UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

COURSE SYLLABUS

Genetic Disease Therapies

2223-1-F0601Q116-F0601Q120M

Aims

Common Aims
The course will be multidisciplinary and organized in modules in order to offer an overview of genetic, molecular, biochemical, physiological and pharmacological aspects of a human disease.
The focus of the course will be the study, from different perspectives, of a genetic disease, from diagnosis to treatment.
In particular, at the end of the course the student will have acquired the following skills:

1. knowledge and understanding: complete overview with different integrated approaches of a specific human genetic disease
2. applied knowledge and understanding: apply what has been learned to the study of numerous human genetic diseases
3. self-judgment: capacity to critically evaluate what has been learned
4. oral competences: oral communication of what has been learned using the correct scientific terminology
5. ability to learn: critical learning and understanding of scientific literature on different aspects of a human disease.
   In the academic year 2022-2023 the genetic disease studied will be Cystic Fibrosis.

Specific aims
For the Pharmacology module (1 CFU), the student will learn about the actual and innovative therapeutic strategies: from the drugs defined as molecular 'corrector' and 'potentiators', to the more recent gene-therapy and cellular-therapy approaches. Further aim will be the analysis of the models currently employed to screen the pharmacological tools.

Contents

The course is organized into lectures, group studies and student presentations on the new therapeutic options for Cystic Fibrosis

Detailed program

The pharmacological issues will be:

- corrector and potentiator drugs
- gene therapy (ODN, gene replacement, gene addition)
- cell-based therapy (stem cells)
- models for the pharmacological screening (organoids, ko mice)

Prerequisites

Basic knowledge of General Pharmacolgy

Teaching form

Lectures, group assignments, students presentations of scientific papers

Textbook and teaching resource

Powerpoint presentations will be available on e-learning

Semester

Second

Assessment method

Written exam.
The exam of the pharmacology module is part of the general exam of the course that will assess the learning of the material discussed in all the modules.
The written exam is organized in:

1. 30 multiple choice questions on the material discussed in the different modules in 30 minutes
2. 1 open question to answer on the material covered in one module in 15 minutes
   A passing grade on part 1) is required to be able to access part 2) of the exam. The exam will be available

on the esamionline platform.

Office hours

Appointment via e-mail

Sustainable Development Goals

GOOD HEALTH AND WELL-BEING