UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

COURSE SYLLABUS

Statistical Models Applied To Clinical Trials

2223-2-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 test with optional oral exam.
The written test will be divided into two sections: the first will require to answer open-ended questions on topics covered in the course, while the second section will propose some exercises based on actual or simulated data to be solved while using the SAS software.
In the first part it will not be possible to consult any type of material, while in the second part it will be possible to consult both the web and all the material made available during the course, including the SAS codes.
The optional oral exam is only accessible to those who successfully complete the written test with a score of at least 18/30.
If the student decides to take the oral exam, passing the written test does not guarantee the passage of the exam.

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