

Course program for: Fundamental concepts in medical statistics with applications to randomized controlled trials, Kampala 7-13 June 2023

NOTE (version 2023-05-11)

- This course is intended to provide an intuitive understanding of some of the fundamental concepts and topics in medical statistics.
- There will be a combination of lectures and hands-on exercises. Time does not permit going deeply into each topic; the course will rather provide an overview.
- The course aims at participants *with prior experience* of using statistical methods in medical research, and is thus not a basic course. Participants are expected to have carefully studied the course text (<https://amzn.eu/d/hqmMitt>) *before* coming to the course.

Wednesday 7

FOCUS OF THE DAY: Overview, and fundamental concepts

09:00 - 10:00 Welcome and startup

- Welcome/introduction round
- Quick overview over course + practical info
- Quick overview over some basic stuff (but note that participants are expected to have a basic understanding of/experience with basic statistical concepts)

10:00 - 12:00 Some fundamentals

- Central limit theorem (most importantly, why it's useful)
- General background on Confidence Intervals
- Testing, p-values, one-sided vs. two-sided
- Confidence intervals vs. testing
- Transformation of data, the log-transform. General discussion of measurement scales

12:00 - 13:00 LUNCH

13:00 - 14:00 Exercises

14:00 - 15:00 Sample size and statistical power / statistical precision

- What it means
- How to restrict the probability of Type I/Type II errors, i.e. achieve sufficient statistical power at a given test significance level
- ... or sometimes better, compute sample size needed for sufficient precision of your effect estimate
- How to compute sample size for study planning, simple situations

15:00 - 16:00 Exercises

Thursday 8

FOCUS OF THE DAY: Generalized linear models (GLMs)

09:00 - 11:00 Linear regression

- Standard linear regression
- Multiple linear regression, confounding, interpretation
- Basic regression diagnostics

11:00 - 12:00 Exercises

12:00 - 13:00 LUNCH

13:00 - 15:00 Binomial regression

- Binomial regression (including logistic regression)
 - Different link functions: logit, log, and identity
 - Corresponding interpretations: logistic, log, and additive
 - Odds Ratio (OR), Relative Risk (RR), and Risk Difference (RD)
 - Basic regression diagnostics
- Poisson regression (Introduced here as a GLM, but will for the most part be covered on Monday, in the context of event history analyses.)

15:00 - 16:00 Exercises

Friday 9

FOCUS OF THE DAY: RCTs, other experimental designs

09:00 - 10:00 Fundamentals

- The causal reasoning behind randomization
- Adjustment for baseline variables? Other adaptations.
- Regression to the mean, need for trial comparator arm, etc.
- Dropouts, intention to treat

10:00 - 11:00 Multiple testing, multiple estimation

- Problems with multiple testing
- “Winner’s curse” in multiple estimation
- p-hacking, multiple subgroups, etc.
- Some extreme examples from genetics

11:00 - 12:00 Exercises

12:00 - 13:00 LUNCH

13:00 - 13:30 Weighted analyses

- Various types of weighting
 - Sampling
 - Frequency
 - Importance (although not really important here)

13:30 - 15:00 Interaction / effect measure modification

- Basic concepts
- Scale dependence
- Examples
- How to estimate, variable coding, such as Stata's approach, and dummy variables
- Testing / estimating differences in effects across subgroups

15:00 - 16:00 Exercises

Saturday 10

FOCUS OF THE DAY: Self study/group work

Monday 12

FOCUS OF THE DAY: Count data, standardization

09:00 - 11:00 Poisson regression

- How and when to use
- How to handle varying exposure time, use of offset
- Examples from Covid-19 pandemic analyses

11:00 - 12:00 exercises

12:00 - 13:00 LUNCH

13:00 - 15:00 Standardization methods

- Standardized averages for continuous data
- Standardized risks and rates
- Standardized Mortality Ratios (SMRs)
- Model-based standardization, examples of how to present results

15:00 - 16:00 Exercises

Tuesday 13

FOCUS OF THE DAY: Survival and event history analysis. Recap of central topics from the course.

09:00 - 11:00 Survival Analysis / Event History Analysis

- Introduction to survival/event history analysis
- Kaplan-Meier
- Cox regression

11:00 - 12:00 Exercises

12:00 - 13:00 LUNCH

13:00 - 14:00 Additional topics in survival analysis

- Alternative models, such as additive hazards model
- Competing risk

14:00 - 15:00 Summary, discussion, evaluation