Estimating population average treatment effects from experiments with noncompliance*

Kellie Ottoboni† Jason Poulos‡

March 13, 2018

Abstract

This paper extends a method of estimating population average treatment effects from randomized controlled trials (RCTs) to settings with noncompliance. We identify the complier–average causal effect for the target population with few additional assumptions. Simulations show the compliance–adjusted estimator performs better than the unadjusted estimator when compliance is relatively low and can be predicted by observed covariates. We apply the proposed estimator to measure the effect of Medicaid coverage on health care use for a target population of adults who may benefit from expansions to the Medicaid program. We draw RCT data from a large-scale health insurance experiment in which a small subset of those randomly selected to receive Medicaid benefits actually enrolled. The RCT sample differs in several dimensions from the target population of individuals who will be covered by government-backed Medicaid expansions. We find substantial differences between sample and population estimates in terms of race, education, and health status subgroups.

*The authors thank Jon McAuliffe and Jas Sekhon and his research group at UC Berkeley for valuable comments. Poulos acknowledges support from the National Science Foundation Graduate Research Fellowship [grant number DGE 1106400].

†Department of Statistics, University of California, Berkeley. email: `kellieotto@berkeley.edu`.

‡*Corresponding author:* Department of Political Science, University of California, Berkeley. email: `poulos@berkeley.edu`.

1 Introduction

Randomized control trials (RCTs) are the gold standard for estimating the causal effect of a treatment. An RCT may give an unbiased estimate of the sample average treatment effect (SATE), but external validity is an issue when the individuals in the RCT are unrepresentative of the actual population of interest. For example, the participants in an RCT in which individuals volunteer to sign up for health insurance may be in poorer health at baseline than the overall population. External validity is particularly relevant to policymakers who want to know how the SATE would generalize to the broader population.

Hartman et al. (2015) propose a method of reweighting the responses of individuals in an RCT 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 is identified from the RCT outcomes. We extend the method to estimate the PATT from RCT data with noncompliance.[1] Noncompliance, which occurs when individuals who are assigned to the treatment group do not comply with the treatment, is a prevalent issue in RCTs. It dilutes the estimated effect of treatment assignment, and the resulting intention–to–treat (ITT) estimate is biased towards zero.

The proposed estimator of PATT involves estimating 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. We use a classification or regression model to estimate the response surface, the conditional expectation of responses in the RCT. We then use the response model to estimate population members' outcomes given their covariates, giving an estimate of the PATT. Explicitly modeling compliance allows us to decompose PATT estimates by subgroup according to covariates common to both RCT and observational datasets.

We apply the proposed estimator to measure the effect of Medicaid coverage on health

1. Similar approaches to the problem of assessing the external validity of RCTs (e.g., Imai, King, and Stuart 2008; Stuart et al. 2011) do not consider settings with noncompliance.

care use for a target population of adults who may benefit from government-backed expansions to the Medicaid program. We draw RCT data from a large-scale health insurance experiment, in which only 30% of those randomly selected to receive Medicaid benefits actually enrolled. We find substantial differences between sample and population estimates in terms of race, education, and health status subgroups.

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

2 Estimator

We are interested in using the outcomes from an RCT to estimate the average treatment effect on the treated for a target population. Treatment in the population is not assigned at random, but rather may depend 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 nonrandomized, 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 $s = 0$ is the population and $s = 1$ is the RCT, and let t be the treatment received. For simplicity, we assume there are only two possible treatments, $t = 1$ for the active treatment and $t = 0$ for the control or no treatment. 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. Treatment is assigned at random by the investigator in the RCT, so we observe both D_i and T_i when $S_i = 1$. For compliers, $D_i = T_i$.

In the population, we suppose that treatment is made available to individuals based on their covariates W_i. 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_i, not T_i, for individuals in the population. Among the population controls, we can't distinguish noncompliers 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 C_i is only observable for individuals assigned to treatment in the RCT.

Assumptions (1) – (4) defined in Appendix A are made by Hartman et al. (2015) to identify PATT from an RCT. We include Assumption (5) to ensure that compliance is independent of sample and treatment assignment for all individuals with covariates W.

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

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

Figure 1 shows Assumptions (2), (3), and (5) in a directed acyclic graph. Treatment assignment T may only depend on C through W, and the potential outcomes (Y_{s0}, Y_{s1}) may

only depend on S through W.

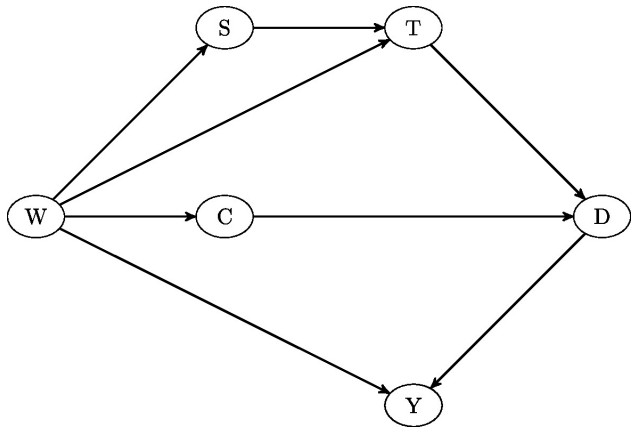

Figure 1: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT.

We additionally include Assumptions (6) – (7), which are made by Angrist, Imbens, and Rubin (1996) to ensure identifiability. The former assumption ensures that crossover is only possible from treatment to control. The latter assumption ensures treatment assignment affects the response only through the treatment received. In particular, the treatment effect may only be nonzero for compliers.

Assumption 6. *No defiers:*

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

Assumption 7. *Exclusion restriction: For noncompliers,*

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

2.2 Population Average Treatment Effect on the Treated (PATT)

The estimand of interest is the average treatment effect on those in the population who receive treatment:

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

It includes individuals who actually receive the treatment, but does not include those who are eligible for treatment and do not accept it (i.e., noncompliers). The following theorem, which we modify from Hartman et al. (2015) to account for noncompliance, 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)*,*

$$\tau_{PATT} = \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{11} \mid S = 1, D = 1, W\right)\right] - \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{10} \mid S = 1, T = 0, C = 1, W\right)\right] \tag{2}$$

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 linearly into two terms and consider each individually.

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

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

The last line follows because of the randomization carried out in the RCT, which guarantees $Y_{10} \perp\!\!\!\perp T \mid (W, S=1)$. Finally, the result follows by plugging these two expressions into Eq. (1). □

2.3 Estimation procedure

There are two challenges in turning Theorem 1 into an estimator of τ_{PATT} in practice. First, we must estimate the inner expectation over potential outcomes of compliers in the RCT. In our empirical example, we use an ensemble of algorithms (Laan, Polley, and Hubbard 2007) to estimate the response surface for compliers in the RCT, given their covariates. Thus, the first term in the expression for τ_{PATT} is estimated by the weighted average of points on the response surface, evaluated for each treated population member's potential outcome under treatment. The second term is estimated by the weighted average of points on the response surface, evaluated for each treated population member's potential outcome under control.

The second challenge is that we cannot observe which individuals are included in the estimation of the second term. In the RCT control group, C is unobservable, as they always receive no treatment ($D=0$). We must estimate the second term of Eq. (2) by predicting who in the control group would be a complier, had they been assigned to treatment. An alternative approach, described in Freedman (2006), is to simply weight the unadjusted PATT estimate by the population compliance rate in order to yield a population average effect of treatment on treated compliers.[2] However, the compliance rate is likely to differ

2. A similar approach is used by Imai, Tingley, and Yamamoto 2013 for estimating average complier

between the sample and population, as well as across subgroups. By explicitly modeling compliance, our approach allows us to decompose PATT estimates by covariate group.

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 $t \in \{0, 1\}$.

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}.

Assumptions (2) and (3) are particularly important here: the success of the proposed estimator hinges on the assumption that the response surface is the same for compliers 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 [2015] estimator, and the Sample Average Treatment Effect on the Treated (SATT) estimate. Our simulation is designed so that the effect of treatment is heterogeneous

indirect effects.

and depends on covariates which are different in the RCT and target population. 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 the RCT 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 with parameter 1/2. 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.[3] 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 surface using a random forests model with features (W_1, W_2, W_3) and D (Breiman 2001). Then population local average treatment effect on the treated is estimated according to the estimation procedure outlined above.

3. This set-up mimics the reality that a population census is usually impossible.

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 average root mean squared error (RMSE) of our proposed estimator for τ_{PATT} that adjusts for noncompliance, the PATT estimator from Hartman et al. (2015) which is unadjusted for noncompliance, and the SATT. All other parameters are held fixed. The unadjusted PATT estimate is obtained by estimating the response surface on all individuals in the RCT and applying Step 4 of our estimation procedure to the nonrandomized trial individuals. The SATT is estimated from the response surface for compliers from Step 3.

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 RMSE 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 RMSE of both estimators increases with the fraction of the total population eligible to participate in the RCT. When less than 70% of the RCT is compliers, the adjusted estimator tends to have lower RMSE than the unadjusted estimator.

Figure 3 compares the RMSE of the two PATT estimators with the SATT (as an estimator of PATT) at varying levels of compliance in the total population. When compliance is low, both PATT estimators have a large RMSE. 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 bias from the large amount of crossover. For low levels of compliance, the SATT actually tends to estimate PATT more closely than either PATT estimator. On the other hand, when the compliance rate is high, both PATT estimators have low RMSE, with he adjusted estimator performing slightly better. Meanwhile, the

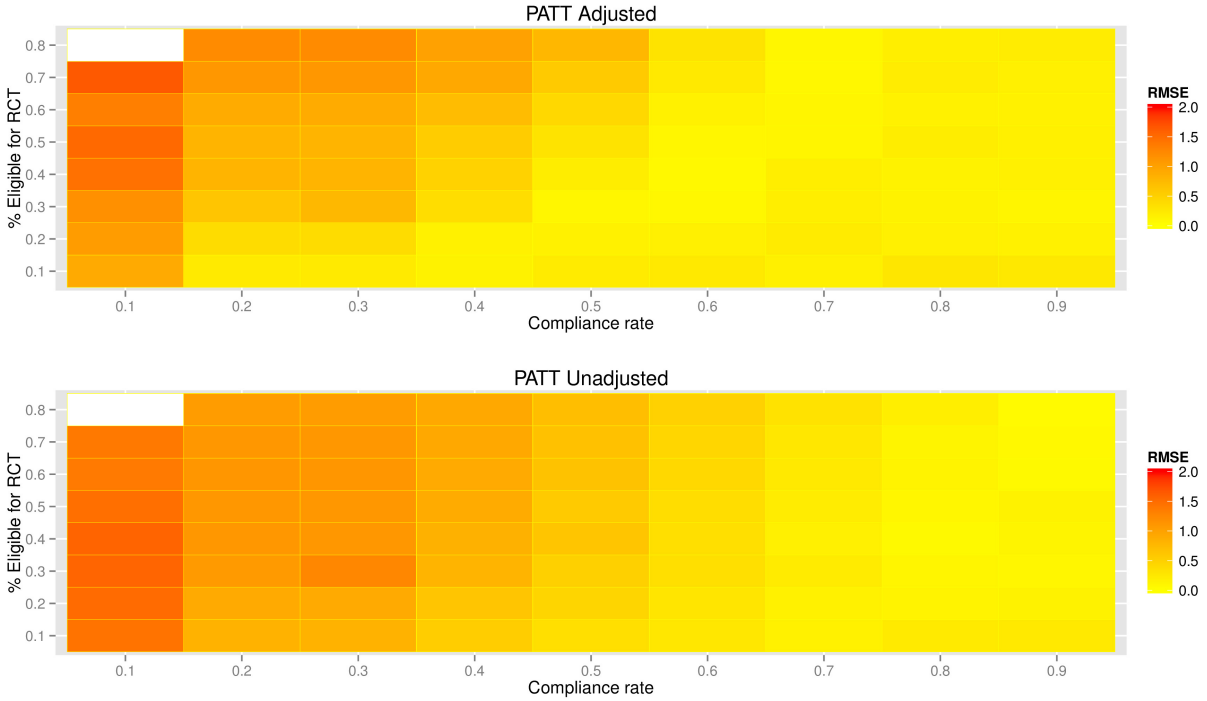

Figure 2: Simulated RMSE, binned by compliance rate and percent eligible for the RCT. Darker tiles correspond to worse estimates of PATT.

median RMSE for the SATT stays relatively constant regardless of compliance rate; the estimator is unbiased for the SATT rather than the PATT. The SATT estimate accounts for the noncompliance, but is unable to account for differences in pretreatment covariates between the RCT sample and target population. This shows that a reweighting approach is needed to extrapolate RCT results to a wider population.

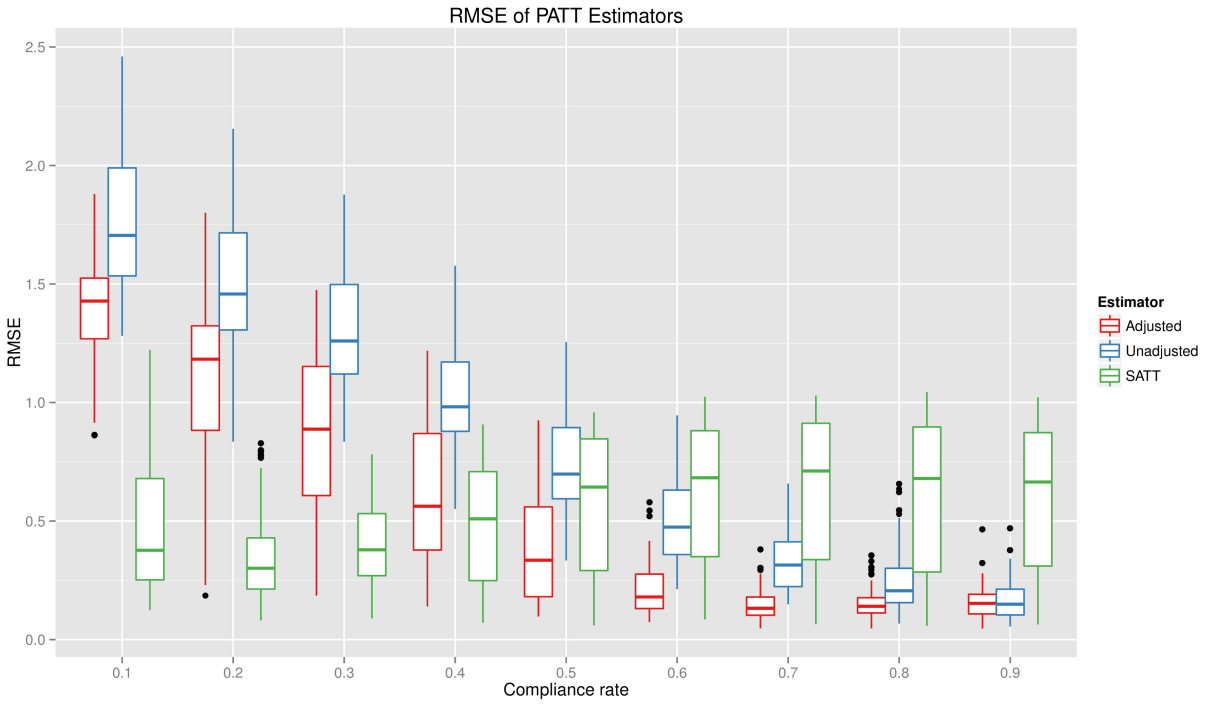

Figure 3: Simulated RMSE of compliance-adjusted PATT, unadjusted PATT, and SATT, according to compliance rates in the total population.

4 Application: Medicaid and health care use

We apply the proposed estimator to measure the effect of Medicaid coverage on health care use for a target population of adults who may benefit from expansions to the Medicaid program. In particular, we examine the population of nonelderly adults in the U.S. with household incomes at or below 138% of the Federal Poverty Level (FPL) — which amounts to $32,913 for a four–person household in 2014 — who may be eligible for Medicaid following

the Affordable Care Act (ACA) expansion.

4.1 RCT sample

We draw RCT data from the Oregon Health Insurance Experiment (OHIE) (Finkelstein et al. 2012; Katherine Baicker et al. 2013; K Baicker et al. 2014; Taubman et al. 2014). In 2008, approximately 90,000 uninsured low-income adults participated in the OHIE to receive Medicaid benefits.[4] Treatment occurred at the household level: participants selected by the lottery won the opportunity for themselves and any household member to apply for Medicaid. Within a sample of 74,922 individuals representing 66,385 households, 29,834 participants were selected by the 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.

Our outcomes of interest are binary 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). 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. The OHIE data include pretreatment covariates for gender, age, race, ethnicity, health status, education, and household income.

4.2 Observational data

We acquire data on the target population from the National Health Interview Study (NHIS) for years 2008 to 2013. We restrict our sample to respondents with income below 138% of

4. 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.

the FPL and who are uninsured or on Medicaid ($N = 11,129$). We select 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.

4.3 Verifying assumptions

In order for τ_{PATT} to be identified, 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 nonrandomized 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 surface, 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 participants selected for Medicaid with NRT participants on Medicaid. Compared to the RCT treated, the target population "treated" group 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 are more common among the population on Medicaid then the RCT treated.

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

	OHIE control $n = 5476$		OHIE treated $n = 5193$		NHIS treated $n = 3382$	
Covariate	**n**	%	**n**	%	**n**	%
Sex:						
Female	3148	57.5	2920	56.2	2380	70.4
Age:						
19-49	1636	29.9	1367	26.3	2429	71.8
50-64	3840	70.1	3826	73.7	953	28.2
Race:						
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
Health status:						
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
Education:						
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:						
$< \$10$k	5476	100.0	3204	61.7	1452	42.9
\$10k-\$25k	0	0.0	1616	31.1	1622	48.0
$> \$25$k	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

Assumptions 4 — 5 are standard in the causal inference literature. A violation of the assumption of no–interference biases the estimate of τ_{PATT} if, for instance, treated participants' Medicaid coverage makes control participants more likely to visit the ER. Interference is less likely in this 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 accurately classify compliance status for 77% of treated RCT participants with only the covariates — and not treatment assignment — as features.[5] This gives evidence in favor of the conditional independence assumption.

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] instrumental variables 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 exclusion restriction (Assumption 7) ensures treatment assignment affects the response only through enrollment in Medicaid. It is reasonable that a person's enrollment in Medicaid, not just their eligibility to enroll, would affect their hospital use. For private health insurance one might argue that eligibility may be be negatively correlated with hospital use, as people with pre-existing conditions are less often eligible yet go to the hospital more frequently. This should not be the case with a federally funded program such as Medicaid.

4.4 Empirical results

We compare PATT and SATT estimates for ER and outpatient use. We obtain estimates for the overall group of participants and subgroups according to sex, age, race, health status, education, and household income. Subgroup treatment effects are estimated by taking differences across response surfaces for a given covariate subgroup, and response surfaces

5. The 10–fold cross–validated risk estimate on the training set is 22.6%. Information on the ensemble's candidate learners and corresponding risk estimates are provided in Table 2.

are estimated with random forests.[6] We use treatment assignment, number of household members, and the subgroup covariates as features in the response models. We generate 95% confidence intervals for these estimates using 1,000 bootstrap samples. 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 1.2% and RCT participants assigned to treatment (unadjusted 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. Treatment effects on ER use tend to be large and significant for RCT participants with health conditions, but negative for the corresponding population participants.

Figure 5 compares treatment effect estimates on outpatient use. For population compliers, the effect of Medicaid coverage on the likelihood of outpatient use is -2.5%. In comparison, the effect is -2% for RCT compliers and -1.2% for RCT participants assigned to treatment. Treatment effects on outpatient use are large and positive for college–educated RCT compliers, but about zero for college–education population compliers.

4.4.1 Comparison with Finkelstein et al. (2012) estimates

We compare our estimates with the estimated treatment effects on number of ER and outpatient visits reported in Appendix Table 28 of Finkelstein et al. (2012). In this table, the authors compare their instrumental variables estimates of the treatment effects on RCT compliers with OLS estimates using a sample of NHIS respondents similar to the one used in our study. The authors report a slightly higher effect of Medicaid on the number of ER

6. The 10–fold cross-validated risk estimate of the random forests model (default settings) range from 0.24 to 0.331.

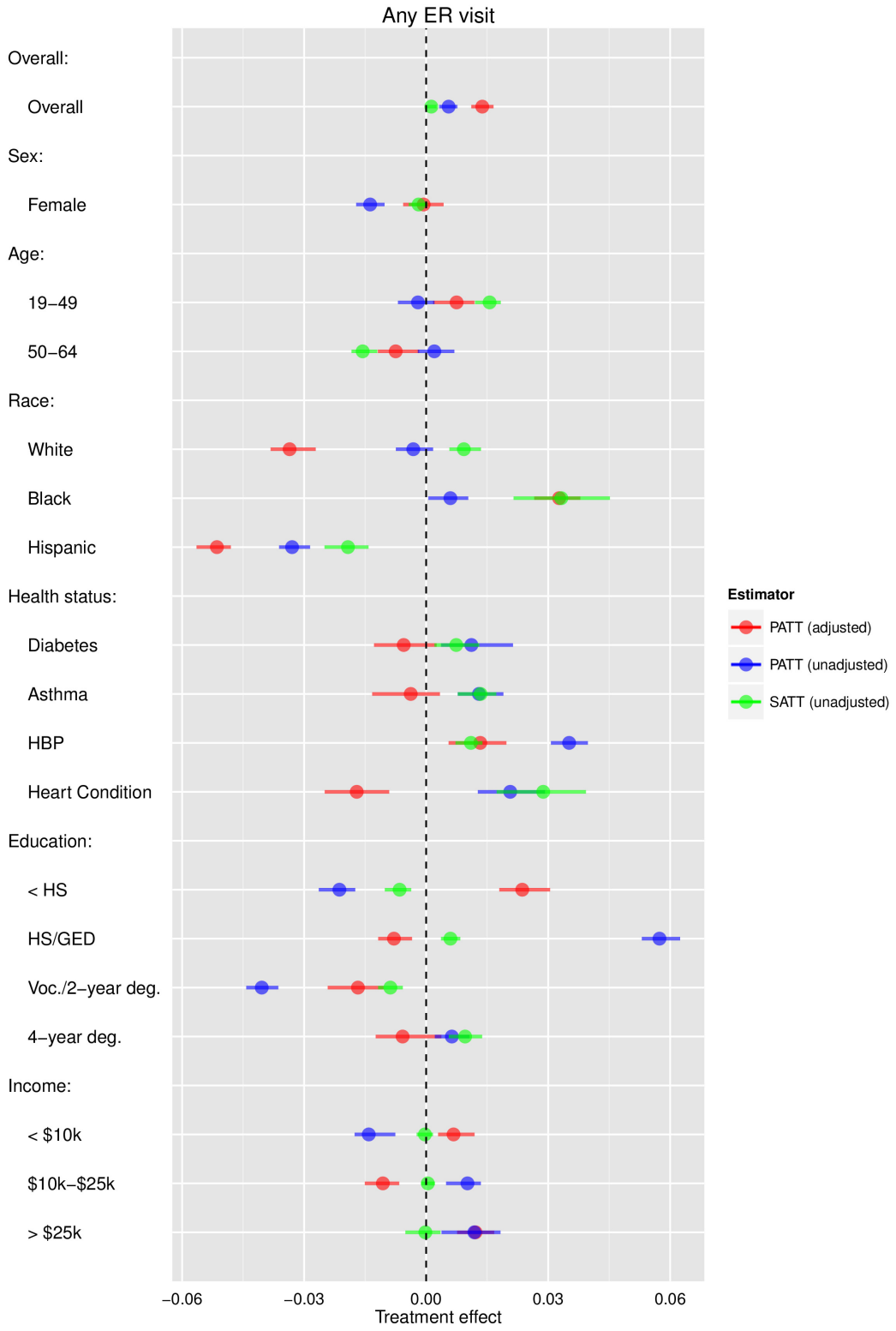

Figure 4: Comparison of estimators: any ER visit. Horizontal lines represent 95% bootstrap confidence intervals.

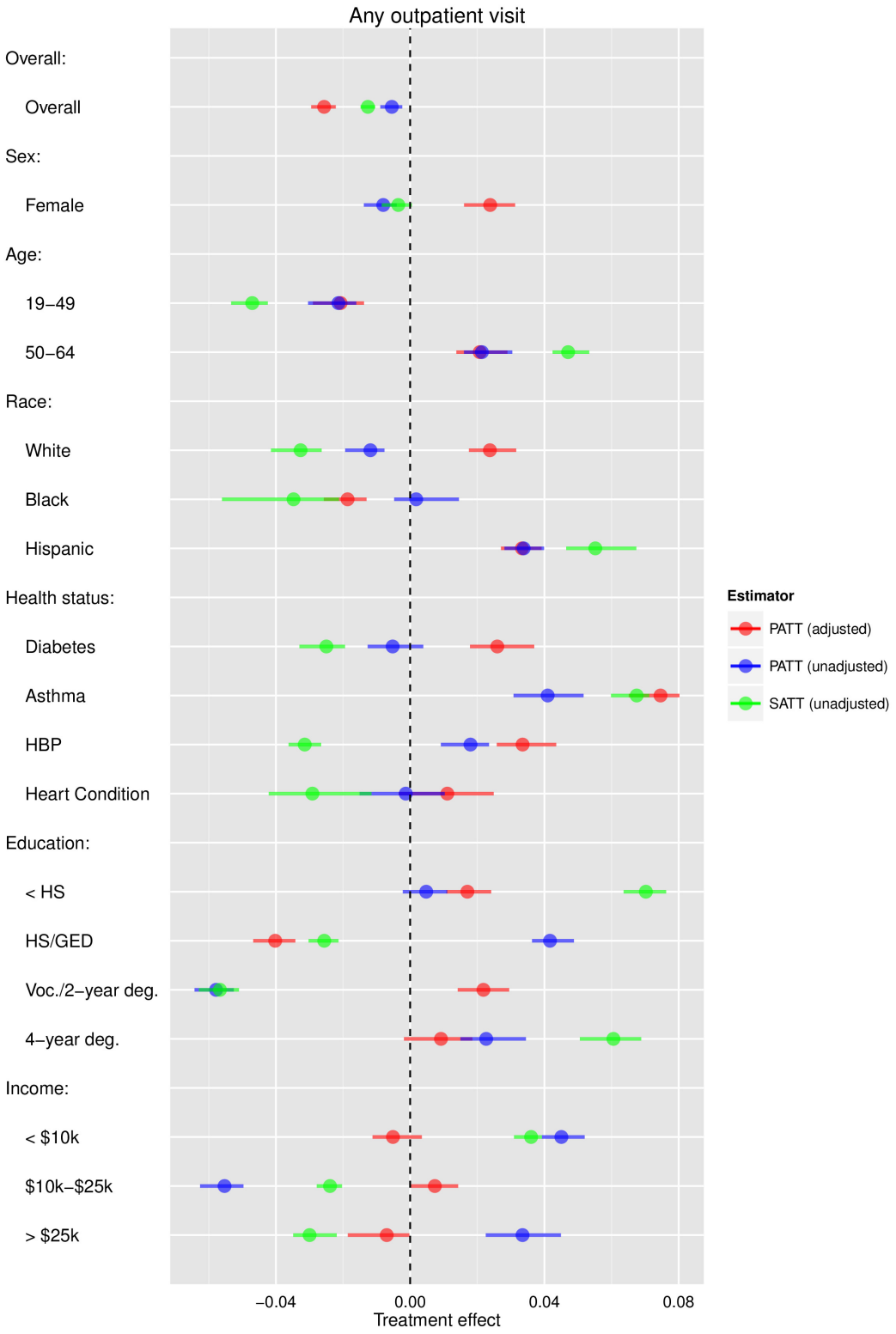

Figure 5: Comparison of estimators: any outpatient visit. Horizontal lines represent 95% bootstrap confidence intervals.

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.

5 Discussion

The simulations presented in Section 3 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 noncompliance. Here, the unadjusted estimator gives the ITT 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 1 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 noncompliers as compliers. Further research should be done into ways to test how well the model of compliance works in the population.

The proposed estimator, adjusting for noncompliance, performs better in simulations than the unadjusted estimator when compliance is low and can be predicted by observed covariates (Figure 3). In the OHIE trial, only about 30% of those selected to receive Medicaid benefits actually enrolled. Our ensemble method for predicting compliance based on observed covariates has 77% accuracy on the training set of participants in the OHIE assigned to treatment. While we don't know how well the compliance model predicts for the control group, the control group should be similar to the treatment group on pretreatment covariates because of the randomization done. 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 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. These differences in baseline covariates make a reweighting method necessary to extend the RCT results to the population.

Explicitly modeling compliance allows us to decompose SATT and PATT estimates by subgroup according to pretreatment 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, education, and health status subgroups. This pattern is expected because RCT participants volunteered for the study and are predominately white and educated. Across all subgroups, the adjusted PATT estimates are generally significant and negative for the effect of Medicaid on ER visits, and significant and positive for the treatment effect on outpatient visits. Intuitively, Medicaid coverage should decrease ER visits — a public "bad" — and increase outpatient visits — a public "good". This pattern does not generally hold for the unadjusted PATT nor SATT estimates, which demonstrates the benefit of using our proposed estimator in settings with noncompliance.

Further research might explore models to more accurately predict compliance in RCTs. Our compliance model accurately classified compliance status for 77% of treated RCT participants using only the pretreatment covariates as features. Accurately predicting compliance is not only essential for yielding unbiased estimates of the compliance–adjusted PATT, it is also useful for researchers and policymakers to know which groups of individuals are unlikely

to comply with treatment.

References

Angrist, Joshua D., Guido W. Imbens, and Donald B. Rubin. 1996. "Identification of Causal Effects Using Instrumental Variables." *Journal of the American Statistical Association* 91, no. 434 (June): 444–455.

Baicker, K, A Finkelstein, J Song, and S Taubman. 2014. "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.

Baicker, Katherine, 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. 2013. "The Oregon experiment—effects of Medicaid on clinical outcomes." *New England Journal of Medicine* 368 (18): 1713–1722.

Breiman, Leo. 2001. "Random forests." *Machine learning* 45 (1): 5–32.

Finkelstein, Amy, Sarah Taubman, Bill Wright, Mira Bernstein, Jonathan Gruber, Joseph P Newhouse, and Heidi Allen. 2012. "The Oregon Health Insurance Experiment: Evidence from the First Year." *The Quarterly Journal of Economics* 127 (3): 1057.

Freedman, David A. 2006. "Statistical Models for Causation What Inferential Leverage Do They Provide?" *Evaluation Review* 30 (6): 691–713.

Hartman, Erin, Richard Grieve, Roland Ramsahai, and Jasjeet S Sekhon. 2015. "From SATE to PATT: Combining experimental with observational studies to estimate population treatment effects." *JR Stat. Soc. Ser. A* 10:1111.

Imai, Kosuke, Gary King, and Elizabeth Stuart. 2008. "Misunderstandings Among Experimentalists and Observationalists about Causal Inference." *Journal of the Royal Statistical Society, Series A* 171, part 2:481–502.

Imai, Kosuke, Dustin Tingley, and Teppei Yamamoto. 2013. "Experimental designs for identifying causal mechanisms." *Journal of the Royal Statistical Society: Series A (Statistics in Society)* 176 (1): 5–51.

Laan, Mark J van der, Eric C Polley, and Alan E Hubbard. 2007. "Super learner." *Statistical Applications in Genetics and Molecular Biology* 6 (1).

Stuart, Elizabeth A, Stephen R Cole, Catherine P Bradshaw, and Philip J Leaf. 2011. "The use of propensity scores to assess the generalizability of results from randomized trials." *Journal of the Royal Statistical Society: Series A (Statistics in Society)* 174 (2): 369–386.

Taubman, Sarah, Heidi Allen, Bill Wright, Katherine Baicker, and Amy Finkelstein. 2014. "Medicaid Increases Emergency Department Use: Evidence from Oregon's Health Insurance Experiment." *Science* 343, no. 6168 (January): 263–268.

Appendix

A Assumptions to identify unadjusted PATT

Assumptions (1) – (4) are made by Hartman et al. (2015) to identify PATT from an RCT.

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

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

Assumption (1) requires that 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:*

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

Assumption (2) ensures the potential outcomes for treatment are independent of sample assignment for individuals with the same covariates W and assignment to treatment.[7] We make a similar assumption for the potential outcomes under control:

7. Throughout, we assume individuals are sampled randomly from an infinite population.

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

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

Assumption 4. *No interference between units: The potential outcomes Y_{ist} for individual i, $s = 0, 1$, $t = 0, 1$ do not depend on T_j, for all $j \neq i$.*

B 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

Note: risk based on the 10–fold cross-validated estimate of the MSE for each algorithm.

C Ensemble method for response models

C.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.

Any ER visit			
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			
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 MSE for each algorithm. 'Weight' is the coefficient for the super learner, which is estimated using nonnegative least squares based on the Lawson-Hanson algorithm.

C.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			
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