Kolloquium „Statistische Methoden in der empirischen Forschung“

Wann: 01. November 2022, 17:00 – 18:30 Uhr

Wo: FU Berlin | FB Wirtschaftswissenschaft | Hörsaal 104a | Garystr. 21, 14195 Berlin | U3, Freie Universität (Thielplatz) | S1, Lichterfelde West

Online-Übertragung: der Link wird auf der Website zur Verfügung gestellt

Cornelia Ursula Kunz (Boehringer Ingelheim)

Adaptive Ph2/3 drug development programs – The pros and cons

Recent statistical research has focused on adaptive designs like seamless Phase 2/3 and enrichments designs in the hope to accelerate the drug development process. The general idea is that these designs lead to a reduction in sample size or a gain in power as compared to classical designs as they use the data more efficiently. However, cases exist where the complexity of an adaptive design might not be worthwhile a small gain in power as compared to less complex fixed designs. Depending on the design specifics there might not even be a gain in power.
For example, seamless Phase 2/3 designs often incorporate many treatment arms in Phase 2. The aims are to establish a dose-response relationship as well as to select a promising treatment arm for Phase 3. In this setting, it is not uncommon to have four or even five treatment arms in Phase 2 while one or two arms will be carried forward to Phase 3.
Type I error control is often ensured by using p-value combination tests. However, due to the many arms, the adjustments to be made are often leading to conservative test statistics. As a result, the overall power of the seamless design might be lower than the overall power for conducting separate Phase 2 and Phase 3 trials or a single-stage design comparing all treatment arms against control without selecting an arm – assuming a fixed total sample size across both phases. Hence, when planning a trial, it might be worthwhile to compare different strategies in terms of overall power across the two phases.