# Different Measures of Average Treatment Effect for Binary Outcome, Estimating by Propensity Scoring

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## **Questions**??

- How to determine whether a treatment/risk factor is on average effective in reducing/increasing disease risk, in a large observational study with a lot of observed confounders?
  Example
- **Z**: **binary** treatment indicator.
- Y: binary outcome.
- X: covariate.
- $Y_i^{(0,1)}$ : potential outcome
- Population average risk:

$$P^{(1)} = \frac{1}{N} \sum_{i=1}^{N} Pr(Y_i^{(1)} = 1)$$

$$P^{(0)} = \frac{1}{N} \sum_{i=1}^{N} Pr(Y_i^{(0)} = 1)$$

- Smoking status
- Lung Cancer
- Age
- Potential cancer status if I ... or not ..
- Population average cancer risk

## **Propensity Scoring (P.S.)**

- Define:  $\mathbf{e}_{\mathbf{T}}(\mathbf{X}) = \mathbf{P}_{\mathbf{r}}(\mathbf{Z}=1|\mathbf{X})$ (e.g. the risk of being smoking at giving age.)
- $e_T(x)$  is a balancing score  $\rightarrow X \coprod Z \mid e_T(x)$ . (e.g. for people with same  $e_T(X)$ , the distribuion of age is same across smoking groups.)
- Typical tool for studying causal inference.
  - $\Box$  The marginal inference of Z to Y (average over X).
- Two conditions for valid causal inference:
  - 1). Treatment assignment is strongly ignorable
  - 2). Close to correctly specified: Z relationship to X.

## P.S. Procedure

- 1. Estimate e(x).
- 2. Take subjects with overlapped e(x) after ordering.
- 3. Subclassification of e(x) into bins.
- 4. If  $f_{X|Z=1, jth bin} \approx f_{X|Z=0, jth bin}$ , hold for all Xs within all subclasses, then move on; o.w, back to step-1.

5. 
$$\widehat{P_j^{(1)}} = average(Y|Z=1, j^{th} \text{ bin})$$
  $\widehat{P^{(1)}} = \sum_{j=1}^{J} \omega_j \widehat{P_j^{(1)}}$   $\widehat{P_j^{(0)}} = average(Y|Z=0, j^{th} \text{ bin})$   $\widehat{P^{(0)}} = \sum_{j=1}^{J} \omega_j \widehat{P_j^{(0)}}$ 

6. **Choose** measure of average treatment effect, and estimate it.

## **Collapsibility**

#### Collapsible:

$$\sum_{j=1}^{J} w_{j} f(P_{k}^{(1)}, P_{k}^{(0)}) = Age < 65$$

$$f(\sum_{j=1}^{J} w_{j} P_{k}^{(1)}, \sum_{j=1}^{J} w_{j} P_{k}^{(0)})$$

$$Age \ge 65$$

$$Age \ge 65$$

$$P_{1}(Y^{(1)} = 1) = 0.4 \quad P_{1}(Y^{(0)} = 1) = 0.2 \quad 1000$$

$$P_{2}(Y^{(1)} = 1) = 0.8 \quad P_{2}(Y^{(0)} = 1) = 0.6 \quad 1000$$

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- A characteristic of the chosen measure.
- Not depends on model.
- Three Types

#### **Hypothetical example: Perfect Randomized Trial**

Z=1 Z=0 Size
$$P_1(Y^{(1)} = 1) = 0.4 \quad P_1(Y^{(0)} = 1) = 0.2 \quad \textbf{1000}$$

$$P_2(Y^{(1)} = 1) = 0.8 \quad P_2(Y^{(0)} = 1) = 0.6 \quad \textbf{1000}$$

$$P(Y^{(1)} = 1) = 0.6 \quad P(Y^{(0)} = 1) = 0.4 \quad \textbf{2000}$$

Young: 
$$OR_1 = \frac{0.4/(1-0.4)}{0.2/(1-0.2)} = 2.67$$

**Old:** 
$$OR_2 = \frac{0.8/(1-0.8)}{0.6/(1-0.6)} = 2.67$$

Marginal: 
$$OR = \frac{0.6/(1-0.6)}{0.4/(1-0.4)} = 2.25$$
  
 $\neq \frac{1}{2}2.67 + \frac{1}{2}2.67$ 

- 1. Collapsible
- Collapsible under assumptions
- Not Collapsible.

# $P^{(1)}$ - $P^{(0)}$ — Average Risk Difference

$$P_j^{(1)} = Pr(Y^{(1)} = 1 | j^{th} \text{ bin}) = \frac{1}{N_j} \sum_{i=1}^{N_j} Pr(Y_i^{(1)} = 1)$$

$$P_j^{(0)} = Pr(Y^{(0)} = 1 | j^{th} \text{ bin}) = \frac{1}{N_j} \sum_{i=1}^{N_j} Pr(Y_i^{(0)} = 1)$$

$$P_j^{(1)} = P_j^{(0)} \sum_{i=1}^{J} P_i^{(1)} \sum_{i=1}^{J} Pr(Y_i^{(0)} = 1)$$

$$\begin{split} P^{(1)} - P^{(0)} &= \sum_{j=1}^{J} \omega_{j} P_{j}^{(1)} - \sum_{j=1}^{J} \omega_{j} P_{j}^{(0)} = \sum_{j=1}^{J} \omega_{j} (P_{j}^{(1)} - P_{j}^{(0)}) \\ &= \frac{1}{N} \sum_{i=1}^{N} [Pr(Y_{i}^{(1)} = 1) - Pr(Y_{i}^{(0)} = 1)] \quad , \quad \omega_{j} = \frac{N_{j}}{N} \end{split}$$

#### Collapsible: overall ← bin-specific ← individual level

- ☐ The difference of average risk
- ☐ A weighted average of bin-specific treatment effect.
- ☐ The average of individual risk difference.

## $P^{(1)}/P^{(0)}$ — Marginal Relative Risk

Not Collapsible, in general.

$$\frac{P^{(1)}}{P^{(0)}} = \frac{\sum_{j=1}^{J} \omega_j P_j^{(1)}}{\sum_{j=1}^{J} \omega_j P_j^{(0)}} \neq \sum_{j=1}^{J} \omega_j \frac{P_j^{(1)}}{P_j^{(0)}}$$

Collapsible, w/ constant treatment effect assumption.

If 
$$\frac{Pr(Y_i^{(1)} = 1)}{Pr(Y_i^{(0)} = 1)} = r$$
 for  $i = 1, 2, ..., N$ , then 
$$\frac{P^{(1)}}{P^{(0)}} = \sum_{j=1}^{J} \omega_j \frac{P_j^{(1)}}{P_j^{(0)}} = r$$

**P.S.:** 
$$\frac{P^{(1)}/(1-P^{(1)})}{P^{(0)}/(1-P^{(0)})}$$
 — Marginal Odds Ratio (OR)

#### Not collapsible:

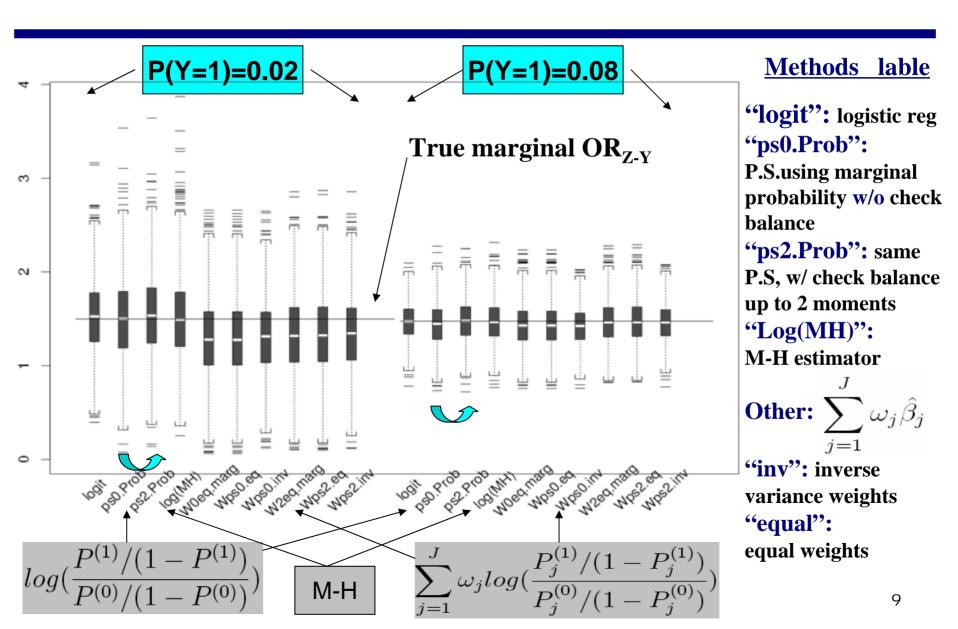
- individual level → bin-specific level
- bin-specific level → overall effect level

w/ or w/o constant treatment effect assumption

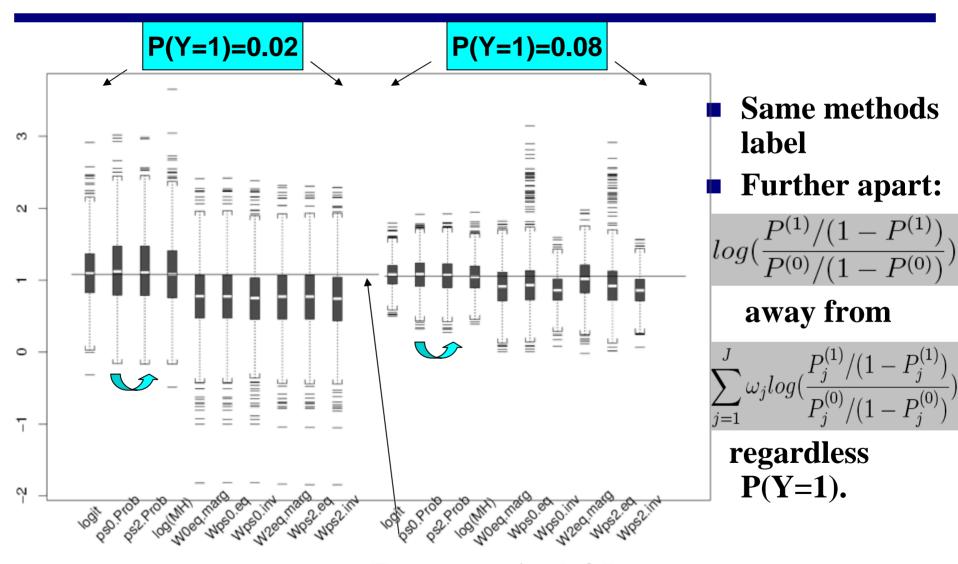
$$\frac{P^{(1)}/(1-P^{(1)})}{P^{(0)}/(1-P^{(0)})} = \frac{\sum_{j=1}^{J} \omega_j P_j^{(1)}/(1-\sum_{j=1}^{J} \omega_j P_j^{(1)})}{\sum_{j=1}^{J} \omega_j P_j^{(0)}/(1-\sum_{j=1}^{J} \omega_j P_j^{(0)})}$$

$$\neq \sum_{j=1}^{J} \omega_j \frac{P_j^{(1)}/(1-P_j^{(1)})}{P_j^{(0)}/(1-P_j^{(0)})} \neq \frac{\sum_{j=1}^{J} \omega_j P_j^{(1)}/(1-P_j^{(1)})}{\sum_{j=1}^{J} \omega_j P_j^{(0)}/(1-P_j^{(0)})}$$

## $Log(Marginal OR_{Z-Y})$ , + constant treatment effect



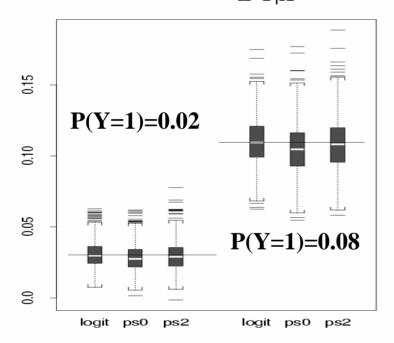
## Log(Marginal OR<sub>Z-Y</sub>), No constant treatment effect



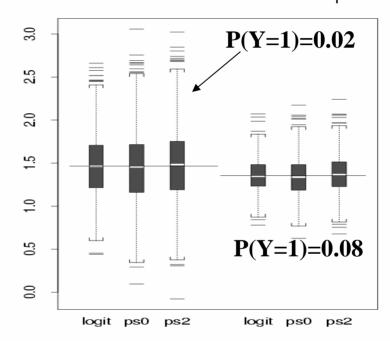
True marginal OR<sub>Z-Y</sub>

## Average Risk Difference & Marginal R.R.

#### $P^{(1)} - P^{(0)}, log(OR_{Z-Y|X}) = 1.5$



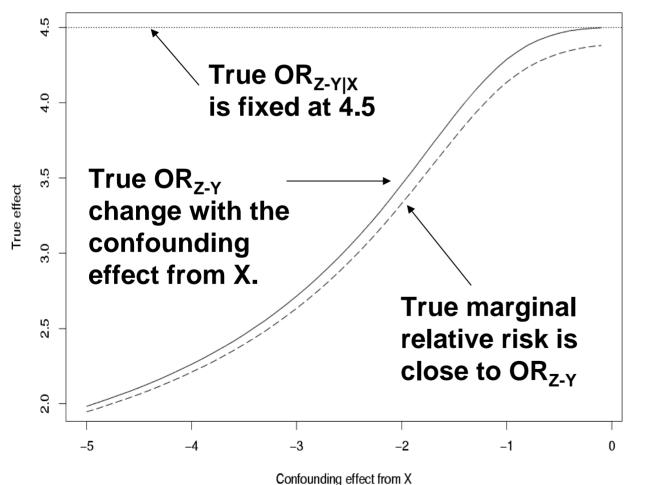
#### $Log(P^{(1)}/P^{(0)}), log(OR_{Z-Y|X})=1.5$



- Under rare disease, ARD is highly influenced by P(Y=1).
- Marginal RR estimated by P.S. performs nice.

## $OR_{Z-Y|X} \neq OR_{Z-Y}$ , even when disease is rare





#### **Setting:**

$$X \sim N(0,1)$$

$$\frac{logit[E(Y|,X)]_{\pm}}{\beta_0 + log(4.5)Z + \beta_x X}$$

$$Pr(Y = 1) = 0.02$$
,  
by adjusting  $\beta_0$   
 $N = 8000$ 

## Summary

- With constant treatment effect + the increasing of disease prevalence, the performance on estimators of weighted average of bin-specific effect type become better. Without constant treatment effect, their performance is bad.
- With the <u>increasing of disease prevalence</u>, model performance for different treatment measures become better.
- P.S: It is not always correct to say "average treament effect is a weighted average of bin-specific treatment effect".
   It really depends on your choice of treatment effect measure.
- In general, it is better to critically examine which treatment effect measure is best for your problem before applying technique to estimate it.

# Thanks!

Questions?