin estimators II

With covariate:

```r
freedman_plugin_estfn2 <- function(data) {
  N <- nrow(data)
  glmbin <- glm(Y ~ Z + x1, data = data, family = binomial(link = "logit"))
  preddat <- data.frame(Z = rep(c(0, 1), each = N))
  preddat$x1 <- rep(data$x1, 2)
  preddat$yhat <- predict(glmbin, newdata = preddat, type = "response")
  bary1 <- mean(preddat$yhat[preddat$Z == 1])
  bary0 <- mean(preddat$yhat[preddat$Z == 0])
  diff_log_odds <- log(bary1 / (1 - bary1)) - log(bary0 / (1 - bary0))
  return(data.frame(estimate = diff_log_odds))
}
```

Let's compare our estimates from the six different estimators

```
      dim    dim_x1       glm    glm_x1  freedman freeman_x1
  -0.4048   -0.4058   -1.8971   -1.9025   -1.8971    -1.9020
```

An example of using DeclareDesign to assess our estimators I

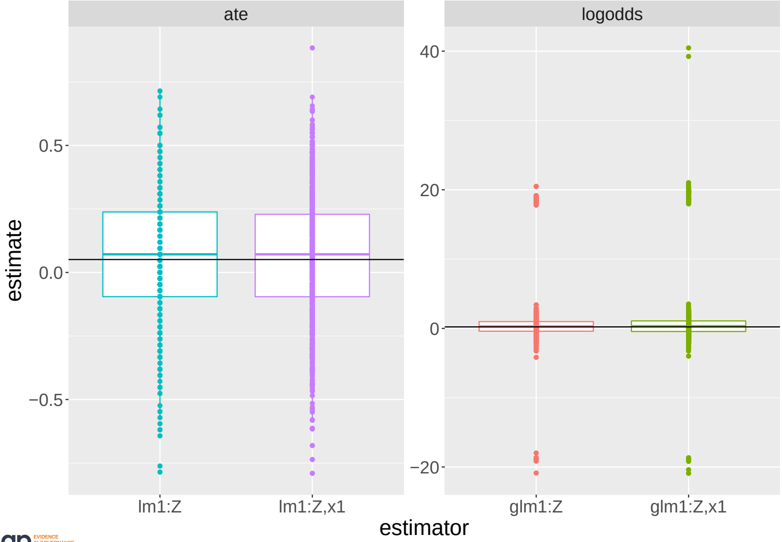
```r
# declare 4 estimators for DD
# first estimator: linear regression with ATE as target
estb1 <- declare_estimator(Y ~ Z,
  model = lm_robust, label = "lm1:Z",
  inquiry = thetarget_ate
)
# second estimator: linear regression with covariate, with ATE as target
estb2 <- declare_estimator(Y ~ Z + x1,
  model = lm_robust, label = "lm1:Z,x1",
  inquiry = thetarget_ate
)
# third estimator: logistic regression, with log odds as target
estb3 <- declare_estimator(Y ~ Z,
  model = glm, family = binomial(link = "logit"),
  label = "glm1:Z", inquiry = thetarget_logodds
)
# fourth estimtor: logistic regression with covariate, with log odds as target
estb4 <- declare_estimator(Y ~ Z + x1,
  model = glm, family = binomial(link = "logit"),
  label = "glm1:Z,x1", inquiry = thetarget_logodds
)
```

An example of using DeclareDesign to assess our estimators II

```r
# Pull together: des_bin is population, potential outcomes, assignment,
# outcome values connected to Z.  We add the two targets and four estimators.
des_bin_plus_est <- des_bin + thetarget_ate + thetarget_logodds +
  estb1 + estb2 + estb3 + estb4
```

Using simulation to assess our estimators

How should we interpret this plot? (Differences in scales make it difficult.)



Which estimator is closer to the truth?

Which estimator works better on this design and these data?

Table 3: Estimator and Test Performance in 5000 simulations of the different estimators and confidence intervals for a binary outcome and completely randomized design.

est	estimand	bias	rmse	power	coverage	sd_est	mean_se
glm1:Z	logodds	0.691	4.099	0.023	0.995	4.226	154.088
glm1:Z,x1	logodds	0.850	4.815	0.016	0.993	4.934	249.506
lm1:Z	ate	0.007	0.182	0.084	0.970	0.239	0.239
lm1:Z,x1	ate	0.010	0.189	0.082	0.970	0.245	0.247

Other topics in estimation

Covariance adjustment: Estimands

In general, simply “controlling for” produces a biased estimator of the ATE **or** ITT estimand. See for example Lin (2013) and Freedman (2008a). Lin (2013) shows how to reduce this bias and, importantly, that this bias tends to be small as the sample size increases.

Conclusion

Final thoughts on basics of estimation

- Counterfactual causal estimands are unobserved functions of potential outcomes.
- Estimators are recipes or computational formulas that use observed data to learn about an estimand.
- Good estimators produce estimates that are close to the true estimand
- (Connecting estimation with testing) Standard errors of estimators allow us to calculate confidence intervals and p-values. Certain estimators have larger or smaller (or more or less correct) standard errors.
- You can assess the utility of a chosen estimator for a chosen estimand by simulation.

Causal effects that differ by groups or covariates

Effects that differ by groups I

If our theory suggests that effects should differ by group, how can we assess evidence for or against such claims?

- We can **design** for an assessment of this theory by creating a block-randomized study — with blocked defined by the theoretically relevant groups.
- We can **plan** for such an assessment by (1) **pre-registering specific subgroup analyses** (whether or not we block on that group in the design phase) and (2) making sure to measure group membership during baseline data collection pre-treatment

Effects that differ by groups II

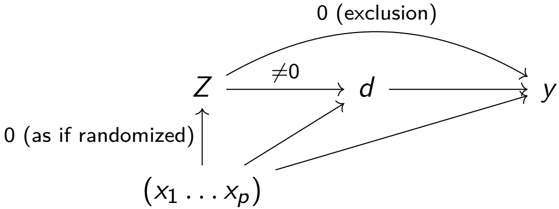
- If we have not planned ahead, subgroup-specific analyses can be useful as explorations but should not be understood as confirmatory: they can too easily create problems of testing too many hypotheses thus inflated false positive rates.
- We **should not use groups formed by treatment**. (This is either "mediation analysis" or "conditioning on post-treatment variables" and deserves its own module).

Causal effects when we do not control the dose

Defining causal effects I

Imagine a door-to-door communication experiment where some houses are randomly assigned to receive a visit. Note that we now use Z and d instead of T.

- Z_i is random assignment to a visit $(Z_i = 1)$ or not $(Z_i = 0)$.
- $d_{i,Z_i=1} = 1$ means that person i would open the door to have a conversation when assigned a visit.
- $d_{i,Z_i=1} = 0$ means that person i would not open the door to have a conversation when assigned a visit.
- Opening the door is an outcome of the treatment.



Defining causal effects II

- $y_{i,Z_i=1,d_{i,Z_i=1}=1}$ is the potential outcome for people who were assigned a visit and who opened the door. ("Compliers" or "Always-takers")
- $y_{i,1,d_{i,Z_i=1}=0}$ is the potential outcome for people who were assigned a visit and who did not open the door. ("Never-takers" or "Defiers")
- $y_{i,0,d_{i,0}=1}$ is the potential outcome for people who were not assigned a visit and who opened the door. ("Defiers" or "Always-takers")
- $y_{i,0,d_{i,0}=0}$ is the potential outcome for people who were not assigned a visit and who would not have opened the door. ("Compliers" or "Never-takers")

Defining causal effects III

We could also write $y_{i,Z_i=0,d_{i,Z_i=1}=1}$ for people who were not assigned a visit but who would have opened the door had they been assigned a visit etc.

In this case we can simplify our potential outcomes:

- $y_{i,0,d_{i,1}=1} = y_{i,0,d_{i,1}=0} = y_{i,0,d_{i,0}=0}$ because your outcome is the same regardless of how you don't open the door.

Defining causal effects IV

We can simplify the ways in which people get a dose of the treatment like so (where d is lower case reflecting the idea that whether you open the door when visited or not is a fixed attribute like a potential outcome).

- Y : outcome ($y_{i,Z}$ or $y_{i,Z_i=1}$ for potential outcome to treatment for person i, fixed)
- X : covariate/baseline variable
- Z : treatment assignment ($Z_i = 1$ if assigned to a visit, $Z_i = 0$ if not assigned to a visit)
- D : treatment received ($D_i = 1$ if answered phone, $D_i = 0$ if person i did not answer the door) (using D here because $D_i = d_{i,1}Z_i + d_{i,0}(1 - Z_i)$)

Defining causal effects V

We have two causal effects of Z: $Z \rightarrow Y$ (δ, ITT, ITT_Y), and $Z \rightarrow D$ (GG call this ITT_D).

And different types of people can react differently to the attempt to move the dose with the instrument.

		$Z = 1$	
		$D = 0$	$D = 1$
$Z = 0$	$D = 0$	Never taker	Complier
	$D = 1$	Defier	Always taker

Defining causal effects VI

The $ITT = ITT_Y = \delta = \bar{y}_{Z=1} - \bar{y}_{Z=0}$.

But, in this design, $\bar{y}_{Z=1} = \bar{y}_1$ is split into pieces: the outcome of those who answered the door (Compliers and Always-takers and Defiers). Write p_C for the proportion of compliers in the study.

$$\bar{y}_1 = (\bar{y}_1|C)p_C + (\bar{y}_1|A)p_A + (\bar{y}_1|N)p_N + (\bar{y}_1|D)p_D. \tag{3}$$

And $\bar{y}_0$ is also split into pieces:

$$\bar{y}_0 = (\bar{y}_0|C)p_C + (\bar{y}_1|A)p_A + (\bar{y}_0|N)p_N + (\bar{y}_0|D)p_D. \tag{4}$$

Defining causal effects VII

So, the ITT itself is a combination of the effects of Z on Y within these different groups (imagine substituting in and then re-arranging so that we have a set of ITTs, one for each type of subject). But, we can still estimate it because we have unbiased estimators of $\bar{y}_1$ and $\bar{y}_0$ within each type.

Learning about the ITT I

First, let's learn about the effect of the policy itself. To write down the ITT, we do not need to consider all of the types above. We have no defiers ($p_D = 0$) and we know the ITT for both Always-takers and Never-takers is 0.

$$\bar{y}_1 = (\bar{y}_1|C)p_C + (\bar{y}_1|A)p_A + (\bar{y}_1|N)p_N \tag{5}$$

$$\bar{y}_0 = (\bar{y}_0|C)p_C + (\bar{y}_0|A)p_A + (\bar{y}_0|N)p_N \tag{6}$$

Learning about the ITT II

First, let's learn about the effect of the policy itself. To write down the ITT, we do not need to consider all of the types above. We have no defiers ($p_D = 0$) and we know the ITT for both Always-takers and Never-takers is 0.

$$
\begin{aligned}
ITT =& \bar{y}_1 - \bar{y}_0 && (7)\\
=& ((\bar{y}_1|C)p_C + (\bar{y}_1|A)p_A + (\bar{y}_1|N)p_N) - && (8)\\
& ((\bar{y}_0|C)p_C + (\bar{y}_0|A)p_A + (\bar{y}_0|N)p_N) && (9)
\end{aligned}
$$

collecting each type together — to have an ITT for each type

$$
\begin{aligned}
=& ((\bar{y}_1|C)p_C - (\bar{y}_0|C)p_C) + ((\bar{y}_1|A)p_A - (\bar{y}_1|A)p_A) + && (10)\\
& ((\bar{y}_1|N)p_N - (\bar{y}_0|N)p_N) && (11)\\
=& \left((\bar{y}_1|C) - (\bar{y}_0|C)\right) p_C + && (12)\\
& \left((\bar{y}_1|A) - (\bar{y}_0|A)\right) p_A + \left((\bar{y}_1|N) - (\bar{y}_0|N)\right) p_N && (13)
\end{aligned}
$$

Learning about the ITT III

$$
\begin{aligned}
ITT =& \bar{y}_1 - \bar{y}_0 && (14)\\
=& ((\bar{y}_1|C)p_C + (\bar{y}_1|A)p_A + (\bar{y}_1|N)p_N) - && (15)\\
& ((\bar{y}_0|C)p_C + (\bar{y}_0|A)p_A + (\bar{y}_0|N)p_N) && (16)\\
=& ((\bar{y}_1|C)p_C - (\bar{y}_0|C)p_C) + ((\bar{y}_1|A)p_A - (\bar{y}_1|A)p_A) + && (17)\\
& ((\bar{y}_1|N)p_N - (\bar{y}_0|N)p_N) && (18)\\
=& ((\bar{y}_1|C) - (\bar{y}_0|C))p_C + ((\bar{y}_1|A) - (\bar{y}_0|A))p_A + && (19)\\
& ((\bar{y}_1|N) - (\bar{y}_0|N))p_N && (20)
\end{aligned}
$$

Learning about the ITT IV

And, if the effect of the dose can only occur for those who open the door, and you can only open the door when assigned to do so then:

$$((\bar{y}_1|A) - (\bar{y}_0|A))p_A = 0 \text{ and } ((\bar{y}_1|N) - (\bar{y}_0|N))p_N = 0 \tag{21}$$

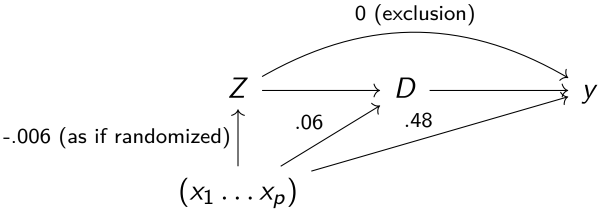
And

$$ITT = ((\bar{y}_1|C) - (\bar{y}_0|C))p_C = (CACE)p_C. \tag{22}$$

The complier average causal effect I

We would also like to learn about the causal effect of answering the door and having the conversation, the theoretically interesting effect.

But this comparison is confounded by x: a simple $\bar{Y}|D=1-\bar{Y}|D=0$ comparison tells us about differences in the outcome due to x in addition to the difference caused by D. (Numbers below from some simulated data)



The complier average causal effect II

```
with(dat, cor(Y, x)) ## can be any number
with(dat, cor(d, x)) ## can be any number
with(dat, cor(Z, x)) ## should be near 0
```

But we just saw that, in this design, and with these assumptions (including a SUTVA assumption) that $ITT = ((\bar{y}_1|C) - (\bar{y}_0|C))p_C = (CACE)p_C$, so we can define $CACE = ITT/p_C$.

How to calculate the ITT and CACE/LATE I

Some example data (where we know all potential outcomes):

X	u	type	Z	pZ	DZ1	YD0Z0	YD1Z0	YD0Z1	YD1Z1	D	
4	1.95	Complier	0	0	1	1.95	2.52	1.95	2.52	0	1
2	0.05	Complier	1	0	1	0.05	0.63	0.05	0.63	1	0

How to calculate the ITT and CACE/LATE II

The ITT and CACE (the parts)

```r
itt_y <- difference_in_means(Y ~ Z, data = dat0)
itt_y
```

```
Design:  Standard
  Estimate Std. Error t value Pr(>|t|) CI Lower CI Upper    DF
Z  0.08725      0.233  0.3745   0.7089  -0.3752   0.5497 97.97
```

```r
itt_d <- difference_in_means(D ~ Z, data = dat0)
itt_d
```

```
Design:  Standard
  Estimate Std. Error t value  Pr(>|t|) CI Lower CI Upper    DF
Z     0.68    0.07307   9.307 8.454e-15   0.5348   0.8252 89.31
```

How to calculate the ITT and CACE/LATE III

All together:[1]

```r
cace_est <- iv_robust(Y ~ D | Z, data = dat0)
cace_est
```

```
            Estimate Std. Error t value Pr(>|t|) CI Lower CI Upper DF
(Intercept)   0.3347     0.1912  1.7502  0.08321 -0.04479   0.7142 98
D             0.1283     0.3404  0.3769  0.70705 -0.54727   0.8039 98
```

```r
## Notice same as below:
coef(itt_y)[["Z"]] / coef(itt_d)[["Z"]]
```

```
[1] 0.1283
```

[1] works when $Z \to D$ is not weak see Imbens and Rosenbaum (2005) for a cautionary tale

Summary of Encouragement/Complier/Dose oriented designs:

- Analyze as you randomized, even when you don't control the dose
- The danger of per-protocol analysis.

References

Freedman, David A. 2008a. "On regression adjustments to experimental data." *Advances in Applied Mathematics* 40 (2): 180–93.

———. 2008b. "Randomization Does Not Justify Logistic Regression." *Statistical Science* 23 (2): 237–49.

Imbens, Guido W., and Paul R. Rosenbaum. 2005. "Robust, Accurate Confidence Intervals with a Weak Instrument: Quarter of Birth and Education." *Journal of the Royal Statistical Society Series A* 168 (1): 109–26.

Lin, Winston. 2013. "Agnostic Notes on Regression Adjustments to Experimental Data: Reexamining Freedman's Critique." *The Annals of Applied Statistics* 7 (1): 295–318.