Estimating population average treatment effects from experiments with noncompliance*

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December 12, 2018

Abstract

This paper improves on the transportability of clinical trial results to a population by extending a method of estimating population average treatment effects in settings with noncompliance. We identify the complier-average causal effect for a 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, using data from a large-scale health insurance experiment in which a small subset of those randomly selected to receive Medicaid benefits actually enrolled.

*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]. This work used the Extreme Science and Engineering Discovery Environment (XSEDE) supercomputer Stampede2 at the Texas Advanced Computing Center (TACC) through allocation SES180010.

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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 treatment effect would generalize to the broader population.

This paper extends the literature on extrapolating clinical trial results to populations to deal with noncompliance. Previous approaches to the problem of extrapolating RCT results to a population (Imai, King, and Stuart 2008; Stuart et al. 2011; Hartman et al. 2015) are designed for settings where there is full compliance with treatment. This paper contributes to the literature by defining the assumptions required to identify complier–average causal effects for the target population and proposing a procedure to recover this estimand.

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 complier–average causal effects for the target population from RCT data with noncompliance. Noncompliance occurs when individuals who are assigned to the treatment group do not comply with the treatment; for individuals assigned to control, we are unable to observe who would have complied had they been assigned treatment. A prevalent issue in RCTs, noncompliance dilutes the estimated effect of treatment assignment, and biases the intention–to–treat (ITT) estimate towards zero.

The proposed estimator 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. Note that our estimation strategy differs from reweighting methods that use propensity scores to adjust the RCT data (Stuart et al. 2011). In this context, the propensity score model predicts participation in the RCT, given pretreatment covariates common to both the RCT and population data. Individuals in the RCT and population are then weighted according to the inverse of the estimated propensity score. We use an ensemble of algorithms to predict the response surface for RCT compliers and use the predicted values from the response surface model to estimate the potential outcomes of population members who received treatment, given their covariates.

When estimating the average causal effect from an RCT, researchers typically divide the ITT estimate by the compliance rate under the identifying assumptions outlined in Angrist, Imbens, and Rubin (1996). When extrapolating RCT results to a population, one might simply weight the PATT estimate by the population compliance rate in order to yield a population average effect of treatment on treated compliers.[1] However, the compliance rate is likely to differ between the sample and population, as well as across subgroups. We propose an alternative approach of actually identifying the likely compliers in the control group. By explicitly modeling compliance, our approach allows researchers to decompose population estimates by covariate group and also predict which population members are likely to comply with treatment. Both of these features are useful for policymakers in evaluating the efficacy of policy interventions for subgroups of interest in a population.

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 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; Section 3 describes the estimation procedure; Section 4

[1] A similar approach is used by Imai, Tingley, and Yamamoto 2013 for estimating average complier indirect effects.

reports the estimator's performance in simulations; Section 5 uses the estimator to identify the effect of extending Medicaid coverage to the low–income adult population in the U.S; Section 6 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 of individualswhose 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_{isd} be the potential outcome for individual i in group s and treatment receipt d. Let S_i denote the sample assignment, where $s = 0$ is the population and $s = 1$ is the RCT. T_i indicates treatment assignment and D_i indicates whether treatment was actually received. 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 in the RCT, $D_i = T_i$.

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, which is only observable for individuals in the RCT treatment group.

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. For individuals in the population, we only observe D_i — not T_i. We frame Assumptions 3 and 4 in terms of C_i and T_i in order to distinguish among the population controls who should have received treatment (i.e., individuals with $T_i = 1$ and $D_i = 0$) from noncompliers assigned to control (i.e., individuals with $T_i = 0$ and $D_i = 0$).

Assumptions 1, 3, 4, and 5 are made by Hartman et al. (2015) to identify PATT from an RCT:

Assumption 1. *Consistency under parallel studies:*

$$Y_{i0d} = Y_{i1d}, \qquad \forall\, i\,,\, d = \{0, 1\}.$$

Assumption 1 requires that each individual i has the same response to treatments, whether i is in the RCT or not. Compliance status C_i is not a factor in this assumption because we assume that compliance is conditionally independent of sample and treatment assignment for all individuals with covariates W_i.

Assumption 2. *Conditional independence of compliance and assignment:*

$$C_i \perp\!\!\!\perp S_i,\, T_i \mid W_i, \qquad 0 < \mathbb{P}(C_i = 1 \mid W_i) < 1.$$

Assumption 2 implies that $P(C_i = 1|S_i = 1, T_i = 1, W_i) = P(C_i = 1|S_i = 1, T_i = 0, W_i)$, which is useful when predicting the probability of compliance as a function of covariates W_i in the first step of our estimation procedure. Together, Assumptions 1 and 2 ensure that potential outcomes do not differ based on sample assignment or receipt of treatment.

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

$$(Y_{i01}, Y_{i11}) \perp\!\!\!\perp S_i \mid (W_i, T_i = 1, C_i = 1), \qquad 0 < \mathbb{P}(S_i = 1 \mid W_i, T_i = 1, C_i = 1) < 1.$$

Assumption 3 ensures the potential outcomes for treatment are independent of sample assignment for individuals with the same covariates W_i and assignment to treatment.[2] We make a similar assumption for the potential outcomes under control:

Assumption 4. *Strong ignorability of sample assignment for controls:*

$$(Y_{i00}, Y_{i10}) \perp\!\!\!\perp S_i \mid (W_i, T_i = 1, C_i = 1), \qquad 0 < \mathbb{P}(S_i = 1 \mid W_i, T_i = 1, C_i = 1) < 1.$$

RCT study designs that apply restrictive exclusion criteria may increase the likelihood that there are unobserved differences between the RCT and target population, which would violate the strong ignorability assumptions.[3]

Interference undermines the framework because it creates more than two potential outcomes per participant, depending on the treatment receipt of other participants (Rubin 1990). We therefore assume no interference between units:

Assumption 5. *The potential outcomes Y_{isd} do not depend on D_j, $\forall j \neq i$.*

Figure 1 shows Assumptions 3, 4, and 2 in a directed acyclic graph. Treatment assignment T_i may only depend on C_i through W_i, and the potential outcomes (Y_{is0}, Y_{is1}) may only depend on S_i through W_i. From the internal validity standpoint, the role of W_i is critical: if any relevant observed covariates are not controlled, then there is a backdoor pathway from T_i back to W_i and into Y_{isd}. We use the same W_i across all identifying assumptions, which implicitly assumes that the observable covariates that determine sample selection

[2] Throughout, we assume individuals are sampled randomly from an infinite population.

[3] Note that Assumptions 3 and 3 also imply strong ignorability of sample assignment for treated and control noncompliers since we assume in that compliance is also independent of sample and treatment assignment, conditional on W_i (Assumption 2). However, we are interested only on modeling the response surfaces for compliers.

also determine population treatment assignment and complier status. This choice reflects a modeling assumption of our estimation procedure described in Section 3.

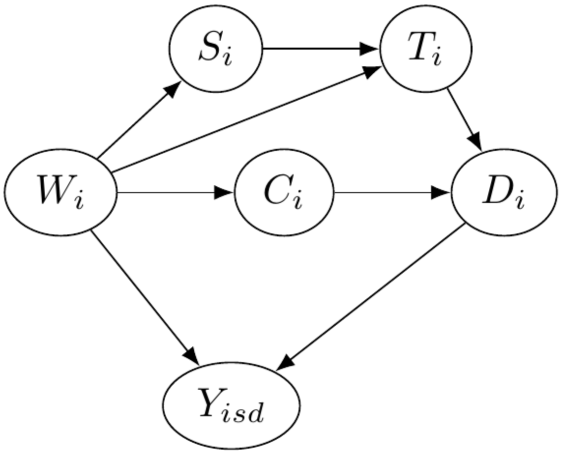

Figure 1: Causal diagram indicating the conditional independence assumptions needed to estimate the PATT-C.

We additionally include Assumptions 6 and 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:

Assumption 6. *No defiers:*

$$T_i \geq D_i, \qquad \forall\, i\,,\, t = \{0, 1\}.$$

Assumption 7 ensures treatment assignment affects the response only through the treatment received. In particular, the treatment effect may only be nonzero for compliers.

Assumption 7. *Exclusion restriction: For noncompliers,*

$$Y_{i11} = Y_{i10}, \qquad \forall\, i.$$

2.2 PATT-C

The estimand of interest is the Population Average Treatment Effect on Treated Compliers (PATT-C):

$$\tau_{\text{PATT-C}} = \mathbb{E}\left(Y_{i01} - Y_{i00} \mid S_i = 0, D_i = 1\right). \tag{1}$$

PATT-C is interpreted as the complier-average causal effect estimated on the RCT sample extrapolated to what we would have observed in the population if treatment received D_i the same. 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.

Theorem 1. *Under Assumptions 1 – 7,*

$$\tau_{PATT\text{-}C} = \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i11} \mid S_i = 1, D_i = 1, W_i\right)\right] - \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i10} \mid S_i = 1, T_i = 0, C_i = 1, W_i\right)\right] \tag{2}$$

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

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

$$\begin{aligned}
\mathbb{E}\left(Y_{i01} \mid S_i = 0, D_i = 1\right) &= \mathbb{E}\left(Y_{i11} \mid S_i = 0, D_i = 1\right) && \text{by Assumption 1}\\
&= \mathbb{E}\left(Y_{i11} \mid S_i = 0, T_i = 1, C_i = 1\right) && \text{by Assumption 6}\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i11} \mid S_i = 0, T_i = 1, C_i = 1, W_i\right)\right] &&\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i11} \mid S_i = 1, T_i = 1, C_i = 1, W_i\right)\right] && \text{by Assumption 3}\\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i11} \mid S_i = 1, D_i = 1, W_i\right)\right] &&
\end{aligned}$$

Intuitively, conditioning on W_i makes sample selection ignorable under Assumption 3. This is the critical connector between the third and fourth lines of the first expectation derivation.

$$\begin{aligned}
\mathbb{E}\left(Y_{i00} \mid S_i = 0, D_i = 1\right) &= \mathbb{E}\left(Y_{i10} \mid S_i = 0, D_i = 1\right) && \text{by Assumption 1} \\
&= \mathbb{E}\left(Y_{i10} \mid S_i = 0, T_i = 1, C_i = 1\right) && \text{by Assumption 6} \\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i10} \mid S_i = 1, T_i = 1, C_i = 1, W_i\right)\right] && \text{by Assumption 4} \\
&= \mathbb{E}_{01}\left[\mathbb{E}\left(Y_{i10} \mid S_i = 1, T_i = 0, C_i = 1, W_i\right)\right] && \text{by Assumption 2}
\end{aligned}$$

The last line follows because Assumption 2 allows us to use RCT controls who would have complied had they been assigned to treatment. Finally, the result follows by plugging these two expressions into Eq. (1). □

3 Estimation procedure

There are two challenges in turning Theorem 1 into an estimator of $\tau_{\text{PATT-C}}$ 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 $\tau_{\text{PATT-C}}$ 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_i is unobservable, as they always receive no treatment ($D_i = 0$). 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 is to simply weight the PATT estimate by the population compliance rate in order to yield a population average effect of treatment on treated compliers. However, the compliance rate is likely to differ between the sample and population, as well as across subgroups. Explicitly modeling compliance allows us to decompose PATT-C estimates by subgroup according to covariates common to both RCT and observational datasets.

The procedure for estimating $\tau_{\text{PATT-C}}$ using Theorem 1 is as follows:

S.1 Using the group assigned to treatment in the RCT ($S_i = 1, T_i = 1$), train a model (or an ensemble of models) to predict the probability of compliance as a function of covariates W_i.

S.2 Using the model from S.1, predict who in the RCT assigned to control *would have* complied to treatment had they been assigned to the treatment group. We use a standard prediction threshold of 50% in order classify compliers, $C_i = 1$.[4]

S.3 For the observed compliers assigned to treatment and predicted compliers assigned to control, train a model to predict the response using W_i and D_i, which gives $\mathbb{E}(Y_{i1d} \mid S_i = 1, D_i = d, W_i)$ for $d \in \{0, 1\}$.

S.4 For all individuals who received treatment in the population ($S_i = 0, D_i = 1$), estimate their potential outcomes Y_{i10} and Y_{i11} using the model from S.3. The mean counterfactual Y_{i11} minus the mean counterfactual Y_{i10} is the estimate of $\tau_{\text{PATT-C}}$.

Assumptions 3 and 4 are particularly important for estimating $\tau_{\text{PATT-C}}$: 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 does not hold, then the potential

[4] Adjusting the prediction threshold upward would result in more accurate classifications, although we do not explore this approach.

outcomes Y_{i10} and Y_{i11} for target population individuals cannot be estimated using the model from S.3.[5]

3.1 Modeling assumptions

In addition to the identification assumptions, we require additional modeling assumptions for the estimation procedure. We assume that the W_i that determine sample selection also determine population treatment assignment and complier status. As pointed out in Section 2.1, we also require that W_i is complete because if any relevant elements of W_i are not controlled, then there is a backdoor pathway from T_i back to W_i and into Y_{isd}. Lastly, we assume that the compliance model is accurate in predicting compliance in the training sample of RCT participants assigned to treatment and also generalizable to RCT participants assigned to control (S.1 and S.2). Section 3.2 below describes our method of evaluating the generalizability of the compliance model.

3.2 Ensemble method

In the empirical application, we use the weighted ensemble method described in Laan, Polley, and Hubbard (2007) for S.1 and S.3 of the estimation procedure. This ensemble method combines algorithms with a convex combination of weights based on minimizing cross-validated error. It is shown to control for overfitting and outperforms single algorithms selected by cross-validation (Polley and Van Der Laan 2010).

We choose a variety of candidate algorithms to construct the ensemble, with a preference towards algorithms that tend to outperform in supervised classification tasks. We also have a preference for algorithms that have a built-in variable selection property. The idea is that we input the same W_i and each candidate algorithm selects the most important covariates for predicting compliance status or potential outcomes. We select three types of candidate algorithms: nonparametric additive regression models (Buja, Hastie, and Tibshirani 1989);

[5] Section 5.3 discusses whether the strong ignorability assumptions are plausible in the empirical application.

L1 or L2-regularized linear models (e.g., Lasso or ridge regression, respectively) (Tibshirani et al. 2012); and random forests (Breiman 2001). L1-regularized linear models are important for our application due to their variable selection properties — Lasso is particularly attractive because it tends to shrink all but one of the coefficients of correlated covariates to zero.

4 Simulations

We conduct a simulation study to compare the performance of the PATT and PATT-C estimators. For comparison, we compare the population estimates to the SATE, which is the ITT effect estimated from the RCT sample adjusted by the sample compliance rate.

Our simulation is designed so that the effect of treatment is heterogeneous and depends on covariates which are different in the RCT and target population. The design satisfies the conditional independence assumptions in Figure 1.

4.1 Simulation design

In the simulation, RCT eligibility, complier status, and treatment assignment in the population depend on multivariate normal covariates $(W_i^1, W_i^2, W_i^3, W_i^4)$ with mean $(0.5, 1, -1, -1)$ and covariances $\text{Cov}(W_i^1, W_i^2) = \text{Cov}(W_i^1, W_i^4) = \text{Cov}(W_i^2, W_i^4) = \text{Cov}(W_i^3, W_i^4) = 1$ and $\text{Cov}(W_i^1, W_i^3) = \text{Cov}(W_i^2, W_i^3) = 0.5$. The first three covariates are observed by the researcher and W_i^4 is unobserved.

The equation for selection into the RCT is

$$S_i = \mathbb{I}(e_2 + g_1 W_i^1 + g_2 W_i^2 + g_3 W_i^3 + e_4 W_i^4 + R > 0),$$

where R is standard normal. The parameter e_2 varies the fraction of the population eligible for the RCT and e_4 varies the degree of confounding with sample selection. We set the constants g_1, g_2, and g_3 to be 0.5, 0.25, and 0.75, respectively.

Complier status is determined by

$$C_i = \mathbb{I}(e_3 + h_2 W_i^2 + h_3 W_i^3 + e_5 W_i^4 + Q > 0),$$

where Q is standard normal, e_3 varies the fraction of compliers in the population, and e_5 varies the degree of confounding with treatment assignment. We set the constants h_2 and h_3 to 0.5.

For individuals in the population ($S_i = 0$), treatment is assigned by

$$T_i = \mathbb{I}(e_1 + f_1 W_i^1 + f_2 W_i^2 + e_6 W_i^4 + V > 0),$$

where V is standard normal. Varying e_1 changes the fraction eligible for treatment in the population and e_6 varies the degree of confounding with sample selection. We set the constants f_1 and f_2 to 0.25 and 0.75, respectively. For individuals in the RCT ($S_1 = 1$), treatment assignment is a sample from a Bernoulli distribution with probability $p = 0.5$. We set treatment received D_i according to T_i and C_i: $D_i = T_i$ if $C_i = 1$ and $D_i = 0$ if $C_i = 0$.

Finally, the response is determined by

$$Y_{isd} = a + bD + c_1 W_i^1 + c_2 W_i^2 + dU.$$

We assume that the treatment effect b is heterogeneous depending on W_i^1: $b = 1$ if $W_i^1 > 0.75$ and $b = -1$ if $W_i^1 \leq 0.75$. We set a, c_1, and d to 1 and c_2 to 2. U is standard normal and $U, V, R, Q, (W_i^1, W_i^2, W_i^3, W_i^4)$ 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 ($S_i = 1$) are selected. Similarly, we sample 5,000 individuals from the population and select those who are not eligible for the RCT ($S_i = 0$): 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_i according to their treatment assignment and complier status and observe their responses Y_{isd}. In this design, the way that we've set S_i, T_i, D_i, C_i, and Y_{isd} ensures that Assumptions 1 – 7 hold.

In the assigned-treatment RCT group ($S_i = 1, T_i = 1$), we train a gradient boosting algorithm (Friedman 2001) on the covariates to predict who in the control group ($S_i = 1, T_i = 0$) would comply with treatment ($C_i = 1$), which 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 gradient boosting with features (W_i^1, W_i^2, W_i^3) and D_i. The PATT-C is estimated according to the estimation procedure outlined above.

4.2 Simulation results

We vary each of the parameters e_1, e_2, e_3, e_4, e_5, and e_6 along a grid of five random standard normal values in order to generate different combinations of rates of compliance, treatment eligibility, RCT eligibility in the population, and confounding. For each possible combination of the six parameters, we run the simulation ten times and compute the average root mean squared error (RMSE) of PATT-C, PATT, and the SATE. All other parameters are held constant. The PATT and PATT-C estimates are obtained by estimating the response surface on all individuals in the RCT and applying S.4 of our estimation procedure to the nonrandomized trial individuals.

Figure 2 shows the relationship between the percent of compliers in the whole population, the percentage of people in the population eligible to participate in the RCT, and the RMSE of the PATT and PATT-C estimators. The PATT estimator performs badly when the compliance rate is low, whereas the PATT-C estimator is comparatively insensitive to changes in the compliance rate. A similar pattern emerges when the compliance rate varies with the population treatment rate (Figure A1).

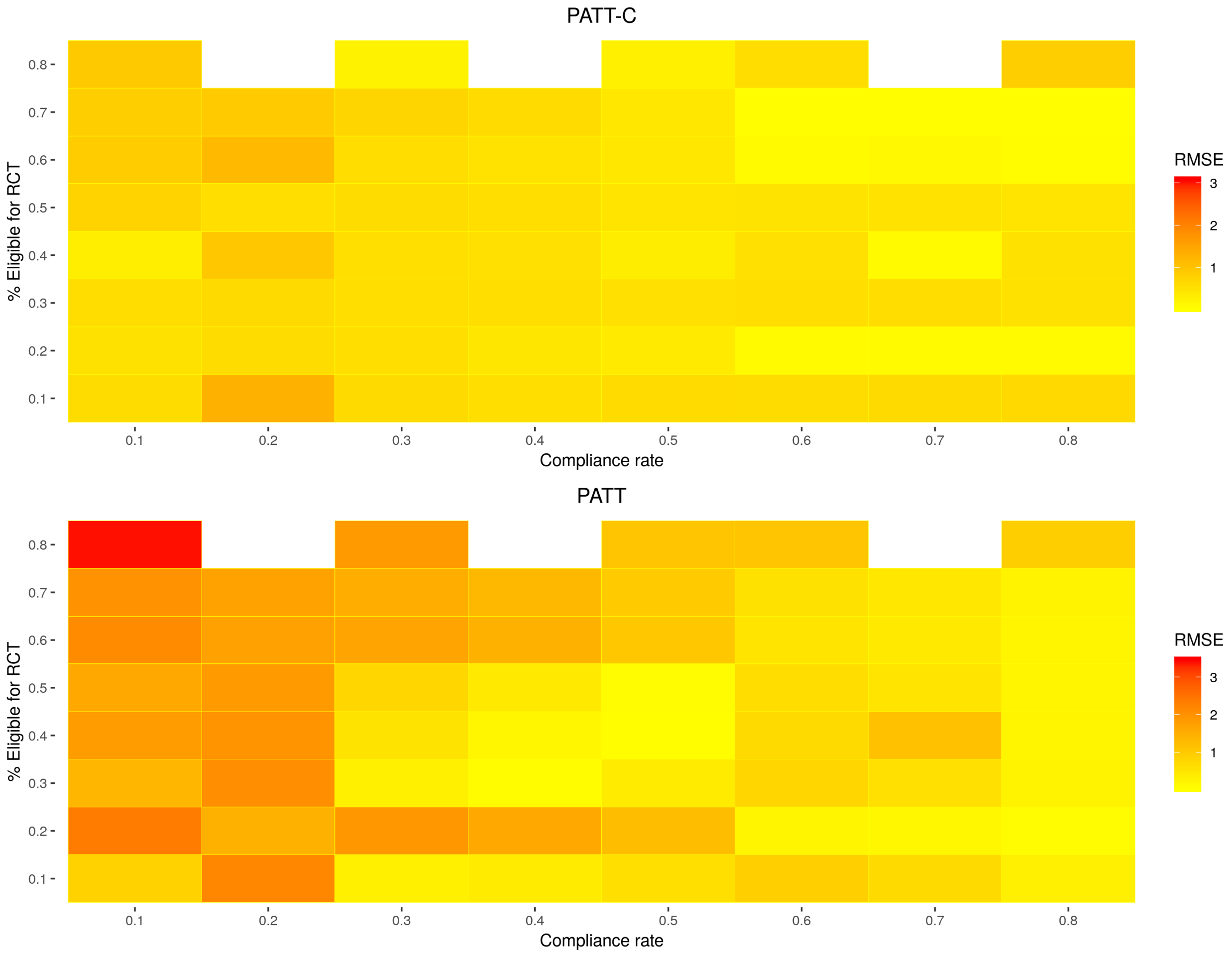

Figure 2: Simulated RMSE, binned by compliance rate and percent eligible for the RCT. Darker tiles correspond to higher errors and white tiles correspond to missing simulated data.

Figure 3 compares the RMSE of PATT and PATT-C with the SATE at varying levels of compliance in the total population. PATT-C is relatively invariant to changes in the compliance rate and outperforms both PATT and SATE in terms of minimizing RMSE when the compliance rate is below 70% and . For high levels of compliance, the SATE actually tends to estimate the average causal effects for the target population as closely than as PATT or PATT-C.

Figures A2, A3, and A4 explore how the degrees of confounding in the mechanisms that determine sample selection, treatment assignment, and compliance affect estimation error. PATT-C tends to be invariant to increases in the degree of confounding, whereas PATT is sensitive to confounding in the sample selection mechanism. The SATE estimates are generally more variable than the population estimates due to the sample estimator's inability to account for differences in pretreatment covariates between the RCT sample and target population.

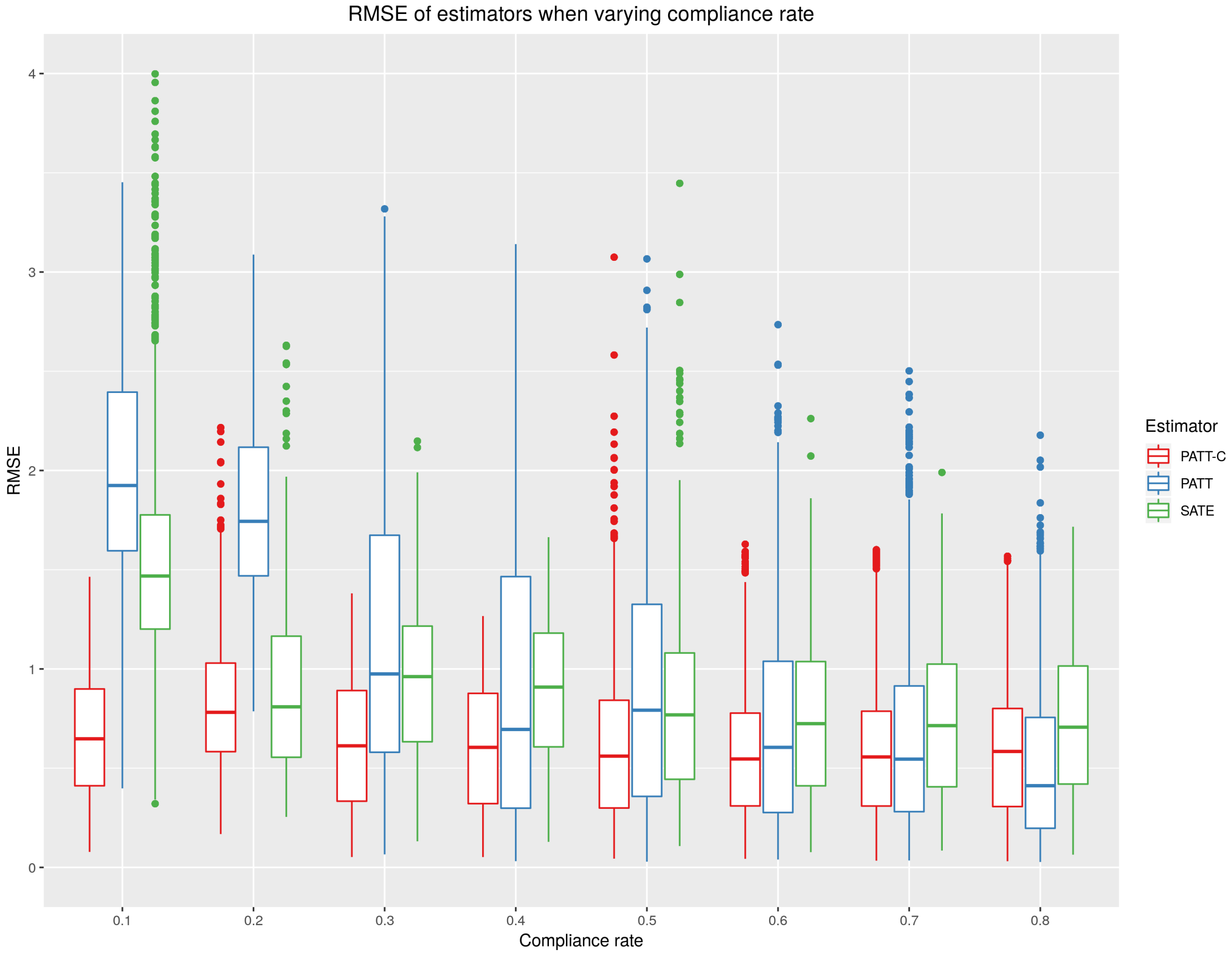

Figure 3: Simulated RMSE of PATT-C, PATT, and SATE, according to compliance rates in the total population.

5 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.

5.1 RCT sample

We draw RCT data from the Oregon Health Insurance Experiment (OHIE) (Finkelstein et al. 2012; Katherine Baicker et al. 2013; 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.[6] 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.[7]

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

[6] 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.

[7] 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.

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.

Our outcomes of interest are binary variables for any emergency room (ER) and outpatient visits in the past 12 months. ER use is an important outcome because it is the main delivery system through which the the uninsured receive health care. The uninsured could potentially receive higher quality and less affordable healthcare through outpatient visits. An important question for policymakers is whether Medicaid expansions will decrease ER utilization by the previously uninsured.

Subsequent research calls in to question the external validity of the OHIE, which resulted in the counterintuitive finding that Medicaid increased ER use among RCT participants (Finkelstein et al. 2012; Taubman et al. 2014). For example, quasi-experimental studies on the impact of the 2006 Massachusetts health reform — which served as a model for the ACA — show that ER use decreased or remained constant following the reform (Miller 2012; Kolstad and Kowalski 2012). A challenge to the external validity of the OHIE is that it's exclusion criteria was likely more restrictive than government health insurance expansions.

5.2 Observational data

We acquire data on the target population from the National Health Interview Study (NHIS) for years 2008 to 2017 (National Center for Health Statistics).[8] We restrict our sample to respondents with income below 138% of the FPL and who are uninsured or on Medicaid and 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.

[8] A possible limitation of this application is that it ignores the complex sampling techniques of the NHIS sample design such as differential sampling, which is discussed in detail in Parsons et al. (2014).

5.3 Verifying assumptions

In order for $\tau_{\text{PATT-C}}$ 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. Medicaid coverage for uninsured individuals was applied in the same manner in the RCT as it is in the population. Differences in potential outcomes due to sample selection might arise, however, 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 3 and 4, which state that potential outcomes for treatment and control are independent of sample assignment for individuals with the same covariates 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.

The final two columsn of Table 1 compares RCT participants selected for Medicaid with population members on Medicaid. Compared to the RCT compliers, the target population "compliers" are 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 by health insurance status.

	OHIE		OHIE		NHIS	
	no insurance		insurance		insurance	
	$n = 4,519$		$n = 6,100$		$n = 6,261$	
Covariate	**n**	%	**n**	%	**n**	%
Sex:						
Female	2,538	56.2	3506	57.5	4,288	68.5
Age:						
19-49	1,288	28.5	1,625	26.6	4324	69.1
50-64	3,231	71.5	4,475	73.4	1,937	30.9
Race:						
White	3,956	87.5	5,183	85.0	3,902	62.3
Black	193	4.3	247	4.0	1,723	27.5
Hispanic	264	5.8	538	8.8	1,570	25.1
Health status:						
Diabetes	459	10.2	637	10.4	866	13.8
Asthma	823	18.2	1,094	17.9	1272	20.3
High blood pressure	1,362	30.1	1,705	27.9	2,166	34.6
Heart condition	120	2.7	189	3.1	529	8.4
Education:						
Less than high school	858	19.0	1,154	18.9	1,942	31.0
High school diploma or GED	2,589	57.3	3,279	53.8	2,076	33.2
Voc. training / 2-year degree	804	17.8	1,186	19.4	1,810	28.9
4-year college degree or more	268	5.9	481	7.9	433	6.9
Income:						
< \$10k	4,518	100.0	4,111	67.4	2,588	41.3
\$10k-\$25k	1	0.0	1,616	26.5	3,098	49.5
> \$25k	0	0.0	373	6.1	575	9.2
Binary response	**n**	%	**n**	%	**n**	%
Any ER visit	1,377	25.4	1,301	25.1	1,659	26.5
Continuous response	$\bar{\mathbf{x}}$	**sd**	$\bar{\mathbf{x}}$	**sd**	$\bar{\mathbf{x}}$	**sd**
# ER visits	0.44	0.95	0.44	0.99	0.48	1.0
# outpatient visits	1.9	3.01	1.9	2.8	2.08	2.3

Strong ignorability assumptions may also be violated due to the fact that the OHIE applied a more stringent exclusion criteria compared to the NHIS sample. While the RCT and population sample both screened for individuals below the FPL, only the RCT required those enrolled to recertify their eligibility status every six months.

A violation of the assumption of no-interference (Assumption 5) biases the estimate of $\tau_{\text{PATT-C}}$ 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 2 is violated if assignment to treatment influences the compliance status of individuals with the same covariates. Our compliance ensemble can accurately classify compliance status for 77% of treated RCT participants with only the covariates — and not treatment assignment — as model inputs.[9] This gives evidence in favor of the conditional independence assumption.

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.

5.3.1 Sensitivity to no defiers assumption

Angrist, Imbens, and Rubin (1996) show that the bias due to violations of Assumption 6 is equivalent to the difference of average causal effects of treatment received for compliers and defiers, multiplied by the relative proportion of defiers, $\mathbb{P}(i \text{ is a defier})/(\mathbb{P}(i \text{ is a complier}]) - \mathbb{P}(i \text{ is a defier}))$.

Table A1 reports the distribution of participants in the OHIE by status of treatment assignment and treatment received. Assumption 6 does not hold due to the presence of

[9] The compliance ensemble is evaluated in terms of 10–fold cross–validated MSE. The distribution of MSE for the ensemble and its candidate algorithms are provided in Table A2.

defiers; i.e., participants who were assigned to control and enrolled in Medicaid during the study period. About 6.7% of the RCT sample were assigned to control but were enrolled in Medicaid ($T_i < D_i$) and 65.5% of the sample complied with treatment assignment ($D_i = T_i$), which results in a bias multiplier of 0.11. Suppose that the difference of average causal effects of Medicaid received on ER use for compliers and defiers is 1.2%. The resulting bias is only 0.1%, which would not meaningfully alter the interpretation of the SATE or PATT-C reported in Section 5.4.

5.4 Empirical results

We compare PATT-C and PATT 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 are estimated with the ensemble mean predictions. 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.

Finkelstein et al. (2012), who report population estimates of the effect of Medicaid coverage on *the number of* ER and out-patient visits using 2004–2009 NHIS data on adults aged 19–64 below 100 percent of the federal poverty line ($n = 15,528$). Finkelstein et al. (2012) estimates Medicaid coverage significantly increases the number of ER visits by 0.08 [0.05, 0.12] and increases the number of outpatient visits by 1.45 [1.33, 1.57]. Table 2 presents our PATT-C estimates, which indicate that Medicaid coverage has a positive, but considerably smaller effect on the number of ER and outpatient visits.

Figures A5, A6, and A7 examine heterogeneous treatment effect estimates on ER and outpatient use in the sample and population. While our study is the first to our knowledge to estimate heterogeniety in treatment effects for the target population, Taubman et al. (2014) and Kowalski (2016) perform subgroup analyses on the RCT sample. Similar to our PATT-C estimates, Taubman et al.'s [2014] subgroup analyses indicate that increases in ER use due

Table 2: Comparison of population and sample estimates.

Estimator \ Outcome	Any ER visit	# ER visits	# outpatient visits
PATT-C	0.01 [0.01, 0.02]	0.0005 [0.0001, 0.0008]	0.002 [0.002, 0.002]
PATT	0.01 [0.01, 0.02]	-0.004 [-0.005, -0.004]	0.02 [0.02, 0.02]
SATE	-0.001 [-0.02, 0.02]	0.005 [-0.04, 0.06]	-0.02 [-0.19, 0.15]

Notes: SATE is the ITT effect adjusted by the sample compliance rate in the RCT. Estimates in brackets represent 95% bootstrap confidence intervals constructed with 1,000 bootstrap samples.

to Medicaid are significantly larger for younger individuals and those with high school-level education.

6 Discussion

Our proposed estimator PATT-C can be interpreted as the complier-average causal effect estimated on the RCT sample extrapolated to what we would have seen in the population if treatment received were the same. The simulations presented in Section 4 show that the PATT-C estimator outperforms its unadjusted counterpart when the compliance rate is low. PATT gives the ITT effect extrapolated those who take treatment in the population, 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 S.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. Further research might also explore models to more accurately predict compliance in RCTs. Accurately predicting compliance is not only essential for yielding unbiased estimates of the average causal effects for target populations, it is also useful for researchers and policymakers to know which groups of individuals are unlikely to comply with treatment.

In the OHIE trial, only about 30% of those selected to receive Medicaid benefits actually enrolled. Our compliance ensemble accurately classified compliance status for 77% of treated RCT participants using only the pretreatment covariates as features. While we don't know how well the compliance ensemble predicts for the control group, the control group should be similar to the treatment group on pretreatment covariates because of the RCT randomization. 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.

In our empirical application, 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 reweighting or response surface methods necessary to extend the RCT results to the population.

Explicitly modeling compliance allows us to decompose population 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.

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Appendix

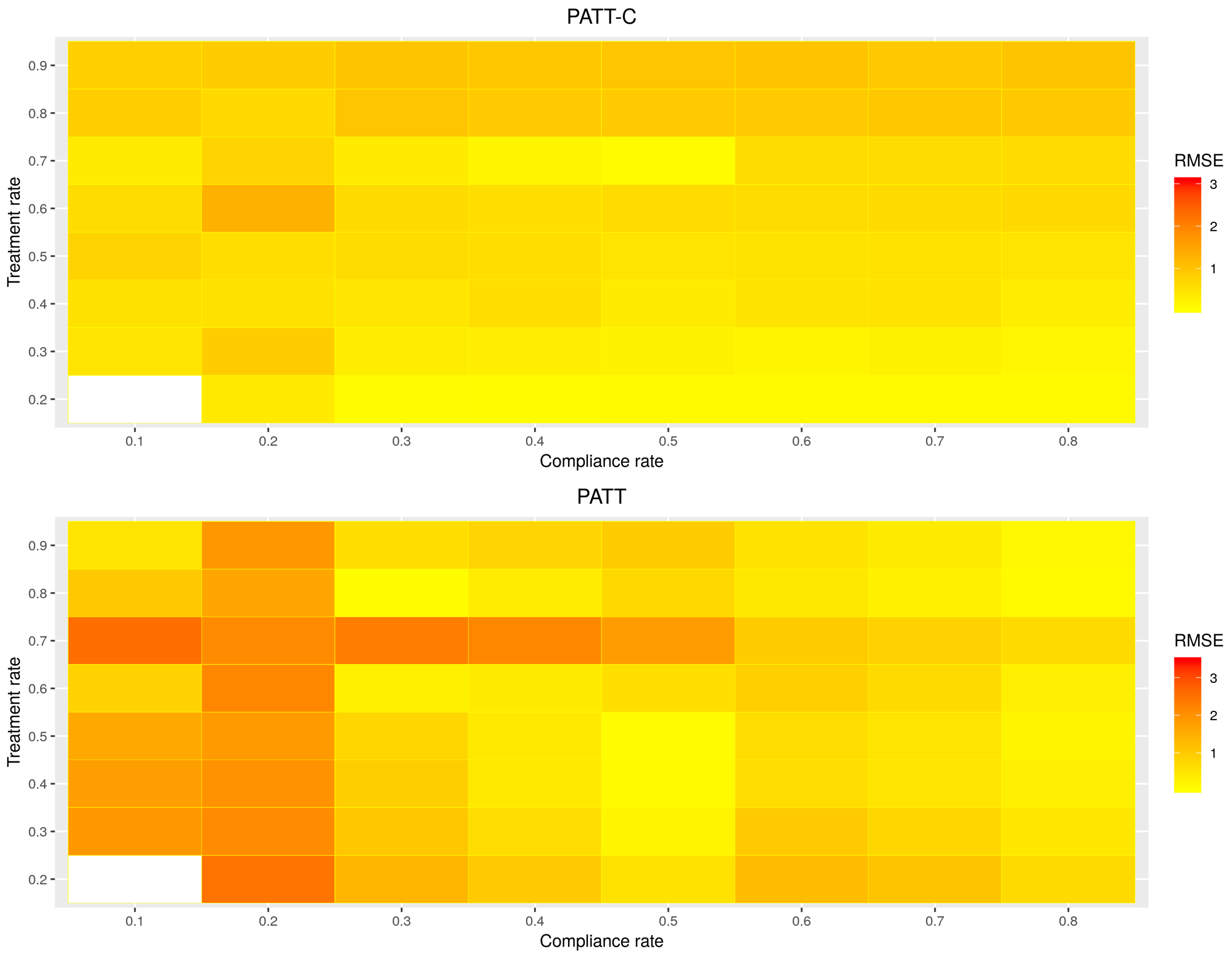

Figure A1: Simulated RMSE, binned by compliance rate and treatment rate.

Table A1: Distribution of OHIE participants by status of treatment assignment (T_i) and treatment received (D_i).

	$D_i = 0$	$D_i = 1$	n
$T_i = 0$	10,010	1,556	11,566
$T_i = 1$	6,446	5,193	11,639
n	16,456	6,749	23,205

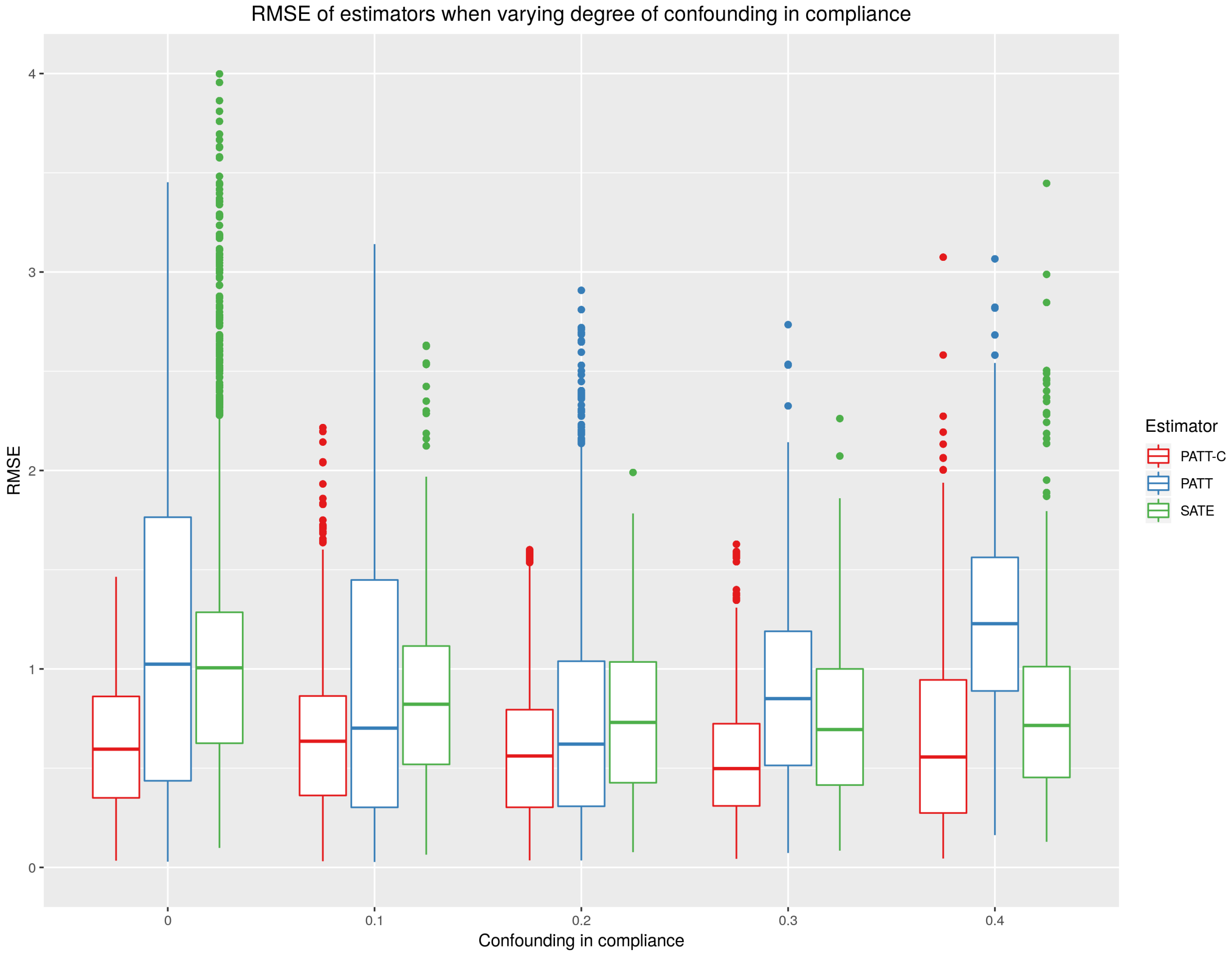

Figure A2: Simulated RMSE of PATT-C, PATT, and SATE, according to degree of confounding in compliance.

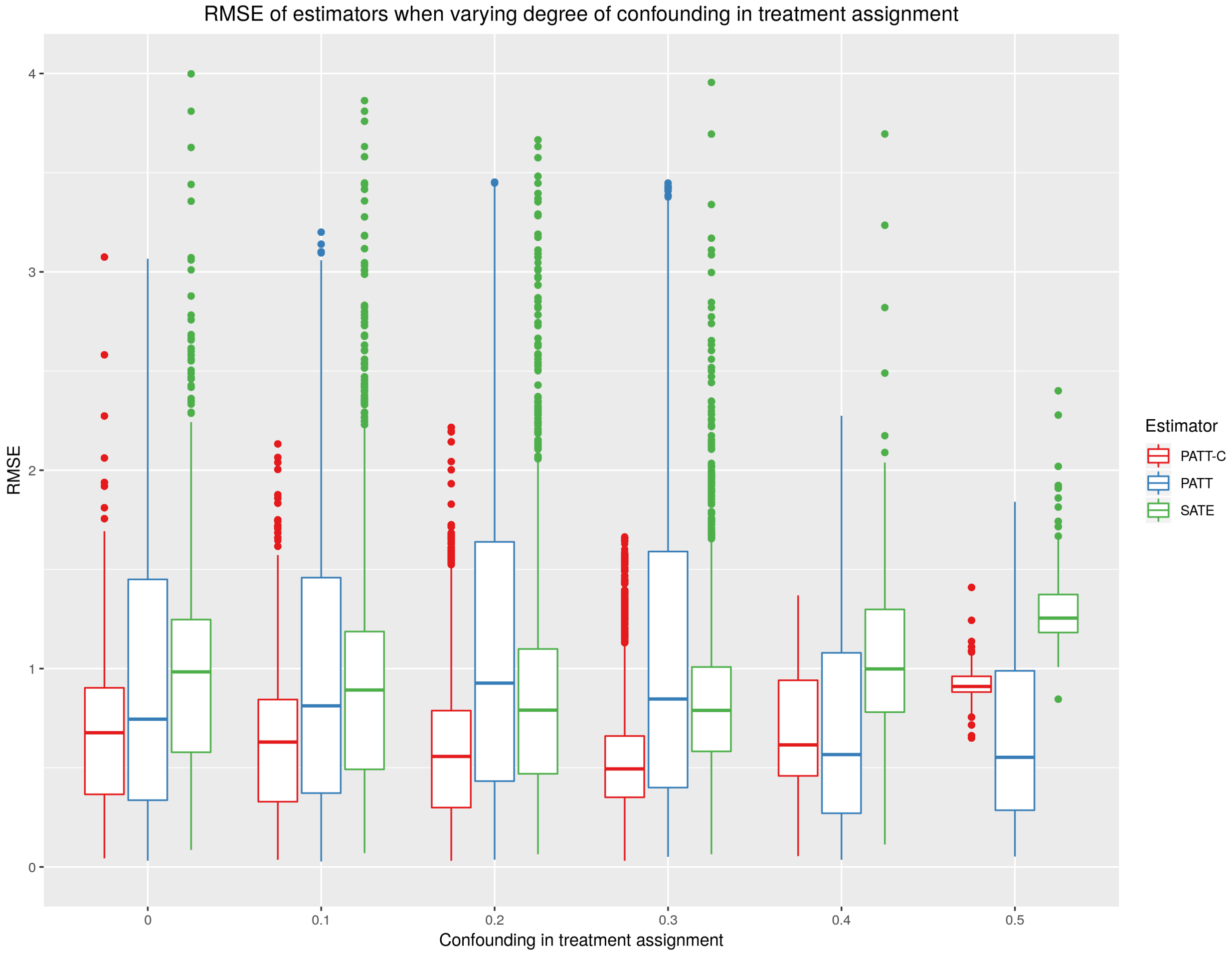

Figure A3: Simulated RMSE of PATT-C, PATT, and SATE, according to degree of confounding in treatment assignment.

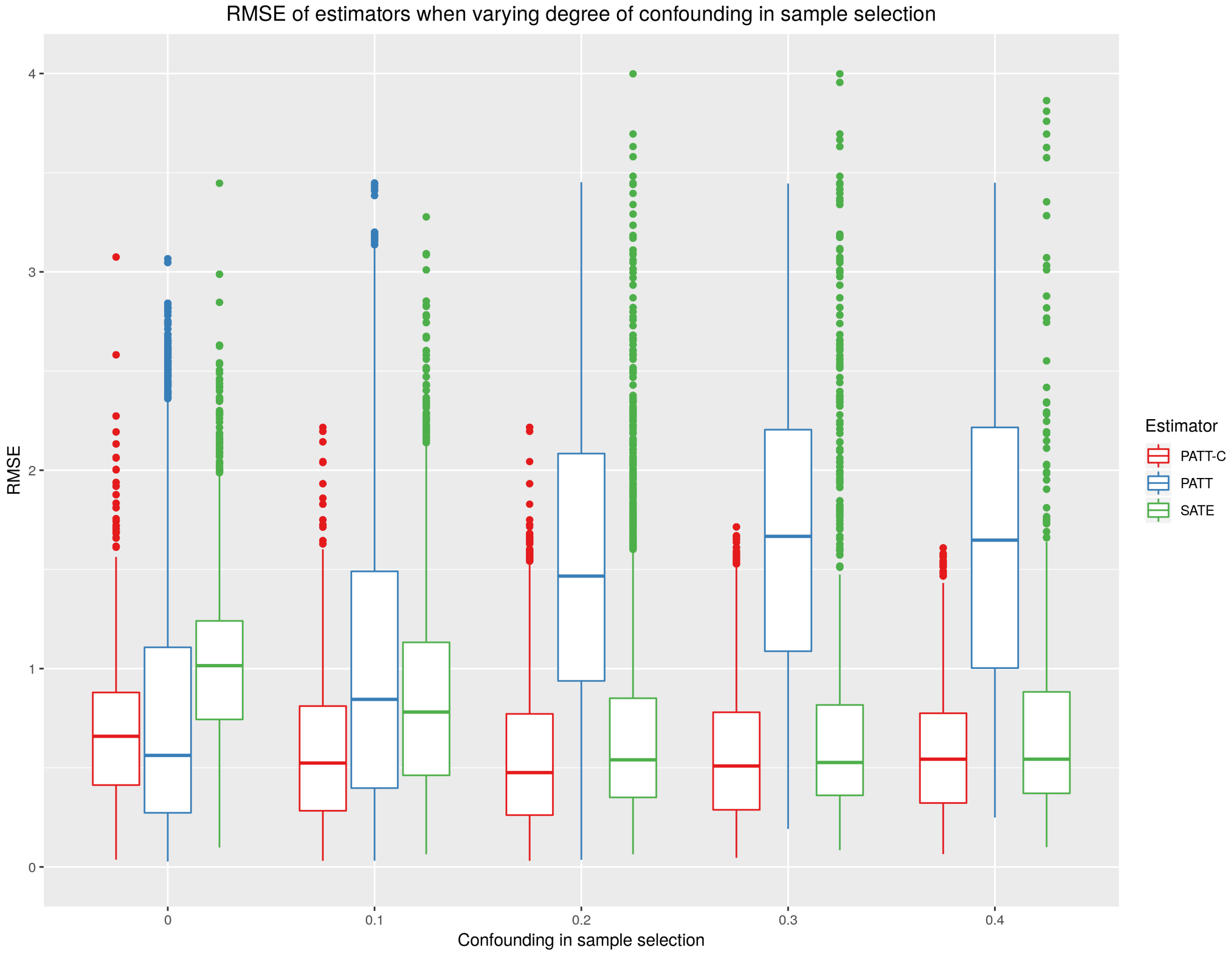

Figure A4: Simulated RMSE of PATT-C, PATT, and SATE, according to degree of confounding in sample selection.

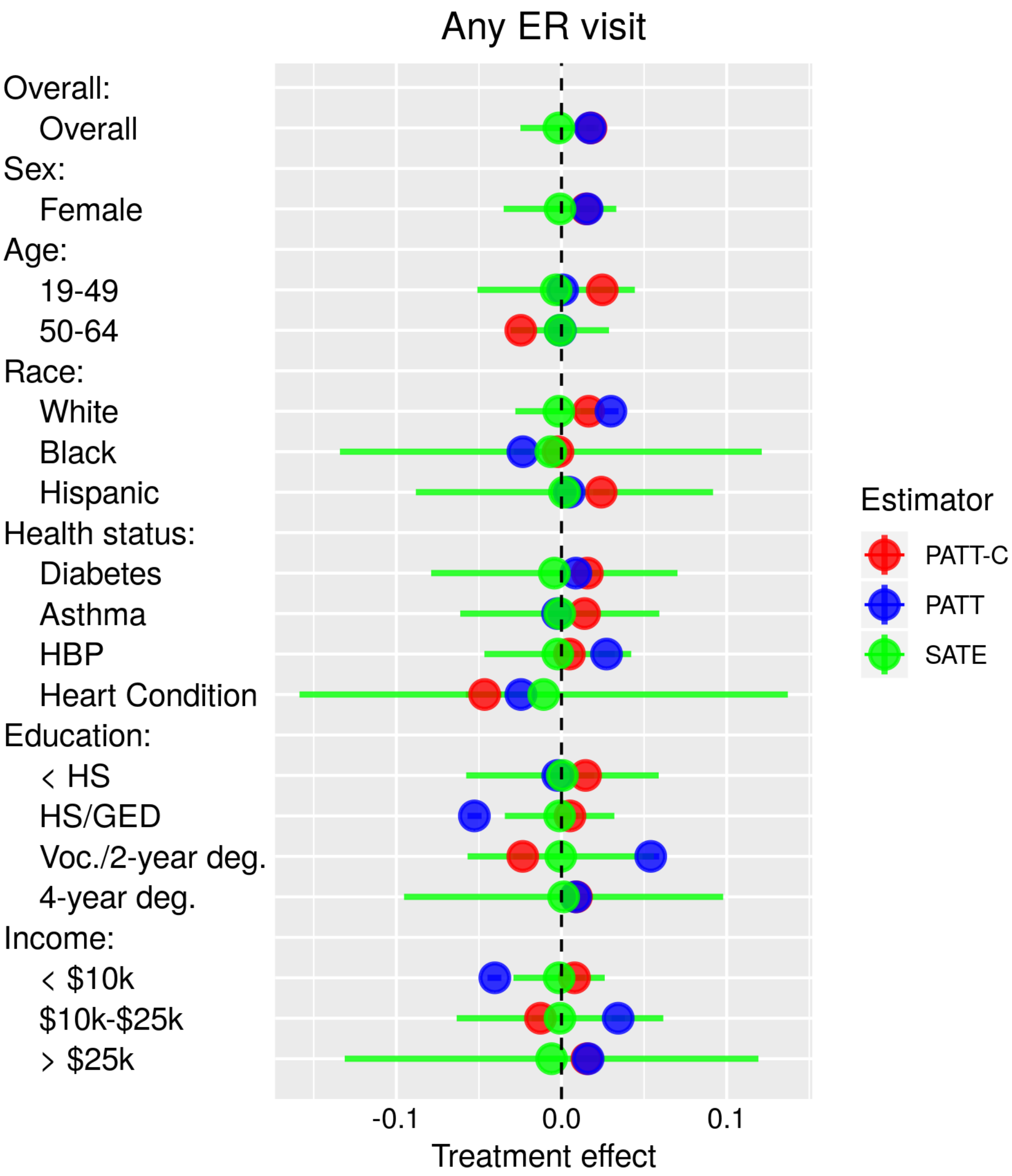

Figure A5: Heterogeneity in sample and population treatment effect estimates: any ER visit. Horizontal lines represent 95% bootstrap confidence intervals constructed with 1,000 bootstrap samples.

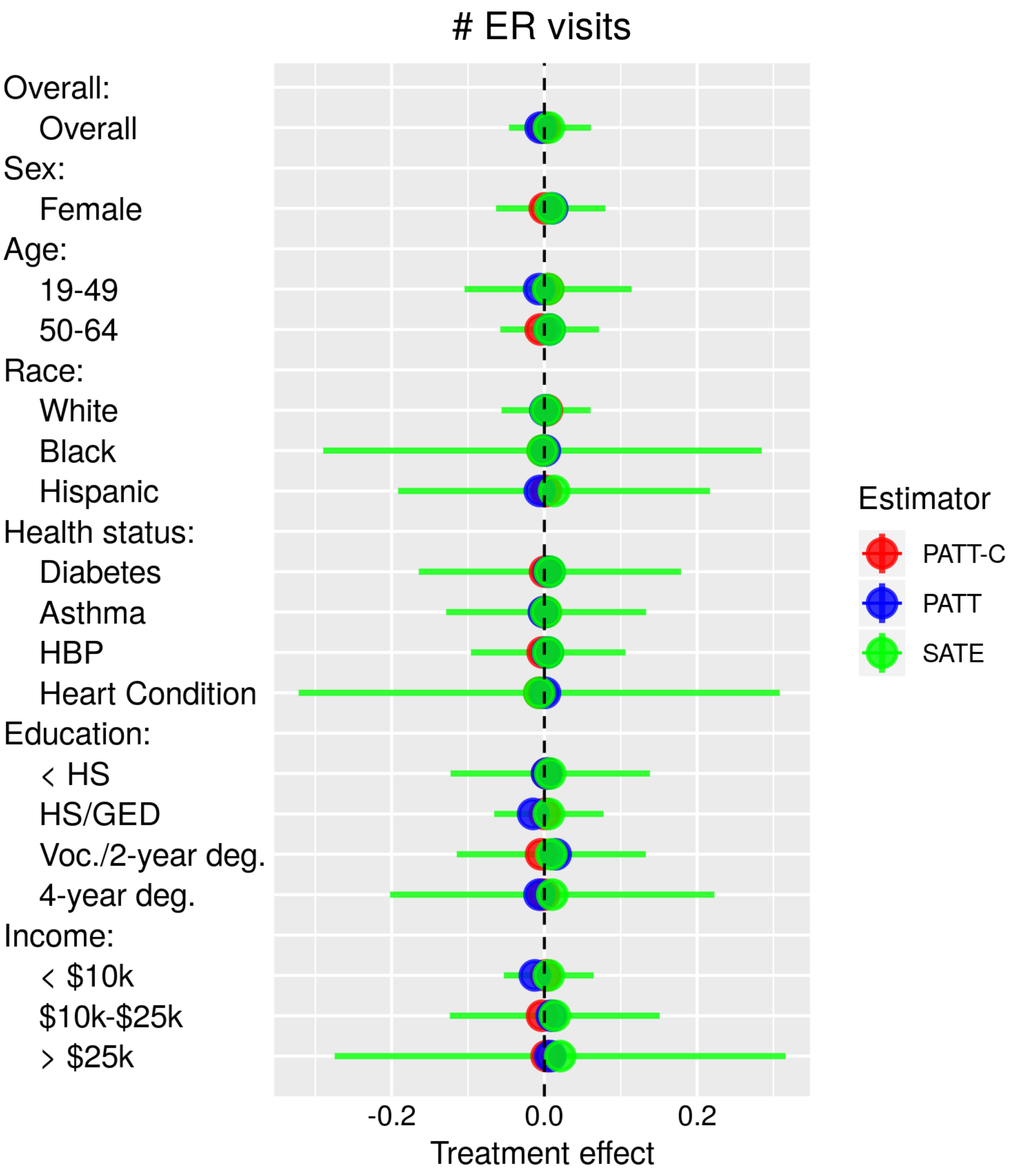

Figure A6: Heterogeneity in sample and population treatment effect estimates: # ER visits.

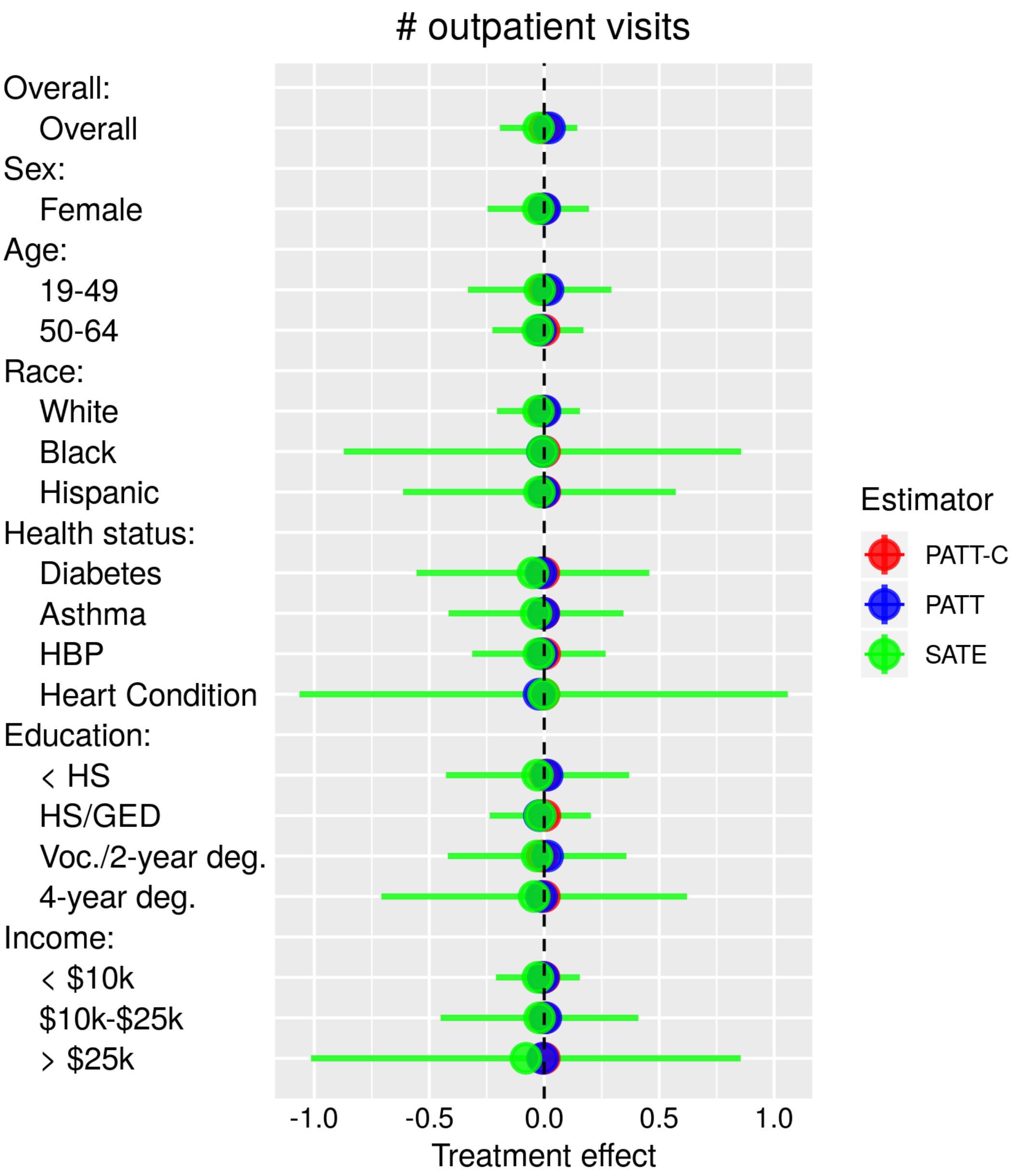

Figure A7: Heterogeneity in sample and population treatment effect estimates: # outpatient visits.

Table A2: Distribution of MSE for compliance ensemble.

Algorithm	Mean	SE	Min.	Max.
Super learner (`SuperLearner`)	0.22	0.001	0.21	0.23
Lasso regression (`glmnet`)	0.22	0.001	0.21	0.23
Random forests (`randomForest`)	0.27	0.002	0.25	0.29
Ridge regression (`glmnet`)	0.22	0.001	0.21	0.23

Notes: MSE is 10-fold cross-validated error for super learner ensemble and candidate algorithms. R package used for implementing each algorithm in parentheses.

Table A3: Error and weights for candidate algorithms in response ensemble for RCT compliers.

Any ER visit

Algorithm	MSE	Weight
Lasso regression (`glmnet`)	0.18	1
Random forests, #*preds.* = 1 (`randomForest`)	0.25	0
Random forests, #*preds.* = 10 (`randomForest`)	0.24	0
Regularized logistic regression, $\alpha = 0.25$ (`glmnet`)	0.19	0
Regularized logistic regression, $\alpha = 0.5$ (`glmnet`)	0.19	0
Regularized logistic regression, $\alpha = 0.75$ (`glmnet`)	0.19	0
Ridge regression (`glmnet`)	0.18	0

# ER visits

Algorithm	MSE	Weight
Additive regression, degree = 3 (`gam`)	0.95	0
Additive regression, degree = 4 (`gam`)	0.95	0
Lasso regression (`glmnet`)	0.95	0.92
Random forests, #*preds.* = 1 (`randomForest`)	0.95	0
Random forests, #*preds.* = 10 (`randomForest`)	0.99	0.08
Regularized linear regression, $\alpha = 0.25$ (`glmnet`)	0.95	0
Regularized linear regression, $\alpha = 0.5$ (`glmnet`)	0.95	0
Regularized linear regression, $\alpha = 0.75$ (`glmnet`)	0.95	0
Ridge regression (`glmnet`)	0.18	0

# outpatient visits

Algorithm	MSE	Weight
Additive regression, degree = 3 (`gam`)	8.40	0
Additive regression, degree = 4 (`gam`)	8.40	0
Lasso regression (`glmnet`)	8.38	0
Random forests, #*preds.* = 1 (`randomForest`)	8.38	0
Random forests, #*preds.* = 10 (`randomForest`)	8.79	0.02
Regularized linear regression, $\alpha = 0.25$ (`glmnet`)	8.38	0
Regularized linear regression, $\alpha = 0.5$ (`glmnet`)	8.38	0
Regularized linear regression, $\alpha = 0.75$ (`glmnet`)	8.38	0.92
Ridge regression (`glmnet`)	8.38	0

Notes: cross-validated error and weights used for each algorithm in super learner ensemble. *MSE* is the ten-fold cross-validated 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. Weights are rounded so that they sum to 1. R package used for implementing each algorithm in parentheses. #*preds.* is the number of predictors randomly sampled as candidates in each decision tree in random forests algorithm. α is a parameter that mixes L1 and L2 norms. degree is the smoothing term for smoothing splines.

Table A4: Error and weights for candidate algorithms in response ensemble for all RCT participants.

Any ER visit

Algorithm	MSE	Weight
Lasso regression (`glmnet`)	0.18	0.96
Random forests, $\#\textit{preds.} = 1$ (`randomForest`)	0.25	0
Random forests, $\#\textit{preds.} = 10$ (`randomForest`)	0.24	0.04
Regularized logistic regression, $\alpha = 0.25$ (`glmnet`)	0.18	0
Regularized logistic regression, $\alpha = 0.5$ (`glmnet`)	0.18	0
Regularized logistic regression, $\alpha = 0.75$ (`glmnet`)	0.18	0

# **ER visits**

Algorithm	MSE	Weight
Additive regression, degree = 3 (`gam`)	0.94	0
Additive regression, degree = 4 (`gam`)	0.94	0
Lasso regression (`glmnet`)	0.93	0.88
Random forests, $\#\textit{preds.} = 1$ (`randomForest`)	0.93	0
Random forests, $\#\textit{preds.} = 10$ (`randomForest`)	0.97	0.11
Regularized linear regression, $\alpha = 0.25$ (`glmnet`)	0.93	0
Regularized linear regression, $\alpha = 0.5$ (`glmnet`)	0.93	0
Regularized linear regression, $\alpha = 0.75$ (`glmnet`)	0.93	0
Ridge regression (`glmnet`)	0.93	0

# **outpatient visits**

Algorithm	MSE	Weight
Additive regression, degree = 3 (`gam`)	8.42	0
Additive regression, degree = 4 (`gam`)	8.42	0
Lasso regression (`glmnet`)	8.41	0
Random forests, $\#\textit{preds.} = 1$ (`randomForest`)	8.41	0.99
Random forests, $\#\textit{preds.} = 10$ (`randomForest`)	8.79	0.01
Regularized linear regression, $\alpha = 0.25$ (`glmnet`)	8.41	0
Regularized linear regression, $\alpha = 0.5$ (`glmnet`)	8.41	0
Regularized linear regression, $\alpha = 0.75$ (`glmnet`)	8.41	0
Ridge regression (`glmnet`)	8.41	0

See notes to Fig.A3.