



UNIVERSITÀ  
DEGLI STUDI DI MILANO-BICOCCA

## SYLLABUS DEL CORSO

### Sistemi Modello di Malattia Umana

2425-1-F0601Q123

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#### 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.

#### Contents

The course will introduce animal models, in particular mouse and zebrafish, with hints at Drosophila, 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

Screening of gene function in organoids

Modeling of the interaction between tissue cells and the immune system in organoids

## **Prerequisites**

A basic very good knowledge of molecular and cellular biology is required, such as that obtained in the course of Genetics of development and differentiation (Laurea magistrale, Masters equivalent) and, prior to that, in the courses of Molecular and cellular biology and Genetics of our Laurea triennale (Bachelors' equivalent).

## **Teaching form**

Lessons in class; about 2 cfu 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

## **Semester**

Second semester

## **Assessment method**

Oral exam

## **Office hours**

By appointment, write to [silvia.nicolis@unimib.it](mailto:silvia.nicolis@unimib.it)

## **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING

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