

Kolloquium „Statistische Methoden in der empirischen Forschung“

Wann: 17. Oktober 2023, 17:00 – 18:30 Uhr

Wo: [Campus Charité Mitte | Hörsaal der Nervenklinik | Bonhoefferweg. 3, 10117 Berlin](#)

Online-Übertragung: der Link wird auf der [Website](#) zur Verfügung gestellt

Tim Friede (Universität Göttingen)

Combining randomized controlled trials and real world data

Innovative approaches to clinical trials include the use of Bayesian statistics. The Bayesian framework lends itself to the integration of data from different sources such as a new randomized controlled trial (RCT) and external real world data (RWD) such as clinical registries. As a prerequisite, we provide some background on Bayesian random-effects meta-analysis (Friede et al, 2017). Then we will discuss the integration of RCT and real RWD by dynamic borrowing (Röver & Friede, 2020). As concerns are frequently raised that large-scale registries might overpower smaller RCT, particular attention will be paid to the weights both sources of information receive (Röver & Friede, 2021). Making use of the R package bayesmeta the approach is motivated and illustrated by examples from rare diseases such as Creutzfeldt-Jakob disease and Alport syndrome. The application of the dynamic borrowing approach in so-called comprehensive cohort studies is considered, in particular in rare diseases and other settings where large-scale RCT are difficult to conduct. Finally, the analysis of clinical registries will be discussed, in particular with regard to data requirements and sample sizes (Friedrich & Friede, 2020). The talk draws on joint work with a number of colleagues including Christian Röver, Sarah Friedrich and Tim Mathes.

Literatur

- Friede T, Röver C, Mathes T (2023) Verknüpfung von randomisierten kontrollierten Studien und Real World Data (Combining randomized controlled trials and real-world data). Prävention und Gesundheitsförderung (in press). <https://link.springer.com/article/10.1007/s11553-023-01016-9>
- Friede T, Röver C, Wandel S, Neuenschwander B (2017) Meta-analysis of few small studies in orphan diseases. Research Synthesis Methods 8: 79–91.
- Friedrich S, Friede T (2020) Causal inference methods for small non-randomized studies: Methods and an application in COVID-19. Contemporary Clinical Trials 99: 106213.
- Röver C, Friede T (2020) Dynamically borrowing strength from another study through shrinkage estimation. Statistical Methods in Medical Research 29: 293–308.
- Röver C, Friede T (2021) Bounds for the weight of external data in shrinkage estimation. Biometrical Journal 63: 1131–1143.