



UNIVERSITÀ  
DEGLI STUDI DI MILANO-BICOCCA

## SYLLABUS DEL CORSO

### Cellular and Gene Therapy

2223-2-F0901D048-F0901D081M

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#### Aims

1. Stem cells biology; Hemopoietic stem cell transplantation as the best success of stem cell therapy; stem cells and tissue regeneration (cardiovascular, orthopaedic, etc.)
2. Cell therapy in cancer and immunocompromised hosts; immunoregulatory cells: from discovery to application in the clinic.
3. Monoclonal antibody: from Koehler & Milstein up to now: a masterpiece in biotech therapy.
4. Introduction to gene therapy; the viral and non-viral vectors; successes and problems in gene therapy.
5. The concept of GMP production: how a cellular or a gene products become a drug.

#### Contents

The aims of the Course is to provide an overview of the current and most relevant applications of biotech in the development of new treatment strategies. The two tracks of the course include the targeting treatment and the development of cellular and gene therapy. The first part will cover the process of identification of new potential targets for treatment by using high-throughout technologies , the screening of active molecules and the preclinical and clinical development. Diseases in the field of cancer will be taken as cases in point. The second part will present the pre clinical and clinical development of a product for cellular and gene therapy in the field of cancer, treatment of infections in immuno-compromised hosts, and tissue regeneration. Emphasis will be given to the knowledge of the process of production under "GMP" conditions.

## **Detailed program**

New perspectives in the use of stem cells

Cellular therapies in the antitumoral therapies

Cellular therapies and immunoregulation

Anti-infectious cellular therapies in the patient with immunodeficiency

Non viral vectors for the gene therapy

The transposons, new methods of gene manipulations

Viral vectors for the gene therapy

Introduction to stem cells

IPS and embrional stem cells: alternatives or complimentary?

Gene and cell therapy for MPS-I: from pre-clinical modelling to patient.

Stem cells and heart: : what is next?

The gene therapy in the non oncological pathologies

Mesenchimal stem cells in the osteo-articular tissue repair

Monoclonal antibodies in therapies: from Koehler & Milstein to the clinic: a useful way

## **Prerequisites**

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

## **Teaching form**

Lessons in attendance, subject to any ministerial changes following the COVID pandemic situation

## **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

## **Semester**

First semester

## **Assessment method**

Written exam: a question with opened answer

Final oral exam with the presentation of a scientific article

## **Office hours**

Contact the teacher by email

## **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING | QUALITY EDUCATION | GENDER EQUALITY

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