Systematic Value Exploration of Propensity Score and Borrowing Approaches

Anduena Rexhepi[1,2], Oliver Sailer[1], Jan Beyersmann[2]

[1] Department of Biostatistics and Data Science, Boehringer Ingelheim Pharma GmbH & Co. KG, Biberach, Germany

[2] Ulm University, Faculty of Mathematics and Economics, Institute of Statistics

Abstract

When designing a randomized clinical trial, it can be efficient to borrow information from the control arm of relevant historical trials where the same control has been carried out. Multiple methods like Propensity Score Matching (A) [1], Bayesian Dynamic Borrowing (B) [2] or a combination of propensity score stratification with Bayesian Dynamic Borrowing (C) [3] can be used to leverage the control arm of a current study. The availability of different approaches inspired the research of the question which technique yields the most efficient and accurate results under the consideration of unmeasured confounders. In order to explore this problem several simulation studies are designed. A linear data generating model with two covariates and a continuous endpoint is defined where one of the covariates is treated as unknown in the analysis afterwards. Through extensive simulations, the Bayesian methods (B) and (C) generally provide smaller MSE estimates with respect to the ATE estimate compared to the PS matching. Especially in scenarios where historical patients might strongly differ from current ones, method (C) appears to be the most efficient one. All methods turn out to mostly yield unbiased ATE estimates only when the historical average of the unmeasured covariate coincides with the current covariate mean. We apply the methods retrospectively to a real case study investigating schizophrenia.

References

[1] Zhao, Q.-Y., Luo, J.-C., Su, Y., Zhang, Y.-J., Tu, G.-W., & Luo, Z. (2021). Propensity score matching with r: Conventional methods and new features. *Annals of translational medicine*, *9*(9).

[2] Schmidli, H., Gsteiger, S., & Neuenschwander, B. Incorporating historical data into randomized controlled trials. In: In *Handbook of statistical methods for randomized controlled trials*. Chapman; Hall/CRC, 2021, pp. 545–566.

[3] Zhu, A. Y., Roy, D., Zhu, Z., & Sailer, M. O. (2023). Propensity score stratified map prior and posterior inference for incorporating information across multiple po- tentially heterogeneous data sources. *Journal of Biopharmaceutical Statistics*, 1–15.