Model-Based Recursive Partitioning for Stratified and Personalised Medicine

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Established statistical procedures for the analysis of primary endpoints in randomised clinical trials assume that there is a universal, i.e. constant, treatment effect that applies to all patients in the trial and - even more importantly - to all future patients potentially to be treated with the novum under consideration. In complex diseases or heterogeneous patient populations this assumption may be incorrect and novel statistical methods are needed to discover if treatment effects differ across patients, and if so, which patient characteristics influence treatment effects.

We propose model-based trees as a method for stratified medicine, i.e. for the discovery of patient subgroups where within subgroups the treatment effects are the same and between subgroups treatment effects differ. Using ensembles of model-based trees (model-based forests) we can detect similarities between patients in terms of treatment effects and use this similarity measure to estimate personalised treatment effects (personalised medicine).