Supplementary Material

1. Proof of Theorem 1:

Our goal is to derive the formula for least square estimate of regression coefficient for W (i.e., $\hat{\beta}_W^{id}$) based on the ideal sample and the variance of $\hat{\beta}_W^{id}$, for observational studies. First, one needs to define the following ordered data matrices for the ideal sample:

$$
\begin{aligned}
&\mathbf{D} = [\mathbf{Y}_{(n^{un}+n^{ob})\times 1}, \mathbf{X}_{(n^{un}+n^{ob})\times(p+2)}] = [\mathbf{Y}_{2n^{ob}\times 1}, \mathbf{X}_{2n^{ob}\times(p+2)}] \\
&\mathbf{X} = [\mathbf{1}_{(n^{un}+n^{ob})\times 1}, \mathbf{V}_{(n^{un}+n^{ob})\times(p+1)}] = [\mathbf{1}_{2n^{ob}\times 1}, \mathbf{V}_{2n^{ob}\times(p+1)}] \\
&\mathbf{V} = [\mathbf{Z}_{(n^{un}+n^{ob})\times p}, \mathbf{W}_{(n^{un}+n^{ob})\times 1}] = \begin{bmatrix} \mathbf{Z}^{ob}_{n^{ob}\times p}, \mathbf{1}_{n^{ob}\times 1} - \mathbf{W}^{ob}_{n^{ob}\times 1} \\ \mathbf{Z}^{ob}_{n^{ob}\times p}, \mathbf{W}^{ob}_{n^{ob}\times 1} \end{bmatrix} \\
&\mathbf{Z} = [\mathbf{Z_1}, \mathbf{Z_2}, ..., \mathbf{Z_p}]_{(n^{un}+n^{ob})\times p} = [\mathbf{Z_1}, \mathbf{Z_2}, ..., \mathbf{Z_p}]_{2n^{ob}\times p}
\end{aligned}
\tag{S1}
$$

and the following ordered mean vectors:

$$
\begin{aligned}
\overline{\mathbf{V}}^{\mathbf{id}} &= [\overline{\mathbf{Z}}^{\mathbf{id}}, \overline{W}^{id}]_{1\times(p+1)} \\
\overline{\mathbf{Z}}^{\mathbf{id}} &= [\overline{Z}_1^{id}, \overline{Z}_2^{id}, \cdots, \overline{Z}_p^{id}]_{1\times p}
\end{aligned}
\tag{S2}
$$

The matrix $\mathbf{X}^{\mathbf{T}}\mathbf{X}$ for the ideal sample could then be modeled as the following block matrix:

$$
\mathbf{X}^{\mathbf{T}}\mathbf{X} = \begin{pmatrix} n^{un} + n^{ob} & (n^{un} + n^{ob})\overline{\mathbf{V}}^{\mathbf{id}} \\ (n^{un} + n^{ob})(\overline{\mathbf{V}}^{\mathbf{id}})^{\mathbf{T}} & \mathbf{V}^{\mathbf{T}}\mathbf{V} \end{pmatrix}
\tag{S3}
$$

The inverse of $\mathbf{X}^{\mathbf{T}}\mathbf{X}$ can be shown to have the following form:

$$\mathbf{(X^T X)^{-1}} = \begin{pmatrix} \frac{1}{n^{un}+n^{ob}} + \mathbf{\bar{V}^{id}} \frac{1}{n^{un}+n^{ob}} \mathbf{(S^{id}_{VV})^{-1}(\bar{V}^{id})^T} & -\frac{1}{n^{un}+n^{ob}} \mathbf{\bar{V}^{id}(S^{id}_{VV})^{-1}} \\ -\frac{1}{n^{un}+n^{ob}} \mathbf{(S^{id}_{VV})^{-1}(\bar{V}^{id})^T} & \frac{1}{n^{un}+n^{ob}} \mathbf{(S^{id}_{VV})^{-1}} \end{pmatrix}$$

(S4)

It should be clear now that, to determine the definite form of $\mathbf{(X^T X)^{-1}}$ I need to find out what $\mathbf{(S^{id}_{VV})^{-1}}$ is. As a variance-covariance matrix for the vector of predictors V, $\mathbf{S^{id}_{VV}}$ can be expressed as the block matrix whose elements is formalized below:

$$\mathbf{S^{id}_{VV}} = \begin{pmatrix} \mathbf{S^{id}_{ZZ}} & \mathbf{S^{id}_{ZW}} \\ \mathbf{S^{id}_{WZ}} & \hat{\sigma}^{id}_{WW} \end{pmatrix}_{(p+1)\times(p+1)} \tag{S5}$$

where:

$$\mathbf{S^{id}_{ZZ}} = \begin{pmatrix} \hat{\sigma}^{id}_{Z_1Z_1} & \cdots & \hat{\sigma}^{id}_{Z_1Z_p} \\ \vdots & \ddots & \vdots \\ \hat{\sigma}^{id}_{Z_pZ_1} & \cdots & \hat{\sigma}^{id}_{Z_pZ_p} \end{pmatrix}_{p\times p}$$

$$\mathbf{S^{id}_{ZW}} = \begin{pmatrix} \hat{\sigma}^{id}_{Z_1W} \\ \vdots \\ \hat{\sigma}^{id}_{Z_pW} \end{pmatrix}_{p\times 1} \tag{S6}$$

$$\mathbf{S^{id}_{WZ}} = \begin{pmatrix} \hat{\sigma}^{id}_{Z_1W} & \cdots & \hat{\sigma}^{id}_{Z_pW} \end{pmatrix}_{1\times p}$$

Furthermore, we define the following covariance vector:

$$\mathbf{S}_{\mathbf{ZY}}^{\mathbf{id}} = \begin{pmatrix} \hat{\sigma}_{Z_1 Y}^{id} \\ \vdots \\ \hat{\sigma}_{Z_p Y}^{id} \end{pmatrix}_{p\times 1} \tag{S7}$$

All aforementioned sample covariances and variances are supposed to be computed according to the following formula:

$$\begin{aligned} \hat{\sigma}_{xy} &= \frac{1}{n}\sum_{i=1}^{n}(x_i - \overline{x})(y_i - \overline{y}) \\ \hat{\sigma}_{xx} &= \frac{1}{n}\sum_{i=1}^{n}(x_i - \overline{x})^2 \end{aligned} \tag{S8}$$

for any variable x or y and any sample size n in this context.

Consequently, the inverse of $\mathbf{S}_{\mathbf{VV}}^{\mathbf{id}}$ can be formulated here:

$$(\mathbf{S}_{\mathbf{VV}}^{\mathbf{id}})^{-1} =$$

$$\begin{bmatrix} (\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1} + (\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}}(\sigma_{WW}^{id} - \mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1} & -(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}}(\sigma_{WW}^{id} - \mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}})^{-1} \\ -(\sigma_{WW}^{id} - \mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1} & (\sigma_{WW}^{id} - \mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}}(\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1}\mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}})^{-1} \end{bmatrix}$$

(S9)

Plugging the above matrix of $(\mathbf{S}_{\mathbf{VV}}^{\mathbf{id}})^{-1}$ into the block matrix of $(\mathbf{X}^{\mathrm{T}}\mathbf{X})^{-1}$ will give us the complete definite form of matrix $(\mathbf{X}^{\mathrm{T}}\mathbf{X})^{-1}$, whose elements are all ideal sample statistics such as ideal sample variances, ideal sample covariances and ideal sample means. To isolate the estimated regression coefficient for W, I only need to use the elements in the last row of $(\mathbf{X}^{\mathrm{T}}\mathbf{X})^{-1}$, which are provide next:

$$(\mathbf{X^T X})^{\mathbf{-1}}{}_{(p+2)1} = \frac{1}{n^{un}+n^{ob}}[(\hat{\sigma}^{id}_{WW} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZW}})^{\mathbf{-1}}\mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}(\bar{\mathbf{Z}}^{\mathbf{id}})^{\mathbf{T}} - \bar{W}^{id}(\hat{\sigma}^{id}_{WW} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZW}})^{\mathbf{-1}}]$$

$$[(\mathbf{X^T X})^{\mathbf{-1}}{}_{(p+2)2}, \cdots, (\mathbf{X^T X})^{\mathbf{-1}}{}_{(p+2)(p+1)}] = -\frac{1}{n^{un}+n^{ob}}(\hat{\sigma}^{id}_{WW} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZW}})^{\mathbf{-1}}\mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}$$

$$(\mathbf{X^T X})^{\mathbf{-1}}{}_{(p+2)(p+2)} = \frac{1}{n^{un}+n^{ob}}(\hat{\sigma}^{id}_{WW} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZW}})^{\mathbf{-1}}$$

(S10)

Because the estimated regression coefficient for W is the last element of $(\mathbf{X^T X})^{\mathbf{-1}}\mathbf{X^T Y}$ which is the dot product between the last row of $(\mathbf{X^T X})^{\mathbf{-1}}$ and $\mathbf{X^T Y}$, the expression of $\mathbf{X^T Y}$ is also needed here:

$$\mathbf{X^T Y} = \begin{bmatrix} (n^{un}+n^{ob})\bar{Y}^{id} \\ \mathbf{Z^T Y} \\ \mathbf{W^T Y} \end{bmatrix} \tag{S11}$$

where:

$$\begin{aligned} \mathbf{Z^T Y} &= (n^{un}+n^{ob})\mathbf{S^{id}_{ZY}} + (n^{un}+n^{ob})\bar{Y}^{id}(\bar{\mathbf{Z}}^{\mathbf{id}})^{\mathbf{T}} \\ \mathbf{W^T Y} &= (n^{un}+n^{ob})\hat{\sigma}^{id}_{WY} + (n^{un}+n^{ob})\bar{W}^{id}\bar{Y}^{id} \end{aligned} \tag{S12}$$

Now one can calculate the estimated regression coefficient for W as the dot product between the last row of $(\mathbf{X^T X})^{\mathbf{-1}}$ and the vector $\mathbf{X^T Y}$. The result is presented below:

$$\hat{\beta}^{id}_{W} = \frac{\hat{\sigma}^{id}_{WY} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZY}}}{\hat{\sigma}^{id}_{WW} - \mathbf{S^{id}_{WZ}}(\mathbf{S^{id}_{ZZ}})^{\mathbf{-1}}\mathbf{S^{id}_{ZW}}} \tag{S13}$$

The variance of $\hat{\beta}_W^{id}$ should be straightforward: it is just the product of the known residual variance σ^2 and the element in the (p+2)th row and the (p+2)th column of $(\mathbf{X}^T\mathbf{X})^{-1}$:

$$Var(\hat{\beta}_W^{id}) = \frac{\sigma^2}{n^{un} + n^{ob}} (\hat{\sigma}_{WW}^{id} - \mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}} (\mathbf{S}_{\mathbf{ZZ}}^{\mathbf{id}})^{-1} \mathbf{S}_{\mathbf{ZW}}^{\mathbf{id}})^{-1} \tag{S14}$$

We note here that $\boldsymbol{S}_{\boldsymbol{WZ}}^{\boldsymbol{id}}$ appears in (A13) and (A14) should be equal to **0** as the correlation between the treatment indicator W and any single covariate in **Z** should be exactly 0, given that each subject in the ideal sample should be thought to participate in both the treatment and control group (which means the conditions $W = 1$ and $W = 0$ appear in every subject). As a result, the expressions of (A13) and (A14) can be greatly simplified for the ideal sample, given $\mathbf{S}_{\mathbf{WZ}}^{\mathbf{id}} = \mathbf{0}$ and $n^{un} = n^{ob}$:

$$\hat{\beta}_W^{id} = r_{wy}^{id} \tag{S15}$$

$$Var(\hat{\beta}_W^{id}) = \frac{1-R^2}{2n^{ob}} \tag{S16}$$

where r_{wy}^{id} denotes the correlation between the treatment status (*W*) and the outcome (*Y*) in the ideal sample. The expression of $\hat{\beta}_W^{id}$ can be further derived based on Cohen et al. (2003):

$$\hat{\beta}_W^{id} = \left(\frac{\bar{Y}_t^{id} - \bar{Y}_c^{id}}{\sigma_Y^{id}}\right) 0.5 \tag{S17}$$

The expression for σ_Y^{id} is derived as:

$$
\begin{aligned}
\sigma_Y^{id} &= \sqrt{0.5[\sigma_t^2 + \pi(1-\pi)(\overline{Y}_t^{un} - \overline{Y}_t^{ob})^2] + 0.5[\sigma_c^2 + \pi(1-\pi)(\overline{Y}_c^{un} - \overline{Y}_c^{ob})^2] + 0.25(\overline{Y}_t^{id} - \overline{Y}_c^{id})^2} \\
&= \sqrt{0.5\sigma_t^2 + 0.5\pi(1-\pi)[(\overline{Y}_t^{un} - \overline{Y}_t^{ob})^2 + (\overline{Y}_c^{un} - \overline{Y}_c^{ob})^2] + 0.5\sigma_c^2 + 0.25(\overline{Y}_t^{id} - \overline{Y}_c^{id})^2}
\end{aligned}
$$

(S18)

Combining (A17) and (A18), $\hat{\beta}_W^{id}$ is written as follows:

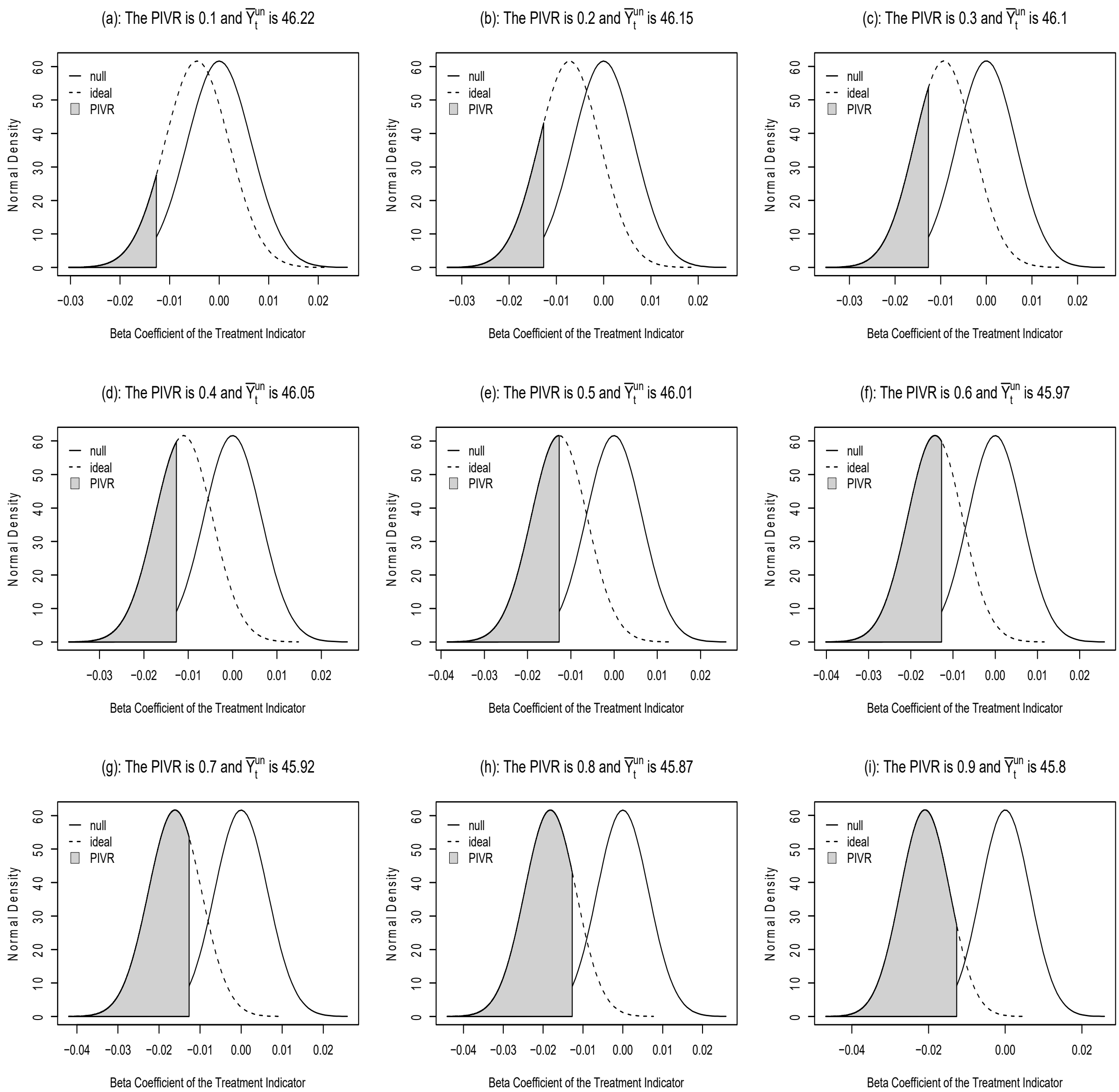
$$
\hat{\beta}_W^{id} = \frac{\bar{Y}_t^{id} - \bar{Y}_c^{id}}{\sqrt{2\hat{\sigma}_t^2 + 2\pi(1\text{-}\pi)[(\bar{Y}_t^{un} - \bar{Y}_t^{ob})^2 + (\bar{Y}_c^{un} - \bar{Y}_c^{ob})^2] + 2\hat{\sigma}_c^2 + (\bar{Y}_t^{id} - \bar{Y}_c^{id})^2}} \tag{S19}
$$

where:

$$
\begin{aligned}
\overline{Y}_t^{id} &= (1-\pi)\overline{Y}_t^{un} + \pi\overline{Y}_t^{ob} \\
\overline{Y}_c^{id} &= \pi\overline{Y}_c^{un} + (1-\pi)\overline{Y}_c^{ob}
\end{aligned} \tag{S20}
$$

Given the expression of $\hat{\beta}_W^{id}$ in (A19) and the expression of $Var(\hat{\beta}_W^{id})$ in (A16), the probit functions of the PIV can be easily derived based on the definitions (2) and (3) in the main text. This concludes the proof of theorem 1.

2. Supplementary Material Figure S1:



Supplementary Material Figure S1: The relationship between the PIVR and retesting the null hypothesis based on the ideal sample for Hong and Raudenbush (2005), assuming $\bar{Y}_c^{un} = 45.2$. The solid curve represents the null hypothesis: $\beta_W = 0$ and the dashed curve represents the alternative hypothesis: $\beta_W = \hat{\beta}_W^{id}$. The grey shaded area symbolizes the PIVR of Hong and Raudenbush (2005).