Causal inference and observational studies – the role of the propensity score

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Causal inference

Estimation of the effect of an exposure/treatment/intervention.

- Medical/surgical interventions
- Vaccines
- Genetic factors

Potential outcomes framework

Each subject has a pair of potential outcomes:

- Y(0) outcome under control treatment.
- Y(1) outcome under active treatment.

Y(1) – Y(0) is the effect of treatment.

Each subject receives one treatment: Z = 0 vs. Z = 1.

We only observe Y: the outcome under the actual treatment received.

Causal inference is thereby reduced to a type of missing-data problem.

What is an RCT estimating?

Under randomization we have that:

The average treatment effect = $E[Y(1) - Y(0)] =$

$E[Y(1)] - E[Y(0)] = E[Y \mid Z = 1] - E[Y \mid Z = 0]$.

Randomization allows unbiased estimation of the average treatment effect.

Observational studies: confounding

In observational studies, there are often systematic differences in the distribution of baseline characteristics between treated and untreated subjects.

Therefore, observed differences in outcomes between treatment groups may be due, in part (or entirely), to differences in the distribution of baseline covariates.

Furthermore, $E[Y(1)] \neq E[Y \mid Z = 1]$

The propensity score

- The propensity score is the probability of treatment assignment conditional on observed baseline covariates: e = Pr(Z=1|X).
- The propensity score is a balancing score:
  - Treated and untreated subjects with the same value of the propensity score will have the same distribution of measured baseline covariates.
  - Comparing outcomes between treated and untreated subjects with similar values of the propensity score allows one to remove the effect of confounding due to measured covariates in observational studies.

Estimating the treatment effect

There are four methods of using the propensity score for estimating treatment effects:

- Matching on the propensity score.
- Stratification on the propensity score.
- Inverse probability of treatment weighting using the propensity score (IPTW).
- ~~Covariate adjustment using the propensity score~~.

Advantages of PS methods (1)

- Matching, weighting and stratification allow one to separate the design of a study from the analysis of a study.
  - In RCTs the separation of design from analysis protects one from introducing bias.
  - Conventional regression adjustment in observational studies does not have this separation.
    - Temptation to work towards the desired/anticipated results.

Advantages of PS methods (2)

More transparent analyses:

- Can explicitly illustrate the comparability of the treated and control subjects.
  - Can compare treated and untreated subjects in the matched/weighted sample.
  - Can compare treated and untreated subjects within propensity score strata.
- With conventional regression adjustment it is difficult to assess whether the analyst has adequately accounted for confounding.

Advantages of PS methods (3)

Can estimate the same measures of effect as are used in RCTs.

- PS methods can estimate both relative and absolute measures of effect:
  - Relative risks
  - Absolute risk reductions
  - Numbers needed to treat/harm.
- Conventional regression adjustment produces only adjusted odds ratios or hazard ratios.

Estimating the PS – new directions

- Usually estimated using a logistic regression model in which treatment selection is regressed on baseline covariates.

- There is a small literature on the use of machine-learning methods for this purpose:
  - Random forests
  - Generalized boosting methods

Extra slides

Average treatment effects

- The average treatment effect (ATE):
  - The average effect of treatment in the population.
  - $E[Y(1) - Y(0)]$
- The average treatment effect in the treated (ATT):
  - The average effect of treatment in those subjects who were ultimately treated.
  - $E[Y(1) - Y(0) \mid Z = 1]$

Propensity score theory

If treatment assignment is strongly ignorable, then, at any value of a balancing score, the difference between the treated and control means is an unbiased estimate of the average treatment effect at that value of the balancing score:

$$E[Y(1)-Y(0) \mid b(\mathbf{x})] = E[Y(1) \mid b(\mathbf{x}), z=1] - E[Y(0) \mid b(\mathbf{x}), z=0]$$

Strongly ignorable treatment assignment

Given a pair of potential outcomes (Y(1),Y(0)), treatment assignment (Z) is strongly ignorable given a vector of covariates *x* if

- $(Y(1),Y(0)) \perp\!\!\!\perp z \mid x$ (No unmeasured confounding)

- $0 < \Pr(z=1 \mid x) < 1$ (Positivity)

Stable Unit Treatment Value Assumption (SUTVA)

The assumption that the value of Y for a unit *u* when exposed to treatment *t* will be the same no matter what mechanism is used to assign treatment *t* to unit *u* and not matter what treatments the other units receive

(Rubin)