Supplementary: On Validation and Planning of An Optimal Decision Rule with Application in Healthcare Studies

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A. Technical Proofs

A.1. Proof of Lemma 1

(A.) First, we show that $\widehat{\beta}$ converges in probability to β_0 as $n \to \infty$, by checking three conditions of the Argmax Theorem:

(a1.) By the regularity condition (C4) that the true value function $V(\beta)$ has twice continuously differentiable at an inner point of maximum β_0.

(a2.) By the consistency conclusion of Zhang et al. (2012) that $\widehat{V}(\beta) = V(\beta) + o_p(1)$, i.e., for $\forall \beta$

$$\widehat{V}(\beta) \xrightarrow{p} V(\beta), \quad \text{as} \quad n \to \infty.$$

(a3.) Since $\widehat{\beta} = \arg\max_{\beta} \widehat{V}(\beta)$, we have the estimated ODR as $d(X, \widehat{\beta}) = I(\phi_X(X)^\top \widehat{\beta} > 0)$ and the corresponding value function $\widehat{V}(\widehat{\beta})$ such that

$$\widehat{V}(\widehat{\beta}) \geq sup_{\beta \in \mathrm{B}} \widehat{V}(\beta).$$

Thus, we have $\widehat{\beta} \xrightarrow{p} \beta_0$ as $n \to \infty$.

(B.) Next, we show that the convergence rate of $\widehat{\beta}$ is $n^{1/3}$, i.e. $n^{1/3}||\widehat{\beta} - \beta_0||_2 = O_p(1)$, where $|| \cdot ||_2$ is L_2 norm, via checking three conditions of the Theorem 14.4: Rate of convergence in (Kosorok, 2008). Here, we first proof the result for the inverse probability weighted estimator, which can be trivially extended to the augmented inverse probability weighted estimator.

(b1.) For every β in a neighborhood of β_0, i.e. $||\beta - \beta_0||_2 < \delta$, by Assumption (10), we take the second order Taylor expansion of $V(\beta)$ at $\beta = \beta_0$,

$$\begin{aligned} V(\beta) - V(\beta_0) =& V'(\beta_0)||\beta - \beta_0||_2 \\ &+ \frac{1}{2} V''(\beta_0)||\beta - \beta_0||_2^2 + o\{||\beta - \beta_0||_2^2\} \\ (\text{by } V'(\beta_0) = 0) =& \frac{1}{2} V''(\beta_0)||\beta - \beta_0||_2^2 + o\{||\beta - \beta_0||_2^2\}. \end{aligned}$$

Since $V''(\beta_0) < 0$, there exist $c_1 = -\frac{1}{2}V''(\beta_0) > 0$ such that $V(\beta) - V(\beta_0) \leq c_1||\beta - \beta_0||_2^2$ holds.

(b2.) First, define

$$f_i(\beta) = \frac{I\{A_i = d(X_i, \beta)\}}{\pi A_i + (1-\pi)(1-A_i)} Y_i.$$

With the fact that

$$\begin{aligned} & I\{A_i = d(X_i, \beta)\} - I\{A_i = d(X_i, \beta_0)\} \\ =& A_i I(\phi_X(X_i)^\top \beta > 0) + (1 - A_i)\{1 - I(\phi_X(X_i)^\top \beta > 0)\} \\ & - [A_i I(\phi_X(X_i)^\top \beta_0 > 0) + (1 - A_i)\{1 - I(\phi_X(X_i)^\top \beta_0 > 0)\}] \\ =& (2A_i - 1)\{I(\phi_X(X_i)^\top \beta > 0) - I(\phi_X(X_i)^\top \beta_0 > 0)\}, \end{aligned} \tag{1}$$

we have,

$$\begin{aligned} & \widehat{V}(\beta) - \widehat{V}(\beta_0) = \frac{1}{n} \sum_{i=1}^n \{f_i(\beta) - f_i(\beta_0)\} \\ =& \frac{1}{n} \sum_{i=1}^n \frac{Y_i\{I(A_i = d(X_i; \beta)) - I(A_i = d(X_i; \beta_0))\}}{\pi A_i + (1-\pi)(1-A_i)} \\ =& \frac{1}{n} \sum_{i=1}^n \frac{Y_i(2A_i - 1)\{I(\phi_X(X_i)^\top \beta > 0) - I(\phi_X(X_i)^\top \beta_0 > 0)\}}{\pi A_i + (1-\pi)(1-A_i)\}}. \end{aligned} \tag{2}$$

Then, we define a class of function $\mathcal{F}_\beta^1(x, a, y) = \left\{ \frac{y(2a-1)}{a\pi + (1-a)\{1-\pi\}} \{(I(\phi_X(x)^\top \beta > 0) - I(\phi_X(x)^\top \beta_0 > 0)\} : ||\beta - \beta_0||_2 < \delta \right\}$.

Let $M_1 = \sup \left| \frac{y(2a-1)}{a\pi + (1-a)\{1-\pi\}} \right|$, by the regularity condition (C1) that Y is bounded, we have $M_1 < \infty$. Then, we define

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the envelope of $\mathcal{F}_\beta^1$ as $F_1 = M_1 \cdot I(1-\delta \le \phi_X(x)^\top \beta_0 \le 1+\delta)$; by by Assumption (6) that the density function of covariate $f_X(x)$ is bounded away from 0 and ∞, thus,

$$
\begin{aligned}
||F_1||_{P,2} =& M_1 \sqrt{P(1-\delta \le \phi_X(x)^\top \beta_0 \le 1+\delta)} \\
=& M_1 \sqrt{f_X(\beta_0) \cdot 2\delta} = M_1 \sqrt{2 f_X(\beta_0)} \delta^{\frac{1}{2}} < \infty.
\end{aligned}
$$

Since $\mathcal{F}_\beta^1$ is an indicate function, by the conclusion of the Lemma 2.6.15 and Lemma 2.6.18 (iii) in (Wellner et al., 2013), $\mathcal{F}_\beta^1$ is a VC (and hence Donsker) class of functions. Thus, the entropy of the class function $\mathcal{F}_\beta^1$ denoted as $J_{[]}^*(1, \mathcal{F}^1)$ is finite, i.e., $J_{[]}^*(1, \mathcal{F}^1) < \infty$.

Next, we consider the following empirical process indexed by β,

$$
\mathbb{G}_n \mathcal{F}_\beta^1 = \frac{1}{\sqrt{n}} \sum_{i=1}^n \{\mathcal{F}_\beta^1(X_i, A_i, Y_i) - \mathrm{E}\mathcal{F}_\beta^1(X_i, A_i, Y_i)\}.
$$

Note that $\mathbb{G}_n \mathcal{F}_\beta^1 = \sqrt{n}[\widehat{V}(\beta) - \widehat{V}(\beta_0) - \{V(\beta) - V(\beta_0)\}]$ by Equation (2). Therefore, by applying Theorem 11.2 in (Kosorok, 2008), we have,

$$
\begin{aligned}
& E^* \sup_{||\beta-\beta_0||_2 < \delta} \sqrt{n} \Big| \widehat{V}(\beta) - V(\beta) - \{\widehat{V}(\beta_0) - V(\beta_0)\} \Big| \\
=& E^* \sup_{||\beta-\beta_0||_2 < \delta} \Big| \mathbb{G}_n \mathcal{F}_\beta^1 \Big| \le c_1 J_{[]}^*(1, \mathcal{F}^1) ||F_1||_{P,2} \\
=& c_1 J_{[]}^*(1, \mathcal{F}^1) M_1 \sqrt{2 f_X(\beta_0)} \delta^{\frac{1}{2}},
\end{aligned}
$$

where E^* is the outer expectation, and c_1 is a finite constant.

Let $C_1^* \equiv c_1 J_{[]}^*(1, \mathcal{F}^1) M_1 \sqrt{2 f_X(\beta_0)}$, since $J_{[]}^*(1, \mathcal{F}^1)$, M_1, and $f_X(\cdot)$ are bounded, we have $C_1^* < \infty$.

Thus, for all n large enough and sufficiently small δ, the centered process $\widehat{V} - V$ satisfies

$$
\begin{aligned}
& E^* \sup_{||\beta-\beta_0||_2 < \delta} \sqrt{n} |\widehat{V}(\beta) - V(\beta) - \{\widehat{V}(\beta_0) - V(\beta_0)\}| \\
\le & C_1^* \delta^{\frac{1}{2}}.
\end{aligned}
$$

Let $\phi_n(\delta) = C_1^* \delta^{\frac{1}{2}}$, and $\alpha = \frac{3}{2} < 2$, check $\frac{\phi_n(\delta)}{\delta^\alpha} = \frac{\delta^{\frac{1}{2}}}{\delta^{\frac{3}{2}}} = \delta^{-1}$ is decreasing not depending on n. Therefore, condition B holds.

(b3.) By $\widehat{\beta} \xrightarrow{p} \beta_0$ as $n \to \infty$ and $\widehat{V}(\widehat{\beta}) \ge sup_{\beta \in \mathrm{B}} \widehat{V}(\beta)$ shown previously, choose $r_n = n^{1/3}$, then r_n satisfies

$$
\begin{aligned}
& r_n^2 \phi_n(r_n^{-1}) = n^{2/3} \phi_n(n^{-1/3}) \\
=& n^{2/3} (n^{-1/3})^{1/2} = n^{2/3 - 1/6} = n^{1/2}.
\end{aligned}
$$

Thus, condition C holds.

By the Theorem 14.4 in (Kosorok, 2008), we have $n^{1/3} ||\widehat{\beta} - \beta_0||_2 = O_p(1)$.

□

A.2. Proof of Proposition 1

To show

$$
\sqrt{n} \{\widehat{V}(\widehat{\beta}) - \widehat{V}(\beta_0)\} = o_p(1),
$$

is sufficient to show $\sqrt{n}\{V(\widehat{\beta}) - V(\beta_0)\} = o_p(1)$ and $\sqrt{n} \Big[\{\widehat{V}(\widehat{\beta}) - \widehat{V}(\beta_0)\} - \{V(\widehat{\beta}) - V(\beta_0)\} \Big] = o_p(1)$.

(a1.) First, by $n^{1/3} ||\widehat{\beta} - \beta_0||_2 = O_p(1)$ and the regularity condition (C4), we take the second order Taylor expansion of $V(\widehat{\beta})$ at β_0, then

$$
\begin{aligned}
& \sqrt{n} \{V(\widehat{\beta}) - V(\beta_0)\} \\
=& \sqrt{n} \big[V'(\beta_0) ||\widehat{\beta} - \beta_0||_2 + \frac{1}{2} V''(\beta_0) ||\widehat{\beta} - \beta_0||_2^2 \\
& + o_p\{||\widehat{\beta} - \beta_0||_2^2\} \big] \\
& (\text{by } V'(\beta_0) = 0) = \sqrt{n} \{ \frac{1}{2} V''(\beta_0) O_E(n^{-\frac{2}{3}}) + o_p(n^{-\frac{2}{3}}) \} \\
=& \frac{1}{2} V''(\beta_0) O_E(n^{-\frac{1}{6}}) = o_p(1).
\end{aligned} \tag{3}
$$

(a2.) Next, recall the result in the proof of Lemma 1 that

$$
E^* \sup_{||\beta-\beta_0||_2 < \delta} \sqrt{n} |\widehat{V}(\beta) - V(\beta) - \{\widehat{V}(\beta_0) - V(\beta_0)\}| \le C_1^* \delta^{\frac{1}{2}},
$$

where C_1^* is a finite constant. Since $||\widehat{\beta} - \beta_0||_2 = O_p(n^{-1/3})$, i.e., $||\widehat{\beta} - \beta_0||_2 = c_4 n^{-1/3}$, where c_4 is a finite constant, we have,

$$
\begin{aligned}
& \sqrt{n} \Big[\{\widehat{V}(\widehat{\beta}) - \widehat{V}(\beta_0)\} - \{V(\beta) - V(\beta_0)\} \Big] \\
\le & E^* \sup_{||\beta-\beta_0||_2 < c_4 n^{-1/3}} \sqrt{n} |\widehat{V}(\beta) - V(\beta) - \{\widehat{V}(\beta_0) - V(\beta_0)\}| \\
\le & C_1^* \sqrt{c_4 n^{-1/3}} = C_1^* \sqrt{c_4} n^{-1/6} = o_p(1).
\end{aligned} \tag{4}
$$

(a3.) Thus, from the results of (3) and (4), we have,

$$
\begin{aligned}
& \sqrt{n} \{\widehat{V}(\widehat{\beta}) - \widehat{V}(\beta_0)\} \\
=& \sqrt{n} \Big[\{\widehat{V}(\widehat{\beta}) - \widehat{V}(\beta_0)\} - \{V(\widehat{\beta}) - V(\beta_0)\} \Big] \\
& + \sqrt{n} \{V(\widehat{\beta}) - V(\beta_0)\} \\
=& o_p(1) + o_p(1) = o_p(1).
\end{aligned}
$$

□

A.3. Proof of Theorem 1: Asymptotic distribution of the test statistic under H_0

Under H_0, we have $d(X, \beta_0) \equiv 1$ and $V(\beta_0) = V_1$. Based on Lemma 1 and Proposition 1, we have

$$
\begin{aligned}
\widehat{\Delta}_n =& \sqrt{n}\{\widehat{V}(\widehat{\beta}) - \widehat{V}_1\} \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \widehat{\beta})\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \widehat{\mu}(X_i, \widehat{\beta})\} \\
&+ \widehat{\mu}(X_i, \widehat{\beta}) - \frac{A_i Y_i}{\pi}\Bigg] \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - \frac{A_i Y_i}{\pi}\Bigg] + o_p(1) \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = 1\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu_1(X_i)\} \\
&+ \mu_1(X_i) - \frac{A_i Y_i}{\pi}\Bigg] + o_p(1) \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{A_i}{\pi}\{Y_i - \mu_1(X_i)\} + \mu_1(X_i) - \frac{A_i Y_i}{\pi}\Bigg] + o_p(1) \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\left\{\frac{A_i}{\pi} - 1\right\}\mu_1(X_i) + o_p(1).
\end{aligned}
$$

By Central Limit Theorem, we have $\widehat{\Delta}_n$ converges in distribution to a normal random variable with mean 0 and variance $\sigma_0^2 = \frac{1-\pi}{\pi} Var\{E(Y|A=1, X)\}$.

□

A.4. Proof of Theorem 2: Asymptotic distribution of the test statistic under $H_{a,n}$

Under $H_{a,n}$, we have $\sqrt{n}\{V(\beta_0) - V_1\} = \Delta$. Based on Lemma 1 and Proposition 1, we have

$$
\begin{aligned}
\widehat{\Delta}_n =& \frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - \frac{A_i Y_i}{\pi}\Bigg] + o_p(1) \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - V(\beta_0) - \frac{A_i Y_i}{\pi} + V_1\Bigg] \\
&+ \sqrt{n}\{V(\beta_0) - V_1\} + o_p(1) \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - V(\beta_0) - \left(\frac{A_i}{\pi} Y_i - V_1\right)\Bigg] \\
&+ \Delta + o_p(1) \\
=&\Delta + \frac{1}{\sqrt{n}}\sum_{i=1}^{n}\phi_i + o_p(1),
\end{aligned}
$$

where

$$
\begin{aligned}
\phi_i =& \frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - V(\beta_0) - \left(\frac{A_i}{\pi} Y_i - V_1\right).
\end{aligned}
$$

Therefore, $\widehat{\Delta}$ converges in distribution to a random random variable with mean Δ and variance $\sigma_\phi^2 = E(\phi_i^2)$.

□

A.5. The degenerate distribution of $\sqrt{n}\{\widehat{V}(\widehat{\beta}) - \widehat{V}^1\}$ under H_0

Following the proof of Theorem 1, by replacing $\widehat{V}_1$ with $\widehat{V}^1$, we have

$$
\begin{aligned}
\widehat{\Delta}_n =& \sqrt{n}\{\widehat{V}(\widehat{\beta}) - \widehat{V}^1\} \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \widehat{\beta})\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \widehat{\mu}(X_i, \widehat{\beta})\} \\
&+ \widehat{\mu}(X_i, \widehat{\beta}) - \frac{A_i}{\pi}\{Y_i - \widehat{\mu}_1(X_i)\} - \widehat{\mu}_1(X_i)\Bigg] \\
&\text{(By Lemma 1 and Proposition 1)} \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = d(X_i, \beta_0)\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu(X_i, \beta_0)\} \\
&+ \mu(X_i, \beta_0) - \frac{A_i}{\pi}\{Y_i - \mu_1(X_i)\} - \mu_1(X_i)\Bigg] + o_p(1) \\
&\text{(Under } H_0\text{, we have } d(X, \beta_0) \equiv 1\text{)} \\
=&\frac{1}{\sqrt{n}}\sum_{i=1}^{n}\Bigg[\frac{I\{A_i = 1\}}{\pi A_i + (1-\pi)(1-A_i)}\{Y_i - \mu_1(X_i)\} \\
&+ \mu_1(X_i) - \frac{A_i}{\pi}\{Y_i - \mu_1(X_i)\} - \mu_1(X_i)\Bigg] + o_p(1) \\
&\text{(With all the terms cancel out, we have)} = o_p(1).
\end{aligned}
$$

Therefore, under the null and the regular assumption, $\sqrt{n}\{\widehat{V}(\widehat{\beta}) - \widehat{V}^1\}$ asymptotically converges in distribution to 0. One may also conclude that the IPW estimators for $\widehat{\beta}$ and the naive rule are asymptotically identical under the null by a similar proof.

Table S1. Simulation results of the proposed test under the Nelder-Mead Method.

		$h = 0.5$		$h = 1$	
SCEN.	RESULTS	$\gamma = 1$	$\gamma = 2$	$\gamma = 1$	$\gamma = 2$
1	V_1	3.00	5.00	4.00	6.00
	$V(\beta_0)$	3.00	5.00	4.00	6.00
	ERR.	5.4%	5.4%	5.4%	5.4%
2	V_1	2.00	3.00	3.00	4.00
	$V(\beta_0)$	2.04	3.08	3.04	4.08
	POW.	19.2%	25.8%	14.4%	19.2%
	$\widehat{\beta}_1$	0.667	0.615	0.663	0.610
	$\widehat{\beta}_2$	0.042	0.018	0.038	0.015
	$\widehat{\beta}_3$	0.026	0.027	0.037	0.022
	$\widehat{\beta}_4$	0.543	0.570	0.545	0.577
	$\widehat{\beta}_5$	-0.507	-0.544	-0.511	-0.543
3	V_1	1.50	2.00	2.50	3.00
	$V(\beta_0)$	1.64	2.28	2.64	3.28
	POW.	92.4%	99.4%	59.8%	89.6%
	$\widehat{\beta}_1$	0.340	0.332	0.344	0.336
	$\widehat{\beta}_2$	0.002	-0.001	0.007	0.001
	$\widehat{\beta}_3$	0.010	0.003	0.006	-0.004
	$\widehat{\beta}_4$	0.628	0.652	0.630	0.647
	$\widehat{\beta}_5$	-0.700	-0.681	-0.696	-0.685

Table S2. Simulation results of the proposed test under the Simulated Annealing.

		$h = 0.5$		$h = 1$	
SCEN.	RESULTS	$\gamma = 1$	$\gamma = 2$	$\gamma = 1$	$\gamma = 2$
1	V_1	3.00	5.00	4.00	6.00
	$V(\beta_0)$	3.00	5.00	4.00	6.00
	ERR.	5.4%	5.8%	5.2%	5.4%
2	V_1	2.00	3.00	3.00	4.00
	$V(\beta_0)$	2.04	3.08	3.04	4.08
	POW.	19.0%	23.6%	12.2%	17.2%
	$\widehat{\beta}_1$	0.599	0.593	0.597	0.590
	$\widehat{\beta}_2$	-0.011	-0.005	-0.003	-0.002
	$\widehat{\beta}_3$	-0.010	0.001	0.007	0.010
	$\widehat{\beta}_4$	0.566	0.570	0.566	0.572
	$\widehat{\beta}_5$	-0.566	-0.569	-0.568	-0.570
3	V_1	1.50	2.00	2.50	3.00
	$V(\beta_0)$	1.64	2.28	2.64	3.28
	POW.	85.8%	97.8%	51.4%	85.8%
	$\widehat{\beta}_1$	0.359	0.351	0.356	0.350
	$\widehat{\beta}_2$	-0.007	0.006	-0.009	0.006
	$\widehat{\beta}_3$	-0.004	0.003	0.009	0.001
	$\widehat{\beta}_4$	0.656	0.659	0.663	0.661
	$\widehat{\beta}_5$	-0.664	-0.665	-0.659	-0.664

B. Additional Results

B.1. Testing and evaluation with linear decision rule under the Nelder-Mead Method.

B.2. Testing and evaluation with linear decision rule under the Simulated Annealing.

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