Estimating population average treatment effects from experiments with noncompliance

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1   Introduction

Randomized control trials (RCTs) are the "gold standard" for estimating the causal effect of a treatment. However, external validity is often an issue when the individuals in an RCT are unrepresentative of the population of interest. For example, RCTs in which participants volunteer to sign up for health insurance coverage may exhibit a sample population that is in poorer health than the target population. External validity is particularly relevant to policymakers who want to know how a sample average treatment effect (SATE) would generalize to the broader population. Hartman et al. propose a method of reweighting the responses of individuals in an RCT study according to the distribution of covariates in the target population in order to estimate the population average treatment effect on the treated (PATT). Under a series of assumptions, the PATT estimate is asymptotically unbiased (Hartman et al. [to appear]).

A prevalent issue in RCTs is non-compliance. One-way crossover from treatment to control occurs when individuals who are assigned to the treatment group refuse the treatment. This serves to dilute the estimated effect of treatment assignment, and the resulting intention to treat estimate is biased towards 0. We propose to extend the method of Hartman et. al. to estimate the PATT in settings with RCT non-compliance. We make the same assumptions needed for the estimator proposed by Hartman et al. [to appear]; these include the stable unit treatment value assumption and strong ignorability. To ensure identifiability, we also make the no defiers and exclusion restriction assumptions as in Angrist et al. [1996].

The estimator of PATT involves the expectation of the response of compliers in the RCT sample, conditional on their covariates, where the expectation is taken over the distribution of population covariates. To estimate the conditional expectation of responses in the RCT, we use an ensemble learning method to estimate the response surface. Then, we use the

response model to estimate population members' outcomes given their covariates, giving an estimate of the PATT.

The paper proceeds as follows: Section 2 presents the proposed estimator, necessary assumptions for its identifiability, and an estimation procedure; Section 3 investigates the estimator's performance in simulations; Section 4 uses the estimator to identify the effect of extending Medicare coverage to the low–income population in the U.S; Section 5 discusses the merits and flaws of the proposed estimator.

2 Estimator

We are interested in using the outcomes from an RCT to estimate the average treatment effect on the treated in some target population. Treatment in the population is not assigned at random, but rather depends on other variables, confounding the effect of treatment on the outcome of interest; RCTs are needed to isolate the effect of treatment. However, strict exclusion criteria for RCTs often result in a sample whose distribution of covariates differs substantially from the target population. Ideally, we would take the results of an RCT and reweight the sample such that the reweighted covariates match the those in the population. In practice, one rarely knows the true covariate distribution in the target population. Instead, we consider data from a non-randomized, observational study in which participants are representative of the target population. Our proposed estimator combines RCT and observational data to overcome these issues.

2.1 Assumptions

Let Y_{ist} be the potential outcome for individual i in group s, where $s = 0$ for the population and $s = 1$ for the randomized control trial, and t be the treatment received. Let S_i denote the sample assignment, T_i denote the treatment assigned, and D_i denote treatment received. Let W_i be individual i's observable pretreatment covariates that are related to the sample selection mechanism for membership in the RCT, treatment assignment in the population, and complier status. Let C_i be an indicator for individual i's compliance to treatment. For a generic value, we drop the subscript i.

We assume that in the RCT, treatment is assigned at random. Then for individuals with $C_i = 1$, we observe $D_i = T_i$. In the population, we suppose that treatment is made available to individuals according to some rule based on their covariates; treatment assignment is not completely random. Individuals with $T_i = 0$ do not receive treatment, while those with $T_i = 1$ may decide whether or not to accept treatment. We only observe D, not T, in the population. Among the population controls, we can't distinguish non-compliers who should have received treatment (individuals with $T_i = 1$ and $D_i = 0$) from those assigned to control (those with $T_i = 0$ and $D_i = 0$). Compliance is only observable for individuals assigned to treatment in the RCT.

We make the following assumptions:

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

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

Each individual i has the same response to treatments, whether i is in the RCT or not.

Assumption 2. *Strong ignorability of sample assignment for treated:*

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

This means that the potential outcomes for treatment are independent of sample assignment for individuals with the same covariates W and assignment to treatment.

Assumption 3. *Strong ignorability of sample assignment for controls:*

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

This means that the potential outcomes for control are independent of sample assignment for individuals with the same covariates W and assignment to treatment.

Assumption 4. *Stable unit treatment value assumption (SUTVA):*
    The potential outcomes Y_{ist} for individual i, $s = 0, 1$, $t = 0, 1$ do not depend on T_j, for all $j \neq i$.

Assumption 5. *Conditional independence of compliance and assignment:*

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

This means that compliance is independent of sample and treatment assignment for all individuals with covariates W.

Assumption 6. *No defiers:*
$$T_i \geq D_i, \forall i$$

This assumption ensures 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.
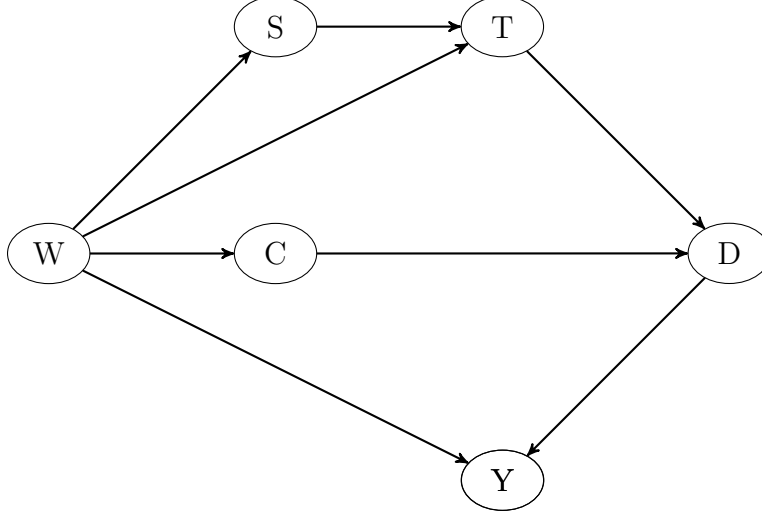
Figure 1: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT.

2.2 Population Average Treatment Effect on the Treated (PATT)

The estimand of interest is

$$\tau_{\text{PATT}} = \mathbb{E}\left(Y_{01} - Y_{00} \mid S = 0, D = 1\right) \tag{1}$$

This is the average treatment effect on those in the population who receive treatment. It includes individuals who actually receive the treatment, but does not include those who are eligible for treatment and do not accept it (non-compliers). The following theorem, which we modify from Hartman et al. [to appear] to handle non-compliance, relates the treatment effect in the population to the treatment effect in the RCT. The result enables us to estimate τ_{PATT} by reweighting the RCT responses according to the distribution of covariates in the population.

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

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

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

Proof. We separate the expectation into two terms and treat each individually.

$$
\begin{aligned}
\mathbb{E}\left(Y_{01} \mid S = 0, D = 1\right) &= \mathbb{E}\left(Y_{11} \mid S = 0, D = 1\right) & \text{by (1)}\\
&= \mathbb{E}\left(Y_{11} \mid S = 0, T = 1, C = 1\right) & \text{by (6)}\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{11} \mid S = 0, T = 1, C = 1, W\right)\right] &\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{11} \mid S = 1, T = 1, C = 1, W\right)\right] & \text{by (2)}\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{11} \mid S = 1, D = 1, W\right)\right] &
\end{aligned}
$$

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$$\mathbb{E}\left(Y_{00} \mid S = 0, D = 1\right) = \mathbb{E}\left(Y_{10} \mid S = 0, D = 1\right) \qquad \text{by (1)}$$
$$= \mathbb{E}\left(Y_{10} \mid S = 0, T = 1, C = 1\right) \qquad \text{by (6)}$$
$$= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{10} \mid S = 1, T = 1, C = 1, W\right)\right] \qquad \text{by (3)}$$
$$= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{10} \mid S = 1, T = 0, C = 1, W\right)\right]$$

The last line follows because of the randomization carried out in the RCT. This ensures $Y_{10} \perp\!\!\!\perp T \mid (W, S = 1)$. $\qquad\square$

2.3 Estimation procedure

Theorem 1 poses two challenges in practice. First, we must construct an estimate of the inner expectation over potential outcomes in the RCT. Here, we use an ensemble of machine learners [van der Laan et al., 2007] to estimate the response curve for compliers, given their covariates.[1] We estimate the outer expectation by taking empirical means. Thus, the first term in the expression for τ_{PATT} is estimated by the weighted average of mean responses in the treatment group in the RCT. The second term is estimated by the weighted average of the mean control response for compliers assigned to control in the RCT. We compute these by evaluating the response curve for each treated individual in the population.

The second challenge is that we cannot observe which individuals are included in the estimation of the second term. We cannot tell who is a complier or non-complier in the control group, as they receive $D = 0$ in either case. We must estimate the second term by predicting who in the control group would be a complier, had they been assigned to treatment. The exact procedure for classification isn't important, as long as predictions are made as accurate as possible.

The procedure for estimating τ_{PATT} using Theorem 1 is as follows:

1. Using the group assigned to treatment in the RCT ($S = 1, T = 1$), train a model to predict complier status C using the covariates W.

2. Using the model from step 1, predict who in the RCT assigned to control *would have* complied to treatment had they been assigned to the treatment group.

3. For the group of observed compliers to treatment and predicted compliers in the control group, train a model to predict the response using as features the covariates W and the treatment T (assigned and observed are the same, for these individuals). This model gives $\mathbb{E}(Y_{1t} \mid S = 1, C = 1, T = t, W)$ for $t = 0, 1$.

4. For all individuals who received treatment in the population ($S = 0, D = 1$), 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 τ_{PATT}.

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[1]The 10–fold cross–validated risk estimate on the training set is 22.6%. Information on the ensemble's candidate learners and corresponding weights and risk estimates are provided in Appendix Table 3.

Assumptions (2) and (3) are particularly important here: the success of the proposed estimator hinges on the assumption that the response curve is the same in the RCT and target population. If this is not true, then the potential outcomes Y_{10} and Y_{11} for target population individuals cannot be estimated using the model from step 3.

3   Simulations

We conduct a simulation study to compare the performance of the proposed estimator, Hartman et. al's estimator, and the instrumental variables estimate of the SATE, as estimators of τ_{PATT}. Our simulation is designed so that the effect of treatment is heterogeneous and depends on other covariates, and the design satisfies the conditional independence assumptions in Figure 1.

3.1   Simulation design

RCT eligibility, complier status, and treatment assignment in the population depend on observed covariates. The observed covariates (W_1, W_2, W_3) are multivariate normal with mean $(0.5, 1, -1)$ and covariances $\text{Cov}(W_1, W_2) = 1$ and $\text{Cov}(W_1, W_3) = \text{Cov}(W_2, W_3) = 0.5$. The equation for selection into studies is

$$S = \mathbb{I}(e_2 + g_1 W_1 + g_2 W_2 + g_3 W_3 + R > 0)$$

where R is standard normal. e_2 controls the fraction of the population eligible for the RCT. We set $g_1, g_2,$ and g_3 to be 0.5, 0.25, and 0.75, respectively. Complier status is determined by

$$C = \mathbb{I}(e_3 + h_2 W_2 + h_3 W_3 + Q > 0)$$

where Q is standard normal. e_3 controls the fraction of compliers in the population. We set h_2 and h_3 to 0.5. For individuals in the population $(S = 0)$, treatment is assigned by

$$T = \mathbb{I}(e_1 + f_1 W_1 + f_2 W_2 + V > 0)$$

Varying e_1 controls the fraction eligible for treatment in the population. V is standard normal. We set f_1 to 0.25 and f_2 to 0.75. For individuals in the RCT $(S = 1)$, treatment assignment is a sample from a Bernoulli distribution. We set treatment received D according to T and C: $D = T$ if $C = 1$ and $D = 0$ if $C = 0$. Finally, the response Y is determined by

$$Y = a + bD + c_1 W_1 + c_2 W_2 + dU$$

We assume that the treatment effect b is heterogeneous depending on W_1: $b = 1$ if $W_1 > 0.75$ and $b = -1$ if $W_1 \leq 0.75$. We set $a, c_1,$ and d to 1 and c_2 to 2. U is standard normal and $U, V, R, Q, (W_1, W_2, W_3)$ are mutually independent.

We generate a population of 30,000 individuals and randomly sample 5,000. Those among the 5,000 who are eligible for the RCT $(S = 1)$ are selected. Similarly, we sample 5,000 individuals from the population and select those who are not eligible for the RCT $(S = 0)$;

these are our observational study participants. This set-up mimics the reality that a population census is usually impossible. We set each individual's treatment received D according to their treatment assignment and complier status and observe their responses Y. In this design, the way that we've set S, T, D, C, and Y ensures that assumptions (1) - (7) hold.

In the assigned-treatment RCT group ($S = 1, T = 1$), we fit a logistic regression to compliance status using the covariates. With this model, we predict who in the control group ($S = 1, T = 0$) has $C = 1$, since this is unobservable. These individuals *would have* complied had they been assigned to the treatment group. For this group of observed compliers to treatment and predicted compliers from the control group of the RCT, we estimate the response curve using an ensemble of models with features (W_1, W_2, W_3) and D. Then population local average treatment effect on the treated is estimated according to the estimation procedure outlined above.

3.2   Simulation results

We vary each of the parameters e_1, e_2, and e_3 along a grid by -2 to 2 in increments of 0.5 in order to generate different combinations of rates of compliance, treatment eligibility, and RCT eligibility in the population of 30,000. For each possible combination of the three parameters, we run the simulation 5 times and compute the mean squared error of our proposed estimator for τ_{PATT} that adjusts for non-compliance, the estimator from Hartman et. al which is unadjusted for non-compliance, and the SATE using the instrumental variables estimator from Angrist et al. [1996], given by the intention-to-treat estimate divided by the rate of compliance. All other parameters are held fixed. The unadjusted estimate is obtained by estimating the response curve on all individuals in the RCT and applying step 4 of our proposed estimation procedure to the non-randomized trial individuals.

Figure 2 shows the relationship between the percent of compliers in the whole population, the percentage of people in the whole population eligible to participate in the RCT, and the mean squared error of the PATT estimators. The pattern of performance is qualitatively similar for the adjusted and unadjusted estimators: both perform best when compliance is high and perform badly when compliance is low. Furthermore, for a fixed rate of compliance, the estimators tend to have lower mean squared error when either a very small or very large fraction of the total population is eligible to participate in the RCT.

Figure 3 compares the mean squared error of the two PATT estimators and the SATE (as an estimator of PATT) at varying levels of compliance in the total population. When compliance is low, both PATT estimators have a large mean squared error and variance. The adjusted estimator has a large variance because the subset of the RCT who are considered compliers is relatively small, whereas the unadjusted estimator has large variance because of the large amount of crossover. For low levels of compliance, SATE actually tends to approximate PATT more closely; the variance due to small sample size contributes less to the mean squared error than the bias due to crossover in the other two estimators. On the other hand, when the compliance rate is high, both PATT estimators perform about equally well with mean squared error close to 0. Meanwhile, the median mean squared
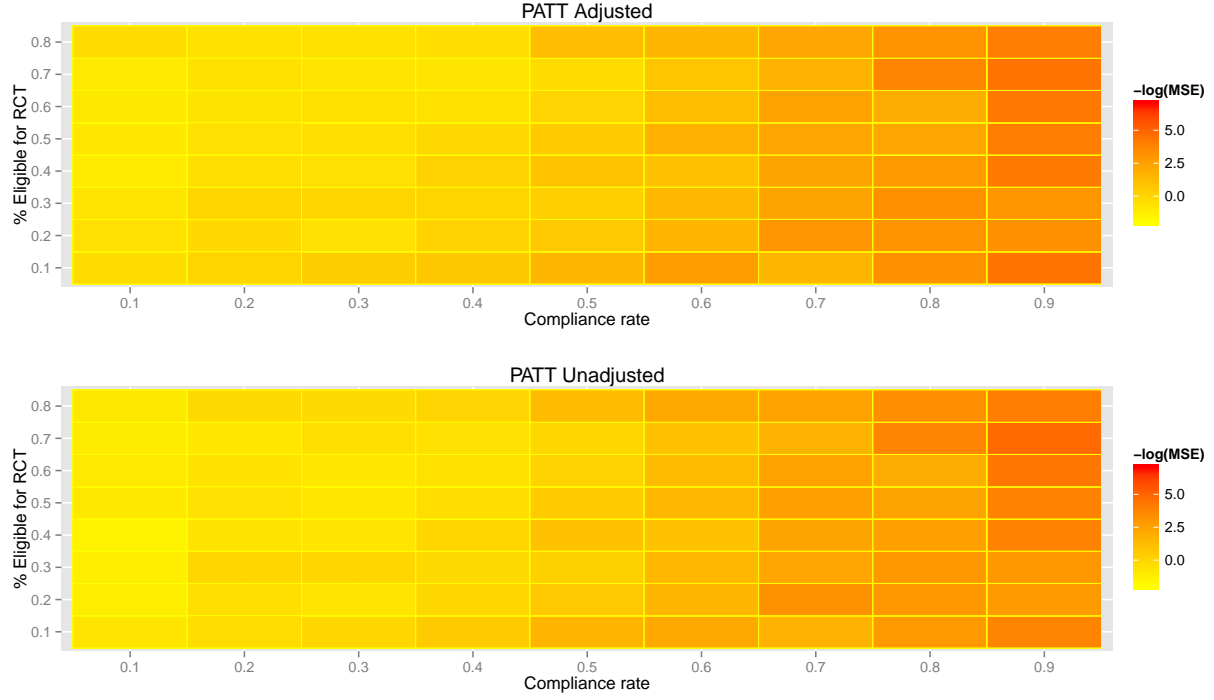
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Figure 2: Simulation mean squared error, transformed by -log. Darker tiles correspond to lower mean squared error.

error for SATE stays relatively constant regardless of compliance rate. This shows that the instrumental variables estimate of SATE is able to adjust for the non-compliance, but is unable to account for differences in pretreatment covariates between the RCT sample and target population.
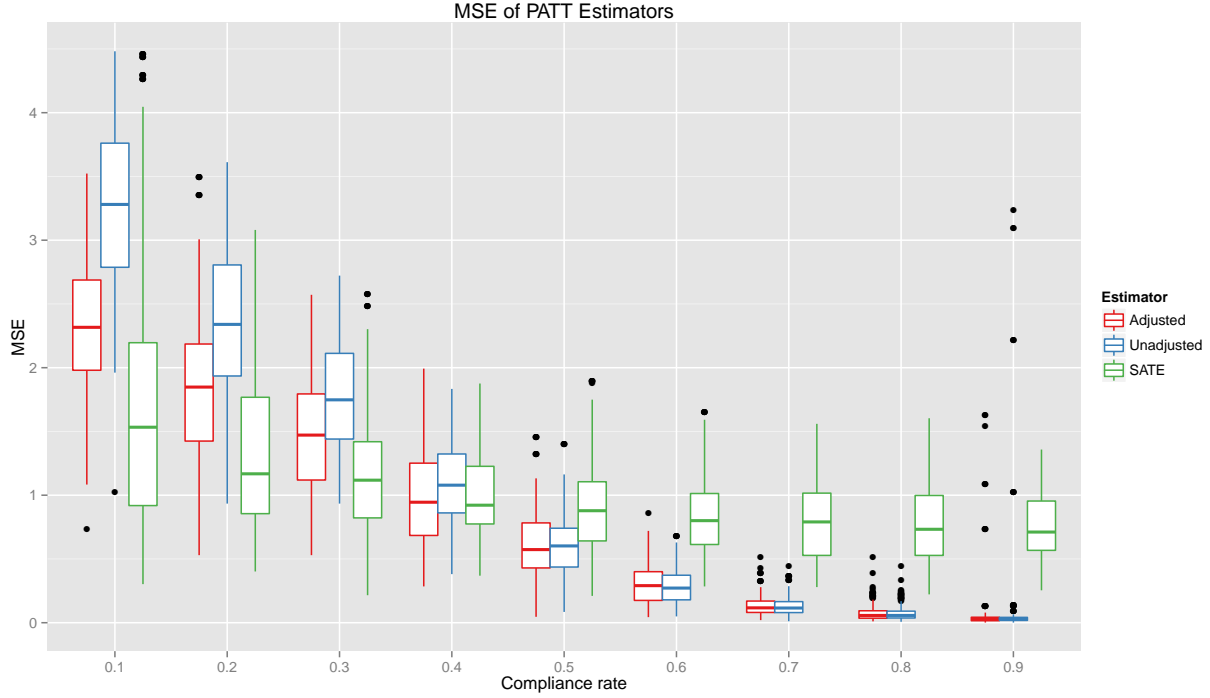
Figure 3: Simulation mean squared error, according to compliance rates in the total population.

4 Application: medicaid and health care use

4.1 Data

We apply the proposed method to estimate the effect of extending Medicaid coverage on health care use. Medicaid is a federally-funded program, so understanding how the RCT estimates generalize to a broader population of individuals who will be covered by other Medicaid expansions is informative for public policy. In particular, we examine the population of nonelderly adults in the U.S. with household incomes at or below 138% of the FPL ($32,913 for a four–person household in 2014) who may be eligible for Medicaid following the Affordable Care Act (ACA) expansion.

Oregon Health Insurance Experiment We draw RCT data from the Oregon Health Insurance Experiment (OHIE) [Finkelstein et al., 2012, Baicker et al., 2013, 2014, Taubman et al., 2014]. In 2008, approximately 90,000 uninsured low-income adults participated in a lottery to receive Medicaid benefits.[2] Treatment occurred at the household level: participants selected by the lottery won the opportunity for themselves and any household member to apply for Medicaid. Sample exclusions brought the sample size down to 74,922 individuals representing 66,385 households. Within the sample, 29,834 participants were selected by the

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[2]Eligible participants include Oregon residents (US citizens or legal immigrants) aged 19 to 64 not otherwise eligible for public insurance, who have been without insurance for six months, and have income below the FPL and assets below $2,000.

lottery; the remaining 45,008 participants served as controls in the experiment. Participants in selected households received benefits if they returned an enrollment application within 45 days of receipt. Among participants in selected households, about 60% mailed back applications and only 30% successfully enrolled.[3] The OHIE data includes pretreatment covariates for gender, age, race, ethnicity, health status, education, and household income.

Our outcomes of interest are indicator variables for any emergency room (ER) and outpatient visits in the past 12 months. The response data originate from a mail survey that was administered to participants over July and August 2009 ($N = 23,741$ survey respondents) Finkelstein et al. [2012]. We use the same definition of insurance coverage as Finkelstein et al. [2012] to form our measure of compliance, which is a binary variable indicating whether the participant was enrolled in any Medicaid program (including the lotteried program) between the notification date and 30 September 2009.

Observational data We employ data on the target population from the National Health Interview Study (NHIS) for Health Statistics for years 2008 to 2013. We restrict our sample to respondents with income below 138% of the FPL and who are uninsured or on Medicaid ($N = 11,129$). We use covariates on respondent characteristics that match the OHIE pretreatment covariates. The outcomes of interest from NHIS are variables on ER and outpatient visits in the past 12 months. We use a recoded variable that indicates whether respondents are on Medicaid as an analogue to the OHIE compliance measure.

4.2   Verifying assumptions

In order for the proposed estimator to perform well, assumptions (1)–(7) must be met. Assumption (1) ensures that potential outcomes for participants in the target population (i.e., respondents in the NHIS sample) would be identical to their outcomes in the RCT if they had been randomly assigned their observed treatment. A violation of the consistency assumption might arise, for instance, if there are differences in the mail surveys used to elicit health care use responses between the RCT and the non-randomized study.

We cannot directly test assumptions (2)–(3), which state that potential outcomes for treatment and control are independent of sample assignment for individuals with the same covariates W and assignment to treatment. The assumptions are only met if every possible confounder associated with the response and the sample assignment is accounted for. In our estimation of the response curve, we use all demographic, socioeconomic, and pre-existing health condition data that were common in the OHIE and NHIS data. Potentially important unobserved confounders include the number of hospital and outpatient visits in the previous year, proximity to health services, and enrollment in other federal programs.

Table 1 compares RCT particiapnts selected for Medicaid with NRT participants on

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[3]About half of the returned applications were deemed ineligible, primarily due to failure to demonstrate income below the FPL. Enrolled participants were required to recertify their eligibility status every six months.

Medicaid. Compared to the RCT treated, the "treated" population is predominantly female, younger, more racially and ethnically diverse, less educated, and live in higher income households. Diagnoses of diabetes, asthma, high blood pressure, and heart disease is more common among the population on Medicaid then the RCT treated. Conditioning on these covariates will improve our estimate of τ_{PATT}.

Covariate	OHIE control $n = 5476$		OHIE treated $n = 5193$		NHIS treated $n = 3382$	
	n	**%**	**n**	**%**	**n**	**%**
Female	3148	57.5	2920	56.2	2380	70.4
Age 19-49	1636	29.9	1367	26.3	2429	71.8
Age 50-64	3840	70.1	3826	73.7	953	28.2
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
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
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
Income < $10k	5476	100.0	3204	61.7	1452	42.9
Income $10k-$25k	0	0.0	1616	31.1	1622	48.0
Income > $25k	0	0.0	373	7.2	308	9.1
Response						
Any ER visit	1393	25.4	1301	25.1	881	26.1
Any outpatient visit	3299	60.2	3081	59.3	2116	62.6

Table 1: Pretreatment covariates and responses for OHIE and NHIS respondents on Medicaid.

Assumptions 4 — 5 are standard in the causal inference literature. A violation of Assumption 4 biases the estimate of τ_{PATT} if, for instance, treated participants' Medicaid coverage makes control participants less likely to visit the ER. Interference is less likely in experimental set–up because treatment occurs at the household level. Assumption 5 is violated if assignment to treatment influences the compliance status of individuals with the same covariates. Our compliance model can accuracy classify compliance for 77% of treated RCT participants with only the covariates —and not treatment assignment — as features.

Assumption 6 does not hold due to the presence of defiers — that is, participants who were assigned to control and enrolled in Medicaid during the study period. While Finkelstein

et al.'s [2012] IVLS estimates of the effect of Medicaid assume one–way crossover, 14% of control participants were actually enrolled in Medicaid during the study period (compared to the 60% of treated participants who did not enroll).The violation of the no defiers assumption does not appear to be appropriately addressed in Finkelstein et al. [2012] nor any of the authors' follow–up papers. We will explore how double–crossover affects our estimate of τ_{PATT} in a future version of this paper.

The exclusion restriction (Assumption 7) ensures treatment assignment affects the response only through assignment to Medicaid and the effect of Medicaid coverage may only be non-zero for compliers. It is reasonable that whether or not someone actually has Medicaid, not their eligibility to enroll, would affect their hospital use. For generic health insurance one might argue that eligibility could be negatively correlated with hospital use, as people with pre-existing conditions may be less likely to be eligible yet go to the hospital more frequently. This should not be the case with a federally funded program such as Medicaid.

4.3 Empirical results

We compare PATT and SATE estimates for ER and outpatient use. We obtain estimates for the overall group of participants and subgroups according to gender, age, race, ethnicity, health status, and education. Subgroup treatment effects are estimated by taking differences across response surfaces for a given covariate group. Response surfaces are estimated with an algorithmic model using random forests [Breiman, 2001].[4] We use treatment assignment, number of household members, gender, age, race, ethnicity, health status, education, and household income as features in the response models. The entire RCT sample size is $N = 23,205$ and the population sample is $N = 11,129$. There are $N = 10,669$ RCT compliers and $N = 3,382$ target population "compliers" on medicaid.

Figure 4 compares treatment effect estimates on ER use. We estimate the effect of Medicaid coverage on the likelihood of visiting the ER is about 1.3% for population compliers (adjusted PATT). In comparison, the effect on RCT compliers (adjusted SATT) is about zero. There are substantial differences between SATT and PATT estimates across covariate groups. For instance, the treatment effect on ER use for white sample participants is positive, but negative for white population participants.

Figures 5 compare treatment effect estimates on outpatient use. For population compliers, the effect of Medicaid coverage on the likelihood of outpatient use is about -2.5%. For RCT compliers, the effect is closer to zero (-1.2%). Treatment effects on outpatient use are large and positive for college–educated RCT compliers, but about zero for college–education population compliers.

We compare our estimates with those reported in Appendix Table 28 of Finkelstein et al. [2012]. In this table, the authors compare their IVLS estimates of the treatment effects on

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[4]Appendix Table 3 compares the risk estimates of the random forests model (default settings) with other candidate learners.

Figure 4: Comparison of estimators: any ER visit.
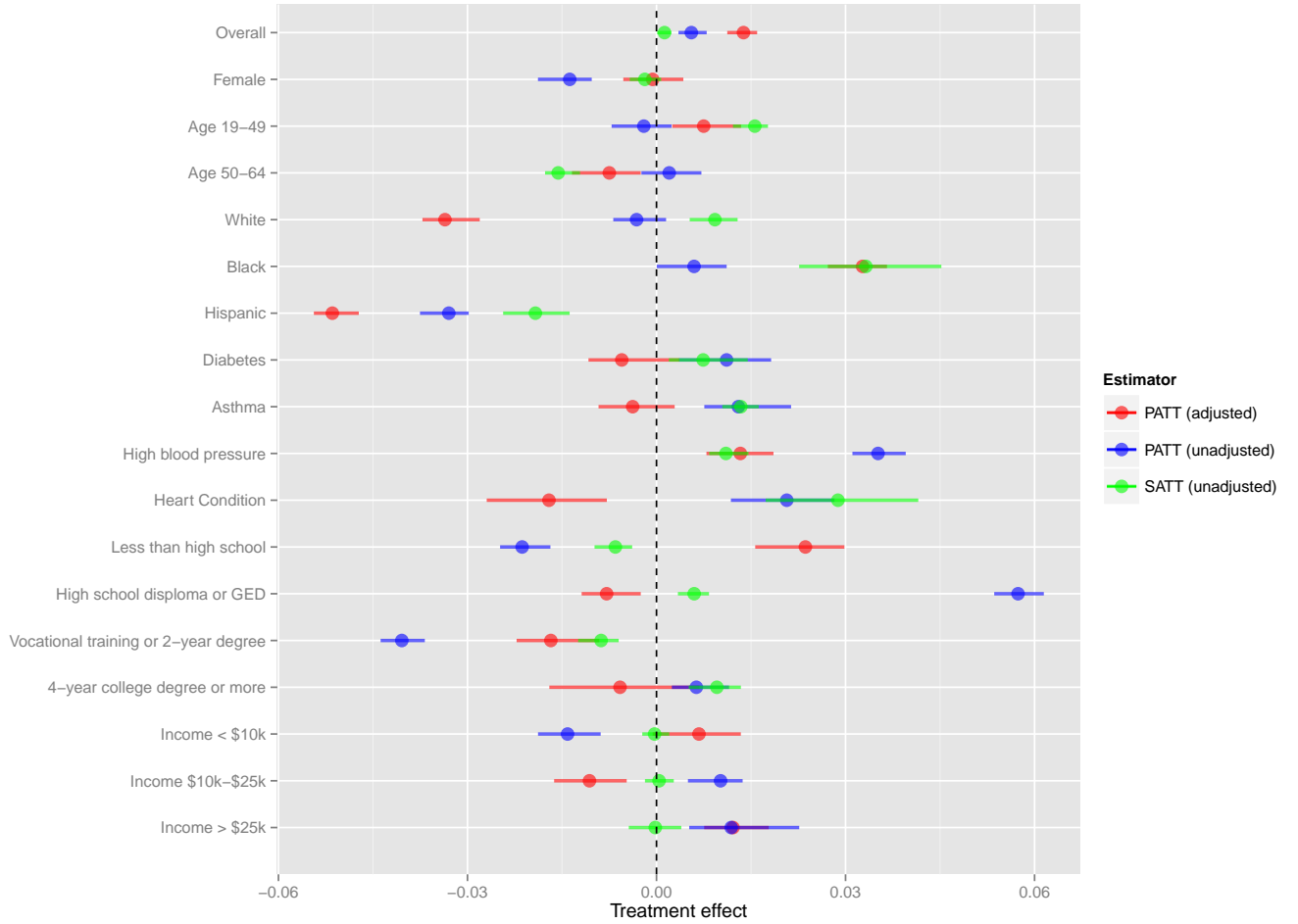
Note: horizontal lines represent 95% bootstrap confidence intervals.

RCT compliers with OLS estimates comparing individuals with and without Medicaid using a sample of NHIS respondents similar to the one used in our study. Finkelstein et al. [2012] report a slightly higher effect of Medicaid on the number of ER visits for the population compared to the RCT sample. The author's effect estimates on the number of outpatient visits is lower for the population compared to the RCT sample. Our estimates generally replicate these patterns.

Figure 5: Comparison of estimators: any outpatient visit.
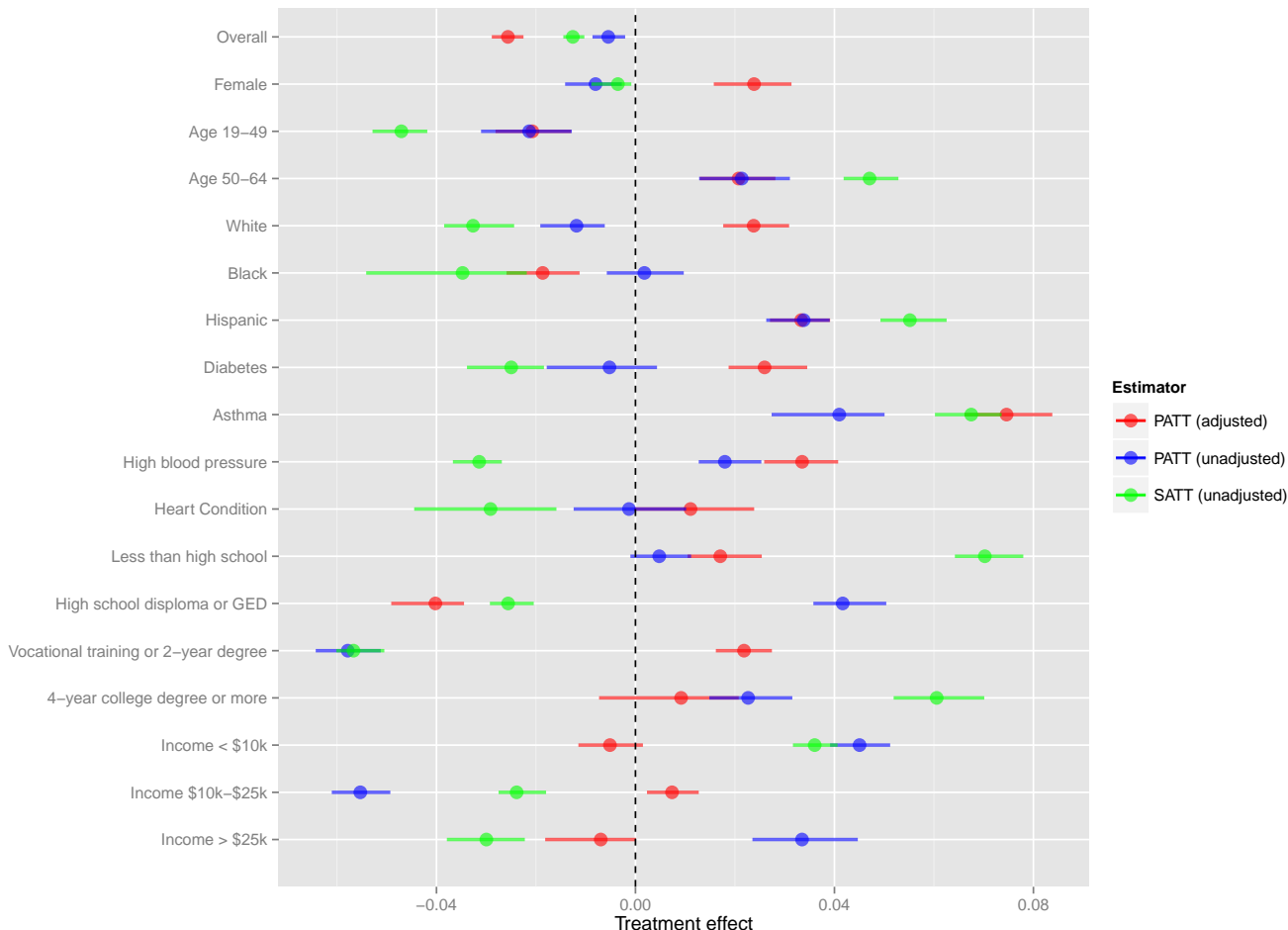
Note: horizontal lines represent 95% bootstrap confidence intervals.

5 Discussion

The treatment effect of Medicaid applies to uninsured adults with income below the FPL who express interest in health insurance coverage. The sample population differs in several dimensions from the target population of individuals who will be covered by other Medicaid expansions, such as the ACA expansion to cover all adults up to 138% of the FPL. For instance, the RCT participants are disproportionately white urban–dwellers [Taubman et al., 2014]. The RCT participants volunteered for the study and therefore may be in poorer health compared to the target population.

Simulations shed light on the conditions under which the proposed estimator should work well. When the rate of crossover from treatment to control is low, the proposed estimator performs better than the estimator which doesn't adjust for non-compliance. Here, the unadjusted estimator gives the intention-to-treat effect, which tends to underestimate the average

treatment effect on compliers. Of course, the simulation results depend on the particular way we parameterized the compliance, selection, treatment assignment, and response schedules. In particular, the strength of correlation between the covariates and compliance governs how well the estimator will perform, since step one of the estimation procedure is to predict who *would be* a complier in the RCT control group, had they been assigned to treatment. If it is difficult to predict compliance using the observed covariates, then the estimator will perform badly because of noise introduced by incorrectly treating non-compliers as compliers. Further research should be done into ways to test how well compliance can be predicted.

In the OHIE trial, only about 30% of those selected to receive Medicaid benefits actually enrolled. The proposed estimator adjusted for non-compliance performs better in simulations than the unadjusted estimator when compliance is low and can be predicted by observed covariates (Figure 3). Our ensemble method for predicting compliance based on observed covariates has 77% accuracy on the training set of individuals in OHIE assigned to the treatment group. While we don't know how well the compliance model predicts for the control group, the model's performance on the training set suggests that compliance is not purely random and depends on observed covariates. This gives evidence in favor of using the proposed estimator.

The proposed method allows us to decompose both SATT and PATT estimates by subgroup according to covariates common to both RCT and observational datasets (e.g, demographic variables, pre–existing conditions, and insurance coverage). We find substantial differences between sample and population estimates in terms of race, and pre–existing condition subgroups. This pattern is expected because the RCT participants are predominately white and volunteered for the study. Our PATT estimates on ER visits is lower than the corresponding SATT estimates, which potentially demonstrates the adverse selection problem in the RCT.

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Appendix

A   Ensemble method for compliance model

Table 2: Risk estimates for compliance model ensemble.

Algorithm	Parameters	Avg.	SE	Min.	Max.
Super Learner (`SuperLearner`)	default	0.226	0.001	0.222	0.231
Generalized boosted models (`gbm`)	default	0.226	0.001	0.222	0.231
Lasso regression (`glmnet`)	$\alpha = 1$	0.227	0.001	0.224	0.233
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.25$	0.227	0.001	0.224	0.232
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.5$	0.227	0.001	0.224	0.233
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.75$	0.227	0.001	0.224	0.233
Neural network (`nnet`)	default	0.227	0.001	0.224	0.232
Random forests (`randomForest`)	default	0.307	0.003	0.293	0.326
Random forests (`randomForest`)	mtry = 1	0.273	0.002	0.268	0.281
Random forests (`randomForest`)	mtry = 5	0.307	0.003	0.294	0.326
Random forests (`randomForest`)	mtry = 10	0.310	0.003	0.294	0.329
Ridge regression (`glmnet`)	$\alpha = 0$	0.227	0.001	0.224	0.232

Notes: risk based on the 10–fold cross-validated estimate of the mean squared error for each algorithm.

B Ensemble method for response models

B.1 Response model on RCT compliers

Table 3: Cross–validated risk and weights used for each algorithm in super learner ensemble for response model on RCT compliers.

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Algorithm	Parameters	Risk	Weight
Generalized boosted models (`gbm`)	default	0.188	0
Lasso regression (`glmnet`)	$\alpha = 1$	0.188	1
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.25$	0.188	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.5$	0.188	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.75$	0.188	0
Neural network (`nnet`)	default	0.234	0
Random forests (`randomForest`)	default	0.24	0
Random forests (`randomForest`)	mtry = 1	0.251	0
Random forests (`randomForest`)	mtry = 5	0.24	0
Random forests (`randomForest`)	mtry = 10	0.241	0
Ridge regression (`glmnet`)	$\alpha = 0$	0.188	0

Any outpatient visit

Algorithm	Parameters	Risk	Weight
Generalized boosted models (`gbm`)	default	0.24	0
Lasso regression (`glmnet`)	$\alpha = 1$	0.24	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.25$	0.24	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.5$	0.24	0.76
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.75$	0.24	0
Neural network (`nnet`)	default	0.242	0
Random forests (`randomForest`)	default	0.331	0
Random forests (`randomForest`)	mtry = 1	0.392	0.23
Random forests (`randomForest`)	mtry = 5	0.333	0
Random forests (`randomForest`)	mtry = 10	0.333	0.008
Ridge regression (`glmnet`)	$\alpha = 0$	0.24	0

'Risk' is the 10–fold cross-validated risk estimate based on mean squared error for each algorithm. 'Weight' is the coefficient for the super learner, which is estimated using non–negative least squares based on the Lawson-Hanson algorithm.

B.2 Response model on all RCT participants

Table 4: Cross–validated risk and weights used for each algorithm in super learner ensemble for response model on all RCT participants.

Any ER visit

Algorithm	Parameters	Risk	Weight
Generalized boosted models (`gbm`)	default	0.188	0
Lasso regression (`glmnet`)	$\alpha = 1$	0.188	0.54
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.25$	0.188	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.5$	0.188	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.75$	0.188	0
Neural network (`nnet`)	default	0.228	0
Random forests (`randomForest`)	default	0.24	0
Random forests (`randomForest`)	mtry = 1	0.252	0
Random forests (`randomForest`)	mtry = 5	0.241	0
Random forests (`randomForest`)	mtry = 10	0.241	0
Ridge regression (`glmnet`)	$\alpha = 0$	0.188	0.459

Any outpatient visit

Algorithm	Parameters	Risk	Weight
Generalized boosted models (`gbm`)	default	0.239	0
Lasso regression (`glmnet`)	$\alpha = 1$	0.239	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.25$	0.239	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.5$	0.239	0
GLM with elasticnet regularization (`glmnet`)	$\alpha = 0.75$	0.239	0
Neural network (`nnet`)	default	0.24	0
Random forests (`randomForest`)	default	0.327	0
Random forests (`randomForest`)	mtry = 1	0.391	0.261
Random forests (`randomForest`)	mtry = 5	0.331	0
Random forests (`randomForest`)	mtry = 10	0.329	0.008
Ridge regression (`glmnet`)	$\alpha = 0$	0.239	0.73