

## SYLLABUS DEL CORSO

### Terapie della Malattia Genetica

2425-1-F0601Q116-F0601Q120M

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#### 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 2024-2025 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 Pharmacology

## **Teaching form**

The course required attendance (10 hours/1 CFU).

It consists in lessons composed by:

- a section of delivered didactics (Didattica erogativa, DE) focused on the presentation of contents by the lecturer.
- a section of interactive teaching (Didattica Interattiva, DI) including integrative didactic interventions by external experts, group works, reverse teaching with the student's personal involvement.  
Visits to centers of excellence in the study of the pathology under consideration are also planned.

## **Textbook and teaching resource**

Powerpoint presentations will be available on e-learning

## **Semester**

Second

## **Assessment method**

The verification and evaluation of the student aims to be \* innovative \* compared to the classic methods.

The exam consists in the elaboration of the knowledges acquired during the course through a poster presentation, similar to what is carried out in scientific meetings.

The student will be evaluated by a commission in terms of: 1) expository skills on what is illustrated in the poster, 2) general knowledge of the pathology treated in the course, 3) first-hand participation in the activities carried out during the course, 4) graphic/display characteristics of the poster.

There are no ongoing tests.

## **Office hours**

Appointment via e-mail ([barbara.costa@unimib.it](mailto:barbara.costa@unimib.it)).

## **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING | QUALITY EDUCATION

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