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Some recent developments in the analysis of adaptive designs with a view on their usefulness in platform trials

Confirmatory platform trials have recently gained considerable attention in the community of pharma statisticians. While similar designs (such as basket trials) are frequently used in earlier phases of clinical development, in particular in oncology, their use for submission purposes is relatively recent. Some of these trials (such as GBM Agile and PANCAN) are multi-sponsor trials.

Currently, there is a lack of experience with the use of platform trials as pivotal trials, in particular regarding type I error control. Familywise-error rate control (FWER) across the entire platform seems to be an unrealistically strict criterion. Some alternative concepts, such as population-wise and treatment-wise error rate control have been suggested.

We will discuss some of the concepts. Subsequently, we will consider how type I error rate control can be guaranteed in platform trials when techniques such as response adaptive randomization or permitting new treatments into an ongoing platform trial are used. It turns out that adaptive design methodology can be adapted to such uses, but a power loss cannot be avoided.