Experimental designs for preclinical dose response experiments

A proper experimental design is key for the validity of statistical results and their interpretations in pharmaceutical research. Research and preclinical experiments build the starting point for further developments and the experimental design needs to be very robust to address the relatively small sample sizes.

One important goal of preclinical experiments is to determine a precise estimation of the dose response shape, while simultaneously demonstrating a proof of concept of the test item. In this setting the Multiple Comparison Procedures and Modeling (MCPMod) technique is utilized. In addition, the topic of Bayesian MCPMod will also be explored in this presentation. This method integrates prior knowledge into the MCPMod process, offering a more comprehensive insight into the dose response relationship.

Further aspects to be covered, include the use of D-optimal design settings to optimize the precision of the dose response estimation. This is based on a minimum variance approach of the estimated model parameters, which also takes the “Three Rs Principle” (Replacement, Reduction, Refinement) into consideration by reducing the required sample size. A particular focus is on the practicability as well as the robustness of the statistical outcomes.

Leonie Hezler, Anja Wiens, Bernd-Wolfgang Igl

2016-2022 Mathematical Biometry at Ulm University (BSc + MSc)

Since 2022 Non-Clinical Statistician at Boehringer Ingelheim Pharma GmbH & Co.KG