

Supplementary Materials for “Robustifying Trial-Derived Optimal Treatment Rules for A Target Population”

Ying-Qi Zhao ¹ and Donglin Zeng ²
and Catherine M Tangen ³ and Michael L LeBlanc ⁴

April 5, 2019

¹Associate Member, Public Health Sciences Division, Fred Hutchinson Cancer Research Center, Seattle, WA, 98109, Email: yqzhao@fredhutch.org.

²Professor, Department of Biostatistics, University of North Carolina at Chapel Hill, Chapel Hill, NC, 27599, Email: dzeng@email.unc.edu.

³Member, Public Health Sciences Division, Fred Hutchinson Cancer Research Center, Seattle, WA, 98109, Email: ctangen@fredhutch.org.

⁴Member, Public Health Sciences Division, Fred Hutchinson Cancer Research Center, Seattle, WA, 98109, Email: mleblanc@fredhutch.org.

There could be the efficiency loss due to the first stage estimation of the contrast function $f^*(x)$. We used Scenario 3 as an example to illustrate this effect in practice, where the true treatment effect was nonlinear. We present the results on MiLD-V based methods in Figure 1, i.e., the true weights $W(X) = |f^*(x)| = |E\{T|A = d^*(x), X = x\} - E\{T|A \neq d^*(x), X = x\}|$. In MiLD-V (est), the true decision rule was estimated as a first step (MiLD-V (est)) with $\hat{f}(X)$ used as the weights. In MiLD-V (truth), the true weights $|f^*(x)|$ were utilized. MiLD-V (truth) yields a better result across all sample sizes, and the variance of MiLD-V (est) is in general larger than the MiLD-V (truth) method. As sample size increases, the difference between two methods decreases, which is expected given that the estimated weights would be improved over increasing sample sizes. We have added this discussion in supplementary material.

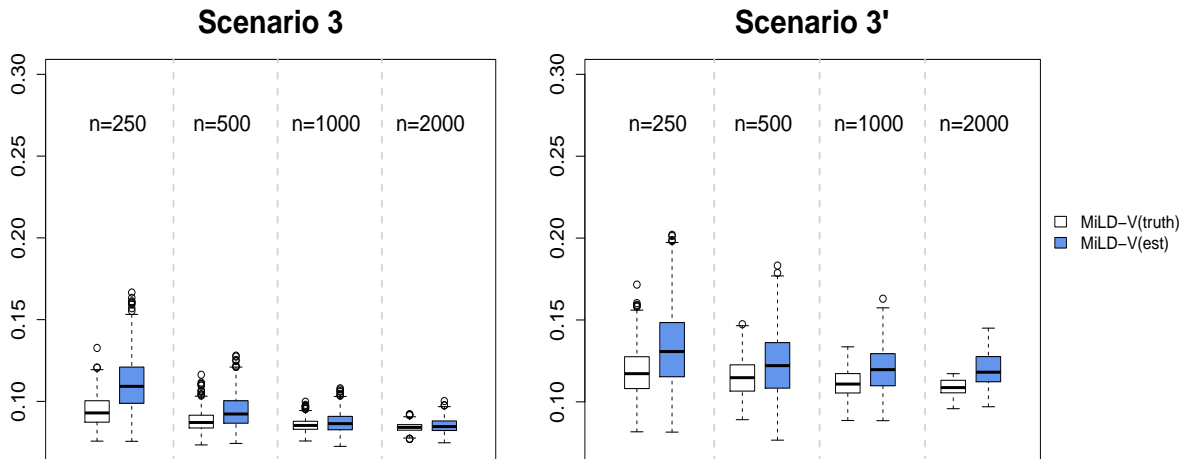


Figure 1: Misallocation rates in Scenarios 3 and 3' using trial data with sample sizes varying from 250 to 2000. The misallocation rate is evaluated by using 500 Monte Carlo repetitions.