Kolloquium „Statistische Methoden in der empirischen Forschung“

Wann: 07. November 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

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Benefit-Risk Assessment in Clinical Trials with Composite Endpoints

Marketing authorization typically requires efficacy to be substantiated while the benefit risk-profile needs to be considered favorable (Directive 2004/27/EC of the European Parliament and of the Council of 31 March 2004). In clinical trial practice that means, the main objective of pivotal trials is to prove superiority or non-inferiority on some primary endpoint of efficacy, and collect information on additional endpoints of efficacy and safety to provide information for the overall benefit-risk profile.

Yet, some clinical endpoints are direct measures of benefit risk, as e.g. overall mortality and patient reported outcomes on quality-of-life. So, the lines are not as clear cut between efficacy and safety as it may seem, and with the increased use of composite endpoints as clinical trial endpoints, it is always possible to combine deliberately measures for efficacy with those of safety. Several methods have been developed since Chuang-Stein and Mohberg (1991) suggested combining efficacy and safety into a ranked outcome.

In this talk, I want to present options for benefit-risk composite endpoints, debates on their pros and cons, and discuss the role that they can play in the benefit-risk assessment drug development.

Literatur

Chuang-Stein C, Mohberg NR. Three measures for simultaneously evaluating benefits and risks using categorical data from clinical trials. Stat Med 1991;10:1349–1359. [PubMed: 1925166]