UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

COURSE SYLLABUS

Cellular and Gene Therapy

2425-2-F0901D048-F0901D081M

Aims

1. Stem cell biology: classification and functional characterization
2. Hemopoietic stem cell transplantation as the best success of stem cell therapy.
3. Cell therapies and regenerative medicine.
4. Cell therapy in cancer, from discovery to the application in the clinic.
5. Monoclonal antibodies: from Koehler & Milstein up to now, a masterpiece in biotech therapy.
6. Introduction to gene therapy: the viral and non-viral vectors/successes and challenges in gene therapy.
7. The concept of ”Good Manifacturing Practices, GMP”: how cellular or gene therapy products (es. CAR-T cells) become a drug.

Contents

The course aims to provide students with a review of selected topics relating to the most relevant biotechnological applications resulting in innovative therapies. The two main pillars consist of development of therapy with somatic and/or genetically modified cells and molecular treatment.
The first part will present aspects of the pre-clinical development of a cell therapy product with somatic and/or genetically modified cells. Examples of applications in the oncology field and in the field of tissue regeneration will be illustrated. Particular attention will be given to the illustration of the procedures and challenges relating to production in "Good-Manufacturing practice-GMP" conditions. The second part will cover the development of a drug directed against a pathogenetically relevant molecular target (CAR-T cells), from its identification using the most advanced array and screening technologies, the synthesis of molecules capable of functionally interfering with it and its validation in in vitro systems and in preclinical models in oncological diseases. Significant examples of applications in the field of tumor diseases will be presented.

Detailed program

Introduction to stem cells: classification and characterization.
New therapeutic perspectives in the use of stem cells.
Induced Pluripotent Stem cells (iPS) and embryonic stem cells: alternative or complementary?
The normal and pathological hematopoietic stem cell niche
Mesenchymal stem cells in the osteo-articular tissue repair.
Monoclonal antibodies in therapies: from Koehler & Milstein to the clinic, an educational journey
Cellular therapies in the antitumoral therapies.
Cellular therapies and immunoregulation.
Gene transfer using non-viral vectors.
The transposons, new methods of gene manipulations.
Viral vectors for the gene therapy.
Gene therapy in non-oncological pathologies, a look at rare genetic diseases.
Cell and gene therapy in Mucopolysaccharidosis type I (MPS-I): from preclinical model to patient.
Gene therapy in oncological diseases.
CAR-T cells, from a biotechnological approach to a clinical reality
Preparation, engineering and characterization of CAR-T cells (in the laboratory)

Prerequisites

Basic knowledge in pathology and immunology. Advanced knowledge in biochemistry, molecular biology and genetics.

Teaching form

The course is divided into 12 in-person learning lessons of 2 hours each and 4 in-person interactive lessons of 2 hours each.

The in-person learning lessons are intended as frontal hours during which the subject is carried out using PowerPoint-type presentations. During the course, students will also be offered interactive lessons in which they will be able to present a critical review of an article taken from a scientific journal in the classroom; this non-mandatory presentation can contribute to the overall evaluation of the student. The articles will be proposed by the teacher.
The course also includes an interactive lesson in the laboratory, where the main technologies used in the production of CAR-T cells will be explained (starting from a peripheral blood bag to obtain a cell-drug for the patient).

Textbook and teaching resource

Updated reviews on all topics will be suggested at each lesson.
On the course page will be uploaded some relevant publications, and the slides (in PDF fromat) of the lessons.

Semester

First semester.

Assessment method

Written exam: an open-ended question.
Final oral exam (in English) with the presentation and the discussion of a scientific article.

Office hours

Make an appointment by email to the teacher, Professor Marta Serafini (email address: marta.serafini@unimib.it)

Sustainable Development Goals

GOOD HEALTH AND WELL-BEING