# Kolloquium „Statistische Methoden in der empirischen Forschung“

Wann: 29. 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

**Stephen Schüürhuis (Charité, Berlin)**

**Adaptive and Group Sequential Designs for Delayed Treatment Responses**

Common statistical theory that is applicable to confirmatory phase III trial designs implicitly comes with the assumption that there is no time gap between the enrollment of a patient and the observation of the outcome of interest and that all patients are enrolled at the same time. In practice, however, patients are recruited successively and there mostly is a latency between the enrollment of a patient and the availability of the primary outcome measure. For single-stage designs, the difference between theory and practice only impacts on the trial duration but not on the statistical analysis and the interpretation thereof. For designs with interim analyses, however, the number of patients already enrolled into the trial and the number of patients with primary outcome measurements available differ during interim. This operational concern can cause issues regarding the statistical analyses of the data.
The main issue is that the use of available methodology either implies that at the time of the interim analysis, there are so-called pipeline patients whose data is not used to make a statistical decision (like stopping early for efficacy) or, if this is not acceptable, the enrollment into the trial needs to be at least paused for interim analysis to avoid pipeline patients. In this presentation, I will illustrate group-sequential and adaptive trial designs particularly suited to the delayed response setting. Oriented towards methods introduced by Hampson and Jennison (2013) [1], the presentation will contain approaches dividing the interim analysis into two statistical analyses, one of those being used to judge on interrupting the enrollment of patients, followed by critical values for hypothesis testing in case the stopping boundaries have been crossed beforehand.

## Literatur

[1] Hampson, L. V., & Jennison, C. (2013). Group sequential tests for delayed responses (with discussion). Journal of the Royal Statistical Society: Series B (Statistical Methodology), 75(1), 3-54.