# Consistent treatment selection based on Manski midpoints







#### **Abstract**

The fundamental difficulty in comparing efficacies of two treatments based on observational data is that those who actually selected or received a particular treatment may give different responses to it than those who did not select or receive it. Thus, based on those who received one treatment, one cannot estimate the effect in case the treatment is applied to the entire population. Manski (1990) showed, that also in this situation, one may derive bounds for the effect size on the entire population. When only observational data are available, Rudas (2015a) suggested, in order to find the better effect, comparing the differences in the numbers the positive versus negative outcomes, in contrast to the usual method, when their ratios are used. He showed that this procedure is consistent in the sense that it never leads to Simpson's paradox. In this research, we develop Manski bounds for these differences, and show that the observed differences are the midpoints of the theoretical ranges obtained.

#### Effects and treatment selection

The task of selecting the best treatment from set  $\mathcal{D}$  of available treatments consists of finding

$$d^* = \arg\max_{d \in \mathcal{D}} u(d),$$

where u(d) is the net benefit of treatment d when selected for the whole population. For two treatments ( $\mathcal{D} = \{0, 1\}$ ), this maps to decision function

$$sgn[\xi(T)] := \begin{cases} -1 & \text{if } u(0) > u(1) \\ 0 & \text{if } u(0) = u(1) \\ +1 & \text{if } u(0) < u(1) \end{cases}$$

where  $\xi$ () is an effect measure and T a table that holds the data.

Often, u(d) is specified as E[Y(d)], where Y(d) is the population distribution of Y under treatment d. For two treatments, this yields effect measure

$$ATE = E[Y(1)] - E[Y(0)],$$
 which under  $Y \in \{0, 1\}$  equals  $P[Y(1) = 1] - P[Y(0) = 1].$ 

In a setup with two treatments and two outcome values, the population is held by the  $2 \times 2$  table

There are four classes  $\mathcal{Z} = \{0, 1, 2, 3\}$  of units based on which of  $\mathcal{D}$  works (Keele and Quinn, 2017),

$Z_i$ = 0: None	$Z_i = 1$ : Only $D = 1$	$Z_i = 2$ : Only $D = 0$	$Z_i = 3$ : Both
$Y_i(O) = O$	$Y_i(O) = O$	$Y_i(O) = 1$	$Y_i(O) = 1$
$Y_i(1) = 0$	$Y_i(1) = 1$	$Y_i(1) = 0$	$Y_i(1) = 1$

		<b>'</b>	$Y_i = 1$			'	'	$Z_i = 2$	<b>'</b>
	$D_i = 0$	$Z_i \in \{0, 1\}$	$Z_i \in \{2, 3\}$	<u> </u>	$D_i = 0$	$(1-\alpha_0)\zeta_0$	$(1 - \alpha_1)\zeta_1$	$(1-\alpha_2)\zeta_2$	$(1-\alpha_3)\zeta_3$
	$D_i = 1$	$Z_i \in \{0, 2\}$	$Z_i \in \{1, 3\}$		$D_i = 1$	$\alpha_0\zeta_0$	$\alpha_1\zeta_1$	$\alpha_2\zeta_2$	$\alpha_3\zeta_3$

where  $\zeta_Z$  is the size of class z in the population or the sample and  $\alpha_Z$  is the fraction choosing or receiving treatment D=1. Thus,

$$ATE = P[Y(1) = 1] - P[Y(0) = 1] = P[Z \in \{1, 3\}] - P[Z \in \{2, 3\}] = P[Z = 1] - P[Z = 2] = \zeta_1 - \zeta_2.$$

For any unit i, at most one of potential outcomes  $\{Y_i(0), Y_i(1)\}$  is observable. In experiments, randomized treatment assignment achieves  $(Y(1), Y(0)) \perp D$ , yielding the conventional estimator of ATE,

$$\beta(T) = P[Y = 1, D = 1] - P[Y = 1, D = 0] = \frac{p_{11}}{p_{10} + p_{11}} - \frac{p_{01}}{p_{00} + p_{01}}.$$

In observational data, the Z-classes may be differentially allocated to the treatments,

$$\exists \{z, z'\} \in \{0, 1, 2, 3\} : P[D = d, Z = z] \neq P[D = d, Z = z'],$$

associating treatment status with the potential outcomes,  $(Y(1), Y(0)) \not\perp\!\!\!\perp D$ .

## Directional collapsibility and consistent treatment selection

Suppose two strata  $S \in \{0, 1\}$  held by tables

$$T_{S=O} = \frac{\begin{vmatrix} q_{00} & q_{01} \\ q_{10} & q_{11} \end{vmatrix}}{ q_{10} | q_{11}} \text{ and } T_{S=1} = \frac{\begin{vmatrix} r_{00} & r_{01} \\ r_{10} & r_{11} \end{vmatrix}}{ r_{10} | r_{11}}, \quad \forall q_{dy}, r_{dy} \in [0, 1], \quad \sum_{d} \sum_{y} q_{dy} = \sum_{d} \sum_{y} r_{dy} = 1.$$

Under a consistent treatment selector

if 
$$\operatorname{sgn}[\xi(T_0)] = \operatorname{sgn}[\xi(T_1)]$$
, then  $\operatorname{sgn}[\xi(T_0)] = \operatorname{sgn}[\xi(T_0 + T_1)]$ .

Rudas (2010) proved that all consistent selectors are indifferent in the sense that

if 
$$sgn[\xi(T_0)] = 0$$
, then  $sgn[\xi(T_0 + T_1)] = sgn[\xi(T_1)]$ ,

and any selector such as  $sgn[\beta(T)]$  that is insensitive to allocation in the sense that

if 
$$T_1 = \frac{n_{00} |n_{01}|}{n_{10} |n_{11}|}$$
,  $T_2 = \frac{w_0 n_{00} |w_0 n_{01}|}{w_1 n_{10} |w_1 n_{11}|}$ ,  $\forall n_{dy} \geq 0$ ,  $\forall w_d \geq 0$ , then  $\operatorname{sgn}\left[\xi(T_1)\right] = \operatorname{sgn}\left[\xi(T_2)\right]$ ,

is not a consistent selector, implying the possibility of association reversal known as Simpson's paradox.

## Manski bounds and midpoints

Manski (1990) showed, that also in settings with observational data and unobserved confounding, one may derive bounds for the effect size on the entire population. For treatment d, the bounds on the response  $(Y_i \in \{0, 1\})$  in the entire population are

$$B[P[Y(d) = 1]] = [2p_{d1} - 1, 1 - 2p_{d0}], \text{ with midpoint } M[P[Y(d) = 1]] = p_{d1} - p_{d0}.$$

The bounds are limiting cases of all possible allocations  $\mathcal{A}$  of the units not in D = d into Y(d) = 0 and Y(d) = 1. Weighting all allocations  $a \in \mathcal{A}$  equally, denoting the cell value under a as  $p_{dy}^a$ ,

$$\mathsf{M} \Big[ \mathsf{P} \big[ \mathsf{Y} (d) = 1 \big] \Big] = \mathsf{E} \big[ p_{d1}^a - p_{d0}^a \big] = \frac{1}{\mathsf{A}} \sum_a \big( p_{d1}^a - p_{d0}^a \big) \,, \quad \mathsf{A} = \mathsf{N} + 1 - \sum_y n_{dy}.$$

Under two treatments ( $\mathcal{D} = \{0, 1\}$ ), denoting the smallest possible size of class z as  $P_{min}[Z = z]$ ,

$$B[ATE] = [(P_{min}[Z = 1] - P_{max}[Z = 2]), (P_{max}[Z = 1] - P_{min}[Z = 2])]$$
$$= [-(p_{01} + p_{10}), (p_{00} + p_{11})],$$

with midpoint equal to half the difference in the midpoints of the two treatments

$$M[ATE] = \frac{1}{2} \Big( (p_{00} + p_{11}) - (p_{01} + p_{10}) \Big) = \frac{1}{2} \Big( (p_{11} - p_{10}) - (p_{01} - p_{00}) \Big)$$
$$= \frac{1}{2} \Big( M \Big[ P[Y(1) = 1] \Big] - M \Big[ P[Y(0) = 1] \Big] \Big) = \frac{1}{2} \delta,$$

where  $\delta$  is the difference measure of association (Rudas, 2015a). Since

if 
$$q_{00} + q_{11} > (<) q_{01} + q_{10}$$
 and  $r_{00} + r_{11} > (<) r_{01} + r_{10}$ ,  
then  $q_{00} + q_{11} + r_{00} + r_{11} > (<) q_{01} + q_{10} + r_{01} + r_{10}$ ,

 $sgn[\delta(T)]$  is a consistent selector, and all consistent selectors are equivalent to it (Rudas, 2010). As

$$\delta = ((1 - \alpha_0)\zeta_0 + \zeta_1 + \alpha_3\zeta_3) - (\alpha_0\zeta_0 + \zeta_2 + (1 - \alpha_3)\zeta_3)$$
  
=  $(\zeta_1 - \zeta_2) + ((1 - \alpha_0)\zeta_0 + \alpha_3\zeta_3) - (\alpha_0\zeta_0 + (1 - \alpha_3)\zeta_3),$ 

even under differential allocation of classes 1 and 2 into the treatments,

$$\delta = ATE \quad \text{if} \quad (1-\alpha_0)\zeta_0 + \alpha_3\zeta_3 = \alpha_0\zeta_0 + (1-\alpha_3)\zeta_3, \quad \text{i.e.} \quad T_{Z_i \in \{0,3\}} = \frac{n_1 + n_3}{n_2 + n_3} \frac{n_1 + n_4}{n_2 + n_3}, \quad \forall n_c \geq 0.$$

Furthermore,  $\delta$ 's sensitivity to allocation into treatments means that

Over an evenly-spaced grid of 72, 576 values  $\{0, 0.2, 0.4, 0.6, 0.8, 1\}$  of  $\{\alpha\}_Z$  and  $\{\zeta\}_Z$ ,

ξ	$sgn[\xi(T)] = sgn[ATE]$	RMSE [if $\beta$ (T) exists]	Bias [if $\beta(T)$ exists]
$\delta$	68%	0.33	0
$\beta$	63%	0.34	O

#### Informative allocation

The treatment selector  $\operatorname{sgn}[\delta]$  is particularly appealing where the treatments' popularity is informative (Rudas, 2010, 2015b). For example, if the goal is to maximize  $P[Y(\{b,d\})=1]$ , where  $\{b,d\}$  is the set of options given to the units in the population to chose from and  $b \notin \mathcal{D}$  is a baseline state, then the more popular treatment d' may be preferable even if  $P[Y(d')=1] < \max_d P[Y(d)=1]$ .

#### Relationship to sensitivity analysis

For any  $2 \times 2$  (D, Y) table holding N observations, there are  $\prod_d \prod_y (n_{dy} + 1)$  possible allocations into the four Z classes. Inspecting all such possible  $2 \times 2 \times 4$  (D, Y, Z) tables with a fixed (D, Y) marginal and half of the cells set to zero relates to conditional volume tests (Diaconis and Efron, 1985). If all allocations receive equal weight,  $\delta$  equals the expected effect. Keele and Quinn (2017) propose a generalization that weights the allocations according to extra-data information, with simulated cell-splitting parameters  $\{\psi\}_{dy}$  on the (D, Z) margin.

#### Statistical properties

Under multinomial sampling, the probability of the correct decision with  $sgn[\delta]$  depends only on N and the population value of  $\delta$ , i.e. the fraction of the population in  $h = p_{00} + p_{11}$ ,

$$P[\delta(T) > 0] = \sum_{k=\lfloor N/2 \rfloor + 1}^{N} \binom{N}{k} h^k (1-h)^{N-k}, \quad \text{approximated for large $N$ by} \quad \phi \bigg( \sqrt{N} \frac{h-0.5}{\sqrt{h(1-h)}} \bigg).$$

where  $\phi$  is the standard normal CDF (Rudas, 2015a), and if

$$sgn[\delta] = +1$$
 and  $\phi(2\sqrt{N}(p_{10} + p_{11} - 0.5)) > 1 - \alpha$ ,

the asymptotic 1 –  $\alpha$  level confidence interval does not contain 0 or –1 (Rudas, 2010).

#### Generalized consistent treatment selection

Generalizations of the selector  $\mathrm{sgn}[\delta]$ 

- To multiple treatments ( $|\mathcal{D}| \ge 2$ ) with  $d^* = \arg\max_{d \in \mathcal{D}} (p_{d1} p_{d0})$ .
- To ordinal  $Y \in \{0, ..., J\}$ ,

$$d' = d^* \text{ if } \forall d \neq d', \ \forall y < J, \ P[Y > y, D = d'] - P[Y \le y, D = d'] > P[Y > y, D = d] - P[Y \le y, D = d].$$

# Summary

- Consistent treatment selectors avoid Simpson's paradox.
- All selectors insensitive to allocation into treatment categories are equivalent and inconsistent.
- All consistent selectors are sensitive to allocation and equivalent in selecting the treatment with the largest midpoint of the Manski bounds on P[Y(d) = 1].

# References

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