



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

PCORI Methodology Standards: Academic Curriculum



JOHNS HOPKINS
SCHOOL *of* MEDICINE



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Module 3a: Defining Causal Effects

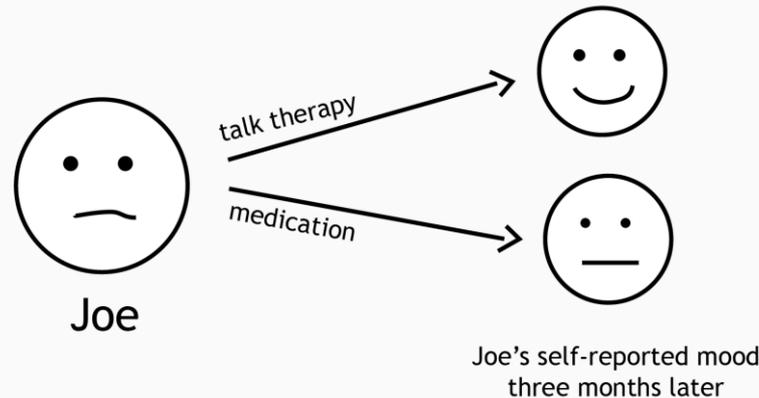
Category 8: Causal Inference Methods

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What Do We Mean by a Causal Effect?

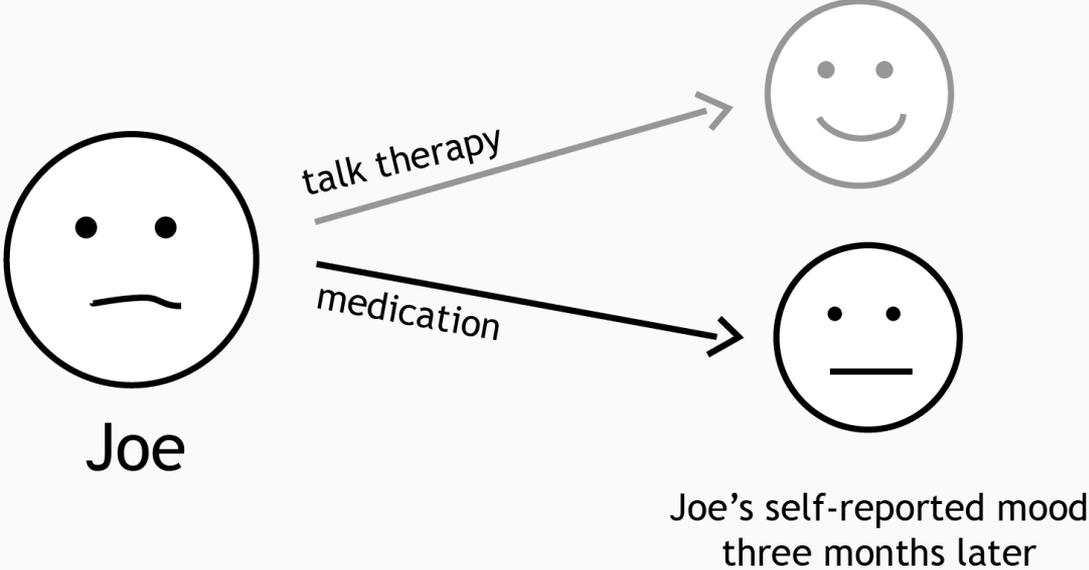
- Does some treatment or intervention lead to a different outcome, relative to the outcome under a different treatment or intervention?
- Imagine Joe, a person with depression. Joe could choose talk therapy or medication.
 - ▶ What is the causal effect of talk therapy vs. medication on his self-reported mood three months later?



More Formally

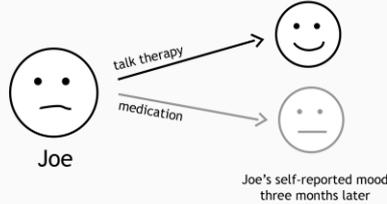
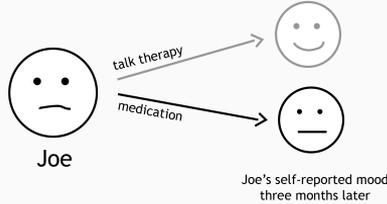
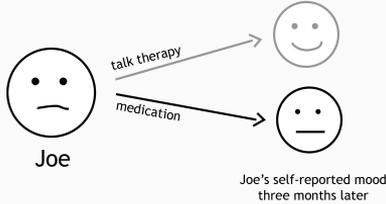
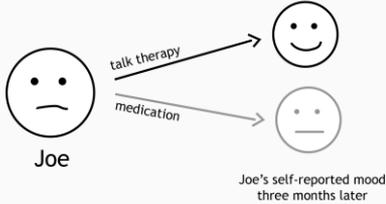
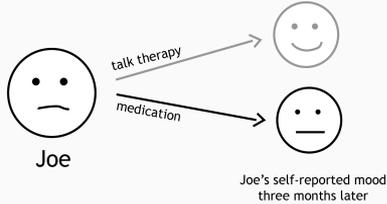
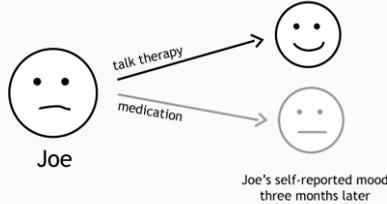
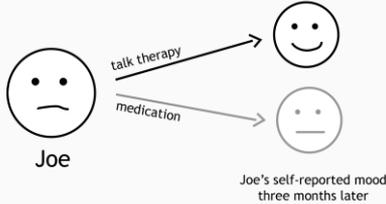
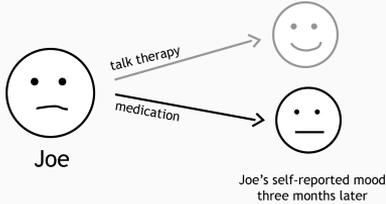
- More formally, a causal effect is defined as a comparison of *potential outcomes*
 - ▶ The outcome someone would have if they had received one treatment
 - ▶ The outcome someone would have if they had received another treatment
- The challenge is that, for a given person at a given point in time, we can see only one of these potential outcomes
 - ▶ The other is “counterfactual”
 - ▶ Joe either takes the medication at this point in time, or he doesn’t

We See Only One Potential Outcome for Each Unit



Multiple Units: Replication

- The way we can learn about causal effects is through “replication”
 - ▶ Multiple people—some who get the treatment, and others who get the comparator
 - ▶ Multiple time points on the same person, with some time points treated and others control



Defining the Population of Interest

- It is very difficult to learn about individual causal effects
- But we can make progress in terms of estimating average effects across a population
 - ▶ For example, what would the average mortality rate be if everyone in a physician's practice were to receive case management services, as compared to the average mortality rate had they not?
 - ▶ For example, how would the average self-reported pain score in a group of patients with severe back pain receiving surgery compare to their pain score had they received medication?

Need for Clear Definitions

- When defining causal effects, it is crucial to clearly define:
 - ▶ The treatment and comparison conditions of interest
 - ▶ The units (population) of interest
 - ▶ The outcomes of interest and precisely how and when they will be measured
- Retain temporal ordering
 - ▶ Membership in the population of interest cannot depend on outcomes or anything that may be affected by the treatment or comparison conditions (Standards CI-1, CI-2)
 - ▶ Outcomes are measured after the treatment or comparison condition is “given” (Standard CI-3)

Example: Treatment of Gestational Diabetes

- Population of interest: pregnant women (ages 15-45) with gestational diabetes mellitus
 - ▶ Used data from a nationwide US employer-based private insurance claims database
- Treatment condition: glyburide given during pregnancy
- Comparison condition: insulin given during pregnancy
- Outcomes of interest: measured at birth or later, including obstetric trauma, cesarean delivery, birth injury, preterm birth, hospitalization in the NICU

Conclusions

- Causal effects are a comparison of potential outcomes
- The challenge of causal inference is that we can't observe all potential outcomes
 - ▶ See only the treatment condition an individual actually receives
 - ▶ Do not see the other potential outcome