Convergence for Individualizing TReatment Using Statistical approaches (CITRUS)

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TI PhD Seminar

Outline

Background

CITRUS =
Convergence for Individualizing TReatment Using Statistical approaches

- Project of the Econometric Institute and Erasmus Medical Center;
- Part of the *Convergence Alliance* between EUR, EMC, TU Delft;
- Fully funded by an *Open Mind Call* grant (awarded: 08/2021).

Introduction

Evidence-based medicine: the *"conscientious, explicit and judicious use of current best evidence in making decisions about the care of individual patients"* (Sackett et al., 1996).

- ➔ Identify *heterogeneous treatment effects* (HTE)!
- ➔ Methods from modern causal inference?
- ➔ CITRUS: Which methods are most eligible for medical data?
- ➔ Simulate common characteristics of medical data.

Introduction

In a perfect world, we'd have...

- *Many* observations: $n \to \infty$;
- Perfect information on relevant covariates (unconfoundedness);
- I.I.D. data from randomized experiment;
- Continuous data.

BUT: The world is not perfect (especially in medicine...)

Introduction

Frank Harrell
@f2harrell

Note to #Statistics paper authors: If you have the word "asymptotic" in your title or abstract, the probability that I'll read the paper is reduced by a factor of 4. I'm only interested in real-world performance of analytical methods. @vandy_biostat

5:55 PM · Sep 29, 2021 · TweetDeck

Introduction

In medical data, we typically have...

- Very finite sample sizes ($n \geq 500$ is rare);
- Noisy to incomplete representation of relevant covariates;
- Non-identically distributed samples;
- Improper randomization (sometimes...);
- Non-continuous data (e.g. categorical).

CITRUS research question:

- To what extent does this affect the performance of each HTE estimation method?

Outline

Setup

Let there be n independent observations $(\mathbf{X}_i, Y_i, W_i)$.

- $\mathbf{X}_i$ is p-dimensional covariate vector;
- Y_i is outcome variable. Binary mortality indicator here: $Y_i = 1$ if i dies.
- W_i is binary treatment assignment variable: $W_i = 1$ if i is in treatment group.

Setup

The DGP for potential outcomes can w.l.o.g. be viewed as

$$Y_i(W_i) = g_i\left(\theta(\mathbf{X}_i)W_i + \nu(\mathbf{X}_i) + \varepsilon_i\right).$$

- $g_i : \mathbb{R} \to \{0, 1\}$ maps to binary outcome space;
- $\theta(\mathbf{X}_i)$ is the HTE function: causal effect of W_i on Y_i;
- $\nu(\mathbf{X}_i)$ is the nuisance function: raw effect of $\mathbf{X}_i$ on Y_i;
- ε_i is zero-mean noise term.

Outline


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Bias In Medicine: Last Week Tonight with John Oliver (HBO)
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References

Sackett, D. L., Rosenberg, W. M., Gray, J. M., Haynes, R. B., and Richardson, W. S. (1996). Evidence-Based Medicine: What it is and What it isn't. *The British Medical Journal*, 312(7023):71–72.