Project Summary Report

YODA Project Protocol #: 2021-4849

Minimax regret for data analysis with application to clinical treatment decisions

Objective

Starting from the seminal works of Wald (1945), Haavelmo (1944), Savage (1951), and later, for instance, Manski (2004), Manski and Tetenov (2016), statistical decision theory has been brought forth to address the limitations of hypothesis testing (e.g., hypothesis testing ignores the magnitude of losses under Type I/II errors). In particular, the maximin-regret criterion has been given special attention. One of its features is that it allows choosing a decision rule (mapping from data to treatment decisions) that performs uniformly well across all states of the world (e.g., across all possible binary outcome distributions) in terms of the suboptimality gap (regret). In other words, it provides an upper bound on the difference between the welfare from the best possible treatment and the treatment chosen based on the data. However, the literature on exact (finite-sample) minimax-regret optimal rules is limited to cases with binary treatments and/or binary outcomes, partly due to the complexity of analytical derivations.

Thus, the main goal of the current study was to derive the exact minimax-regret optimal rules for a broader class of outcomes such as bounded outcomes and **apply those to the actual clinical trials**. Conditional on achieving the main goal, the secondary goals included extensions to the multiple treatments and missing data.

Methods

Following Schlag (2006) we have completed minimax-regret optimality proofs for the Correlated Binomial Average Rule (CBAR) for *bounded outcomes*. We have also developed CBAR extensions to regret-squared loss function (akin to risk-averse decision makers). However, we failed to prove the admissibility (i.e., that there is no other rule that has the same maximum regret but performs better outside of the worst-case scenario states). Moreover, using simulated data (prior to applying CBAR to real clinical trial data), ***CBAR and its versions underperformed for large subsets of bounded distributions (e.g., beta-distributed outcomes) compared to the Empirical Success (ES) rule (Manski, 2004) and the normal-based rule (Kitagawa et al., 2023)***, even though the last two rules are derived binary and normally distributed outcomes. Hence, the main goal was not achieved, and we did not apply the derived rules to the real data provided by YODA.

Results

No results were released/published as the derived methods did not perform adequately on simulated data.

Conclusions

N/A

References

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