UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

# SYLLABUS DEL CORSO

# Cellular and Gene Therapies From Bench To Bedside

**2526-3-H4102D134**

## Aims

The course aims to introduce the concept of "advanced therapies" or "ATMPs" (Advanced Therapy Medicinal Products), starting from the molecular bases that characterize cell and gene therapy up to their use in clinical applications. ATMPs are biological medicines obtained by processing/manipulating genes, tissues or cells. Given their nature, they fall into the branch of personalized medicine, precisely because these drugs are "built" on the clinical history of individual patients. The course will focus on the biological foundations of stemness, pluripotency, plasticity and inducibility of cells and the morphological, genetic and use characteristics of viral vectors and recent genome editing techniques.
The aim of the course is to introduce students to the application potential of biotechnological and medical innovations, bringing them closer to a sector still considered "frontier". We also aim to highlight the new concept of drug and therapy that derives from the cell and gene therapy approaches implemented in the last two decades, both in a positive and negative sense. Furthermore, it is intended to stimulate a critical sense towards the regulatory and legislative aspects that regulate it.

**Knowledge and Understanding** – By the end of the course, the student will have acquired advanced knowledge and critical understanding of preclinical and clinical tools in translational medicine based on cell therapy, immunotherapy, and gene therapy.

**Applying Knowledge and Understanding** – By the end of the course, the student will be able to interpret experiments and clinical trials in oncohematology, understand how preclinical results can be translated into innovative clinical strategies, and apply the acquired translational concepts to the design and development of new targeted therapies.

**Judgement Autonomy** – By the end of the course, the student will be able to critically evaluate scientific literature, interpret and comment on data from clinical sources and immunotherapy/gene therapy trials with a critical mindset, and formulate original hypotheses in the field of translational research.

**Communication Skills** – By the end of the course, the student will have acquired appropriate terminology to clearly and coherently present scientific data to specialist audiences. They will also be able to effectively

communicate research findings in clinical settings and engage in discussions on the proposed topics.

## Contents

The course offers a series of lessons in which learning the main cell and gene therapy approaches, focusing on innovative translational therapies that have become a clinical reality from the laboratory bench. Aspects relating to the preclinical development of a cell therapy product based on stem cells (isolated from bone marrow or cord blood) and gene therapy based on cells engineered to be directed against a pathogenetically relevant molecular target (CAR-T cells) will be illustrated. Examples of clinical applications of these approaches in the field of oncology and rare genetic diseases will be illustrated, also focusing on the procedures relating to the production of these cellular products.

## Detailed program

The program aims to lead the student to:
a) learn methodologies, technical requirements and clinical application principles for the preparation of cell and gene therapy products;
b) learn the biological, morphological and functional characteristics of cell types (specifically stem cells and T lymphocytes derived from different biological resources, such as bone marrow, peripheral blood, cord blood) and viral vectors used in advanced therapies;
c) critically compare the advantages, disadvantages and risks of cellular and gene therapeutic applications, also in relation to reference therapies;
d) know the general rules for regulating the definition, production and use of products for advanced therapies.

## Prerequisites

For the purposes of complete understanding of the topics covered in the course, basic knowledge of cell biology, pathology, immunology and physiology is to be considered particularly useful - although not mandatory.

## Teaching form

The in-person learning lessons are intended as frontal hours during which the subject is carried out using PowerPoint-type presentations.

## Textbook and teaching resource

For each explained topic, articles in scientific journals that can be consulted will be indicated in class. Some relevant publications and slides (in PDF format) of the lessons will be uploaded on the course page.

## Semester

Second semester

## Assessment method

Course attendance

## Office hours

Request an appointment with the teacher via email (marta.serafini@unimib.it)

## Sustainable Development Goals

GOOD HEALTH AND WELL-BEING