

# 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 policy-makers 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 assume monotonicity (no defiers) and exclusion restriction 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.** *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.

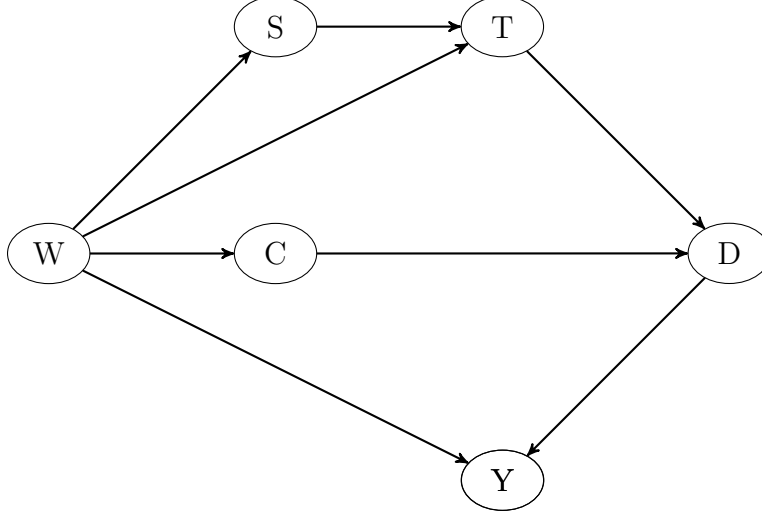


Figure 1: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT.

## 2.2 Population Average Treatment Effect on the Treated

The estimand of interest is

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

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  $\tau_{\text{PATT}}$  by reweighting the RCT responses according to the distribution of covariates in the population.

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

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

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.

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

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

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

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

## 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 random forests to estimate the response curve for compliers, given their treatment assignment and covariates. We estimate the outer expectation by taking empirical means. Thus, the first term in the expression for  $\tau_{\text{PATT}}$  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  $\tau_{\text{PATT}}$  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  $\tau_{\text{PATT}}$ .

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  $\tau_{\text{PATT}}$ . 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

$$S = \mathbb{I}(e_2 + g_1W_1 + g_2W_2 + g_3W_3 + R > 0)$$

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

$$C = \mathbb{I}(e_3 + h_2W_2 + h_3W_3 + Q > 0)$$

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

$$T = \mathbb{I}(e_1 + f_1W_1 + f_2W_2 + V > 0)$$

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

$$Y = a + bD + c_1W_1 + c_2W_2 + dU$$

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 a random forest 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  $\tau_{\text{PATT}}$  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

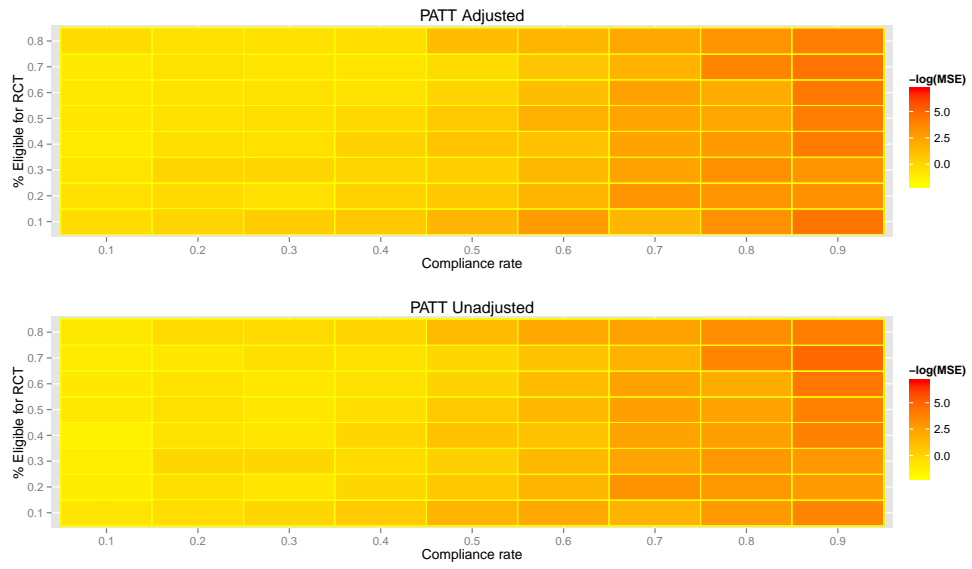


Figure 2: Simulation mean squared error, transformed by  $-\log$ . Darker tiles correspond to lower mean squared error.



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.

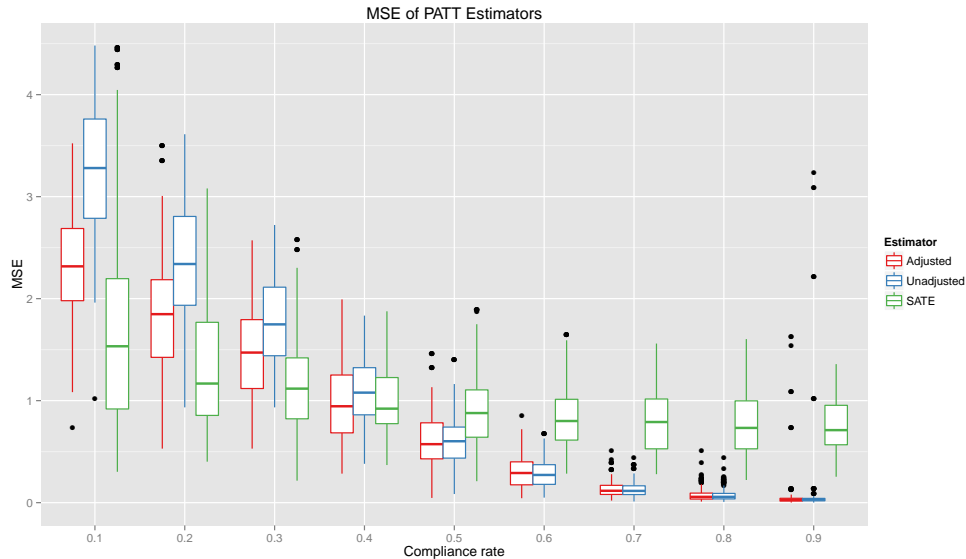


Figure 3: Simulation mean squared error, according to compliance rates in the total population.

## 4 Application: Medicaid and Hospital 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.<sup>1</sup> Treatment occurred at the household level: participants selected by the lottery won the opportunity for themselves and any household member

<sup>1</sup>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.

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 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.<sup>2</sup> The OHIE data includes pretreatment covariates for gender, age, race, ethnicity, health status, education, and household income.

We use two health care use responses: emergency room (ER) and outpatient visits in the past 12 months.<sup>3</sup> These 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 study years 2008–2013. We restrict our sample to respondents with income below 138% of the FPL and who are uninsured or on Medicaid ( $N = 3,914$ ). 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. Table 1 shows the distribution of pre-treatment covariates in the two studies. **TO DO: DESCRIBE WHAT IS**

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<sup>2</sup>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.

<sup>3</sup>The variables that capture the total number of visits have been censored so that no individual value has fewer than ten observations and are truncated at the 2\*99th percentile.

**NOTABLE IN THE TABLE, HOW IT RELATES TO CONFOUNDING.** Future studies on this subject should attempt to control for other potential confounders, such as number of hospital and outpatient visits in the previous year, proximity to health services, and enrollment in other federal programs.

**TO DO: TALK ABOUT THESE: THERE IS NO THEORETICAL REASON OR EVIDENCE AGAINST THE ASSUMPTIONS OF NON-INTERFERENCE BETWEEN EXPERIMENTAL UNITS (ASSUMPTION 4) AND CONDITIONAL INDEPENDENCE OF COMPLIANCE AND TREATMENT ASSIGNMENT (ASSUMPTION 5); THESE ARE STANDARD ASSUMPTIONS.**

|                               | OHIE<br>control<br><i>n</i> = 5476 |          | OHIE<br>treated<br><i>n</i> = 5193 |          | NHIS<br>treated<br><i>n</i> = 3382 |          |
|-------------------------------|------------------------------------|----------|------------------------------------|----------|------------------------------------|----------|
| <b>Covariate</b>              | <b>n</b>                           | <b>%</b> | <b>n</b>                           | <b>%</b> | <b>n</b>                           | <b>%</b> |
| 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 \$0-\$10000            | 5476                               | 100.0    | 3204                               | 61.7     | 1452                               | 42.9     |
| Income \$10001-\$25000        | 0                                  | 0.0      | 1616                               | 31.1     | 1622                               | 48.0     |
| Income > \$25000              | 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     |

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

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.

Assumption 6 does not hold due to the presence of non-compliers in the control group — 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 monotonicity 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 PATT estimates in a future version of this paper.

### 4.3 Empirical results

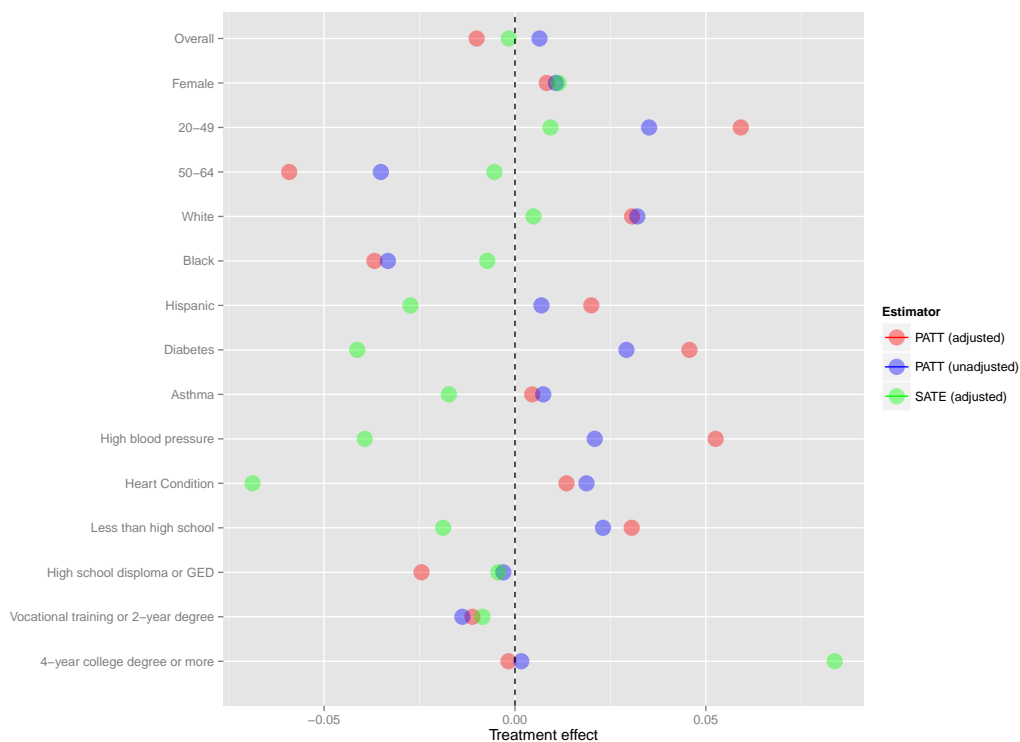
We compare PATT and SATE estimates for ER and outpatient use. We obtain estimates for the overall group of participants and subgroups according to gender, age, race, ethnicity, health status, and education. Subgroup treatment effects are estimated by taking differences across response surfaces for a given covariate group. Response surfaces are estimated with random forests prediction models using the default settings [Breiman, 2001]. We use treatment assignment, number of household members, gender, age, race, ethnicity, health status, education, and household income as features in our response models. Our entire RCT sample has size  $N = 23,205$  and the population has  $N = 38,579$ . There are  $N = 10,297$  RCT compliers and  $N = 3,914$  target population “compliers” on Medicaid.

Figures 4a and 4b compare estimates for a binary response indicating whether the participant visited an ER and the number of ER visits in the past 12 months, respectively. We estimate the effect of Medicaid coverage on the likelihood of visiting the ER is about -1% for population compliers (adjusted PATT). In comparison, the treatment effects on RCT compliers (adjusted SATE) are about zero. There are substantial differences between SATE and PATT estimates across covariate groups. For instance, while there are large negative treatment effects on ER use for sample participants with pre-existing conditions, treatment effects for population participants with pre-existing conditions are large and positive. While the treatment effects on any ER use for college-educated RCT compliers is 9%, treatment effects on college-educated population compliers is about 0.

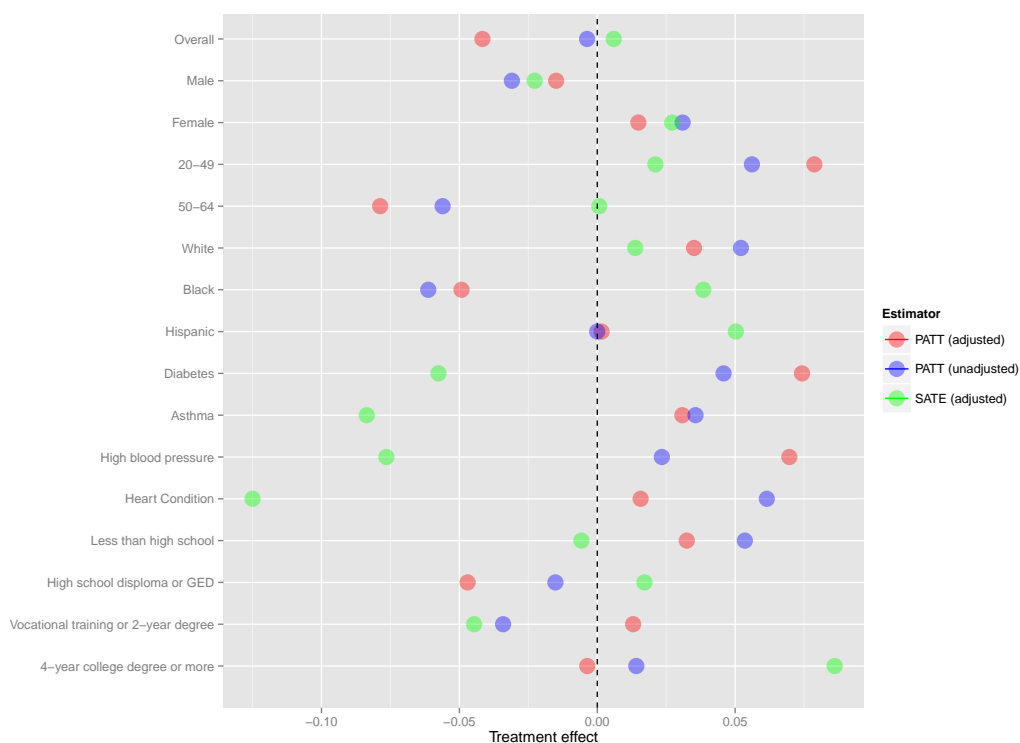
Figures 5a and 5b compare treatment effect estimates on outpatient use. For population compliers overall, the effect of Medicaid coverage on the likelihood of outpatient use is about 2%. For RCT compliers overall, the effect is about 0. Sample and population estimates are generally similar according to pre-existing conditions. Treatment effects on outpatient use are large and negative 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 RCT compliers with observational estimates comparing individuals with and without Medicaid using a sample of NHIS respondents similar to the one used in our study. That authors report OLS estimates of the effect of Medicaid on outpatient use for the population

is slightly lower than the RCT sample; observational estimates of Medicaid on ER visits is slightly higher than for the RCT sample. Our estimates do not demonstrate the relationship between RCT and population estimates. One reason for the failure to replicate is that we are using censored data on the number of visits.

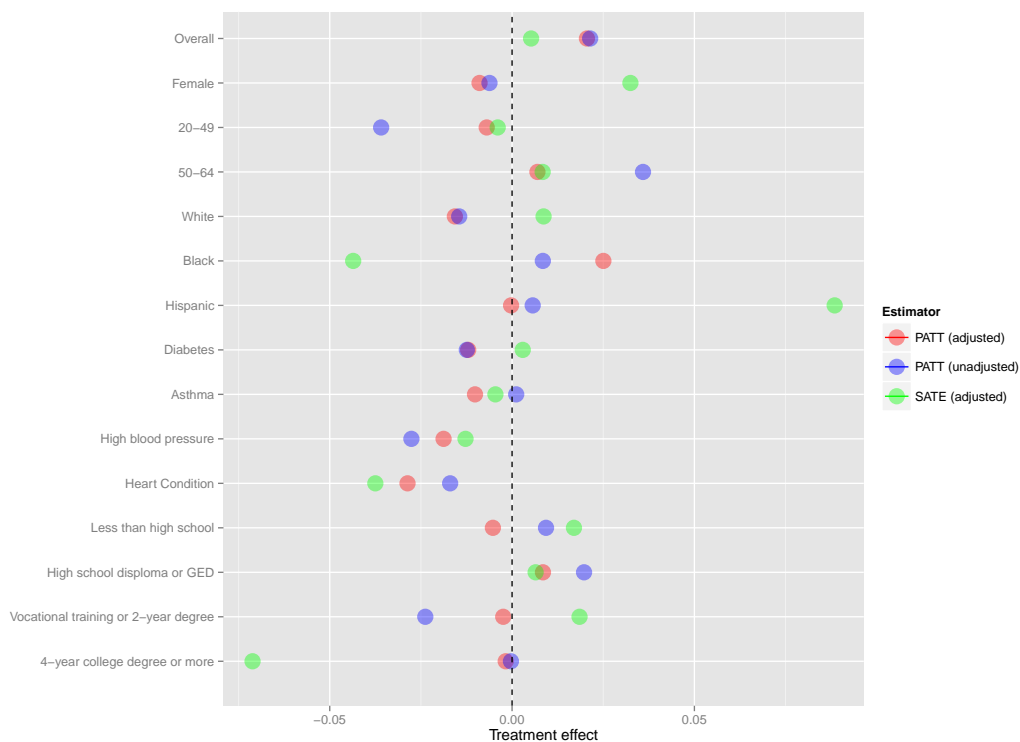


(a) Any ER visit.

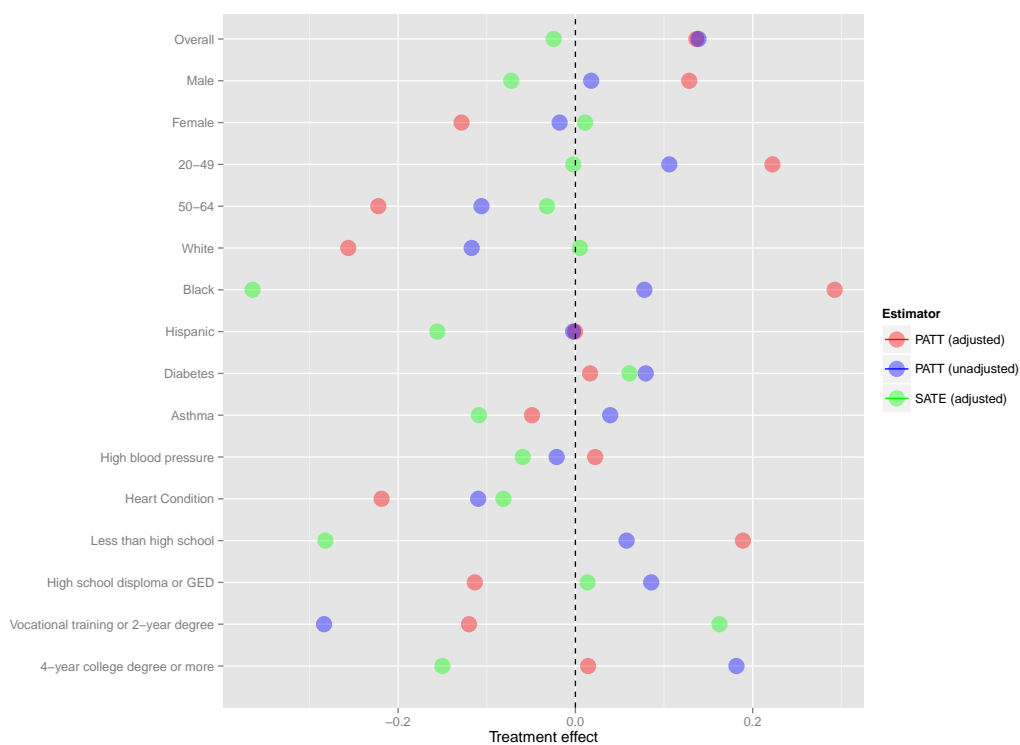


(b) Number of ER visits.

Figure 4: Comparison of estimators for treatment effect on ER use.



(a) Any outpatient visit.



(b) Number of outpatient visits.

Figure 5: Comparison of estimators for treatment effect on outpatient use.

## 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 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 successfully, and successful enrollment depended in part on income levels. The proposed estimator that adjusts for non-compliance seems to perform better than the unadjusted estimator when compliance is low and can be predicted by observed covariates (Figure 3). Our random forest for predicting compliance based on observed covariates has about 61.5% accuracy on the training set of individuals in OHIE assigned to the treatment group. While we don't know well how the model predicts for the control group, its performance on the training set suggests that compliance is not purely random, but depends on observed covariates. This gives evidence in favor of using the proposed estimator. Future work should be done to construct a better model to predict compliance, both in the treated group used for training and the controls in the RCT.

The proposed method allows us to decompose both SATE 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). Since the RCT participants are predominately white and volunteered for the study, we expected to find substantial differences between sample and population estimates in terms of race, and pre-existing condition subgroups. We find large negative treatment effects on ER use for sample participants with pre-existing conditions, yet the treatment effects for population participants with pre-existing conditions are large and positive. Overall, we expected the PATT estimates to be lower than corresponding SATE estimates due to the adverse selection problem in the RCT. We find the PATT estimates are lower than SATE for number of ER visits, but the PATT



estimates are higher than SATE for outpatient usage.

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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 ( <b>SuperLearner</b> )                | default         | 0.226 | 0.001 | 0.222 | 0.231 |
| Generalized boosted models ( <b>gbm</b> )            | default         | 0.226 | 0.001 | 0.222 | 0.231 |
| Lasso regression ( <b>glmnet</b> )                   | $\alpha = 1$    | 0.227 | 0.001 | 0.224 | 0.233 |
| GLM with elasticnet regularization ( <b>glmnet</b> ) | $\alpha = 0.25$ | 0.227 | 0.001 | 0.224 | 0.232 |
| GLM with elasticnet regularization ( <b>glmnet</b> ) | $\alpha = 0.5$  | 0.227 | 0.001 | 0.224 | 0.233 |
| GLM with elasticnet regularization ( <b>glmnet</b> ) | $\alpha = 0.75$ | 0.227 | 0.001 | 0.224 | 0.233 |
| Neural network ( <b>nnet</b> )                       | default         | 0.227 | 0.001 | 0.224 | 0.232 |
| Random forests ( <b>randomForest</b> )               | default         | 0.307 | 0.003 | 0.293 | 0.326 |
| Random forests ( <b>randomForest</b> )               | mtry = 1        | 0.273 | 0.002 | 0.268 | 0.281 |
| Random forests ( <b>randomForest</b> )               | mtry = 5        | 0.307 | 0.003 | 0.294 | 0.326 |
| Random forests ( <b>randomForest</b> )               | mtry = 10       | 0.310 | 0.003 | 0.294 | 0.329 |
| Ridge regression ( <b>glmnet</b> )                   | $\alpha = 0$    | 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.

| <b>Any ER visit</b>  |                 |       |        |
|--|-----------------|-------|--------|
| Algorithm  | Parameters      | Risk  | Weight |
| Generalized boosted models ( <code>gbm</code> )            | default         | 0.188 | 0      |
| Lasso regression ( <code>glmnet</code> )                   | $\alpha = 1$    | 0.188 | 1      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.25$ | 0.188 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.5$  | 0.188 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.75$ | 0.188 | 0      |
| Neural network ( <code>nnet</code> )                       | default         | 0.234 | 0      |
| Random forests ( <code>randomForest</code> )               | default         | 0.24  | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 1        | 0.251 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 5        | 0.24  | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 10       | 0.241 | 0      |
| Ridge regression ( <code>glmnet</code> )                   | $\alpha = 0$    | 0.188 | 0      |
| <b>Any outpatient visit</b>                                |                 |       |        |
| Generalized boosted models ( <code>gbm</code> )            | default         | 0.24  | 0      |
| Lasso regression ( <code>glmnet</code> )                   | $\alpha = 1$    | 0.24  | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.25$ | 0.24  | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.5$  | 0.24  | 0.76   |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.75$ | 0.24  | 0      |
| Neural network ( <code>nnet</code> )                       | default         | 0.242 | 0      |
| Random forests ( <code>randomForest</code> )               | default         | 0.331 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 1        | 0.392 | 0.23   |
| Random forests ( <code>randomForest</code> )               | mtry = 5        | 0.333 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 10       | 0.333 | 0.008  |
| Ridge regression ( <code>glmnet</code> )                   | $\alpha = 0$    | 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.

| <b>Any ER visit</b>  |                 |       |        |
|--|-----------------|-------|--------|
| Algorithm  | Parameters      | Risk  | Weight |
| Generalized boosted models ( <code>gbm</code> )            | default         | 0.188 | 0      |
| Lasso regression ( <code>glmnet</code> )                   | $\alpha = 1$    | 0.188 | 0.54   |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.25$ | 0.188 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.5$  | 0.188 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.75$ | 0.188 | 0      |
| Neural network ( <code>nnet</code> )                       | default         | 0.228 | 0      |
| Random forests ( <code>randomForest</code> )               | default         | 0.24  | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 1        | 0.252 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 5        | 0.241 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 10       | 0.241 | 0      |
| Ridge regression ( <code>glmnet</code> )                   | $\alpha = 0$    | 0.188 | 0.459  |
| <b>Any outpatient visit</b>                                |                 |       |        |
| Generalized boosted models ( <code>gbm</code> )            | default         | 0.239 | 0      |
| Lasso regression ( <code>glmnet</code> )                   | $\alpha = 1$    | 0.239 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.25$ | 0.239 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.5$  | 0.239 | 0      |
| GLM with elasticnet regularization ( <code>glmnet</code> ) | $\alpha = 0.75$ | 0.239 | 0      |
| Neural network ( <code>nnet</code> )                       | default         | 0.24  | 0      |
| Random forests ( <code>randomForest</code> )               | default         | 0.327 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 1        | 0.391 | 0.261  |
| Random forests ( <code>randomForest</code> )               | mtry = 5        | 0.331 | 0      |
| Random forests ( <code>randomForest</code> )               | mtry = 10       | 0.329 | 0.008  |
| Ridge regression ( <code>glmnet</code> )                   | $\alpha = 0$    | 0.239 | 0.73   |