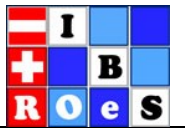
Einladung zum Biometrischen Kolloquium

Wiener Biometrische Sektion
der Internationalen Biometrischen Gesellschaft
Region Österreich – Schweiz



TIM FRIEDE

Department of Medical Statistics, University Medical Center Göttingen (Germany)

SOME STATISTICAL ASPECTS OF CLINICAL TRIALS FOR PERSONALIZED MEDICINE

18. November 2016 um 10:00 Uhr

Jugendstilhörsaal, Rektoratsgebäude (BT88)
Medizinische Universität Wien, Spitalgasse 23, 1090 Wien

Abstract:

Some statistical aspects of clinical trials for personalizedmedicine

Tim Friede

In personalized medicine patient populations are stratified with a viewto improve treatment outcome in terms of efficacy and tolerability.Stratification is often carried out by biomarkers. A particular case arenested subgroups that might arise from using several thresholds of acontinuous marker. Efficient testing strategies for normal data arederived under homoscedasticity and heteroscedasticity assumptions acrossthe subgroups. Furthermore, procedures for sizing a study with severalnested subgroups are presented. These depend among other quantities onnuisance parameters such as the variances of the outcomes in thesubgroups and the prevalences of the subgroups. Knowledge of these mightbe very scarce in the planning phase of such a trial resulting in aconsiderable risk of choosing an inappropriate sample size. To mitigatethese risks an internal pilot study design is proposed and itsproperties including type I error rate, power and sample sizedistribution are explored in Monte Carlo simulations. Adaptiveenrichment designs allow to restrict recruitment to certain subgroupsfollowing interim analyses. Approaches to hypothesis testing in suchdesigns are reviewed and their properties are compared in a simulationstudy. Finally, an adaptive enrichment design including an internalpilot study is presented and its characteristics are discussed.