

# Estimating population average treatment effects from experiments with noncompliance

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# Overview

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# Motivation

- ▶ RCTs are the gold standard for estimating the causal effect of a treatment
- ▶ Noncompliance to treatment assignment biases estimates of the sample average treatment effect (SATE) towards 0
- ▶ External validity is an issue when RCT participants don't reflect the target population

# Motivation

- ▶ Idea: reweight responses in the treatment group of RCT compliers to estimate population average treatment effect on the treated (PATT)
- ▶ Hartman et al. [2015] develop a nonparametric reweighting method to extend SATE to PATT
- ▶ We extend this method to the case of one-way crossover

# Estimating treatment effects

- ▶ Neyman-Rubin framework: each  $i = \{1, \dots, N\}$  participants have four potential outcomes,  $Y_{ist}$  for  $s = 0, 1$  and  $t = 0, 1$ 
  - ▶  $S$  = study assignment:  $S=1$  for RCT,  $S=0$  for population/observational study
  - ▶  $T$  = treatment assignment:  $T = 1$  for treatment,  $T = 0$  for control
  - ▶  $D$  = treatment received
- ▶ Other variables
  - ▶  $W$  = observed covariates
  - ▶  $C$  = compliance to treatment

# Estimating treatment effects

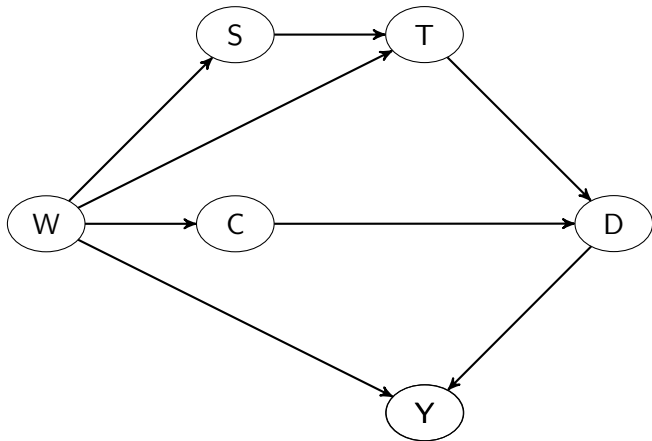


Figure: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT.

# Estimating treatment effects

## Theorem

*Under assumptions (1) - (7),*

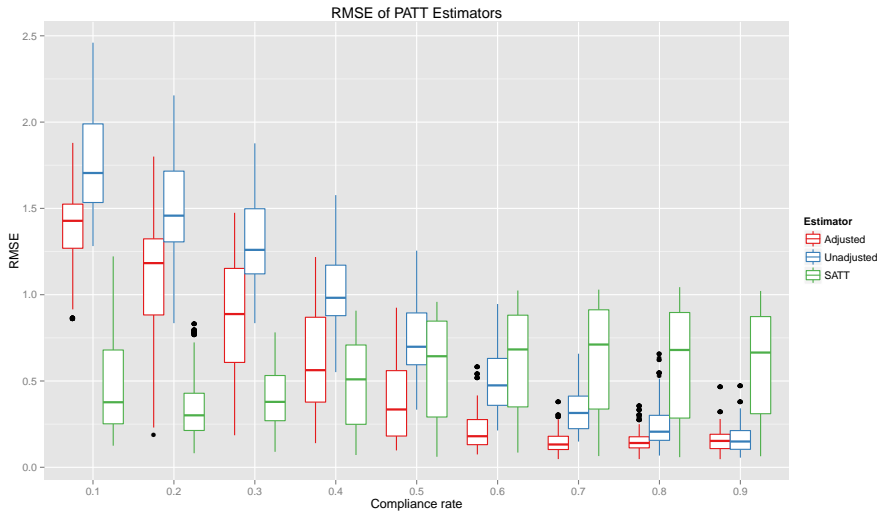
$$\tau_{PATT} = \mathbb{E}_{01} [\mathbb{E}(Y_{11} \mid S = 1, T = 1, C = 1, W)] - \mathbb{E}_{01} [\mathbb{E}(Y_{10} \mid S = 1, T = 0, C = 1, W)]$$

*where  $\mathbb{E}_{01} [\mathbb{E}(\cdot \mid \dots, W)]$  denotes the expectation with respect to the distribution of  $W$  in the treated individuals in the target population.*

# Simulation Design

- ▶ Generate a population of 30,000 with 3 observable covariates  $W$
- ▶ Set  $S$ ,  $T$ ,  $C$ ,  $Y$  to be linear functions of  $W$ , with some Gaussian noise
- ▶ Heterogeneous treatment effect: magnitude of effect depends on one of the covariates
- ▶ Sample 5,000 “randomizables” for RCT and 5,000 “observables” for observational study. Enroll individuals according to  $S$
- ▶ Predict would-be compliers in the RCT control group using logistic regression
- ▶ Estimate response curve in RCT compliers using a random forest
- ▶ Use model to estimate potential outcomes for treated individuals in the observational study





# Application: Oregon Health Insurance Experiment (OHIE)

- ▶ In 2008,  $\approx 90,000$  uninsured low-income adults participated in a lottery to receive Medicaid benefits [Finkelstein et al., 2012]
  - ▶ Selected participants won the opportunity for themselves and any household member to apply for Medicaid
  - ▶ 29,834 participants were selected by the lottery; remaining 45,008 served as controls
  - ▶ Compliance measured by whether participant enrolled in Medicaid program during study period
- ▶ Two health care use responses from mail survey ( $N = 23,741$ ): emergency room (ER) and primary care visits in past 12 months

# Observational data

- ▶ Data on the target population comes from National Health Interview Study (NHIS) for Health Statistics for 2009–2013
- ▶ Restrict to respondents with income below 138% of FPL and on Medicaid ( $N = 3,914$ )
- ▶ Extract covariates and responses that match OHIE

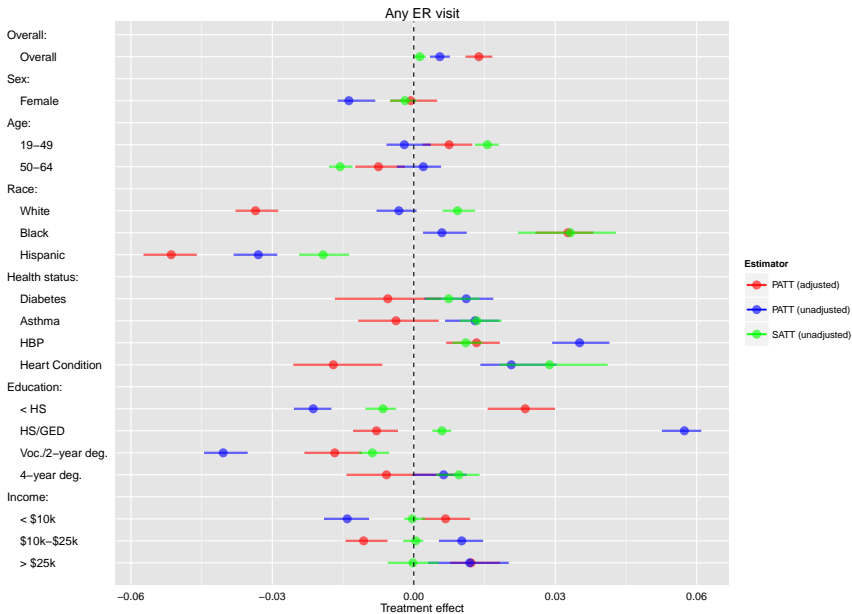
	OHIE control <i>n</i> = 5476		OHIE treated <i>n</i> = 5193		NHIS treated <i>n</i> = 3382	
<b>Covariate</b>	<b>n</b>	<b>%</b>	<b>n</b>	<b>%</b>	<b>n</b>	<b>%</b>
<i>Sex:</i>						
Female	3148	57.5	2920	56.2	2380	70.4
<i>Age:</i>						
19-49	1636	29.9	1367	26.3	2429	71.8
50-64	3840	70.1	3826	73.7	953	28.2
<i>Race:</i>						
White	4829	88.2	4393	84.6	1991	58.9
Black	243	4.4	197	3.8	1050	31.1
Hispanic	301	5.5	476	9.2	910	26.9
<i>Health status:</i>						
Diabetes	581	10.6	539	10.4	452	13.4
Asthma	1036	18.9	887	17.1	652	19.3
High blood pressure	1670	30.5	1418	27.3	1143	33.8
Heart condition	170	3.1	141	2.7	285	8.4
<i>Education:</i>						
Less than high school	1056	19.3	950	18.3	1183	35.0
High school diploma or GED	3081	56.3	2775	53.4	1076	31.8
Voc. training / 2-year degree	969	17.7	1031	19.9	934	27.6
4-year college degree or more	370	6.8	437	8.4	189	5.6
<i>Income:</i>						
< \$10k	5476	100.0	3204	61.7	1452	42.9
\$10k-\$25k	0	0.0	1616	31.1	1622	48.0
> \$25k	0	0.0	373	7.2	308	9.1
<b>Response</b>						
Any ER visit	1393	25.4	1301	25.1	881	26.1
Any outpatient visit	3299	60.2	3081	59.3	2116	62.6

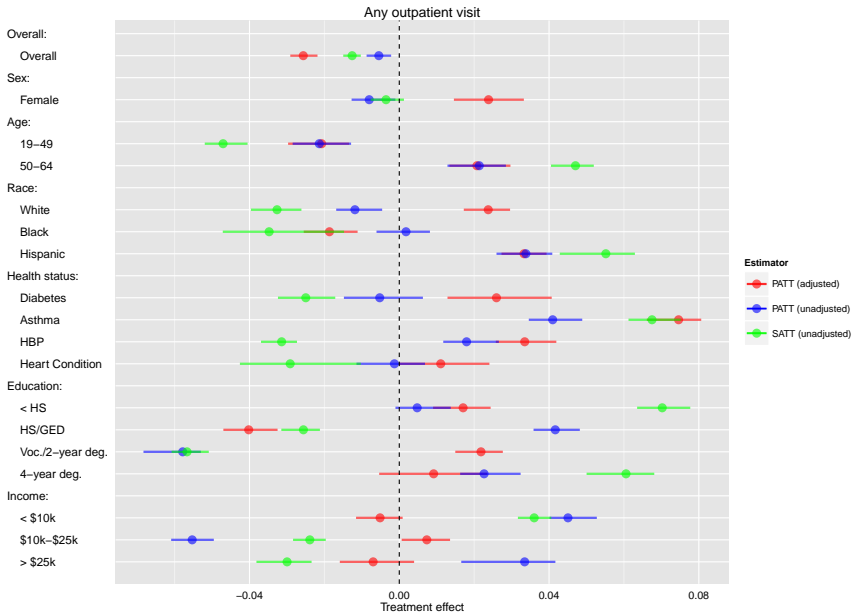
# Checking Assumptions

- ▶ Monotonicity is violated: two-way crossover occurred in OHIE
  - ▶ 60% of treated did not enroll in Medicaid
  - ▶ 14% of controls enrolled in Medicaid during the study period
  - ▶ Cross-over from control to treatment is low relative to other direction
- ▶ Key assumption is strong ignorability: response given covariates is the same in the RCT and population
  - ▶ Other potential confounders: unmeasured health conditions, proximity to health services, enrollment in other federal programs, etc.
- ▶ Conditional independence of compliance and treatment assignment
  - ▶ Ensemble model of compliance has 77% accuracy on the treatment group
  - ▶ No way of checking that it predicts equally well for controls

# Estimation Procedure

1. For RCT units assigned to treatment, use an ensemble method to model compliance, given covariates
2. Using compliance model, predict compliers among RCT controls
3. For observed and predicted RCT compliers, train random forests model to predict response using covariates and treatment as features
4. Using response model, estimate potential outcomes for population on Medicaid
5.  $\tau_{\text{PATT}}$  is the difference in means of the potential outcomes







# Results

- ▶ Effect of Medicaid coverage on likelihood of visiting the ER is 1.3% and outpatient use is -2.5% for population compliers
- ▶ Substantial variation in treatment effects across subgroups
  - ▶ Within subgroups, effects on ER visits tend to be negative; effects on outpatient visits tend to be positive and significant
- ▶ SATT and PATT estimates differ substantially
  - ▶ For example, increase in outpatient visits for college-educated RCT compliers, but no difference for college-educated people on Medicaid in the population
  - ▶ Patterns match those found by [Finkelstein et al., 2012]

# Conclusions

- ▶ In simulations, the proposed estimator of PATT performs better than unadjusted estimator in simulations when compliance is low and can be predicted by observed covariates
- ▶ We combine experimental data from OHIE and observational population data from NHIS to estimate the PATT of Medicaid coverage on health care use
- ▶ We find substantial differences between sample and population estimates in terms of race, education, and health status subgroups

- Amy Finkelstein, Sarah Taubman, Bill Wright, Mira Bernstein, Jonathan Gruber, Joseph P Newhouse, Heidi Allen, Katherine Baicker, Oregon Health Study Group, et al. The oregon health insurance experiment: Evidence from the first year. *The quarterly journal of economics*, 127(3):1057, 2012.
- National Center for Health Statistics. National health interview survey. <http://www.cdc.gov/nchs/nhis.htm>. April 2015.
- Erin Hartman, Richard Grieve, Roland Ramsahai, and Jasjeet S Sekhon. From sate to patt: combining experimental with observational studies to estimate population treatment effects. *JR Stat. Soc. Ser. A*, 10:1111, 2015.

## Appendix: estimator assumptions

### Assumption 1

*Consistency under parallel studies: for all  $i$  and for  $t = 0, 1$ ,*

$$Y_{i0t} = Y_{i1t}$$

## Appendix: estimator assumptions (cont.)

### Assumption 2

*Strong ignorability of sample assignment for treated:*

$$(Y_{01}, Y_{11}) \perp\!\!\!\perp S \mid (W, T = 1, C = 1), 0 < \mathbb{P}(S = 1 \mid W, T = 1, C = 1) < 1$$

### Assumption 3

*Strong ignorability of sample assignment for controls:*

$$(Y_{00}, Y_{10}) \perp\!\!\!\perp S \mid (W, T = 1, C = 1), 0 < \mathbb{P}(S = 1 \mid W, T = 1, C = 1) < 1$$

Potential outcomes are independent of sample assignment for individuals with the same covariates  $W$  and assignment to treatment.

## Appendix: estimator assumptions (cont.)

### Assumption 4

*Stable unit treatment value assumption (SUTVA):*

$$Y_{ist}^{L_i} = Y_{ist}^{L_j}, \forall i \neq j$$

*where  $L_j$  is the treatment and sample assignment vector for unit  $j$ .*

### Assumption 5

*Conditional independence of compliance and assignment:*

$$C \perp\!\!\!\perp T = 1 \mid W, 0 < \mathbb{P}(C = 1 \mid W) < 1$$

## Appendix: estimator assumptions (cont.)

### Assumption 6

*Monotonicity:*

$$T_i \geq D_i, \forall i$$

This assumption implies that there are no defiers and that crossover is only possible from treatment to control.

### Assumption 7

*Exclusion restriction: For non-compliers*

$$Y_{11} = Y_{10}$$

The treatment assignment affects the response only through the treatment received. In particular, the treatment effect may only be non-zero for compliers.