



**UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA**

SYLLABUS DEL CORSO

Sistemi Modello di Malattia Umana

2627-1-F0602Q123

Aims

This advanced course will familiarize students with a diversity of advanced in vivo and in vitro models of human disease, to understand and enable their use in the understanding of disease mechanisms, and the consequent design of therapy approaches.

1. Knowledge and ability to understand: the course will provide knowledge and understanding related to the development of in vitro and in vivo models for the study of the mechanisms at the basis of human disease.
2. Ability to apply knowledge and understanding: the student will apply knowledge and understanding acquired to the study of specific examples presenting the use of model systems for the investigation of frontier problems connected to the understanding of human disease, as illustrated by original scientific papers
3. Autonomy of judgement: the student will develop autonomous critical sense by the study of original papers and their critical discussion with the class
4. Communication abilities: the students will be invited to present a synthesis of scientific papers connected to the problems treated in the course, by a presentation to the class followed by discussion
5. Learning ability: The student will develop the ability to learn in a problem-solving way, understanding how the development of a model is experimentally realized

Contents

The course will introduce animal models, in particular mouse and zebrafish, with hints at *Drosophila* and *C. elegans*, and cellular models, mostly those based on human pluripotent stem cells, embryonic (human embryonic stem cells, hESC) and obtained by reprogramming (human induced pluripotent stem cells, hiPSC). Transversally

through the models, approaches will be presented for their genetic manipulation by gene targeting and genome editing, transgenesis, viral transduction, transfection, as a basis for both the generation of the models themselves, and their experimental study with understanding and therapeutic objectives.

Detailed program

The mouse as a model, and targeted genome modification of the mouse genome in disease modeling: knock-out, knock-in of pathological mutations, conditional knock-out, genome editing, transgenesis, to understand specific diseases, with special attention to how these allowed the understanding of mechanisms of pathology, and, in some cases, the development of targeted therapies.

Zebrafish as a model system, and the modification of the zebrafish genome by transgenesis and germline modification, yielding a vertebrate model to follow embryonic development in detail in a free environment.

Screening of gene function in animal and cellular models

Human pluripotent stem cells, reprogramming and their differentiation to specific cell types and organoids relevant for the disease

Genetic manipulation of pluripotent stem cells and organoids

Prerequisites

A basic very good knowledge of molecular and cellular biology and genetics is required.

Teaching form

Lessons in class; around 4 lessons, for a total of 8-10 hours, will be devoted to interactive activities, by the preparation of slides presentations regarding original research papers, to be discussed with the class.

Textbook and teaching resource

Original scientific papers and presentations.

Semester

Second semester

Assessment method

Oral exam. Previous oral presentations done to the class (if done) will be part of the evaluation. The exam will begin with the discussion of a problem chosen by the student, and will continue with two more questions on

different subjects treated during the course. No intermediate evaluations are foreseen.

Office hours

By appointment, write to sara.mercurio@unimib.it.

Sustainable Development Goals

GOOD HEALTH AND WELL-BEING
