N-of-1 trials and other modern study designs -- winter semester 2021-2022

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Traditionally, treatment guidelines and health intervention recommendations are developed based on results of large cohort studies or randomized controlled trials (RCTs). However, the analysis of such studies only yields estimates of average effects. Hence, these results do not allow meaningful predictions whether an intervention will help a given single individual. In the advent of digital solutions, personalized approaches have been on the rise. N-of-1 trials and other modern study designs allow to derive individual treatment effects, but also to use the data to obtain and improve the precision of population-level effect estimates of health interventions.

This seminar covers N-of-1 trials and other modern study designs such as micro-randomized trials. After an overview of different study types and their characteristics, the main focus of the class will be on methodological approaches for planning and analyzing N-of-1 trials. At the beginning of the class, we will gather data from N-of-1 trials that will be used throughout the course to illustrate the statistical methods.

Topics:

- Overview of classic and modern study designs
- Introduction to N-of-1 trials
- Micro-randomized trials & other modern study designs
- Ethics, data privacy and other requirements of digital studies
- Standard methods for individual analysis of N-of-1 trials
- Standard methods for aggregated analysis of N-of-1 trials
- Bayesian regression models for N-of-1 trials
- Meta analysis & network meta analyses
- Sample size calculation for N-of-1 trials
- Adaptive designs
- Statistical methods for the analysis of micro-randomized trials

Format:

- Introductory lectures with discussion of main concepts of N-of-1 trials and study designs
- Weekly readings of a paper as homework and discussion in class. One group of students will give a short presentation and lead the discussions
- Joint statistical analysis of N-of-1 trial data gathered in class by applying the discussed statistical models.
- Final project with presentation in class

Learning goals:

At the end of the course, the students will be able to

- understand the main concepts of planning & conducting N-of-1 trials and selected other study designs
- perform individual-level and aggregated analysis of N-of-1 trials using state-of-the-art methods

Course format:	Seminar
SWS:	2
Credits:	3 ECTS
Prerequisites:	Courses in statistics/machine learning/data science and in programming using R (e.g. "Biostatistics & Epidemiological Data Analysis using R" class)
Final grade:	30% participation in class, 30% paper presentation, 40% project