

## 2.4 Personalised Treatment Effects

Alicia Curth

[amc253@cam.ac.uk](mailto:amc253@cam.ac.uk)

### Personalised therapeutics

- estimate effect of a treatment/intervention on an individual patient
- Tutorial goal:
  - Estimating the effect of a treatment on an individual
    - Will a given treatment work for an individual patient?
      - Age, weight, blood, pressure into observational data (offline)
  - Answering such questions from observational data is tricky
    - Missing counterfactual and confounding

### Observational data

- Collected from actual clinical practice
  - Patient features -  $X$
  - Treatment assignment -  $W$
  - Outcome -  $Y$
- This is not randomised
  - Depends on clinical practice
  - Based on observable characteristics
  - Propensity function
- Potential outcomes framework (Neyman-Rubin)
  - $X$ : Each patient  $i$  has features
  - 
  - Two potential outcomes:
    - Treated outcome: treated
    - Control outcome: not treated
      - Will only observe factual outcomes – not the counterfactual outcomes
- Conditional average treatment effect (CATE)/ Individualised treatment effect (ITE)
  - [http://www.personal.ceu.hu/staff/Robert\\_Lieli/cate.pdf](http://www.personal.ceu.hu/staff/Robert_Lieli/cate.pdf)

### Causal effect challenges

- No labels – not supervised
- A solution:
  - Modelling potential outcome regressions
  - Data from treated
- Challenges:
  - Confounding -> covariate shift
    - Training distribution != testing distribution
    - Solutions:
      - Domain-adversarial training
      - Importance weighting
      - Not focus

- Unobserved labels: can target outcomes but not treatment effect
- Potential outcomes framework
  - Main assumptions:
    - no unmeasured confounders (ignorability)
      - E.g. smoking is not recorded but treatment was assigned based on smoking status
    - Common support
      - Some randomness across treatment and not treatment
      - Treatment can be deterministically assigned

### How to model individualized treatment effects? – potential outcome regression

- <https://arxiv.org/abs/2106.03765>
- Indirect learners: PO regression for CATE estimation
  - T-learner
  - Fit two separate regression surfaces using any ML method and only data of each treatment group, then use difference
- PO-specific regression heads
- T-learner
- TARnet
- Implicit inductive biases in existing indirect learners
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### CATE estimation

- Soft, Hard, and flexible approach
- Soft:
  - Generic indirect learner loss function > change regularization scheme

### Evaluation

- Semi-synthetic simulation studies
- <https://datasets-benchmarks-proceedings.neurips.cc/paper/2021/hash/2a79ea27c279e471f4d180b08d62b00a-Abstract-round2.html>

### Meta-learner

- Meta-learning != Meta-learner
- Two approaches
  - Indirect approach
    - For CATE: T-learner/ S-learner/ Hybrid S-learner
  - Direct approach

### Key takeaways:

### What's next?

Handling unobserved confounding?

<https://proceedings.mlr.press/v119/bica20a.html>

Survival outcomes

Treatment outcome: multiple and continuous  
Longitudinal data

**Additional notes:**

- Nonparametric Estimation of Heterogeneous Treatment Effects: From Theory to Learning Algorithms
  - <https://arxiv.org/abs/2101.10943>
- On Inductive Biases for Heterogeneous Treatment Effect Estimation
  - <https://arxiv.org/abs/2106.03765>
- Metalearners for estimating heterogeneous treatment effects using machine learning
  - <https://www.pnas.org/doi/10.1073/pnas.1804597116>
- Towards optimal doubly robust estimation of heterogeneous causal effects
  - <https://arxiv.org/abs/2004.14497>
- Estimating individual treatment effect: generalization bounds and algorithms
  - <https://arxiv.org/abs/1606.03976?context=stat>
- CATENets: <https://github.com/AliciaCurth/CATENets>
- Learn more:
  - <https://www.vanderschaar-lab.com/individualized-treatment-effect-inference/>