Kolloquium „Statistische Methoden in der empirischen Forschung“

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Wo: Online

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Using Historical Data to Predict Health Outcomes

The gold standard for the investigation of the efficacy of a new therapy is a randomized controlled trial (RCT) [1]. This is costly, time consuming [2] and not always practicable (e.g. for lethal conditions with limited treatment possibilities [1]) or even possible in a reasonable time frame (e.g. in rare diseases due to small sample sizes [3]). At the same time, huge quantities of available control-condition data in analyzable format [4-8] of former RCTs or real-world data (RWD), i.e., patient-level data gathered outside the conventional clinical trial setting [9], are neglected if not often completely ignored [2]. To overcome these shortcomings, alternative study designs using data more efficiently would be desirable.

Assuming that the standard therapy and its mode of functioning is well known and large volumes of patient data exist, it is possible to set up a sound prediction model to determine the treatment effect of this standard therapy for future patients. When a new therapy is intended to be tested against the standard therapy, the vision would be to conduct a single-arm trial and to use the prediction model to determine the effect of the standard therapy on the outcome of interest of patients receiving the test treatment only, instead of setting up a two-arm trial for this comparison. While the advantages of using historical data to estimate the counterfactual are obvious (increased efficiency, lower cost, alleviating participants’ fear of being on placebo)[10, 11], bias could be caused by confounding (e.g. by indication, severity, or prognosis) or a number of other data issues that could jeopardize the validity of the non-randomized comparison [12].

The aim is to investigate if and how such a design - the prediction design - may be used to provide information on treatment effects by leveraging existing infrastructure and data sources (historical data of RCTs and/or RWD). Therefore, we investigate under what assumptions a linear prediction model could be used to predict the counterfactual of patients precisely enough to construct a test for evaluating the treatment effect for normally distributed endpoints. In particular, it is investigated what amount of data is necessary (of historical data/for single-arm trial). Via simulation studies, it is examined how sensible the design acts towards violations of the assumptions. The results are compared to reasonable (conventional) benchmark scenarios, e.g., the setting of a single-arm study with pre-defined threshold or a setting, where a propensity score matching [13-16] was performed.

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