UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

COURSE SYLLABUS

Statistical Models Applied To Clinical Trials

2526-2-F8203B009

Learning objectives

The aim of the course is to deepen the student's knowledge of the statistical design and analysis of a clinical trial.

Knowledge and understanding

This course will provide knowledge and understanding regarding:

- data simulation in SAS

- sample size and power analysis of a clinical study

- statistical analysis of a clinical study in the case of survival outcome, repeated events, continuous outcome with baseline and follow-up measurements, correlated data

- statistical analysis strategy in non-inferiority and bioequivalence clinical studies

Applying knowledge and understanding

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

- independently use data simulation in SAS

- identify the fundamental elements for power and sample size calculation

- analyze clinical studies in the case of survival outcomes, repeated events, continuous outcome with baseline and follow-up measurements, correlated data

The course will provide sound basis for planning and analyzing a clinical study with the help of SAS tools.

Contents

1. Simulations of clinical study data in SAS
2. Models for the analysis of clinical studies with binary and count outcomes
3. Models for the analysis of clinical studies with survival outcomes
4. Models for the analysis of clinical studies with continuous outcomes measured at both the beginning and end of the study
5. Sample size and statistical power of a clinical study: a simulation-based approach
6. Non-inferiority and bioequivalence clinical studies
7. Models for the analysis of clinical studies with correlated data

Detailed program

1. Simulations of clinical data in SAS
1.1 Importance of simulations as a working tool for biostatisticians
1.2 Basic elements and techniques for data simulation
1.3 Using simulations to evaluate sampling distributions, validity of statistical techniques, and properties of a statistical design

2. Models for the analysis of clinical studies with binary and count outcomes
2.1 Logistic regression model for the analysis of single events
2.2 Poisson and negative binomial models for the analysis of repeated events

3. Models for the analysis of clinical studies with survival outcomes
3.1 Introduction to clinical studies with survival outcomes
3.2 Survival curves
3.3 Link between the Poisson model and the analysis of survival data using the exponential model
3.4 Parametric and semi-parametric models
3.5 Methods for analyzing a randomized clinical trial in the presence of non-proportional hazards

4. Models for the analysis of clinical studies with continuous outcomes measured at both the beginning and end of the study
4.1 Introduction to pre-post clinical studies
4.2 Comparison of models for the analysis of a randomized pre-post clinical trial (post analysis, difference analysis, analysis of covariance, percent change analysis)

5. Sample size and statistical power of a clinical study: a simulation-based approach

6. Non-inferiority and bioequivalence clinical studies
6.1 Difference between the analysis of a superiority clinical study and the analysis of a non-inferiority clinical study
6.2 Bioequivalence clinical studies: definitions, outcomes used, and analysis models

7. Models for the analysis of clinical studies with correlated data
7.1 Introduction to the analysis of correlated data
7.2 Linear mixed-effects models for the analysis of correlated continuous outcomes

Prerequisites

None

Teaching methods

Classes can be conducted in two formats: in the first, the instructors will present the concepts (**lecture mode**); in the second, the instructors will interact with the students by proposing problems based on real or simulated clinical data, to be solved in groups using the specified software (primarily SAS). Students will solve and discuss the problems, with corrections made together with the instructors (**interactive mode**).
The ratio between lecture and interactive hours will be approximately 2:1. Some lecture-mode classes will be conducted remotely (with an approximate ratio of 1:1 between in-class and remote lessons).

Assessment methods

The exam will take place in one day and will be divided into two sections:
- in the first section, responses to open-ended questions on the course topics will need to be written;
- in the second section, at the computer, setting up a simulation study in SAS will be required.
In both sections, consulting any type of material or accessing the web will not be allowed.

Textbooks and Reading Materials

The course material (book excerpts, articles, SAS code, datasets) will be distributed during the course.

Semester

Semester I, cycle I

Teaching language

Italian

Sustainable Development Goals

GOOD HEALTH AND WELL-BEING