

ASA-BI-NESS Statistics Webinar Series



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**March 28,
Thursday
9-10 am EST**

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Title

Considerations on Model-Based Approaches for Proof of Concept in Multi-Armed Studies

Abstract

Prior to larger Phase 2b dose-finding or confirmatory Phase 3 studies, initial signs of efficacy are established in small proof of concept (PoC) studies. Multiple dose groups are frequently not considered in these studies, as the sample size shall be kept at a minimum due to the uncertainty on the efficacy of the drug. The inclusion of multiple dose groups reduces the power for the PoC, while adding valuable insight on the dose-response relation. Model-based methods support efficient sharing of data between treatment groups, what could also reduce the loss of efficiency in multi-armed PoC-studies. The commonly used MCPMod approach consists of a model-based trend test to establish any drug-related effect, followed by a dose-response modeling step. In particular the model-based trend test serves as a PoC-criterion in multi-armed. Generally not only the existence of any drug related effects is of interest, but also the size of effects. Lalonde et al. (2007) use confidence intervals on a minimum reference and a targeted effect as quantitative decision criteria in clinical development. An extension to multi-armed studies using model-based tests is in the Lalonde framework not straight forward, as the model assumption would typically bias the effect estimator, such that the Go/No-Go criterion might not be well controlled. Scope of this presentation will be the discussion of benefits and limitations when utilizing MCPMod-type approaches for testing the existence of targeted effects.

Professional Biography

Tobias is a Scientific Director at Janssen Pharmaceuticals, working as an internal consultant on innovative statistical study designs. Prior to joining Janssen in 2018, Tobias has spent 5 years in industry as statistical consultant for ICON Clinical Research. Having developed ADDPLAN DF, a software for the design, simulation and analysis of adaptive dose-finding studies, Tobias has gained extensive experience in the methods and applications of MCPMod. Prior to joining industry, Tobias worked as scientific researcher at the University of Magdeburg on methodology for designing experiments. Tobias did his PhD on the topic of optimal experimental designs for nonlinear response models at the University in Magdeburg (Germany).

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- American Statistical Association (Boston, Connecticut, Florida, New Jersey, Princeton/Trenton, and Washington chapters)
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- New England Statistical Society (NESS)

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