Statistical Inference for Heterogeneous Treatment Effects Discovered by Generic Machine Learning in Randomized Experiments

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Motivation and Overview

- Two methodological revolutions over the past two decades
  1. randomized experiments (field/lab/survey)
  2. machine learning

- Causal machine learning (causal ML)
  1. estimation of heterogeneous treatment effects
  2. development of individualized treatment rules

- Experimental evaluation of causal ML
  1. ML algorithms may not work well in practice
  2. assumption-free uncertainty quantification is essential

I will show how to experimentally evaluate heterogeneous treatment effects discovered by \textit{generic} causal ML
Setup

- **Notation:**
  - $n$ experimental units
  - $T_i \in \{0, 1\}$: binary treatment
  - $Y_i(t)$ where $t \in \{0, 1\}$: potential outcomes
  - $Y_i = Y_i(T_i)$: observed outcome
  - $X_i$: moderator of interest

- **Assumptions:**
  1. no interference between units:
     \[ Y_i(T_1 = t_1, \ldots, T_n = t_n) = Y_i(T_i = t_i) \]
  2. randomization of treatment assignment:
     \[ \{ Y_i(1), Y_i(0) \} \perp \perp T_i \]
  3. random sampling of units:
     \[ \{ Y_i(1), Y_i(0) \} \overset{i.i.d.}{\sim} \mathcal{P} \]
Exploration of Heterogeneous Treatment Effects

- Two commonly used treatment prioritization scores
  1. Conditional average treatment effect (CATE):

\[ \tau(x) = \mathbb{E}(Y_i(1) - Y_i(0) \mid X_i = x) \]

2. Baseline risk:

\[ \lambda(x) = \mathbb{E}(Y_i(0) \mid X_i = x) \]

- Estimate a score with ML algorithm using an external data set

\[ f : \mathcal{X} \rightarrow S \subset \mathbb{R} \]

- Group Average Treatment Effect (GATES; Chernozhukov et al. 2019)

\[ \tau_k = \mathbb{E}(Y_i(1) - Y_i(0) \mid p_{k-1} \leq S_i = f(X_i) < p_k) \]

for \( k = 1, 2, \ldots, K \) where \( p_k \) is a cutoff (\( p_0 = -\infty, p_K = \infty \))
How can we make valid statistical inference for GATES without assuming that the scores are correctly estimated by ML algorithm?

A natural difference-in-means estimator for GATES:

$$\hat{\tau}_k = \frac{K}{n_1} \sum_{i=1}^{n} Y_i T_i \hat{f}_k(X_i) - \frac{K}{n_0} \sum_{i=1}^{n} Y_i (1 - T_i) \hat{f}_k(X_i),$$

where $$\hat{f}_k(X_i) = 1\{S_i \geq \hat{p}_k(s)\} - 1\{S_i \geq \hat{p}_{k-1}\}$$

Bias bound and exact variance are derived, accounting for the estimation uncertainty of cutoffs.

Under mild regularity conditions (e.g., continuity of CATE at thresholds), the distribution of $$\hat{\tau}_k$$ is asymptotically normal.
Statistical Hypothesis Tests for Subgroups

1. Nonparametric test of treatment effect homogeneity:
   - Null hypothesis:
     \[ H_0 : \tau_1 = \tau_2 = \cdots = \tau_K. \]
   - Test statistic:
     \[ \hat{\tau}^\top \Sigma^{-1} \hat{\tau} \xrightarrow{d} \chi^2_K \]
     where \( \hat{\tau} = (\hat{\tau}_1 - \hat{\mu}, \cdots, \hat{\tau}_K - \hat{\mu})^\top \)

2. Nonparametric test of rank-consistent treatment effect heterogeneity:
   - Null hypothesis:
     \[ H^*_0 : \tau_1 \leq \tau_2 \leq \cdots \leq \tau_K. \]
   - Test statistic:
     \[ (\hat{\tau} - \mu^*(\hat{\tau}))^\top \Sigma^{-1} (\hat{\tau} - \mu^*(\hat{\tau})) \xrightarrow{d} \bar{\chi}^2_K. \]
     where \( \mu^*(x) = \arg\min_{\mu} \|\mu - x\|^2_2 \) subject to \( \mu_1 \leq \mu_2 \leq \cdots \leq \mu_K \).
Cross-fitting procedure:

1. randomly split the data into $L$ folds: $\mathcal{Z}_1, \ldots, \mathcal{Z}_L$
2. estimate the score using $L - 1$ folds: $\hat{f}_{-\ell}$
3. estimate GATES with the hold-out set: $\hat{\tau}_k^{(\ell)}(\hat{f}_{-\ell})$
4. repeat the process for each $\ell$ and average

$$\hat{\tau}_k(F; n - m) = \frac{1}{L} \sum_{\ell=1}^{L} \hat{\tau}_k^{(\ell)}(\hat{f}_{-\ell})$$

where $F : \mathcal{Z} \rightarrow \mathcal{F}$ is a generic but stable ML algorithm with $\mathcal{Z}_{\text{train}} \in \mathcal{Z}$ and $\hat{f}_{\mathcal{Z}_{\text{train}}} = F(\mathcal{Z}_{\text{train}}) \in \mathcal{F}$

Estimand: average performance of $F$

$$\tau_k(F; n - m) = \mathbb{E}[\mathbb{E}\{ Y_i(1) - Y_i(0) \mid p_{k-1}(\hat{f}_{\mathcal{Z}_{\text{train}}}^{n-m}) \leq \hat{f}_{\mathcal{Z}_{\text{train}}}^{n-m}(X_i) < p_k(\hat{f}_{\mathcal{Z}_{\text{train}}}^{n-m})\}].$$

Unbiasedness: $\mathbb{E}(\hat{\tau}_k(F; n - m)) = \tau_k(F; n - m)$

Finite-sample (conservative) variance estimator (Imai and Li, JASA, 2023)
Simulation Study

- A highly nonlinear specification from the 2016 ACIC competition
  - 58 covariates (3 categorical, 5 binary, 27 counts, 13 continuous)
  - sample size: $n = 4802$
  - use empirical distribution of $X_i$ as true distribution

- Machine learning algorithms
  - Causal forest and Lasso
  - $L = 5$ and also use 5-fold cross validation for tuning

- Fixed score (see the paper) and estimated one with cross-fitting
## Simulation Results: Bias and Coverage

|                | $n = 100$ |               | $n = 500$ |               | $n = 2500$ |               |
|----------------|-----------|---------------|-----------|---------------|------------|---------------|
|                | bias      | s.d.          | coverage  | bias          | s.d.       | coverage      |
| **Causal Forest** |           |               |           |               |            |               |
| $\hat{\tau}_1$ | -0.05     | 2.97          | 94.0%     | -0.01         | 1.57       | 95.6%         |
| $\hat{\tau}_2$ | -0.06     | 2.58          | 95.9      | -0.04         | 1.08       | 98.2          |
| $\hat{\tau}_3$ | -0.01     | 2.56          | 96.7      | -0.05         | 1.06       | 97.7          |
| $\hat{\tau}_4$ | -0.12     | 2.87          | 97.4      | 0.05          | 1.15       | 97.9          |
| $\hat{\tau}_5$ | 0.14      | 3.45          | 94.1      | 0.00          | 1.62       | 96.0          |
| **LASSO**      |           |               |           |               |            |               |
| $\hat{\tau}_1$ | -0.13     | 3.20          | 97.6%     | -0.03         | 1.49       | 96.0%         |
| $\hat{\tau}_2$ | 0.04      | 2.28          | 97.5      | -0.07         | 1.03       | 97.9          |
| $\hat{\tau}_3$ | -0.13     | 2.35          | 96.6      | -0.02         | 1.00       | 97.9          |
| $\hat{\tau}_4$ | -0.00     | 2.54          | 96.8      | 0.04          | 1.17       | 96.8          |
| $\hat{\tau}_5$ | 0.11      | 3.62          | 96.2      | 0.05          | 1.81       | 95.0          |

- Reduction in standard errors compared with fixed $F$ of the same evaluation size is more than 50% in some cases.
### Simulation Results: Size and Power of Tests

|                  | $n = 100$ |                      | $n = 500$ |                      | $n = 2500$ |                      |
|------------------|-----------|----------------------|-----------|----------------------|-----------|----------------------|
|                  | rejection rate | median $p$-value | rejection rate | median $p$-value | rejection rate | median $p$-value |
| **Causal Forest** |           |                     |              |                     |              |                     |
| Homogeneity      | 1.4%      | 0.79                | 4.6%      | 0.71                | 51.4%      | 0.04                |
| Rank-consistency | 1.4%      | 0.70                | 0.8%      | 0.85                | 0.0%       | 0.98                |
| **LASSO**        |           |                     |              |                     |              |                     |
| Homogeneity      | 0.6%      | 0.88                | 1.8%      | 0.85                | 9.0%       | 0.66                |
| Rank-consistency | 1.0%      | 0.72                | 0.6%      | 0.77                | 0.2%       | 0.89                |

- Heterogeneous but rank-consistent effects
- More conservative and lower power than fixed case
- When sample size is large, cross-fitting yields higher power
Empirical Application

- National Supported Work Demonstration Program (LaLonde 1986)
  - Temporary employment program to help disadvantaged workers by giving them a guaranteed job for 9 to 18 months

- Data
  - sample size: $n_1 = 297$ and $n_0 = 425$
  - outcome: annualized earnings in 1978 (36 months after the program)
  - 7 pre-treatment covariates: demographics and prior earnings

- Setup
  - ML algorithms: Causal Forest, BART, and LASSO
  - Sample-splitting: 2/3 of the data as training data
  - Cross-fitting: 3 folds
### GATES Estimates (in 1,000 US Dollars)

|                      | $\hat{\tau}_1$ | $\hat{\tau}_2$ | $\hat{\tau}_3$ | $\hat{\tau}_4$ | $\hat{\tau}_5$ |
|----------------------|----------------|----------------|----------------|----------------|----------------|
| **Sample-splitting** |                |                |                |                |                |
| BART                 | 2.90           | -0.73          | -0.02          | 3.25           | 2.57           |
|                      | [-2.25, 8.06]  | [-5.05, 3.58]  | [-3.47, 3.43]  | [-1.53, 8.03]  | [-3.82, 8.97]  |
| Causal Forest        | 3.40           | 0.13           | -0.85          | -1.91          |
|                      | [-1.29, 3.40]  | [-5.37, 5.63]  | [-5.22, 3.52]  | [-5.16, 1.34]  |
| LASSO                | 1.86           | 2.62           | -2.07          | 1.39           |
|                      | [-3.59, 7.30]  | [-1.69, 6.93]  | [-5.39, 1.26]  | [-2.95, 5.73]  |
| **Cross-fitting**    |                |                |                |                |                |
| BART                 | 0.40           | -0.15          | -0.40          | 2.52           | 2.19           |
|                      | [-3.79, 4.59]  | [-2.54, 2.23]  | [-3.37, 2.56]  | [-0.99, 6.03]  |
| Causal Forest        | -3.72          | 1.05           | 5.32           | -2.64          |
|                      | [-6.52, -0.93] | [-2.28, 4.37]  | [2.63, 8.01]   | [-5.07, -0.22] |
| LASSO                | 0.65           | 0.45           | -2.88          | 1.32           |
|                      | [-3.65, 4.94]  | [-3.28, 4.18]  | [-5.38, -0.38] | [-1.83, 4.48]  |

- Greater statistical power with cross-fitting
- ML algorithms are not necessarily reliable
## Results of Hypothesis Tests

|                | Causal Forest | BART | LASSO |
|----------------|---------------|------|-------|
|                | stat | $p$-value | stat | $p$-value | stat | $p$-value |
| **Sample-splitting** |      |         |      |         |      |         |
| Homogeneity     | 9.78 | 0.08   | 2.76 | 0.74   | 5.26 | 0.36   |
| Rank-consistency| 3.07 | 0.32   | 1.13 | 0.66   | 3.14 | 0.30   |
| **Cross-fitting** |      |         |      |         |      |         |
| Homogeneity     | 30.29 | 0.00   | 2.32 | 0.80   | 10.79 | 0.06   |
| Rank-consistency| 0.06 | 0.69   | 0.04 | 0.89   | 0.45 | 0.71   |
Concluding Remarks

- Causal machine learning (ML) is rapidly becoming popular
  - estimation of heterogeneous treatment effects (HTEs)
  - development of individualized treatment rules (ITRs)

- Safe deployment of causal ML requires uncertainty quantification
  - experimental evaluation of HTEs and ITRs
  - no modeling assumption
  - no resampling (computationally efficient)
  - applicable to any complex causal ML algorithms
  - good small sample performance

- Open source software: evalITR: Evaluating Individualized Treatment Rules at CRAN https://CRAN.R-project.org/package=evalITR

- More information: https://imai.fas.harvard.edu/research/