# Estimating population average treatment effects from experiments with noncompliance

Kellie Ottoboni, Jason Poulos

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**Abstract**

This is the abstract...

# 1   Introduction

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

A prevalent issue in RCTs is non-compliance. One-way crossover from treatment to control occurs when individuals who are assigned to the treatment group refuse the treatment. This serves to dilute the 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. estimate the PATT in settings with RCT non-compliance. After deriving the estimator and investigating its performance in simulations, we apply the method to identify the effect of extending Medicare coverage to the low–income population in the U.S.

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.

# 2   Estimator

TO DO: SOME INTRO

## 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 assigned. Let $T_i$ denote the treatment assigned and $D_i$ denote treatment received. Let $W_i$ be individual $i$'s observable covariates related to the sample selection mechanism for membership in the RCT. 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 (individuals with $T_i = 1$ and $D_i = 0$) from compliers (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 $t = 0, 1$,*

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

**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):*

$$Y_{ist}^{L_i} = Y_{ist}^{L_j}, \forall i \neq j$$

*where $L_j$ is the treatment and sample assignment vector for unit $j$.*

This means that the treatment assignment for all other individuals $j$ does not affect the potential outcomes of individual $i$.

**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 (ER): 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.
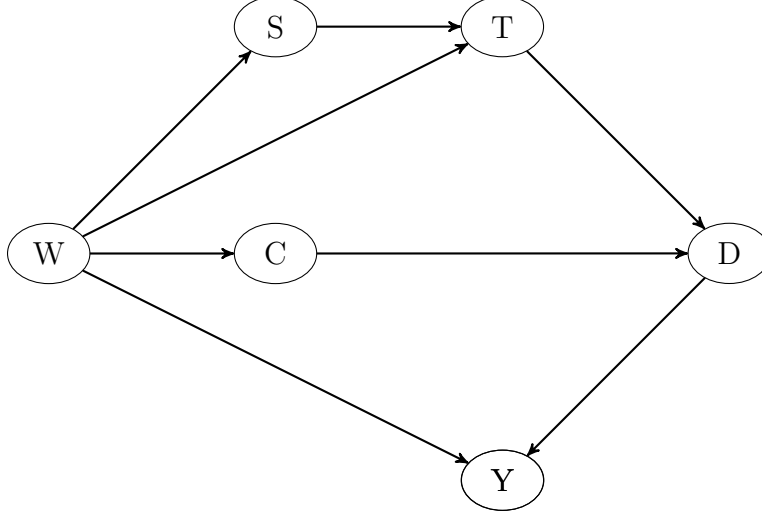
Figure 1: Causal diagram which satisfies the conditional independence assumptions needed to estimate the PATT.

## 2.2  Population Average Treatment Effect on the Treated

TO DO: MOVE SOME OF THIS TO APPENDIX? The estimand of interest is

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

This is the average treatment effect on those in the population who receive treatment. It includes individuals who actually receive the treatment, but does not include those who are eligible for treatment and do not accept it (non-compliers).

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

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

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

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

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

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

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

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

# 3 Empirical results

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

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

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

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

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

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

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

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

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

We generate a population of 30,000 individuals and randomly sample 5,000. Those among the 5,000 who are eligible for the RCT $(S = 1)$ are selected. Similarly, we sample 5,000 individuals from the population and select those who are not eligible for the RCT $(S = 0)$; these are a sample of the "target population". 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 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, 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. TO DO: FOR LOW LEVELS OF COMPLIANCE, SATE ACTUALLY TENDS TO APPROXIMATE PATT MORE CLOSELY; THE VARIANCE DUE TO SMALL SAMPLE SIZE IS LESS THAN THE NOISE ADDED BY NON-COMPLIANCE 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. The median mean squared error for SATE stays relatively constant regardless of compliance rate
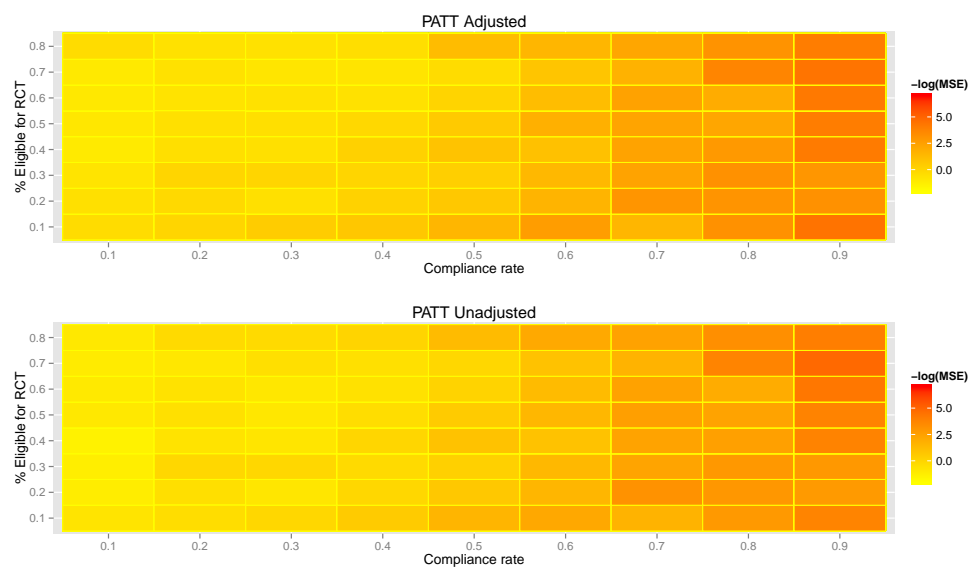
Figure 2: Simulation mean squared error, transformed by -log. Darker tiles correspond to lower mean squared error.
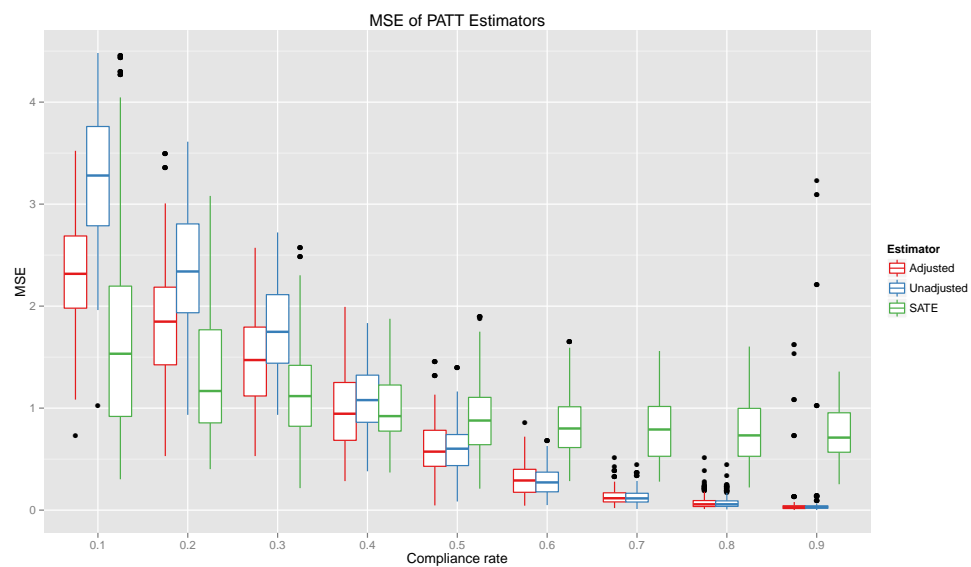


Figure 3: Simulation mean squared error, according to compliance rates in the total population.

# 4 Data

We apply the proposed method to estimate the effect of extending Medicaid coverage on emergency–room use and other health outcomes. 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.

**Oregon Health Insurance Experiment**  We draw RCT data from the Oregon Health Insurance Experiment Finkelstein et al. [2012], Taubman et al. [2014]. In 2008, approximately 90,000 uninsured low-income adults participated in a lottery to receive Medicaid benefits.[1] Treatment occurred at the household level: participants selected by the lottery won the opportunity for themselves and any household member to apply for Medicaid.[2] In total, about 35,000 participants (representing about 30,000 households) were selected by the lottery; the remaining participants were not able to apply for Medicaid and served as controls in the experiment. Participants in selected households received benefits if they returned an enrollment application within 45 days of receipt. Among participants in selected households, about 60% mailed back applications and only 30% successfully enrolled.[3] The RCT data includes demographic variables such as age, gender, ethnicity, pre-existing medical conditions, education, employment, income, and insurance coverage.

The Oregon Health Study obtained data on the number of emergency room visits for every RCT participant who resides near twelve hospitals in the Portland area ($N = 24,646$). The authors find Medicaid coverage increased emergency-room use over an 18–month period by 40% relative to the control group Taubman et al. [2014].

**Observational data**  We have data on the target population from two surveys conducted by the Center for Disease Control: the Behavioral Risk Factors Surveillance Study (BRFSS) for Disease Control and Prevention and the National Health Interview Study (NHIS) for Health Statistics. We will restrict our analysis to adults aged 19-64 whose income is below 100% of the FPL, comparing those with Medicaid to uninsured adults. The outcomes of interest from NHIS are amount of hospital use: outpatient visits, ER visits, and inpatient hospital admissions. The BRFSS outcomes are self-reported health ratings. Both surveys record individuals' demographic covariates, which match those measured in the RCT.

## 4.1 Assumptions

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

[2]Since randomization is applied on the household level, we will need to account for the number of each participant's household members when specifying the probability of treatment assignment.

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

## 4.2 Results

TO DO:

# 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 Affordable Care Act 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.

TO DO: Update tense 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 expect to find substantial differences between sample and population estimates in terms of ethnicity and pre–existing condition subgroups. Overall, we expect the PATT estimate to be lower than the SATE estimate due to the adverse selection problem in the RCT.

discuss implications of the simulation results

- 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.

- unadjusted is estimating 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.

- 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

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the unadjusted estimator when compliance is low and can be predicted by observed covariates (Figure 3). This gives evidence in favor of using the proposed estimator in this situation.

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# Appendix