

Heidelberger Kolloquium Medizinische Biometrie, Informatik und Epidemiologie

Sehr geehrte Damen und Herren,

hiermit laden wir Sie herzlich ein zu dem Vortrag:

“Mastering innovative trial designs: Master protocols, basket, umbrella and platform trials“

von

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am Montag, 28.05.2018, 16.15 Uhr

im Kommunikationszentrum des DKFZ, Hörsaal **K1**, Im Neuenheimer Feld 280, 69120 Heidelberg

With the advent of novel targeted therapies and complex combination therapies, especially in oncology, new trial designs were developed to allow quicker and more specific investigations. These designs are subsumed as master protocols, with basket and umbrella trials being specific subtypes. Master protocols have in common that they are composed of multiple sub-studies each with a specific target population, usually defined based on biomarkers or indication. Platform trials additionally allow opening new sub-studies or new arms within a sub-study within an ongoing trial.

This talk exemplarily introduces different trial designs, discusses statistical benefits and challenges, and puts a special emphasis on regulatory issues.

Master protocols might offer operational benefits such as a unified biomarker screening platform and thus easier enrolment of patients. Statistical benefits which are commonly discussed include an increased power by sharing controls or by conducting pooled analyses, and the potential to transfer evidence from larger to smaller sub-studies. These strategies, however, might not always be clinically justified and thus depend on a good clinical rationale and regulatory agreement. The need to control for bias is emphasised. Situations where a trial-wise type 1 error control is necessary are contrasted with situations where the type 1 error can be controlled for sub-trials, and ways to control the type 1 error are sketched.

The statistical challenges are further exacerbated if group sequential or adaptive designs are used, which is especially the case in platform trials. Possible solutions to retain the validity and integrity of the trial and to allow for sound decisions on the basis of master protocols are presented.

Alle Interessenten sind herzlich eingeladen!

Gezeichnet: Dickhaus, Gabrysch, Kieser, Knaup, Kopp-Schneider, Wellek

Organisation: Birgit Schleweis

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