

Randomized trials w/ non-compliance

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Setup: Randomize trial (RT)

Z ... randomization to treatment (e.g. 1 if we randomize to treatment, 0 if we randomize to placebo/no treatment)

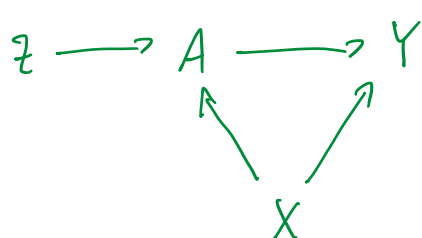
A ... treatment received (1 if receive treatment, 0 otherwise)

Y ... outcome

NOTE: Not everyone assigned to treatment ($A=1$) will actually receive the treatment (non-compliance) b/c we could have ($Z=0$).

DAG:

- non-compliance makes the RT like an observational study (i.e. we might have confounding).
- there could be confounding based on treatment received.
- reasonable to assume that treatment assignment does not directly affect outcome Y .



step 1: use X to determine treatment.

step 2: draw random Z to choose whether or not to apply advised treatment from step 1.

Potential treatment:

- observed data: (Z, A, Y)
- for a given subject: they were assigned treatment Z and received treatment A

A ... observed treatment

Z ... assigned treatment

- each subject has 2 potential values of treatment:

1. $A^{z=1}$: value of treatment if randomized to $z=1$.

2. $A^{z=0}$: value of treatment if randomized to $z=0$.

\Rightarrow every subject has 2 potential treatments: (A^0, A^1) & the value of Z determines the actual received treatment

Average Causal Effect of treatment assignment on treatment received:

- pop. causal effect b/c we don't condition on any variables
 $E(A^1 - A^0)$ (equals to 1 under perfect compliance)

\hookrightarrow estimable from observed data b/c:

$$\underbrace{E(A^1) = E(A|Z=1)}_{\text{b/c of randomization of } Z} \quad \& \quad E(A^0) = E(A|Z=0)$$

Perfect compliance iff $A^1=1$ \forall patients and $A^0=0$ \forall patients.

- **Causal Effect:** Contrast in potential outcomes on a common population.

- Consistency needed to estimate $E(A^1 - A^0)$ from observed data:

$$\hookrightarrow A^1 = A \text{ if } Z=1 \quad \& \quad A^0 = \bar{A} \text{ if } Z=0$$

Average causal effect of treatment assignment on the outcome:

- $E(Y^{Z=1} - Y^{Z=0})$: "intention-to-treat" effect

+ $Z=1$ means subject is assigned to receive treatment.

+ $Z=0$ means " " not receive treatment.

- if perfect compliance, then $E(Y^{Z=1} - Y^{Z=0}) \equiv$ causal effect of the treatment

- $E(Y^{Z=1} - Y^{Z=0})$ is identifiable/estimable from observed data, as, by randomization and consistency:

$$\underbrace{E(Y^{Z=1})}_{\text{avg. value of the outcome, had everyone in the population be assigned } Z=1.} = E(Y|Z=1), \quad E(Y^{Z=0}) = E(Y|Z=0)$$

both are observed data

What about causal effect of treatment received on the outcome?

- the instrumental variable Z can be thought of as strong encouragement to receive treatment (i.e. $Z=1 \rightarrow A=1$, $Z=0 \rightarrow A=0$)

- Z determines treatment assignment. As such, it affects treatment received.

- but Z does not affect the outcome directly.