

# Supplementary Web Appendix for: Robust estimation of encouragement-design intervention effects transported across sites

Kara E. Rudolph, Mark J. van der Laan

## 1 Identification proofs

### 1.1 ITTATE

$\psi_1$  is the intent-to-treat average effect of the instrument on the outcome for participants in the site without follow-up data ( $S = 0$ ), defined to be  $\psi_1 = E(Y^1 - Y^0 | S = 0)$ , where for each  $a \in \{0, 1\}$ ,  $Y^a$  denotes the counterfactual outcome that would be observed if instrument  $A = a$  were assigned and if  $Y$  were observed for participants with  $S = 0$ .

The assumptions needed for identifiability are:

1.  $E_0(Y | S = 0, W, A, Z) = E_0(Y | S = 1, W, A, Z)$ ,
2.  $A$  is independent of  $(Z^a, Y^a)$ , given  $W, S = 0$ , and
3.  $P_0(S = 1, A = a | W, Z) > 0$   $P_{0, W, Z | A=a, S=0}$ -a.e. This is the positivity assumption and means that every  $P(S = 1, A = a | W, Z)$  that one could draw from the true joint distribution of  $W, Z$  given  $A = a$  and  $S = 0$  must be greater than 0.

Proof.

$$\begin{aligned} \Psi_1(P) &\equiv E_0([E_0\{E_0(Y|S = 1, W, A = 1, Z)|S = 0, W, A = 1\} \\ &\quad - E_0\{E_0(Y|S = 1, W, A = 0, Z)|S = 0, W, A = 0\}]|S = 0) \end{aligned}$$

By assumption 1,

$$\begin{aligned} &\equiv E_0([E_0\{E_0(Y|S = 0, W, A = 1, Z)|S = 0, W, A = 1\} \\ &\quad - E_0\{E_0(Y|S = 0, W, A = 0, Z)|S = 0, W, A = 0\}]|S = 0) \end{aligned}$$

By assumption 2,

$$\begin{aligned} &P(Z = z | S = 0, W, A = a) = P(Z^a = z | S = 0, W), \text{ so} \\ &\equiv E_0[E_0\{E_0(Y^1 | S = 0, W, Z^1) | W, S = 0\} \\ &\quad - E_0\{E_0(Y^1 | S = 0, W, Z^1) | W, S = 0\} | S = 0] \\ &\equiv E_0(Y^1 - Y^0 | S = 0) \end{aligned}$$

By assumption 3, we have that  $\psi_1$  is defined.  $\square$

## 1.2 CACE

$\psi_3$  is the complier average effect of the exposure on the outcome in the site without long-term follow-up data, defined to be  $\psi_3 = E(Y^1 - Y^0 | Z^1 - Z^0 = 1, S = 0)$ , where for each  $a \in \{0, 1\}$ ,  $Y^a$  denotes the counterfactual outcome that would be observed if instrument  $A = a$  were assigned and if  $Y$  were observed for participants with  $S = 0$ , and  $Z^a$  denotes the counterfactual exposure that would be observed if instrument  $A = a$  were assigned.

The assumptions needed for identifiability are:

1.  $E_0(Y | S = 0, W, A, Z) = E_0(Y | S = 1, W, A, Z)$ ,
2.  $A = f_A(U_A)$  is independent of  $(Z^a, Y^a)$ , given  $W, S = 0$ ,
3.  $Y^{az} = Y^z$ , which is the exclusion restriction assumption, stating that the instrument  $A$  only affects the outcome  $Y$  through the exposure  $Z$ ,
4.  $Z^1 - Z^0 \geq 0$ , which is the monotonicity assumption, meaning that the instrument  $A$  cannot decrease exposure, and
5.  $P_0(S = 1, A = a | W, Z) > 0$   $P_{0,W,Z|A=a,S=0}$ -a.e and  $E_0(Z^1 - Z^0 | S = 0, W) \neq 0$ . This first part means that every  $P(S = 1, A = a | W, Z)$  that one could draw from the true joint distribution of  $W, Z$  given  $A = a$  and  $S = 0$  must be greater than 0. The second part means that the nontransported average effect of the instrument on the exposure of participants with  $S = 0$  does not equal 0.

Proof.

$$\begin{aligned}\Psi_3(P) &\equiv \frac{\Psi_1(P)}{\tilde{\Psi}(P)} \\ &\equiv \frac{\{E_0([E_0\{E_0(Y|S=1, W, A=1, Z)|S=0, W, A=1\} \\ &\quad - E_0\{E_0(Y|S=1, W, A=0, Z)|S=0, W, A=0\}]|S=0)\} \\ &\quad / [E_0\{E_0(Z|S=0, W, A=1) - E_0(Z|S=0, W, A=0)|S=0\}]\end{aligned}$$

By assumption 1,

$$\begin{aligned}&\equiv \frac{\{E_0([E_0\{E_0(Y|S=0, W, A=1, Z)|S=0, W, A=1\} \\ &\quad - E_0\{E_0(Y|S=0, W, A=0, Z)|S=0, W, A=0\}]|S=0)\} \\ &\quad / [E_0\{E_0(Z|S=0, W, A=1) - E_0(Z|S=0, W, A=0)|S=0\}]\end{aligned}$$

By assumption 2,

$$\begin{aligned}&P(Z=z | S=0, W, A=a) = P(Z^a=z | S=0, W), \text{ so} \\ &\equiv \frac{(E_0[E_0\{E_0(Y^1 | S=0, W, Z^1) | S=0, W\} \\ &\quad - E_0\{E_0(Y^0 | S=0, W, Z^0) | S=0, W\} | S=0]) \\ &\quad / [E_0\{E_0(Z^1|S=0, W) - E_0(Z^0|S=0, W)|S=0\}]}{E_0(Y^1 - Y^0 | S=0)} \\ &\equiv \frac{E_0(Y^1 - Y^0 | S=0)}{E_0(Z^1 - Z^0 | S=0)} \\ &\equiv \frac{\{E_0(Y^1 - Y^0|Z^1 - Z^0 = 1, S=0)P_0(Z^1 - Z^0 = 1|S=0) \\ &\quad + E_0(Y^1 - Y^0|Z^1 - Z^0 = 0, S=0)P_0(Z^1 - Z^0 = 0|S=0) \\ &\quad + E_0(Y^1 - Y^0|Z^1 - Z^0 = -1, S=0)P_0(Z^1 - Z^0 = -1|S=0)\}}{E_0(Z^1 - Z^0 | S=0)}\end{aligned}$$

By assumption 3,

$$\begin{aligned}&\equiv \frac{\{E_0(Y^1 - Y^0|Z^1 - Z^0 = 1, S=0)P_0(Z^1 - Z^0 = 1|S=0) \\ &\quad + E_0(Y^1 - Y^0|Z^1 - Z^0 = -1, S=0)P_0(Z^1 - Z^0 = -1|S=0)\}}{E_0(Z^1 - Z^0 | S=0)}\end{aligned}$$

By assumption 4,

$$\equiv \frac{E_0(Y^1 - Y^0|Z^1 - Z^0 = 1, S=0)P_0(Z^1 - Z^0 = 1|S=0)}{E_0(Z^1 - Z^0 | S=0)}$$

By assumption 4,

$$\begin{aligned}&Z^1 - Z^0 \text{ can only have values in } \{0, 1\}, \\ &\text{so the conditional expression is a conditional probability.} \\ &\equiv \frac{E_0(Y^1 - Y^0|Z^1 - Z^0 = 1, S=0)E(Z^1 - Z^0 | S=0)}{E_0(Z^1 - Z^0 | S=0)} \\ &\equiv E_0(Y^1 - Y^0|Z^1 - Z^0 = 1, S=0)\end{aligned}$$

By assumption 5, we have that  $\psi_3$  is defined.  $\square$

## 2 Robustness results

We derived each efficient influence curve  $D(P)(o)$  of  $\Psi$  on the nonparametric model by noting that it is given by the Gateaux derivative of  $\Psi$  at  $P$  in the direction  $(\delta_o - P\delta_o)$ , where  $\delta_o$  is the probability distribution that puts mass 1 on  $o$  (Gill et al., 1989):  $D(P)(o) = \lim_{\epsilon \rightarrow 0} \Psi[P + \epsilon\{\delta_o - P(o)\}] - \Psi(P)/\epsilon$ . In addition, we note that the efficient influence curve (EIC) for a parameter  $\Psi(P) = \Psi\{Q(P)\}$  is not affected by model assumptions on nuisance parameters whose tangent space is orthogonal to the tangent space of  $Q$ , so the EIC of the nonparametric model equals the EIC for the actual model.

### 2.1 ITTATE

#### 2.1.1 Efficient influence curve

The following result provides the conditions under which an efficient influence curve-based estimator is consistent. Let  $Q_W(w|s) = P(W \leq w | S = s)$ ,  $\bar{Q} = \{Q_W, \bar{Q}(1, W, A, Z)\}$ , and  $q_W(w|s) = \frac{dQ_W}{d\mu}(w)$  (i.e., density with respect to the appropriate dominating measure  $\mu$ ). Let  $G_Z\bar{Q}(W) = E\{\bar{Q}(1, W, a, Z) | A = a, W, S = 0\}$ . Recall that a subscript of 0 added to any of the above notation denotes values under the true data distribution,  $P_0$ .

**Result 1.** *We have*

$$P_0 D_{\Psi_1}^a(P) = \Psi_1^a(P_0) - \Psi_1^a(P) + R_2^a(P, P_0),$$

where

$$R_2^a(P, P_0) = \sum_{j=1}^4 R_{2j}^a(P, P_0),$$

and

$$\begin{aligned} R_{21}^a &= Q_{W,0|S=0} \left\{ \frac{g_{A,0|S=0} P_0(S=0)}{g_{A|S=0} P(S=0)} - 1 \right\} (G_{0,Z} - G_Z) \bar{Q} \\ R_{22}^a &= Q_{W|S=0} G_Z \left\{ \frac{g_{A,0|S=1} q_{W,0|S=1} P_0(S=0)}{g_{A|S=1} q_{W|S=1} P(S=0)} \frac{g_{Z,0|S=1}}{g_{Z|S=1}} - 1 \right\} (\bar{Q}_0 - \bar{Q}) \\ R_{23}^a &= (Q_{W,0|S=0} - Q_{W|S=0}) G_Z (\bar{Q} - \bar{Q}_0) \\ R_{24}^a &= Q_{W,0|S=0} (G_{Z,0} - G_Z) (\bar{Q} - \bar{Q}_0). \end{aligned}$$

Inspection of the second order term  $R_2^a$  gives three scenarios under which an estimator  $\Psi_1^a(P_n)$  that solves the efficient influence equation  $P_n D_{\Psi_1}^a(P_n)$  will be consistent (robustness result). First, if  $Q_W, G_Z, g_A, q_{W|S=1}$ , and  $P(S=0)$  are correctly specified in the sense that  $(Q_{W|S=0} - Q_{W,0|S=0}) G_Z (\bar{Q} - \bar{Q}_0) = 0$ ,  $G_Z = G_{Z,0}$ ,  $g_{A|S=1} = g_{A,0|S=1}$ ,  $q_{W|S=1} = q_{W,0|S=1}$ , and  $P(S=0) = P_0(S=0)$ . In other words, the  $Y$  model may be misspecified if all other models are correct. Second, if  $\bar{Q}$  and  $G_Z$  are correctly specified in the sense that  $\bar{Q} = \bar{Q}_0$ ,  $G_Z = G_{Z,0}$ . In other words, the  $S$  and  $A$  models may be misspecified if the  $Y$  and  $Z$  models are correct. Third, if  $\bar{Q}$  and  $g_A$  (and  $P(S=0)$ ) are

correctly specified in the sense that  $\bar{Q} = \bar{Q}_0$ ,  $g_{A|S=0} = g_{A,0|S=0}$ ,  $P(S=0) = P_0(S=0)$ . In other words, the  $S$  and  $Z$  models may be misspecified if the  $Y$  and  $A$  models are correct.

**Corollary 1.** *If  $P_0 D_{\Psi_1^a}(P) = 0$  and one of three above scenarios holds, then  $\Psi_1^a(P) = \Psi_1^a(P_0)$ .*

The implication of this corollary is that if  $P_n$  of  $P_0$  converges to a  $P$  so that  $P_0 D(P) = 0$  (which will be true for a TMLE since a TMLE solves  $P_n D(P_n) = 0$ ) and one of the above three scenarios holds, then  $\Psi_1^a(P_n)$  is consistent for  $\Psi_1^a(P_0)$ .

## 2.2 EACE

The following result provides the conditions for consistency based on the efficient influence curve.

**Result 2.**

$$P_0 D_{\Psi_2^z}(P) = \Psi_2^z(P_0) - \Psi_2^z(P) + R_2^z(P, P_0),$$

where

$$R_2^z(P, P_0) = R_{21}^z(P, P_0) + R_{22}^z(P, P_0) + R_{23}^z(P, P_0),$$

and

$$\begin{aligned} R_{21}^z(P, P_0) &= \int \frac{g_0(S=1, Z=z | W) - g(S=1, Z=z | W)}{g(S=1, Z=z | W)} (\bar{Q}_0 - \bar{Q})(W) \frac{g_S(S=0 | W)}{P(S=0)} dP_0(W) \\ R_{22}^z(P, P_0) &= (Q_W - Q_{0,W})(\bar{Q}_0 - \bar{Q}) \\ &\quad + \int \frac{g_S(S=0 | W)}{P(S=0)} (\bar{Q}_0 - \bar{Q}) d(P - P_0)(W) \\ R_{23}^z(P, P_0) &= \frac{(P_0 - P)(S=0)}{P(S=0)} (Q_{0,W} - Q_W) \bar{Q}. \end{aligned}$$

Considering the case that  $P(S=1) = P_0(S=1)$  so that  $R_{23}^z = 0$ , we have two scenarios under which an estimator that solves the efficient influence curve will be consistent (robustness result). First, if  $\bar{Q} = \bar{Q}_0$  (i.e., the  $Y$  model is correct). Second, if  $P(S=1, Z=z | W) = P_0(S=1, Z=z | W)$  and  $(Q_W, P_W) = (Q_{0,W}, P_{0,W})$  (i.e., the  $S$  and  $Z$  models are correct).

**Corollary 2.** *If  $P_0 D_{\Psi_2^z}(P) = 0$  and one of two above scenarios holds, then  $\Psi_2^z(P) = \Psi_2^z(P_0)$ .*

The implication of this corollary is that if  $P_n$  of  $P_0$  converges to a  $P$  so that  $P_0 D(P) = 0$  (which will be true for a TMLE since a TMLE solves  $P_n D(P_n) = 0$ ) and one of the above two scenarios holds, then  $\Psi_2^z(P_n)$  is consistent for  $\Psi_2^z(P_0)$ .

## 2.3 CACE

### 2.3.1 Efficient influence curve

We know the efficient influence curve of  $\Psi_1$  and  $\tilde{\Psi}$ , so by the delta method, the efficient influence curve of  $\Psi_3$  is given by the following ratio:

$\Psi_3(P) = \frac{\Psi_1(P)}{\tilde{\Psi}(P)}$ , where  $\tilde{\Psi}(P)$  is the statistical estimand of the average effect of the instrument on the treatment.

$$\begin{aligned} D_{\Psi_1, \tilde{\Psi}}^a(P) &= \left\{ \frac{\Psi_1(P)}{\tilde{\Psi}(P)} \right\}' \\ &= \frac{\tilde{\Psi}(P)\Psi_1(P)' - \Psi_1(P)\tilde{\Psi}(P)'}{\tilde{\Psi}(P)^2} \\ &= \frac{\tilde{\Psi}(P)D_{\Psi_1}(P) - \Psi_1(P)D_{\tilde{\Psi}}(P)}{\tilde{\Psi}(P)^2} \\ &= \frac{1}{\tilde{\Psi}(P)}D_{\Psi_1}(P) - \frac{\Psi_1(P)}{\tilde{\Psi}(P)^2}D_{\tilde{\Psi}}(P) \end{aligned}$$

## 3 Alternative ITTATE TMLE

Let  $Pa(Z)$  represent variables that are parents of  $Z$ . If  $Z$  is binary, then we can use that for any function  $S\{Z | Pa(Z)\}$  with conditional mean zero, given  $Pa(Z)$ , we have

$$S\{Z | Pa(Z)\} = [S\{1 | Pa(Z)\} - S\{0 | Pa(Z)\}][Z - E\{Z | Pa(Z)\}]$$

(Van der Laan and Robins, 2003). Therefore, we can rewrite  $D_Z^a(P)$  as follows:

$$\begin{aligned} D_{Z, \Psi_1}^a(P) &= \frac{I(A = a, S = 0)}{g_A(A = a | W, S = 0)P(S = 0)} \{ \bar{Q}(1, W, a, Z = 1) - \bar{Q}(1, W, a, Z = 0) \} \\ &\quad \times \{ Z - g_Z(Z = 1 | S = 0, W, a) \} \\ &\equiv C_Z(g_A, \bar{Q}) \{ Z - g_Z(Z = 1 | S = 0, W, a) \}. \end{aligned}$$

As in the TMLE shown in the main text, consider submodel  $\text{Logit}\bar{Q}_n^0(\epsilon) = \text{Logit}\bar{Q}_n^0 + \epsilon C_Y(g_{Z,n}^0, g_{S,n}^0)$ , and let  $\epsilon_n^0$  be the fitted coefficient for this clever covariate  $C_Y$  in the univariate logistic regression model using  $\text{Logit}\bar{Q}_n^0$  as offset, using the binary log-likelihood loss function multiplied with  $I(S = 1, A = a)$  (i.e., only using the observations with  $S_i = 1, A_i = a$ ). The updated estimator is denoted with  $\bar{Q}_n^1 = \bar{Q}_n^0(\epsilon_n^0)$ . Consider the submodel

$$\text{Logit}\bar{g}_{Z,n}^0(\epsilon) = \text{Logit}\bar{g}_{Z,n}^0 + \epsilon C_Z(g_{A,n}, \bar{Q}_n^0).$$

Let  $\epsilon_{1n}^0$  be the fitted coefficient using logistic regression of  $Z = z$  on  $W$  among the observations with  $S_i = 0, A_i = a$ , using predicted values  $\text{Logit}\bar{g}_{Z,n}^0$  as an offset. This defines now  $g_{Z,n}^1 = g_{Z,n}^0(\epsilon_{1n}^0)$ . This process can be iterated: Let  $k = 0$ , set  $\bar{Q}_n^{k+1} = \bar{Q}_n^k(\epsilon_n^k)$  and  $g_{Z,n}^{k+1} = g_{Z,n}^k(\epsilon_{1n}^k)$ , set  $k \leftarrow k + 1$ , and repeat until convergence. Assume that  $\epsilon_n^k, \epsilon_{1n}^k$  converge to

zero as  $k \rightarrow \infty$  or that at a step  $K$  we have that  $P_n D_{Z, \Psi_1}^a(g_{A,n}, g_{Z,n}^K, g_{S,n}, \bar{Q}_n^K, Q_{W,n|S=0}) = o_P(1/\sqrt{n})$ . Let  $g_{Z,n}^*, \bar{Q}_n^*$  denote the resulting final fits. The TMLE of  $\psi_{0,1}^a$  is defined by the substitution estimator  $\psi_{n,1}^{a*} = \Psi_1^a(g_{Z,n}^*, \bar{Q}_n^*, Q_{W,n|S=0})$ . This TLME solves

$$P_n D_{Z, \Psi_1}^a(g_{S,n}, g_{A,n}, g_{Z,n}^*, \bar{Q}_n^*, Q_{W,n|S=0}) = 0 \text{ or } o_P(1/\sqrt{n}).$$

#### 4 Alternative variance estimate of $\Psi_3$

Alternatively,  $\text{var}(\Psi_3)$  can be estimated using the multivariate delta method.

$$\begin{aligned} \text{Var}\left(\frac{\Psi_1}{\dot{\Psi}}\right) &= \left(\nabla \frac{\Psi_1}{\dot{\Psi}}\right)' \text{Cov}(\Psi_1, \tilde{\Psi}) \left(\nabla \frac{\Psi_1}{\dot{\Psi}}\right) \\ &= \begin{bmatrix} 1 & -\mu_1 \\ \tilde{\mu} & \tilde{\mu}^2 \end{bmatrix} \begin{bmatrix} \sigma_1^2 & \sigma_1 \tilde{\sigma} \\ \sigma_1 \tilde{\sigma} & \tilde{\sigma}^2 \end{bmatrix} \begin{bmatrix} \frac{1}{\tilde{\mu}} \\ \frac{-\mu_1}{\tilde{\mu}^2} \end{bmatrix} \\ &= \begin{bmatrix} \frac{\sigma_1^2}{\tilde{\mu}} - \frac{\mu_1 \sigma_1 \tilde{\sigma}}{\tilde{\mu}^2}, & \frac{\sigma_1 \tilde{\sigma}}{\tilde{\mu}} - \frac{\tilde{\sigma}^2 \mu_1}{\tilde{\mu}^2} \end{bmatrix} \begin{bmatrix} \frac{1}{\tilde{\mu}} \\ \frac{-\mu_1}{\tilde{\mu}^2} \end{bmatrix} \\ &= \frac{\sigma_1^2}{\tilde{\mu}^2} - \frac{\mu_1 \sigma_1 \tilde{\sigma}}{\tilde{\mu}^2} - \frac{\mu_1 \sigma_1 \tilde{\sigma}}{\tilde{\mu}^3} + \frac{\tilde{\sigma}^2 \mu_1^2}{\tilde{\mu}^4} \\ &= \frac{1}{\tilde{\mu}^2} \left( \sigma_1^2 - \frac{2\mu_1 \sigma_1 \tilde{\sigma}}{\tilde{\mu}} + \frac{\tilde{\sigma}^2 \mu_1^2}{\tilde{\mu}^2} \right) \\ &= \frac{\mu_1^2}{\tilde{\mu}^2} \left( \frac{\sigma_1^2}{\mu_1^2} - \frac{2\sigma_1 \tilde{\sigma}}{\mu_1 \tilde{\mu}} + \frac{\tilde{\sigma}^2}{\tilde{\mu}^2} \right) \end{aligned}$$

## 5 Simulation results

Table 1: Results from data-generating mechanism 1 without positivity violations. TMLE estimator performance under correct and incorrect model specification across 10,000 simulations in terms of percent bias, estimator standard error  $\times \sqrt{n}$ , 95% confidence interval coverage, and mean squared error.  $N=500$ . The estimator standard error  $\times \sqrt{n}$  should be compared to the efficiency bound, which is 0.47 for the ITTATE, 1.42 for the CACE, and 0.51 for the EACE.

Specification	%Bias	SE $\times \sqrt{n}$	95%CI Cov	MSE
bound, which is 1.49 for the ITTATE, 4.50 for the CACE, and 1.60 for the EACE.				
ITTATE				
All models correct	-0.09	1.53	94.75	0.0048
S model misspecified	0.03	1.39	94.91	0.0039
Z model misspecified	-0.09	1.51	94.40	0.0048
Y model misspecified	-0.03	1.56	94.85	0.0049
S,Z models misspecified	0.11	1.37	94.80	0.0038
S,Z,Y models misspecified	6.82	1.39	94.80	0.0039
EACE				
All models correct	-1.27	1.62	94.70	0.0052
S model misspecified	-0.83	1.46	93.60	0.0047
Z model misspecified	-1.27	1.49	92.82	0.0052
Y model misspecified	-1.17	1.65	95.40	0.0052
S,Z models misspecified	-0.88	1.35	93.09	0.0042
S,Z,Y models misspecified	13.98	1.37	94.03	0.0041
CACE				
All models correct	0.55	4.86	96.02	0.0495
S model misspecified	0.57	4.40	96.11	0.0397
Z model misspecified	0.55	4.81	95.93	0.0495
Y model misspecified	0.56	4.86	95.81	0.0503
S,Z models misspecified	0.78	4.35	96.27	0.0380
S,Z,Y models misspecified	7.16	4.41	96.34	0.0390



Table 2: Results from data-generating mechanism 2 with practical positivity violations. TMLE estimator performance under correct and incorrect model specification across 10,000 simulations in terms of percent bias, estimator standard error  $\times\sqrt{n}$ , 95% confidence interval coverage, and mean squared error.  $N=500$ . The estimator standard error  $\times\sqrt{n}$  should be compared to the efficiency bound, which is 0.85 for the ITTATE, 3.58 for the CACE, and 1.29 for the EACE.

Specification	%Bias	SE $\times\sqrt{n}$	95%CI Cov	MSE
ITTATE				
All models correct	3.32	2.68	92.09	0.0165
S model misspecified	-0.06	1.40	95.18	0.0039
Z model misspecified	3.32	2.75	92.68	0.0165
Y model misspecified	7.72	2.91	93.84	0.0167
S,Z models misspecified	-0.25	1.40	95.42	0.0038
S,Z,Y models misspecified	18.87	1.42	94.97	0.0040
EACE				
All models correct	-11.13	3.21	87.31	0.0246
S model misspecified	-2.40	1.88	83.77	0.0122
Z model misspecified	-11.13	2.63	84.38	0.0246
Y model misspecified	-4.54	3.73	91.34	0.0242
S,Z models misspecified	0.55	1.38	78.59	0.0095
S,Z,Y models misspecified	-52.27	1.41	75.96	0.0102
CACE				
All models correct	8.15	12.41	94.84	0.3519
S model misspecified	2.89	6.40	96.94	0.0811
Z model misspecified	8.15	14.42	95.24	0.3519
Y model misspecified	12.93	12.41	94.76	0.3550
S,Z models misspecified	4.10	7.35	97.66	0.1225
S,Z,Y models misspecified	29.38	7.64	98.09	0.1337

Table 3: Results of truncation of the clever covariate to lessen sensitivity to practical positivity violations (from data-generating mechanism 2). Estimator performance under correct model specification across 10,000 simulations in terms of percent bias, estimator standard error  $\times \sqrt{n}$ , 95% confidence interval coverage, and mean squared error.

Truncation Level	%Bias	SE $\times \sqrt{n}$	95%CI Cov	MSE
N=5,000. The estimator standard error $\times \sqrt{n}$ should be compared to the efficiency bound, which is 2.68 for the ITTATE, 11.33 for the CACE, and 4.09 for the EACE.				
ITTATE				
No modification	-0.88	2.68	94.85	0.0015
Truncation at 0.01/100	-0.87	2.68	94.87	0.0015
Truncation at 0.05/20	-0.87	2.48	93.71	0.0014
Truncation at 0.1/10	-0.74	2.05	89.45	0.0012
EACE				
No modification	0.18	3.60	91.36	0.0029
Truncation at 0.01/100	2.29	3.23	92.50	0.0024
Truncation at 0.05/20	2.71	2.40	89.78	0.0016
Truncation at 0.1/10	2.60	1.90	84.96	0.0013
CACE				
No modification	2.57	11.42	94.96	0.0265
Truncation at 0.01/100	2.58	11.41	94.96	0.0265
Truncation at 0.05/20	2.59	10.57	93.80	0.0250
Truncation at 0.1/10	2.75	8.72	89.74	0.0221
N=500. The estimator standard error $\times \sqrt{n}$ should be compared to the efficiency bound, which is 0.85 for the ITTATE, 3.58 for the CACE, and 1.29 for the EACE.				
ITTATE				
No modification	3.32	2.68	92.09	0.0165
Truncation at 0.01/100	3.37	2.67	92.11	0.0165
Truncation at 0.05/20	3.02	2.59	92.45	0.0158
Truncation at 0.1/10	3.53	2.00	87.86	0.0128
EACE				
No modification	-11.13	3.21	87.31	0.0246
Truncation at 0.01/100	-8.19	3.03	87.64	0.0237
Truncation at 0.05/20	-9.18	3.02	87.21	0.0234
Truncation at 0.1/10	-0.60	1.86	82.70	0.0139
CACE				
No modification	8.15	12.41	94.84	0.3519
Truncation at 0.01/100	8.20	12.38	94.84	0.3513
Truncation at 0.05/20	7.85	12.01	95.01	0.3354
Truncation at 0.1/10	7.84	9.34	91.35	0.2729

## 6 Baseline covariates used in MTO application

An extensive set of baseline characteristics were included in applying our transport estimators to the MTO research question.

- Adolescent characteristics: age, gender, race, number of family members.
- Characteristics related to the child's behavior and learning: child was suspended or expelled from school during 2 years prior to baseline, child had gone to a special class or school or had gotten special help in school for behavioral or emotional problems during 2 years prior to baseline, child had gone to a special class or school or had gotten special help in school for a learning problem during 2 years prior to baseline, someone from school asked to discuss problems the child had with schoolwork or behavior during the 2 years prior to baseline, child enrolled in special class for gifted and talented students, child had problems that made it difficult to get to school or play active games/sports.
- Adult family member characteristics included: level of education, marital status, age at birth of the adolescent, work status, receipt of AFDC/TANF, car status, disability status.
- Neighborhood characteristics: family lived in neighborhood for at least 5 years; felt neighborhood streets were unsafe at night; household member had been assaulted, threatened with a knife or gun, or robbed during the 6 months prior to baseline; chat with a neighbor at least once per week; would likely tell neighbor if neighbor's child was getting into trouble; family living in neighborhood; friends in neighborhood; neighborhood satisfaction.
- Reported reasons for participating in MTO: to get away from drugs or gangs, to have access to better schools.
- Moving-related characteristics: confidence about finding an apartment in a different part of the city, moved more than 3 times during the 5 years prior to baseline, and previous application for Section 8 voucher.

## 7 R code

### 7.1 Code for TMLE functions

```

1 ## a variable needs to be named a and have values 0/1
2 ## site variable needs to be named 'site' and needs to have value 0 for the
   site where the outcome data is not used and value 1 for the site where
   the outcome data is used
3 ## z variable needs to be named z and have values 0/1
4 ## y variable needs to be named y and have values 0/1
5 ## w variables in a dataframe named w and with names w1:wx
6

```

```

7 ittatetmle<-function(a, z, y, site, w, aamodel, asitemodel, azmodel,
  aoutmodel, aq2model){
8   datw<-w
9   n.dat<-nrow(datw)
10
11  #calculate components of clever covariate
12  cpa <- predict(glm(formula=aamodel, family="binomial", data=data.frame(
  cbind(datw, a=a))), newdata=datw, type="response")
13  cps <- predict(glm(formula=asitemodel, data=data.frame(cbind(site=site,
  datw)), family="binomial"), type="response")
14
15  zmodels0 <- glm(formula=azmodel, data=data.frame(cbind(a=a, z=z, site=
  site, datw)), subset=site==0, family="binomial")
16  zmodels1 <- glm(formula=azmodel, data=data.frame(cbind(a=a, z=z, site=
  site, datw)), subset=site==1, family="binomial")
17  data_new0<-data_new1<-datw
18  data_new0$a<-0
19  data_new1$a<-1
20
21  dga1s0<-dbinom(z, 1, prob=predict(zmodels0, newdata=data_new1, type="
  response"))
22  dga1s1<-dbinom(z, 1, prob=predict(zmodels1, newdata=data_new1, type="
  response"))
23  dga0s0<-dbinom(z, 1, prob=predict(zmodels0, newdata=data_new0, type="
  response"))
24  dga0s1<-dbinom(z, 1, prob=predict(zmodels1, newdata=data_new0, type="
  response"))
25
26  #calculate clever covariate
27  g0w<-(1-cpa)*(dga0s1/dga0s0)*(cps/(1-cps))
28  g1w<-cpa*(dga1s1/dga1s0)*(cps/(1-cps))
29  h0w<-((1-a)*I(site==1))/g0w
30  h1w<-(a*I(site==1))/g1w
31
32  ymodel<-glm(formula=aoutmodel, family="binomial", data=data.frame(cbind(
  datw, a=a, z=z, site=site, y=y)), subset=site==1)
33
34  #initial prediction
35  q<-cbind(predict(ymodel, type="link", newdata=data.frame(cbind(datw, a=a,
  z=z))), predict(ymodel, type="link", newdata=data.frame(cbind(datw, a
  =0, z=z))), predict(ymodel, type="link", newdata=data.frame(cbind(datw
  , a=1, z=z))))
36
37  epsilon<-coef(glm(y ~ -1 + offset(q[,1]) + h0w + h1w, family="binomial",
  subset=site==1))
38
39  #update initial prediction
40  q1<- q + c((epsilon[1]*h0w + epsilon[2]*h1w), epsilon[1]/g0w, epsilon[2]
  /g1w)
41
42  predmodela0<-suppressWarnings(glm(formula=paste("plogis(q1)", aq2model,
  sep="~"), data=data.frame(cbind(w, a=a, site=site, q1=q1[,2])), subset
  =site==0 & a==0, family="binomial"))

```

```

43 predmodela1<-suppressWarnings(glm(formula=paste("plogis(q1)", aq2model,
44   sep="~"), data=data.frame(cbind(w,a=a, site=site, q1=q1[,3])), subset
   =site==0 & a==1, family="binomial"))
44 predmodelaa<-suppressWarnings(glm(formula=paste("plogis(q1) ~", aq2model,
   "+a", sep=""), data=data.frame(cbind(w, site=site, q1=q1[,1], a=a)),
   subset=site==0, family="binomial"))
45
46 #get initial prediction for second regression model
47 q2pred<-cbind(predict(predmodelaa, type="link", newdata=data.frame(cbind(
   datw, a=a))), predict(predmodela0, type="link", newdata=datw),
   predict(predmodela1, type="link", newdata=datw))
48
49 cz<-cbind(ifelse(a==0,I(site==0)/(1-cpa), I(site==0)/cpa), I(site==0)/(1-
   cpa), I(site==0)/cpa)
50
51 epsilon2<-suppressWarnings(coef(glm(plogis(q1[,1]) ~ -1 + offset(q2pred
   [,1]) + cz[,2] + cz[,3], family="binomial", subset= site==0)))
52 for(k in 1:2){
53   epsilon2[k]<-ifelse(is.na(epsilon2[k]), 0, epsilon2[k])
54 }
55
56 q2<- q2pred + c((epsilon2[1]*cz[,2] + epsilon2[2]*cz[,3]), epsilon2[1]/
   (1-cpa), epsilon2[2]/cpa)
57
58 tmleest<-mean(plogis(q2[,3][site==0]))-mean(plogis(q2[,2][site==0]))
59
60 ps0<-mean(I(site==0))
61
62 eic<-(((hlw/ps0) - (h0w/ps0))*(y - plogis(q[,1]))) + (((a*cz[,3]/ps0) -
   ((1-a)*cz[,2]/ps0))* (plogis(q[,1]) - plogis(q2pred[,1]))) + ((I(site
   ==0)/ps0)*((plogis(q2pred[,3]) - plogis(q2pred[,2])) - tmleest))
63
64 return(list("est"=tmleest, "var"=var(eic)/n.dat, "eic"=eic))
65
66 }
67
68 eatetmle<-function(a, z, y, site, w, nsitemodel, nzmodel, noutmodel){
69   datw<-w
70   n.dat<-nrow(w)
71
72   #calculate components of clever covariate
73   cps <- predict(glm(formula=nsitemodel, data=data.frame(cbind(site=site,
   datw)), family="binomial"), type="response")
74   cpz<-predict(glm(formula=nzmodel, data=data.frame(cbind(a=a, z=z, datw)),
   family="binomial"), type="response")
75
76   #calculate clever covariate
77   g0w<-((1-cpz)*cps)/(1-cps)
78   glw<- (cpz*cps)/(1-cps)
79   h0w<-((1-z)*I(site==1))/g0w
80   hlw<- (z*I(site==1))/glw
81

```

```

82 ymodel<-glm(formula=noutmodel, family="binomial", data=data.frame(cbind(
      datw, a=a, z=z, site=site, y=y)), subset=site==1)
83
84 data_new0<-data_new1<-datw
85 data_new0$z<-0
86 data_new1$z<-1
87 #initial predicition
88 q<-cbind(predict(ymodel, type="link", newdata=data.frame(cbind(datw, a=a,
      z=z))), predict(ymodel, type="link", newdata=data_new0), predict(
      ymodel, type="link", newdata=data_new1))
89
90 epsilon<-coef(glm(y ~ -1 + offset(q[,1]) + h0w + hlw, family="binomial",
      subset=site==1))
91
92 #update initial prediction
93 q1<- q + c((epsilon[1]*h0w + epsilon[2]*hlw), epsilon[1]/g0w, epsilon[2]/
      glw)
94
95 tmleest<-mean(plogis(q1[,3][site==0]))-mean(plogis(q1[,2][site==0]))
96
97 #get efficient influence curve values for everyone
98 ps0<-mean(I(site==0))
99
100 eic<-(((z*hlw/ps0) - ((1-z)*h0w/ps0))*(y - plogis(q[,1]))) + (I(site==0)/
      ps0*plogis(q1[,3])) - (I(site==0)/ps0*plogis(q1[,2])) - (tmleest/ps0)
101
102 return(list("est"=tmleest, "var"=var(eic)/n.dat, "eic"=eic[site==0]))
103 }
104
105 notransporttmle<-function(a,z,w, site, ntamodel, ntzmodel){
106   datw<-w
107
108   n.dat<-nrow(datw)
109   ps0<-mean(I(site==0))
110
111   #calculate components of clever covariate
112   cpa <- predict(glm(formula=ntamodel, data=data.frame(cbind(a=a, site=site
      , datw)), subset=site==0, family="binomial"), newdata=datw, type="
      response")
113
114   g0w<-1-cpa
115   glw<-cpa
116
117   #clever covariates
118   h0w<-I(site==0)*(1-a)/(g0w*ps0)
119   hlw<-I(site==0)*a/(glw*ps0)
120
121   zmodel<-glm(formula=ntzmodel, family="binomial", data=data.frame(cbind(a=a
      , z=z, site=site, datw)), subset=site==0)
122
123   data_new0<-data_new1<-datw
124   data_new0$a<-0
125   data_new1$a<-1

```

```

126
127 q<-cbind(predict(zmodel, type="link", newdata=data.frame(cbind(a=a, z=z,
128   datw))), predict(zmodel, type="link", newdata=data_new0), predict(
129   zmodel, type="link", newdata=data_new1)
130
131 epsilon<-coef(glm(z ~ -1 + offset(q[,1]) + h0w + hlw , family="binomial",
132   subset= site==0))
133
134 q1<- q + c((epsilon[1]*h0w + epsilon[2]*hlw), I(site==0)*epsilon[1]/(g0w*
135   ps0), I(site==0)*epsilon[2]/(glw*ps0))
136
137 tmleest<-mean(plogis(q1[,3][ site ==0]))-mean(plogis(q1[,2][ site ==0]))
138
139 eic<-(((a*hlw) - ((1-a)*h0w))*(z - plogis(q[,1])) + ((I(site==0)/ps0)*((
140   plogis(q1[,3]) - plogis(q1[,2])) - tmleest))
141
142 return(list("est"=tmleest, "var"=var(eic)/n.dat, "eic"=eic))
143 }
144
145
146
147 catetmle<-function(ca, cz, cy, csite, cw, czmodel, csitemodel, coutmodel,
148   cq2model){
149   datw<-cw
150   n.dat<-nrow(datw)
151   ps0<-mean(I(csite==0))
152   camodel<-"a ~ 1"
153
154   nottransportate<-nottransporttmle(a=ca, z=cz, site=csite, w=cw, ntamodel=
155     camodel, ntzmodel=czmodel)
156   ittate<-ittatetmle(a=ca, z=cz, y=cy, site=csite, w=cw, aamodel=camodel,
157     asitemodel=csitemodel, azmodel=czmodel, aoutmodel=coutmodel, aq2model
158     =cq2model)
159   cate<-ittate$est/nottransportate$est
160   varcate<-((ittate$est^2/nottransportate$est^2)*(((ittate$var*n.dat)/ittate$
161     est^2) - ((2*cov(cbind(ittate$eic, nottransportate$eic))[1,2])/(ittate
162     $est*nottransportate$est)) + ((nottransportate$var*n.dat)/
163     nottransportate$est^2))
164   eic<-((ittate$eic/nottransportate$est) - (ittate$est/(nottransportate$est^2)
165     )*nottransportate$eic)
166
167   return(list("est"=cate, "var"=var(eic)/n.dat, "eic"=eic))
168 }

```

## Functions.R

## 7.2 Code for example application

```

1 source("Functions.R")
2
3 n<-5000
4
5 site<-rbinom(n, 1, .5)

```

```

6
7 race<-rbinom(n,1, .4 + (.2*site))
8
9 crime<-rnorm(n, .1*site, 1)
10 discrimination<-rnorm(n, 1+(.2*site), 1)
11
12 #instrument
13 voucher<-rbinom(n, 1, .5)
14
15 #exposure
16 move0<-rbinom(n,1, plogis( -log(1.6) - log(1.1)*crime -log(1.3)*
17   discrimination))
18 move1<-rbinom(n,1, plogis( -log(1.6) +log(4) - log(1.1)*crime -log(1.3)*
19   discrimination))
20 move<-ifelse(voucher==1, move1, move0)
21
22 #outcomes
23 inschoola0<-rbinom(n,1, plogis(log(1.6) + (log(1.9)*move0) -log(1.3)*
24   discrimination - log(1.2)*race + log(1.2)*race*move0) )
25 inschoola1<-rbinom(n,1, plogis(log(1.6) + (log(1.9)*move1) - log(1.3)*
26   discrimination - log(1.2)*race+ log(1.2)*race*move1) )
27 inschoola<-ifelse(voucher==1, inschoola1, inschoola0)
28
29 dat<-data.frame( w2=crime, w3=discrimination, w1=race, site=site, a=
30   voucher, z=move, y=inschoola)
31
32 wmat<-data.frame(w1=dat$w1, w2=dat$w2, w3=dat$w3)
33
34 amodel<-"a ~ 1"
35 sitemodel<-"site ~ w1 + w2 + w3 "
36 zmodel<-"z ~ a + w2 + w3 "
37 outmodel<-"y ~ z + w1 +w3 + z:w1"
38 outmodelnoz<-"y ~ a + w1+w3+ a:w1"
39 q2model<-"w1 + w2 + w3 "
40
41 ittatetmletransportest<-ittatetmle(a=dat$a, z=dat$z, y=dat$y, site=dat$site
42   , w=wmat, aamodel=amodel, asitemodel=sitemodel, azmodel=zmodel,
43   aoutmodel=outmodel, aq2model=q2model)$est
44 catetmletransportest<-catetmle(ca=dat$a, cz=dat$z, cy=dat$y, csite=dat$site
45   , cw=wmat, csitemodel=sitemodel, czmodel=zmodel, coutmodel=outmodel,
46   cq2model=q2model)$est
47 eatetmletransportest<-eatetmle(a=dat$a, z=dat$z, y =dat$y, site=dat$site, w
48   =wmat, nsitemodel=sitemodel, nzmodel=zmodel, noutmodel=outmodel)$est

```

examp.R

## Bibliography

- Gill, R. D., Wellner, J. A. and Præstgaard, J. (1989) Non-and semi-parametric maximum likelihood estimators and the von mises method (part 1)[with discussion and reply]. *Scandinavian Journal of Statistics*, 97–128.



Van der Laan, M. J. and Robins, J. M. (2003) *Unified methods for censored longitudinal data and causality*. Springer.