

# UNIVERSITÀ DEGLI STUDI DI MILANO-BICOCCA

## **SYLLABUS DEL CORSO**

## Clinical Research

2526-4-H4102D059-H4102D198M

#### **Aims**

The course aims to explore theoretical and practical aspects of the statistical analysis of clinical data with a particular focus on the application of causal inference methods to observational studies with survival outcomes.

#### Knowledge and understanding

At the end of the course the student should know the fundamental concepts about:

- · methods to describe survival outcomes
- methods to assess the association between an exposure and a survival outcome
- basic concepts in causal inference
- methods to assess the marginal treatment effect in observational studies

#### Applying knowledge and understanding

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

- estimate and compare survival functions using non-parametric methods (Kaplan-Meier estimator and Log-Rank test)
- fit a Cox regression model and interpret the coefficients
- apply propensity score based methods to assess the marginal treatment effect in observational studies

## Making judgements

The student should be able to select the study design depending on the aim and to choose the proper statistical analysis method depending on the nature of data. Furthermore, the student should be able to understand and evaluate, from a statistical point of view, the solidity of findings of published studies.

Communication skills

The student will be able to correctly report the results of a study, justifying the methods used for the statistical analysis.

#### **Contents**

The course will explain basic concepts in survival analysis, main quantities of interest and non-parametric estimators. Cox regression model.

Furthermore, an introduction to causal inference methods to assess the association between a (binary) exposure and a survival outcome in observational studies will be provided.

Real examples will be considered and practical guidance on the application of the methods will be provided. Analysis with R software will be shown to demostrate the application of the methods.

# **Detailed program**

#### Introduction

Recap on basic concepts in statistics (study designs, desccriptive methods, statistical inference, regression methods).

#### Review of survival analysis

Basic theory in survival analysis: complexities of life time data, survival/incidence functions, rate, hazard function, Kaplan Meier estimator, epidemiological rate (exponential) estimator, Cox regression model.

#### Introduction to causal inference

Basic concepts in causal inference: counfounders bias, effect modification, Direct Acyclic Graphs (DAGs), Average Treatment Effect (ATE)

Causal inference methods: Propensity Score (PS), PS-matching, PS-weighting (Inverse Probability Weighting IPW)

#### Additional content (not mandatory)

R commands to apply causal inference methods for the estimation of a marginal treatment effect on real data with survival outcome

#### **Prerequisites**

· Basic descriptive and inferential statistics.

#### **Teaching form**

Lectures and R labs in presence.

# Textbook and teaching resource

Course slides, datasets and R lab commands and outputs will be available on the elearning page.

#### Semester

Second semester

#### **Assessment method**

Written exam with multiple choice questions. Optional oral exam, with the discussion of the written exam.

## Office hours

Upon request by email, in the Webex room of the teacher.

# **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING