Heidelberger Kolloquium
Medizinische Biometrie, Informatik und Epidemiologie

Sehr geehrte Damen und Herren,

hiermit laden wir Sie herzlich ein zu dem Vortrag:

"Using principal stratification to address post-randomization events – a case study"

von
Baldur Magnusson
Novartis Pharma AG, Basel

am Montag, 09.12.2019, 16.15 Uhr

Im **K13**,Turm Süd **Marsilius Arkaden**, Im Neuenheimer Feld 130.2, 69120 Heidelberg

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The treatment effect in subgroups of patients is often of interest in randomized controlled clinical trials, as this may provide useful information on how to treat which patients best. When a specific subgroup is characterized by the absence of certain events that happen postrandomization, a naive analysis on the subset of patients without these events may be misleading. The principal stratification framework allows one to define an appropriate causal estimand in such settings. Statistical inference for the principal stratum estimand hinges on scientifically justified assumptions, which can be included with Bayesian methods through prior distributions. Our motivating example is a large randomized placebo-controlled trial of siponimod in patients with secondary progressive multiple sclerosis. The primary objective of this trial was to demonstrate the efficacy of siponimod relative to placebo in delaying disability progression for the whole study population. However, the treatment effect in the subgroup of patients who would not relapse during the trial is relevant from both a scientific and patient perspective. Assessing this subgroup treatment effect is challenging as there is strong evidence that siponimod reduces relapses. We describe in detail the scientific question of interest, the principal stratum estimand, the corresponding analysis method for binary endpoints, and sensitivity analyses.

Alle Interessenten sind herzlich eingeladen!

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

Organisation: Birgit Schleweis
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