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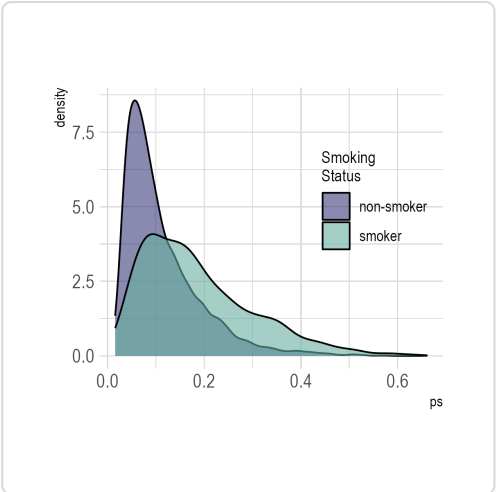

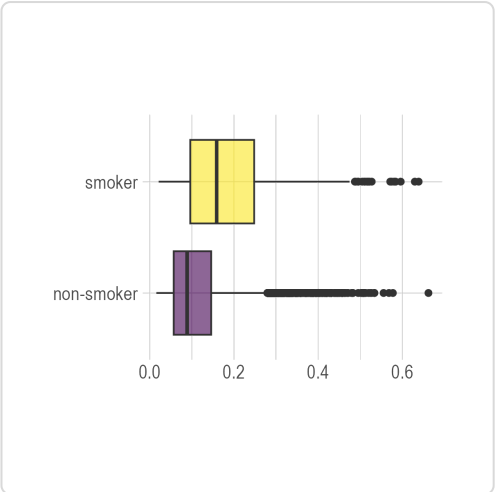

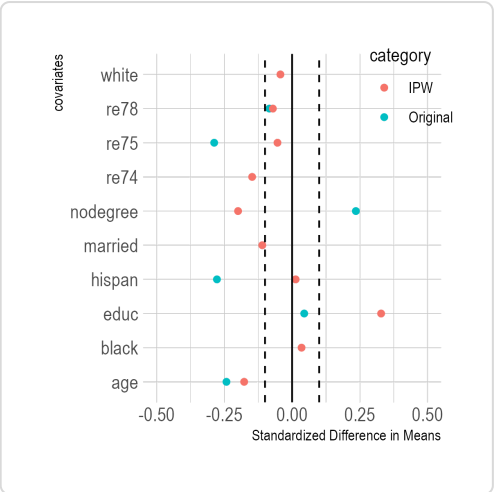

Propensity Score in Pharmacoepidemiolgy

Ignacio Leiva-Escobar, MSc

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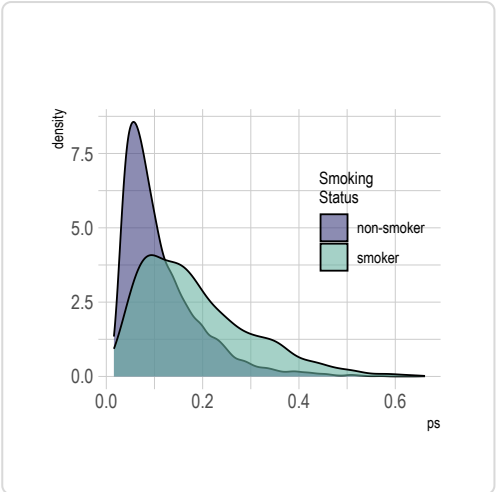
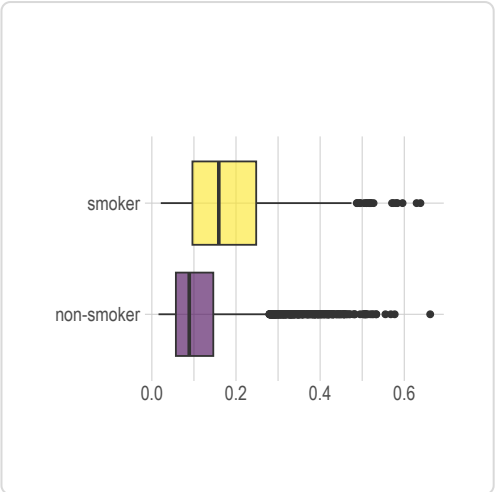
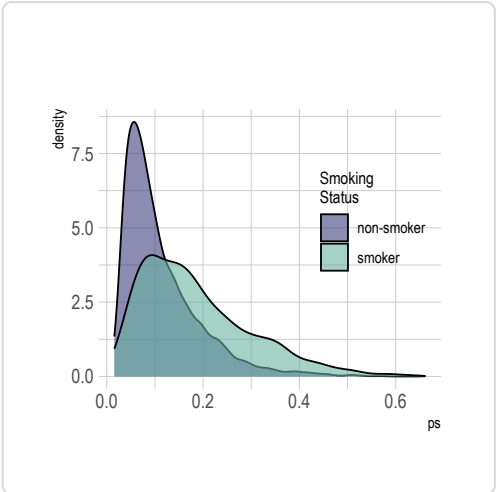






Propensity Score in Pharmacoepidemiolgy

Ignacio Leiva-Escobar, MSc

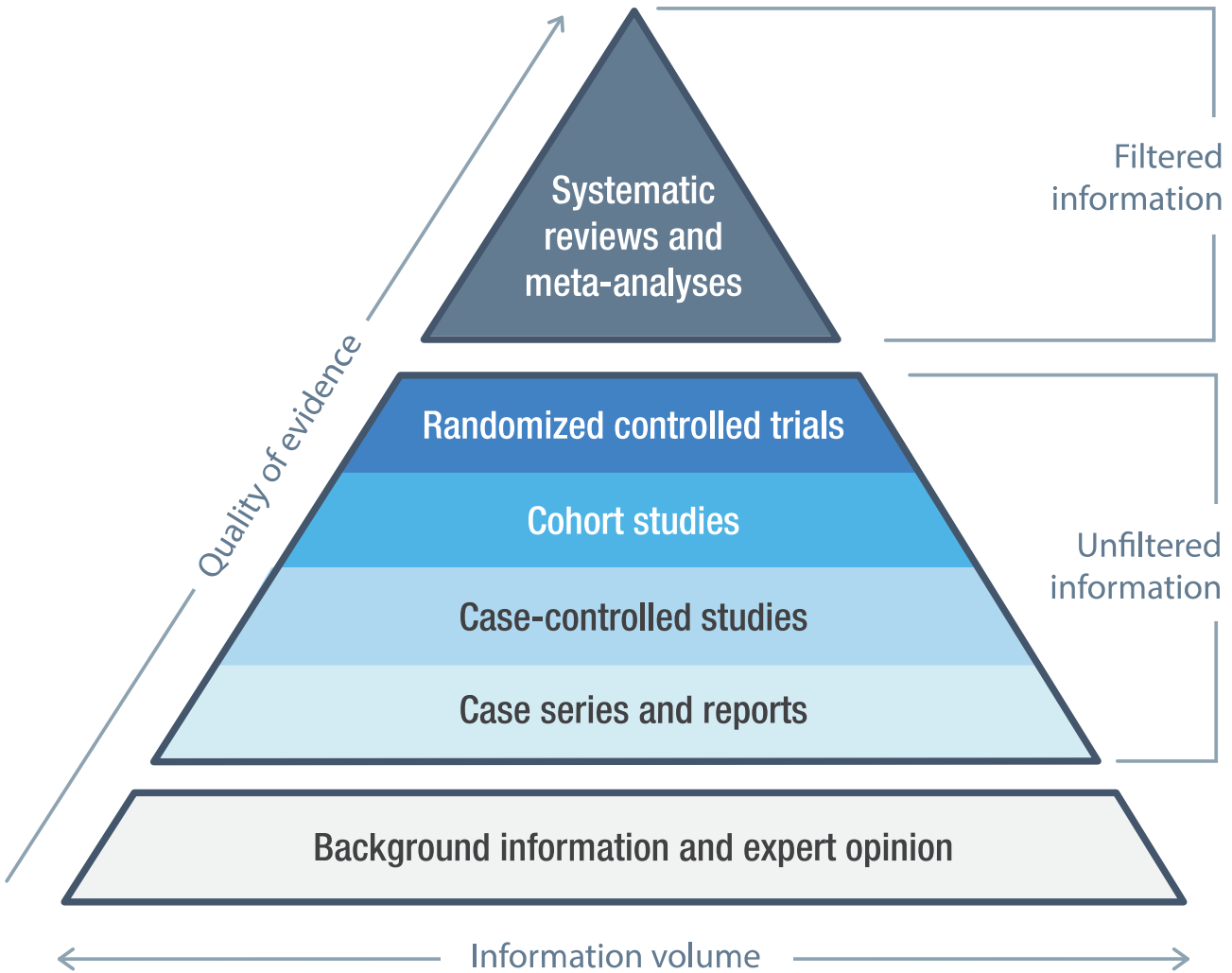
Goals

> **Briefly introduce the concept of casal effect**

> **Understand the role of propensity score in pharmacoepidemiology**

Causal Effect Estimation

Level of evidence



Source: https://openmd.com

Causal Effect Estimation

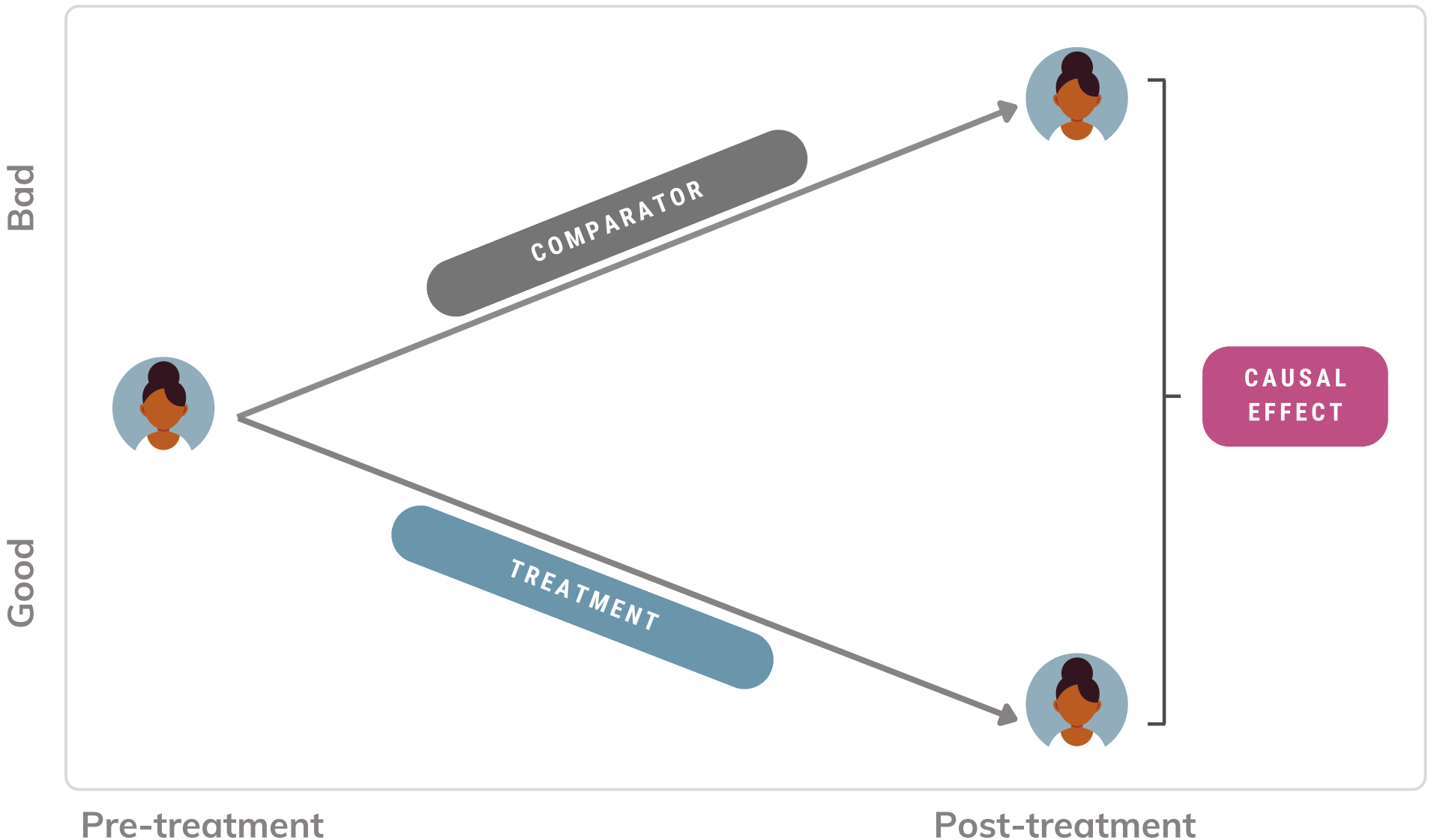
`Causal effect` are of interest in many field

- Prove ***causation*** over merely ***correlation***

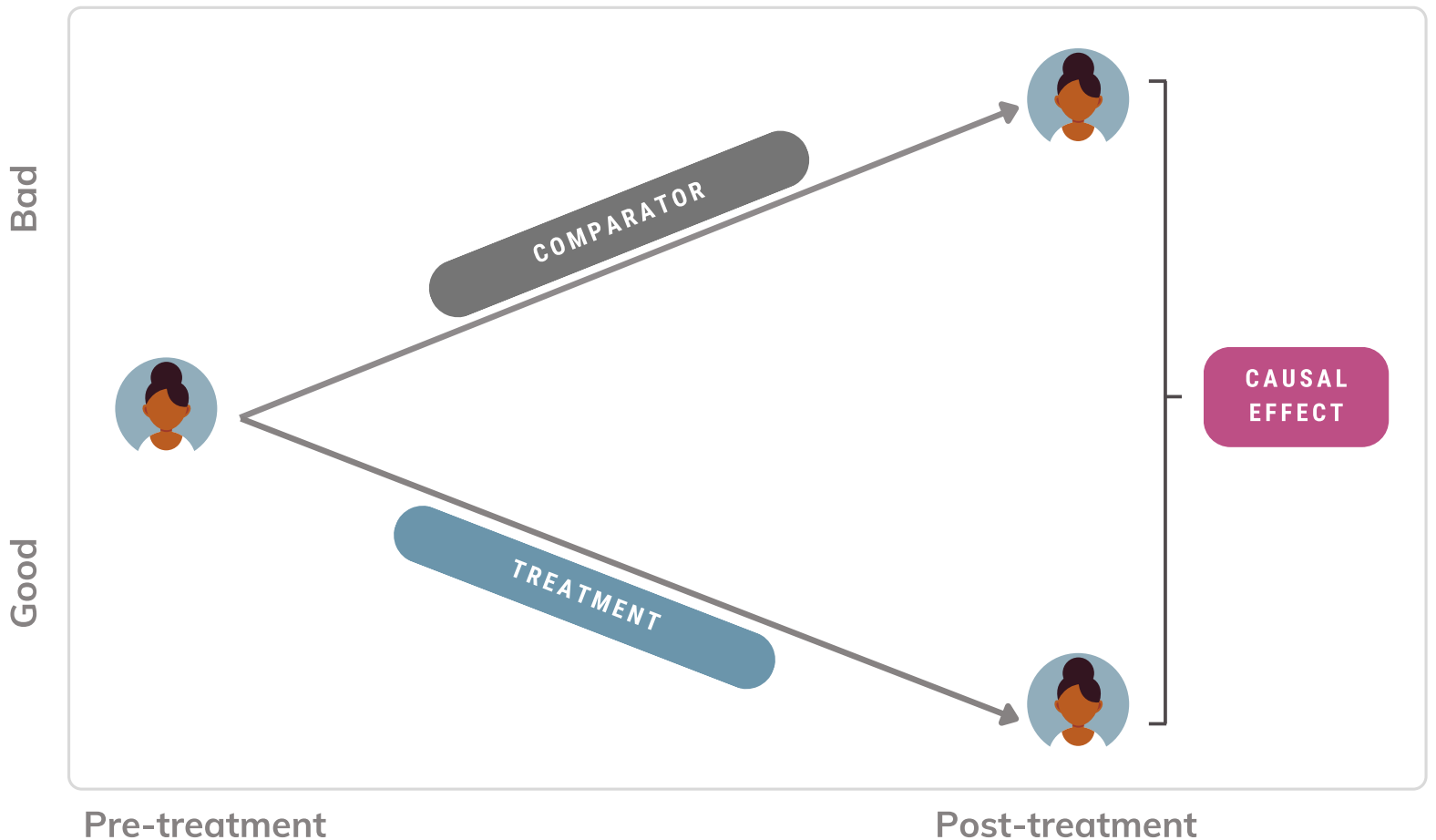
Causal effect describes the `effect` of an exposure on an outcome

- New treatment
- Prevention program
- New policy

How to get causal effect estimates

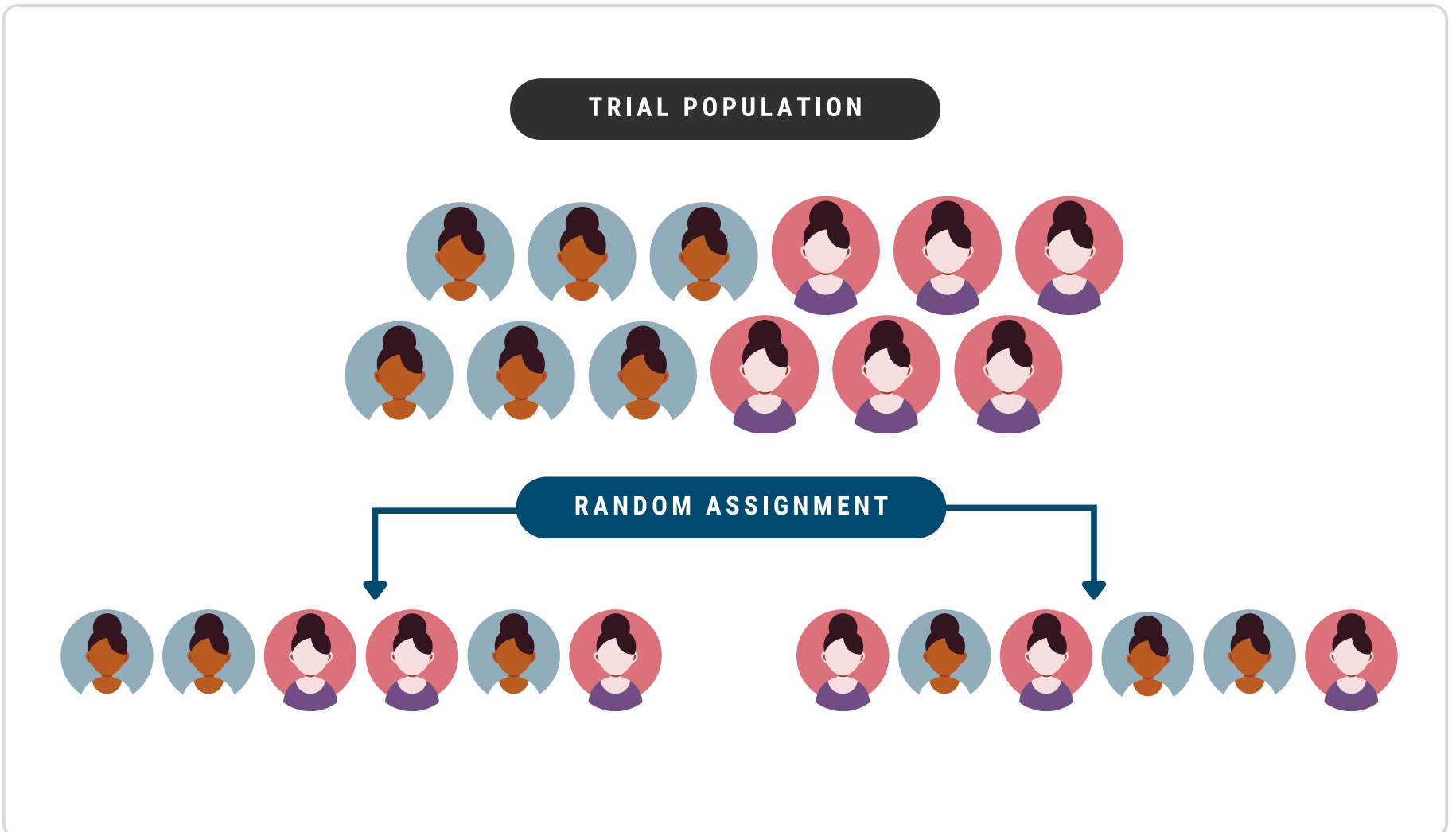


How to get causal effect estimates

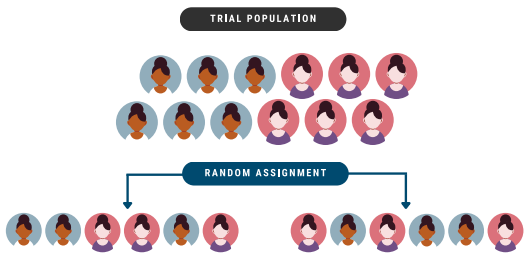


Fundamental problem of causa inference

Random Assignment



Random Assignment

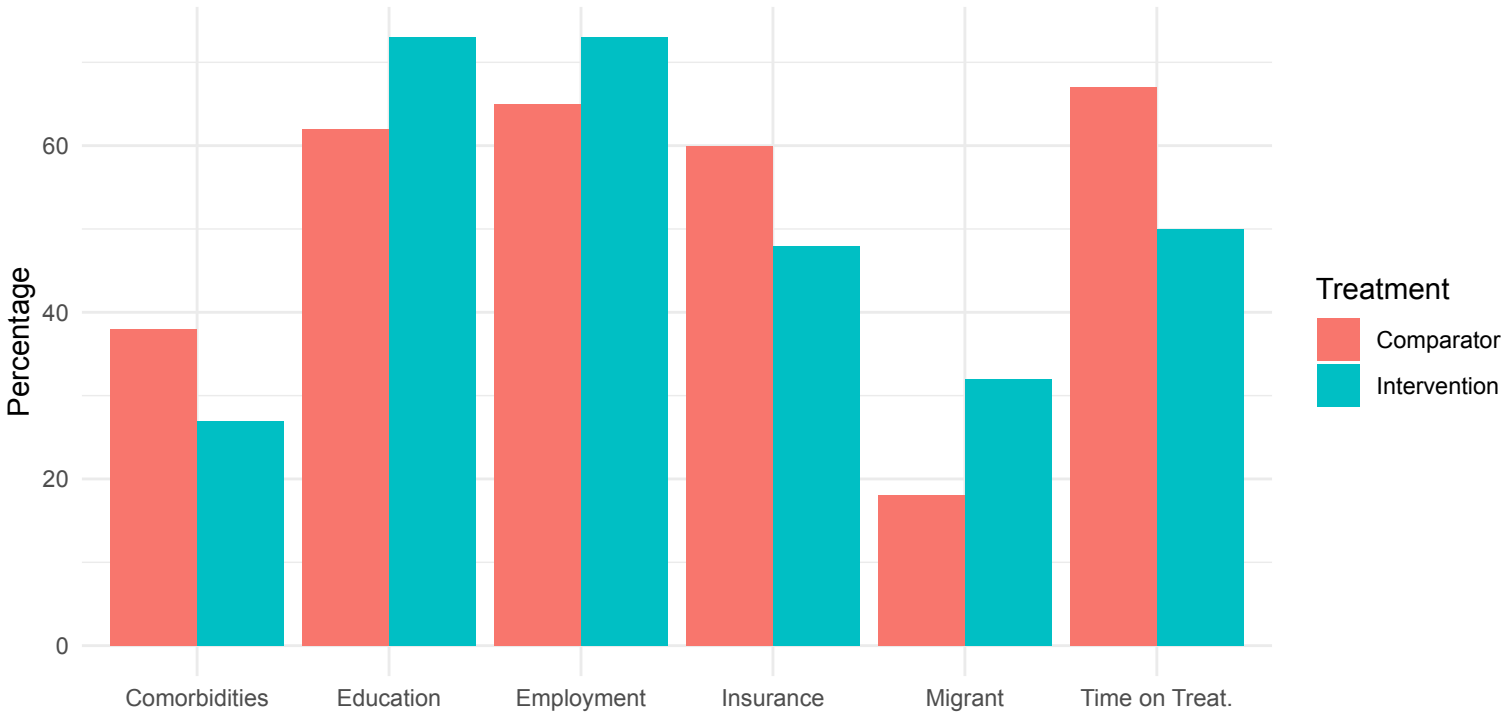


Gold standard for estimating causal effects:

- Randomization (if it works) makes groups being compared balance on baseline characteristics. (observed and unobserved)
- Treatment assignment is unrelated to potential outcomes (ignorability assumption)

Observational studies

- An alternative when randomization is not always feasible
- Selection bias is a major concern (imbalanced groups)



Observational studies

- An alternative when randomization is not always feasible
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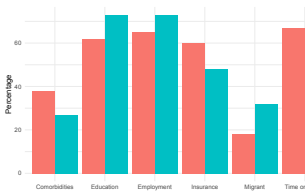


Observational studies provide another way to get causal effects:

- Treatment assignment is not controlled by the researcher
- Groups being compared are usually imbalanced
- Causal inference methods to try to replicate what a randomised study does

Propensity Score

Propensity scores

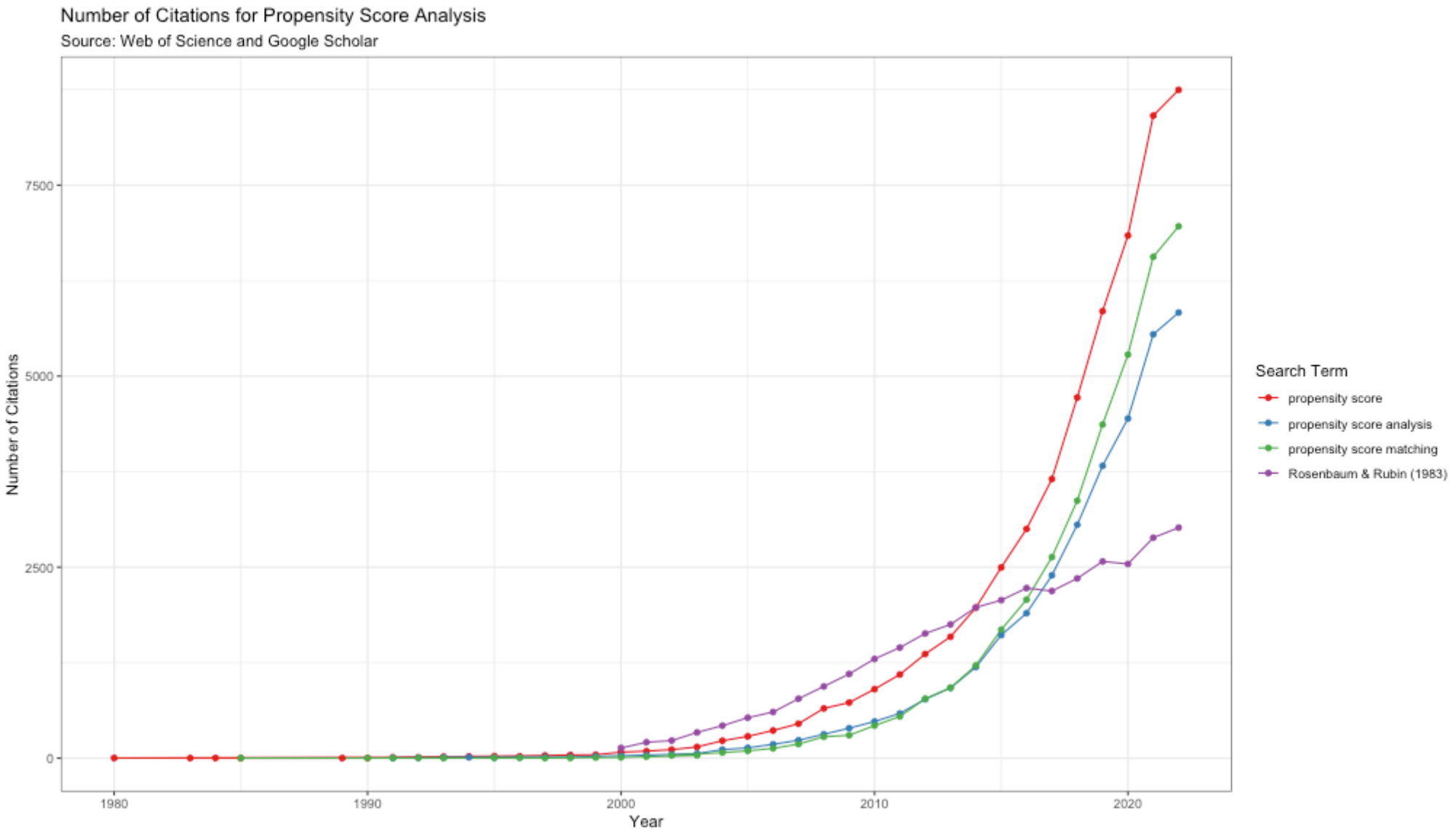
Propensity scores help us deal with the challenge of selection.

The propensity score is an individual's probability of receiving the treatment given pretreatment characteristics[1]

$$P(X) = Pr(Z = 1|X)$$

Used to create balance between treatment and comparison conditons. Balancing on $P(X)$ can balance distribution of X between treatment and comparison group.

Popularity of PS

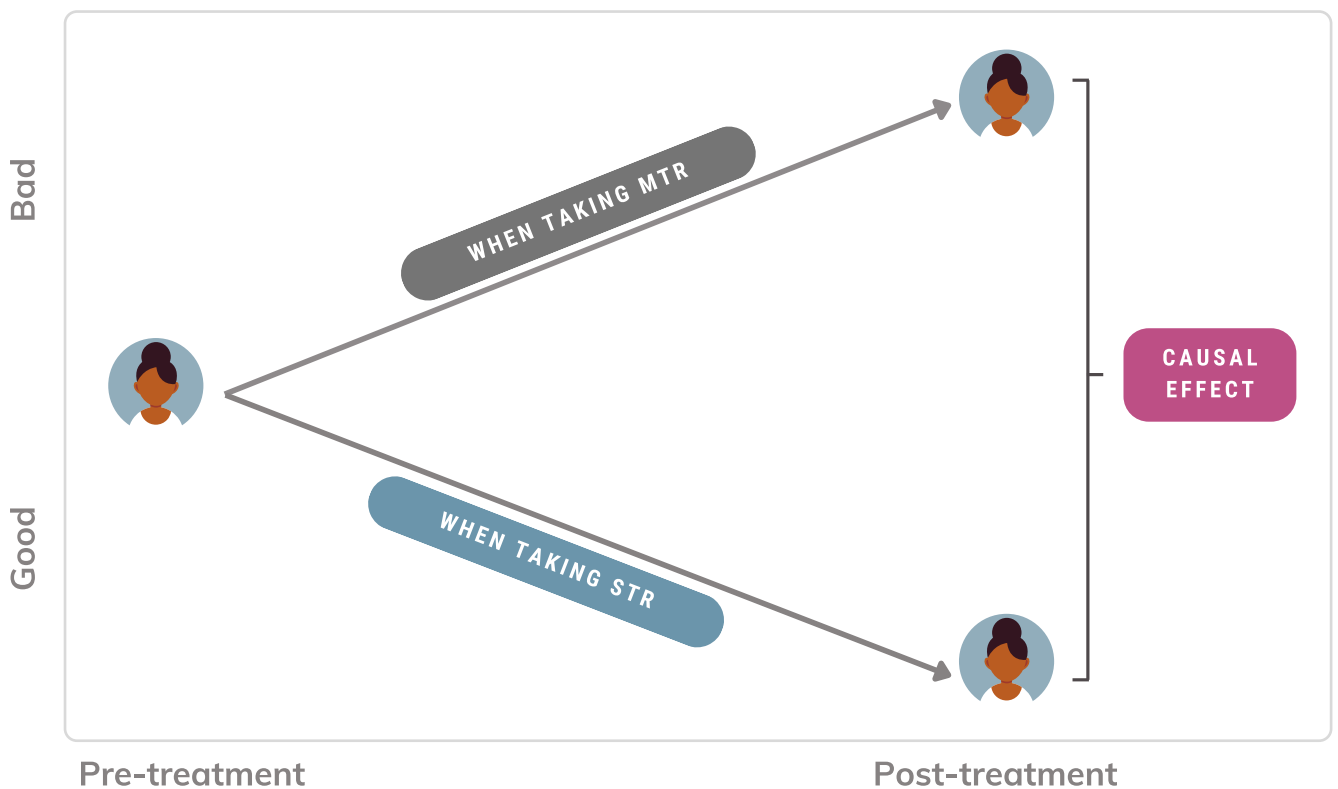


Motivating example

- Single-tablet regimens (STR) are associated with higher adherence rates, decreased hospitalizations, and a higher proportion of patients achieving undetectable viral loads compared to multi-tablet regimens (MTR)[1]
- STRs can lead to improved therapy satisfaction, better symptom control, enhanced health status, reduced healthcare resource utilization, and cost-effectiveness compared to MTRs[2]

Motivating example

The aim is to investigate whether a single-tablet regimens (STR) affects adherence and HIV viral supression, in comparison with multi-tablet regimens (MTR).

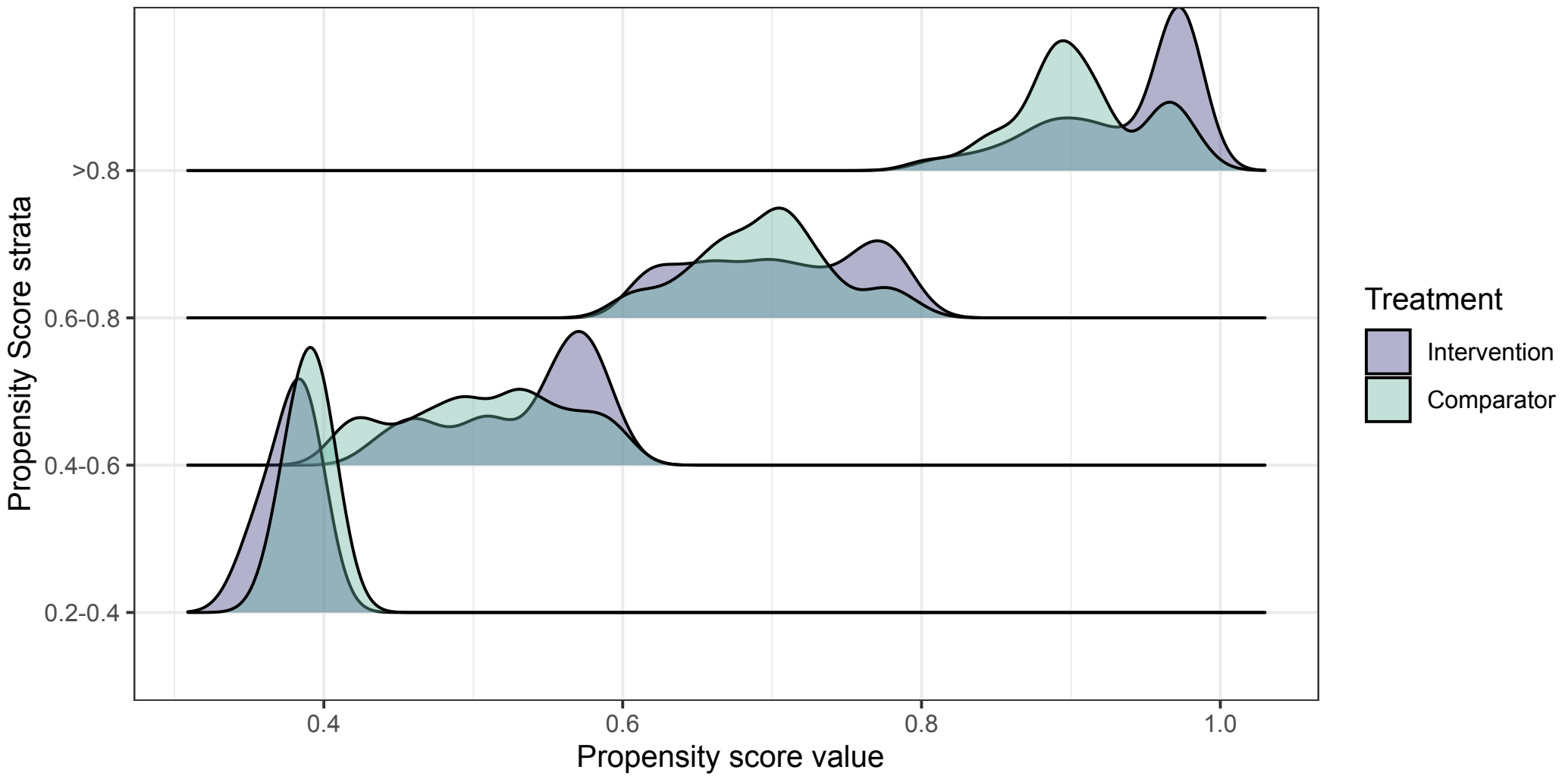


Application of PS

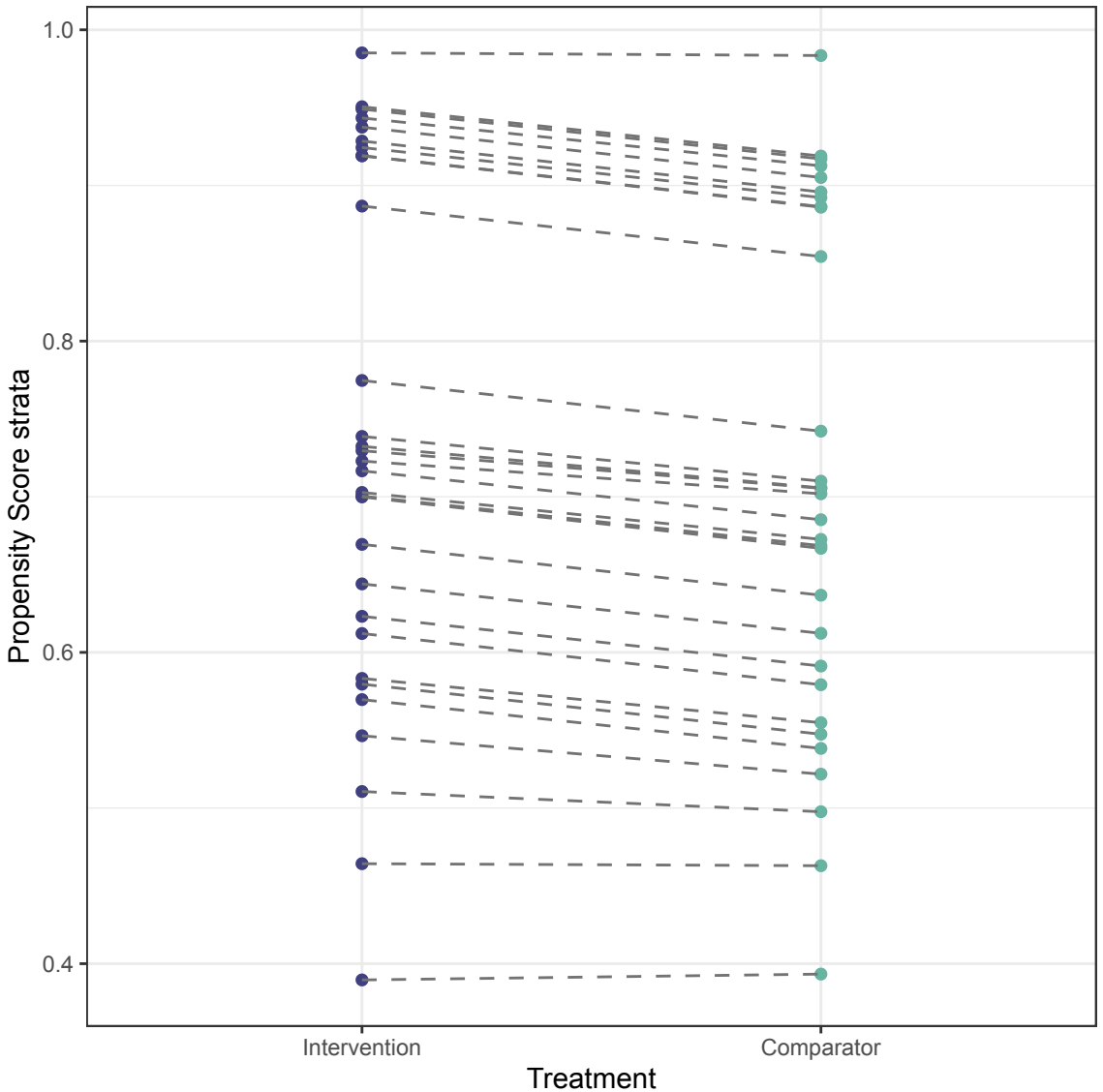
PS can be used in different ways to estimate the treatment effect[1]

- Stratification
- Matching
- Weighting
- Regression adjustment

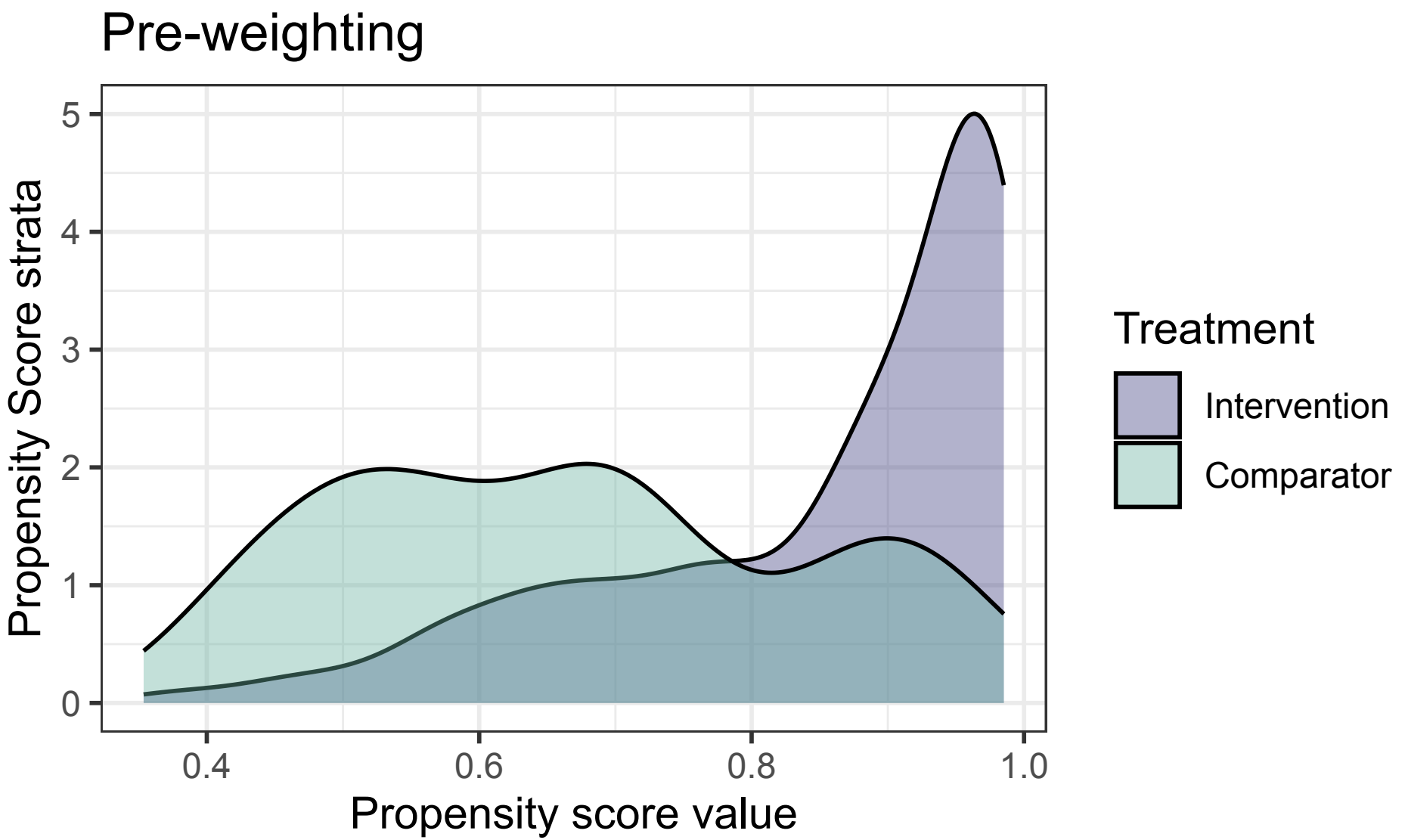
Stratification



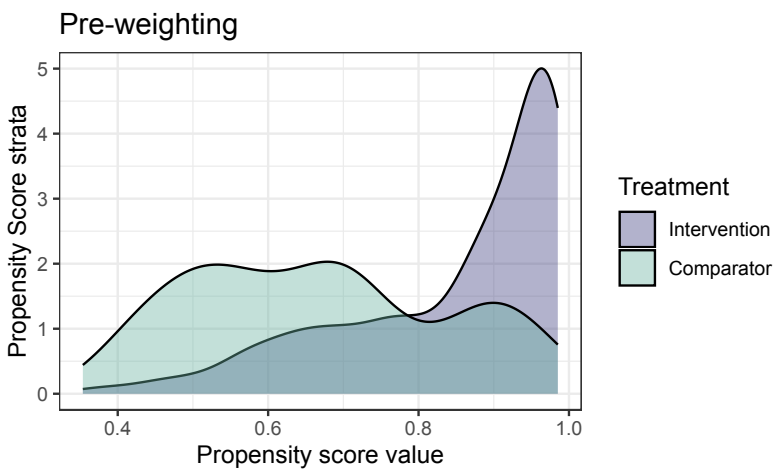
Matching


1.0
0.8
0.6
0.4
Propensity Score strata
Intervention
Comparator
Treatment

Weighting


Pre-weighting
Propensity Score strata
5
4
3
2
1
0
0.4
0.6
0.8
1.0
Propensity score value
Treatment
Intervention
Comparator

Weighting


Pre-weighting
Propensity Score strata
0
1
2
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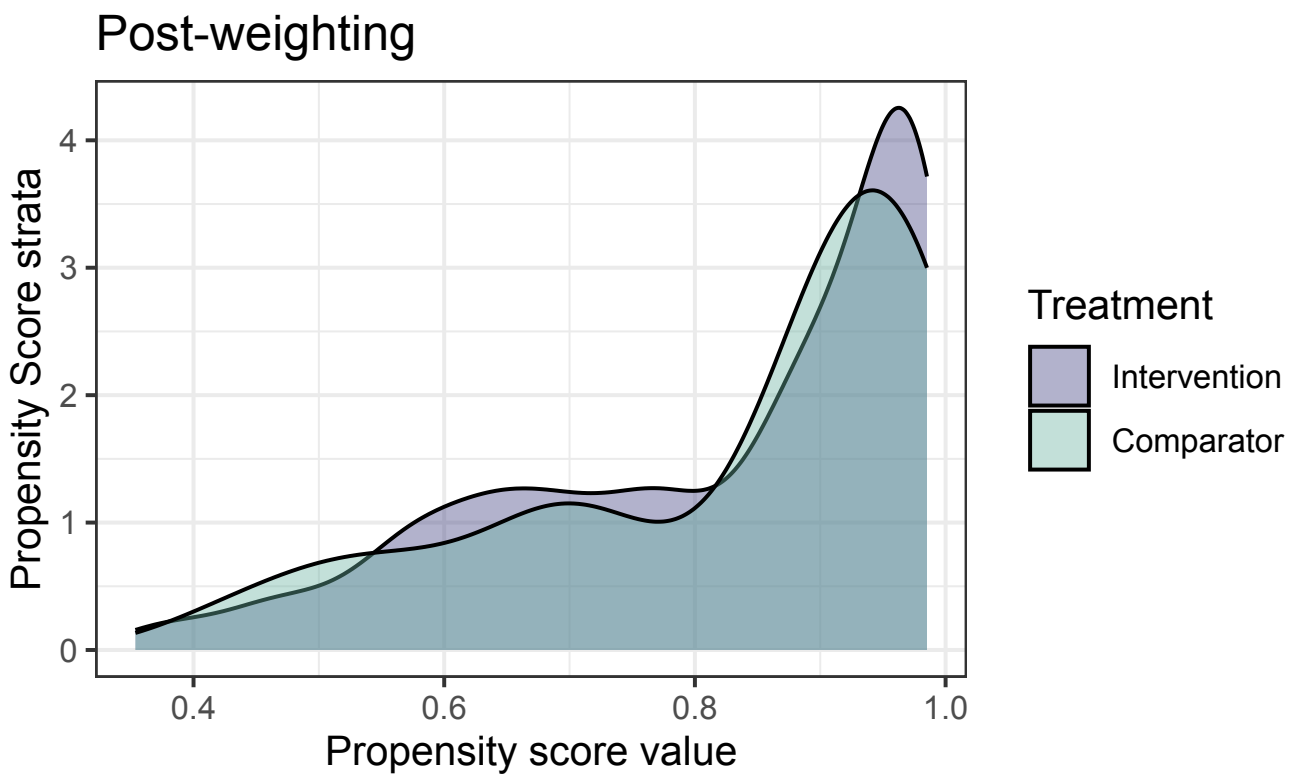
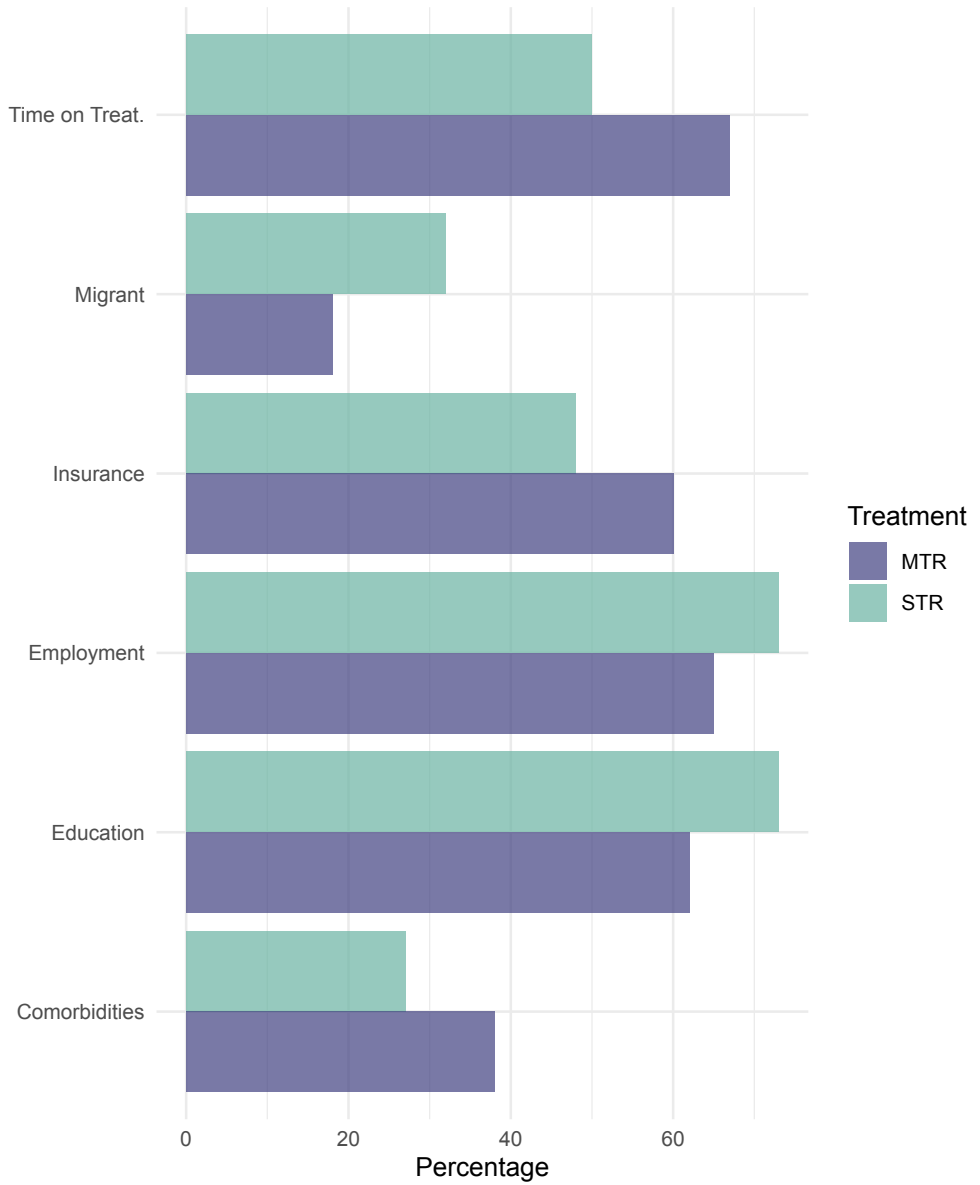
5
0.4
0.6
0.8
1.0
Propensity score value
Treatment
Intervention
Comparator


Post-weighting
Propensity Score strata
0
1
2
3
4
0.4
0.6
0.8
1.0
Propensity score value
Treatment
Intervention
Comparator

Adventages and disadventages

The HIV example

- 803 patients
- Outcome: Adherence and viral suppression
- Exposure: Single-tablet regimens (STR) vs multi-tablet regimens (MTR)
- Covariates: 16 - Sociodemographic, clinical, and treatment-related variables



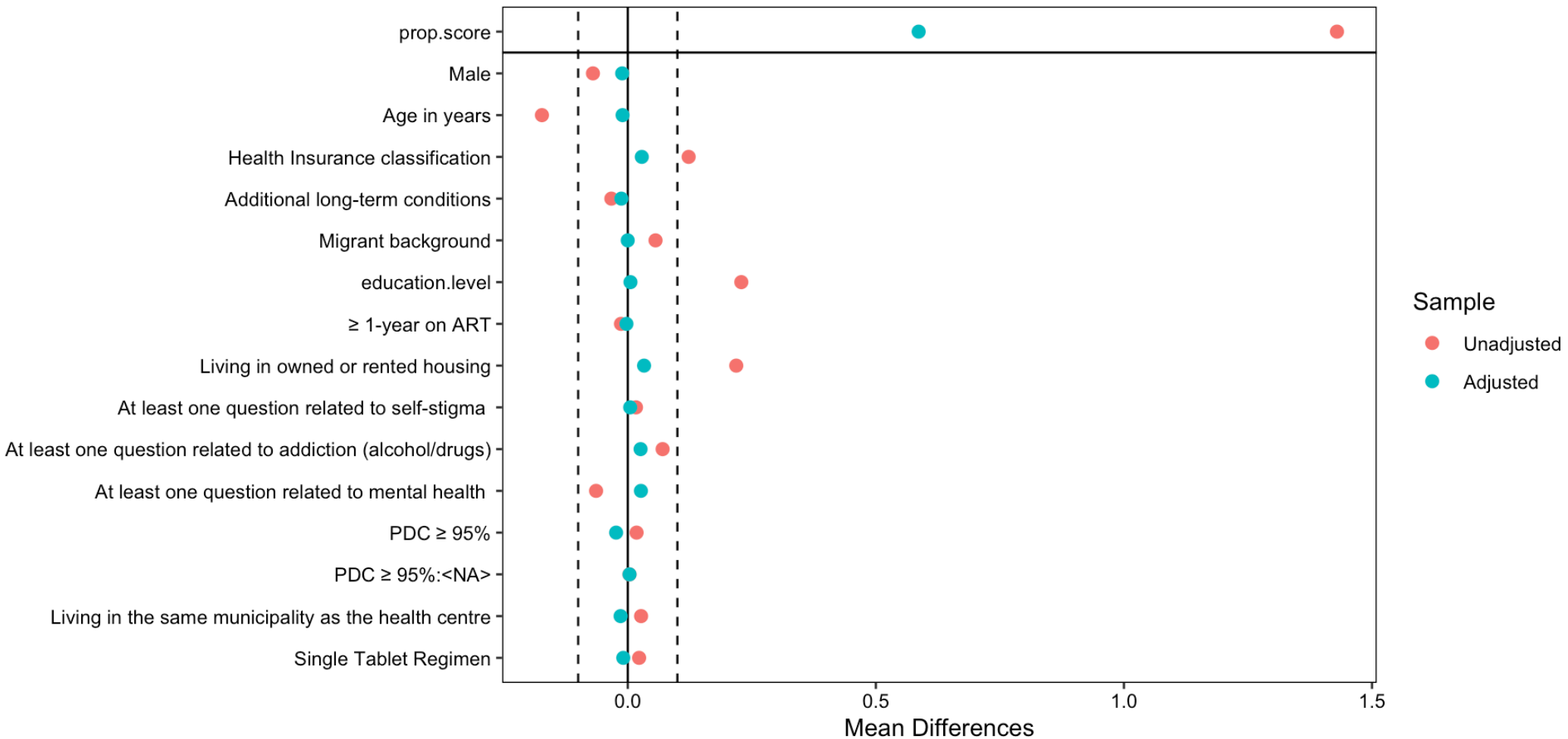
Variable selection for PS

Variable selection based on subject matter knowledge and considering the following criteria:[1]

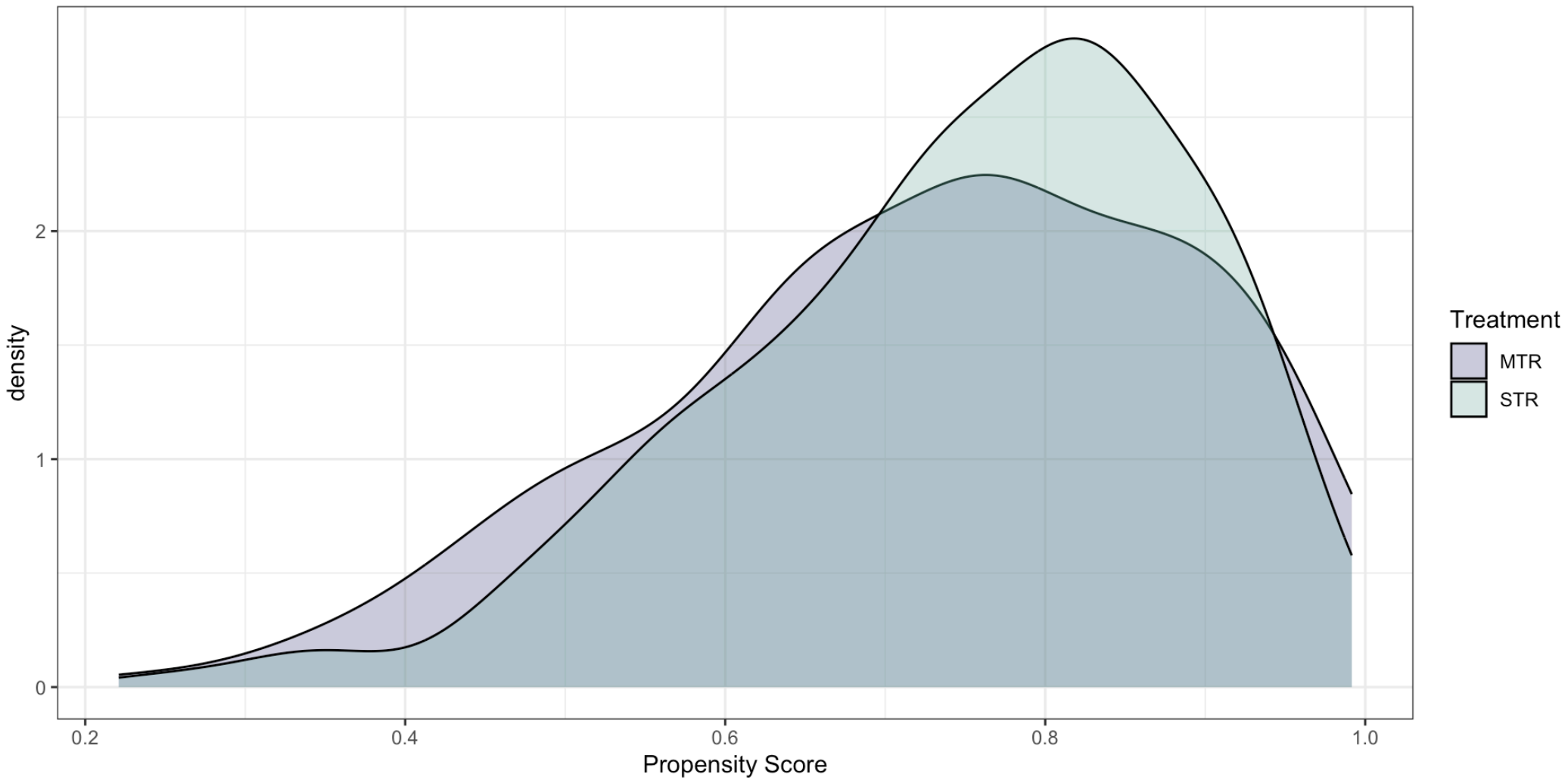
- Related to the treatment and the outcome (confounders)
- Variables only related to the outcome

Some variables included were: age, sex, time on treatment, employment, insurance, comorbidities, migrant, education, and others.

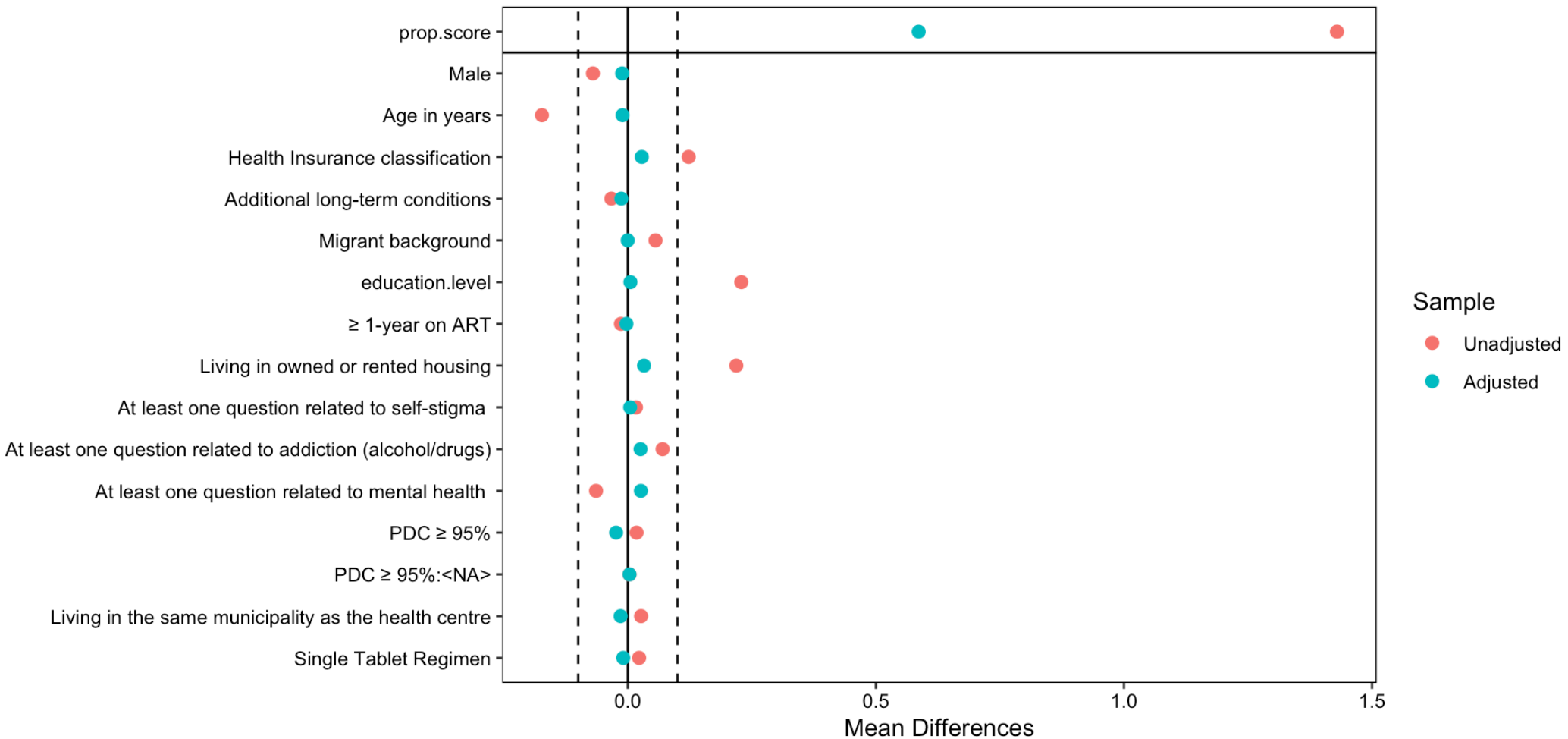
Propensity score assessment



Propensity score assessment



Different PS approaches



Average treatment effect

for being virally non-suppressed

wiggted 0.607 0.292 -1.71 8.75e- 2 0.342 0.89 unad STR 0.540 0.248 -2.49 1.29e- 2 0.331 0.77 match STR 0.784 0.496 -0.492 6.23e- 1 0.35 1.67

for being adherent weight STR 1.20 0.231 0.548 5.84e- 1 1.10 1.79 unadjusted STR 1.81 0.196 3.03 2.44e- 3 1.44 2.66 matched STR 1.30 0.196 3.03 2.44e- 3 1.20 1.95

Potential limitations

We only included variables that were available in the dataset. (un measured confounders) We are assumining that variables are measured without error.

however, comparing with other methods for analysing observational data, PS offers to get closer to causal inference.

Conclusion

Propensity scores cannot replace randomization but are a good alternative for analyzing non-randomized treatment studies and have epistemiological advantages over conventional regression modelling. PS matching, in particular, has a number of advantages. The most important of these is the ability to compare risk factors in the two treatment groups explicitly.

One thing, however, must always be remembered: like conventional regression models, propensity scores can only adjust for patient characteristics that are known and have actually been measured. Only randomized controlled trials can achieve equal distributions of unknown confounding factors too.