

# Estimating population average treatment effects from experiments with noncompliance

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# Motivation

- ▶ RCTs are the “gold standard” for estimating the causal effect of a treatment
  - ▶ External validity is an issue when RCT participants don't reflect the target population
  - ▶ Non-compliance to treatment assignment biases estimates of the sample average treatment effect (SATE) towards 0
- ▶ Idea: reweight responses in the treatment group of RCT compliers to estimate population average treatment effect on the treated (PATT)
  - ▶ Hartman et al. [to appear] 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
  - ▶  $Y$  = response

# Estimating treatment effects

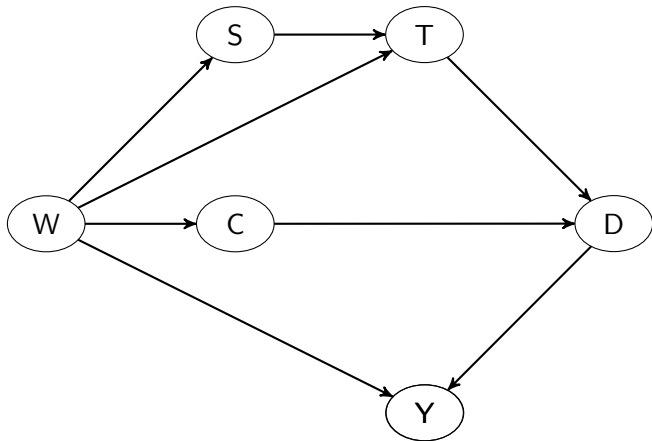


Figure: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT.

# Estimating treatment effects (cont.)

## Theorem

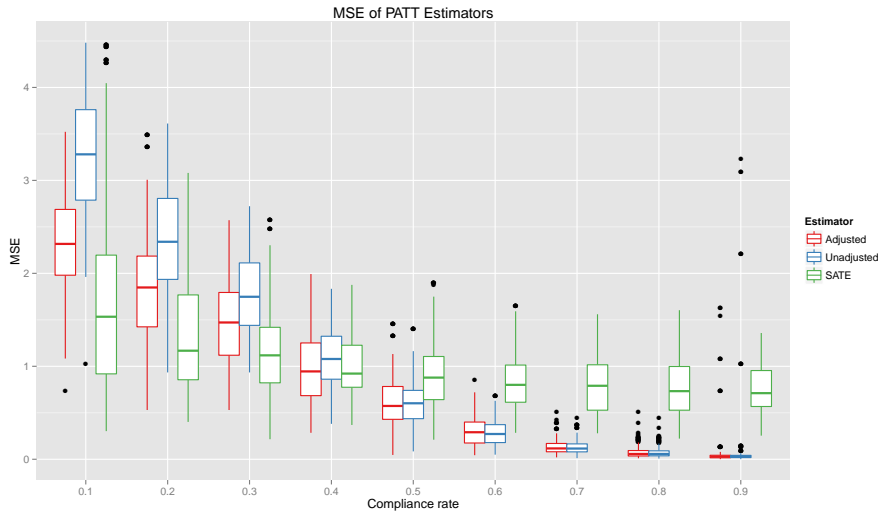
*Under assumptions (1) - (7),*

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

*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 in the observational study to estimate  $\tau_{\text{PATT}}$



## 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]
- ▶ Participants selected by the lottery won the opportunity for themselves and any household member to apply for Medicaid
- ▶ After sample exclusions, 29,834 participants were selected by the lottery; remaining 45,008 served as controls
- ▶ Two health care use responses from mail survey ( $N = 23,741$ ): emergency room (ER) and primary care visits in past 12 months
- ▶ Compliance measure: indicator for whether participant was enrolled in Medicaid program during study period



# Observational data

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

	OHIE control <i>n</i> = 5104		OHIE treated <i>n</i> = 5193		NHIS treated <i>n</i> = 3914	
<b>Covariate</b>	<b>n</b>	<b>%</b>	<b>n</b>	<b>%</b>	<b>n</b>	<b>%</b>
Female	2970	58.2	2920	56.2	2712	69.3
20-49	1307	25.6	1367	26.3	1418	36.2
50-64	3797	74.4	3826	73.7	2496	63.8
White	4420	86.6	4393	84.6	2308	59.0
Black	227	4.5	197	3.8	1192	30.4
Hispanic	331	6.5	476	9.2	1054	26.9
Diabetes	518	10.2	539	10.4	689	17.6
Asthma	986	19.3	887	17.1	748	19.1
High blood pressure	1486	29.1	1418	27.3	1581	40.4
Heart condition	159	3.1	141	2.7	396	10.1
Less than high school	994	19.5	950	18.3	1555	39.7
High school diploma or GED	2908	57.0	2775	53.4	1193	30.5
Vocational training / 2-year degree	922	18.1	1031	19.9	945	24.1
4-year college degree or more	280	5.5	437	8.4	221	5.7
<b>Response</b>						
Any ER visit	1289	25.2	1323	25.5	860	22.0
Any primary care visit	3044	59.6	3125	60.2	3175	81.1

# Checking Assumptions

- ▶ Monotonicity is violated: two-way crossover occurred in OHIE
  - ▶ Lottery winners: 60% attempted to enroll in Medicaid; only about 30% successfully enrolled
  - ▶ Lottery losers: 14% enrolled in Medicaid some other way during study period
  - ▶ Cross-over from control to treatment is low relative to other direction
- ▶ Key assumption is strong ignorability: model of response given covariates is same in the RCT and in the population
  - ▶ No way to check that we've included all possible confounders
  - ▶ We have included all the confounders we have data on

## Estimation Procedure

1. Using OHIE lottery winners ( $S = 1, T = 1$ ), train a random forest [?] to predict complier status.
2. Predict which lottery losers in the OHIE *would have* signed up for Medicaid had they been eligible
3. For the group of RCT compliers (both observed and predicted), train a random forest to predict hospital use using the covariates and Medicaid enrollment as features.
4. For all individuals who enrolled in Medicaid in the NHIS, estimate their potential outcomes  $Y_{10}$  and  $Y_{11}$  using the model from step 3. The mean counterfactual  $Y_{11}$  minus the mean counterfactual  $Y_{10}$  is the estimate of  $\tau_{\text{PATT}}$ .

Figure: Any ER visit.

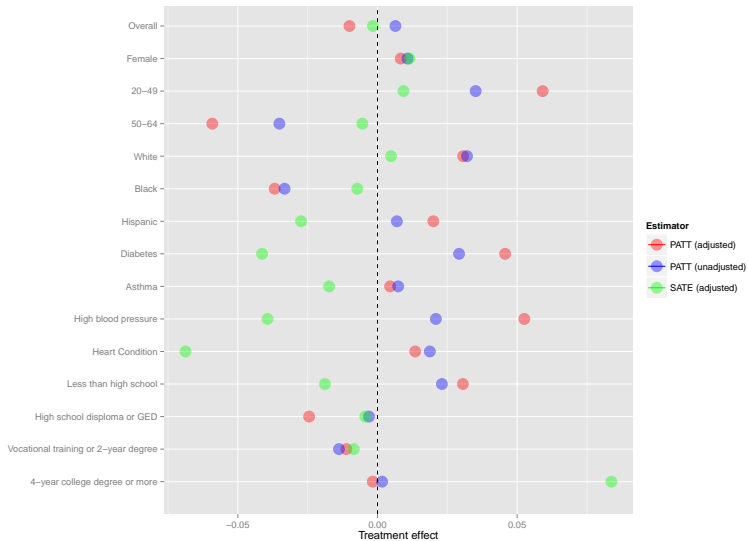
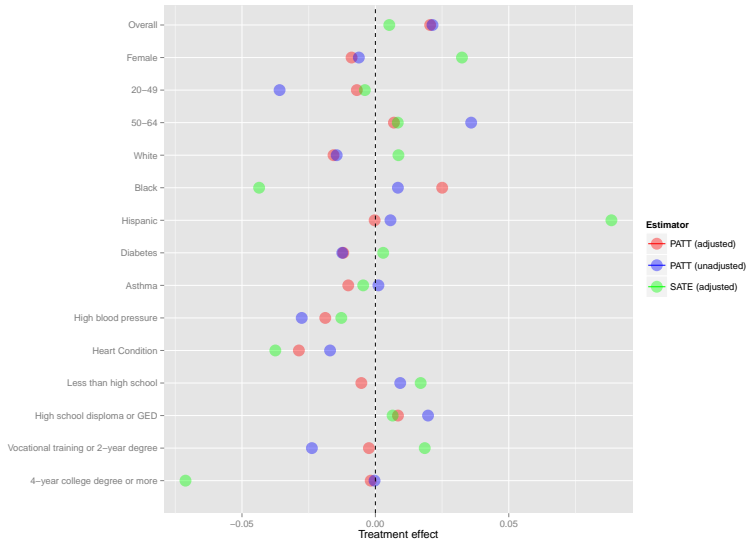


Figure: Any primary care visit.



# Conclusions

- ▶ If compliance can be predicted by observed covariates, then it makes sense to use the proposed estimator
- ▶ statement or two about the OHIE/NHIS conclusions

- K Baicker, A Finkelstein, J Song, and S Taubman. The impact of medicaid on labor market activity and program participation: Evidence from the oregon health insurance experiment. *The American economic review*, 104(5):322, 2014.
- Katherine Baicker, Sarah L Taubman, Heidi L Allen, Mira Bernstein, Jonathan H Gruber, Joseph P Newhouse, Eric C Schneider, Bill J Wright, Alan M Zaslavsky, and Amy N Finkelstein. The oregon experiment effects of medicaid on clinical outcomes. *New England Journal of Medicine*, 368(18):1713–1722, 2013.
- 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. *Journal of the Royal Statistical Society, Series A*, to appear.
- Sarah Taubman, Heidi Allen, Bill Wright, Katherine Baicker, Amy Finkelstein, and the Oregon Health Study Group. Medicaid increases emergency department use: Evidence from oregon's health insurance experiment. *Science*, 343(6168):263–268, January 2014.



# Estimating treatment effects (cont.)

## Assumption 1

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

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

# Estimating treatment effects (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.

# Estimating treatment effects (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:*

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

## Estimating treatment effects (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.