UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

SYLLABUS DEL CORSO

Modelli Statistici Applicati alle Sperimentazioni Cliniche

2122-2-F8203B036

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;
- Clinical trial phases.

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

- Data simulation in SAS

- Poisson and Negative binomial regression for repeated-events analysis
- Sample size and statistical power of a clinical study: a simulation approach
- An introduction to the clinical trial phases
- How to analyze controlled trials with baseline and follow-up measurements
- Analysis of clustered data

Detailed program

1. Data simulation with SAS

1.1 Simulation as an important tool for biostatisticians

1.2 Data simulation basic techniques

1.3 Use of simulations to evaluate sample distributions, validity of statistical techniques and properties of a statistical design

2. Analysis of discrete outcomes

1.1 Poisson regression and negative-binomial regression for the analysis of repeated events

1.2 Poisson regression and negative-binomial regression for the analysis of single events

1.3 Poisson model and survival analysis

3. Statistical power and sample size of a clinical study: a simulation-based approach

4. Clinical research methodology

4.1 Statistical models for phase I, II and III clinical trials

4.2 Superiority and non-inferiority trials

4.3 Adaptive trials

5. Analysis of pre-post studies

6. Analysis of correlated data

6.1 Introduction to correlated data

6.2 Linear mixed models for correlated continuous outcomes

6.3 Generalized linear mixed models for correlated binary outcomes

Prerequisites

None

Teaching methods

Lectures

Computer lab with applications in SAS

Assessment methods

Written exam – project work (only for attending students)

During the course a couple of biostatistical problems that could be solved by means of a simulation study will be proposed.

The simulation study will be set up in the classroom by working groups of two people. Then, each student will choose and deepen one of the proposed problems at home.

A written final report with the structure of a research paper should be provided.

There are no intermediate exams.

The written exam will test the student's ability to set up a research work in a group and then independently develop a document that describes the data used, the aims of the study, the justification of the used methods, and the critical discussion of the results.

Oral exam

The oral exam will be based on all the materials (book excerpts, research papers, slides) provided during the course.

The oral exam will test the student’s knowledge of the main methods used in the biomedical field to plan and analyze clinical studies.

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