The Institute for Medical Informatics, Statistics and Documentation
announces a seminar/guest lecture held by Dr. Hans Ulrich Burger

Date: 22. (14:30 – 16:00) and 23.October (10:30-12:00)
Location: MC2.Q.01.019 - SR62

22.10. 14:30-16:00

Sample Size Determination: Power and Minimal Detectable Difference

The first presentation introduces and discusses two different ways to justify the size of a study., one is the usual power calculation, we are all familiar with, and the other approach is based on the minimal detectable difference. The reasons for introducing the concept of the minimal detectable difference is to align statistical significance with clinical relevance. The presentation then discusses two examples. The first example deals with the size of a cardiovascular outcome study where typically a realistic effect is close to a minimal clinically relevant difference. And the second example discusses the sample size calculation of a confirmatory trial in Alzheimer's disease where different issues occur, making again the concept of a minimal detectable difference important.

Non-inferiority assessments: Overview and issues

The second presentation discusses the assessment of non-inferiority in clinical trials. It presents some of the underlying methodology (high level), issues and also provides a number of examples. Major issues will be discussed like the choice of the population, the constancy assumption and the determination of the non-inferiority limit. All these issues point basically to the overarching issue of assay sensitivity in a non-inferiority trial. For the determination of the non-inferiority limit key approaches will be introduced, a basic definition based on clinical relevance and two retained effect methods, the 95-95% method and the synthesis method. Three examples will finally illustrate the importance of all these elements.

Medical University of Graz, Neue Stiftingtalstraße 6, 8010 Graz, www.medunigraz.at

legal form: corporate body under public law according to the universities act 2002.
information: newsletter of the university, DVR-Nr. 210 9494.
UID: ATU57511179, banking account: UniCredit Bank Austria AG IBAN: AT931200050094840004, BIC: BKAUATWW
Raiffeisen Landesbank Steiermark IBAN: AT443800000000049510, BIC: RZSTAT2G

23.10. 10:30-12:00

The Estimand framework: An introduction

The first presentation is an introduction into the estimand framework. It first discusses why the framework is needed and was developed in an ICH guideline. And then it introduces all the elements of the framework and how they are implemented. The key elements of an estimand - the five attributes - will be discussed in detail. Specific focus will be on intercurrent events and the different strategies how to handle them. An example on the implementation of the framework in primary progressive MS will finally illustrate the framework and its usefulness.

The Estimand framework: Successes, Issues and Examples

The second presentation on the estimand framework will provide first a critical review where the industry and regulatory bodies around the world stand with respect to the implementation of the framework, what has so far worked well and what worked less well. The presentation will then focus on the two challenging areas of the framework and highlight there the issues, the choice of the intercurrent event handling strategy and the subsequent analytical approach. All the issues will then be discussed in three examples. The first one is a discussion of the treatment policy strategy, when it makes sense and when not. The second one deals with the principal stratum strategy in an example in MS. And finally, the third example deals with the implementation of the framework, when a strategy is easy to implement and when it becomes complex. All examples together highlight the challenges but also the usefulness of the estimand framework.

We look forward seeing you!

Univ.-Prof. DI Dr. Andrea Berghold