Statistical Planning and Evaluation of Translational Trials

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Short Bio: I am a professor of Biostatistics and the director of the Institute of Biometry and Clinical Epidemiology of the Charité Berlin. In my research, I specialize in statistical methods for pre-clinical and translational phases and small sample size inference. Furthermore, I serve as the deputy chair of the working group Non-Clinical Statistics of the IBS-DR. By training, I am a mathematician.

Abstract:
Any trial should start with careful planning, especially with sample size calculations. The planning phase of an experiment is key since errors in statistical planning can have severe consequences on both the results and conclusions drawn from the data. In translational research (preclinical and early clinical), false conclusions highly affect subsequent trials, and thus, mistakes proliferate, an unethical outcome. In statistical practice, most studies are planned based on t-tests and Wald-type statistics (including ANOVA) and make some strict distributional assumptions. Sample sizes are typically small and if planning assumptions are not met, the trials are either underpowered or too large, result in wrong conclusions and waste resources, and might even be misleading. On the other hand, nonparametric ranking methods (such as the Wilcoxon- Mann-Whitney test, Brunner-Munzel test, multiple contrast tests, and their generalizations) are excellent alternatives to such parametric approaches. However, sample size formulas and detailed power analyses are yet to be implemented for broad classes of such tests. In this talk, we discuss statistical planning and evaluation methods for translational trials. Real data sets illustrate the methods.

References

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