Bayesian dynamic borrowing of external information: What can be gained in terms of frequentist power?

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The research is motivated by a basket trial in precision medicine in which adults with a specific molecular tumor profile are treated with targeted therapy and response to therapy is assessed. The population of children with this specific molecular profile is too small to warrant a separate pediatric trial. This motivates the implementation of a pediatric stratum in the adult trial. This setting suggests that information from the adult trial can be used for the pediatric stratum as "historical information". An overview of different methods for borrowing from historical data has been given by Viele et al. (2014). A number of novel adaptive methods have been proposed in recent years that dynamically discount the amount of information borrowed from historical data based on the conformity between the historical and current data. Adaptive power priors represent one of the approaches suited for this situation where the discounting factor can be selected by, e.g., an empirical Bayes approach as suggested by Gravestock et al. (2017). However, even in case of dynamic borrowing no power can be gained when strict frequentist type I error control is required. We exemplify this finding in the case of the pediatric arm of an adult trial and a dichotomous outcome. We discuss that this counter-intuitive limitation is true in any situation in which a uniformly most powerful test exists and show situations for which this applies.

References:

Gravestock, I., Held, L.; COMBACTE-Net consortium (2017). Adaptive power priors with empirical Bayes for clinical trials. Pharm Stat. 16(5): 349-360.

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