



UNIVERSITÀ  
DEGLI STUDI DI MILANO-BICOCCA

## SYLLABUS DEL CORSO

### Translational Approach To Onco-Hematological Diseases

2627-2-F0902D013

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#### Aims

The course aims to illustrate the technologies and approaches based on molecular therapy, immunotherapy, and gene therapy currently adopted in preclinical and clinical practice for the treatment of various oncological and non-oncological diseases. Particular emphasis will be placed on the development pathway of these approaches from the laboratory to the clinic, highlighting the crucial role of the biotechnologist in the design and implementation of such strategies.

**Knowledge and Understanding** – By the end of the course, the student will have acquired advanced knowledge and a critical understanding of the molecular and cellular mechanisms underlying oncohematological diseases, as well as the tools of translational medicine based on molecular therapies, cell therapies, 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, translate preclinical results into innovative clinical strategies, and apply acquired translational concepts to the design and development of new targeted therapies.

**Making Judgements**– By the end of the course, the student will be able to critically assess scientific literature, interpret and discuss data from clinical sources and from molecular and cellular biology experiments, and formulate original hypotheses in the field of translational research.

**Communication Skills** – By the end of the course, the student will have acquired appropriate scientific terminology and will be able to clearly and effectively present scientific data to specialized audiences. The student will also be able to communicate research findings in clinical settings and actively participate in discussions on course topics.

**Learning Skills** – By the end of the course, the student will have developed the ability to independently stay updated on the latest innovations in oncohematology, as reported in scientific journals, thereby broadening their professional and scientific competencies.

The topics covered in the course are as follows.

1. Stem cell biology; hemopoietic stem cell transplantation as the best success of stem cell therapy. Cell therapy and regenerative medicine.
2. Cell therapy in cancer, from discovery to application in the clinic.
3. Monoclonal antibodies: from Koehler & Milstein up to now, a masterpiece in biotech therapy.
4. Introduction to gene therapy; the viral and non-viral vectors; successes and challenges in gene therapy.
5. The concept of "Good Manufacturing Practices, GMP": how cellular or gene therapy products (es. CAR-T cells) become a drug.
6. Use of TKIs in different neoplastic diseases
7. Mechanisms of resistance to TKIs
8. Methods to identify and analyze genetic lesions causally connected to the transformed phenotype
9. DNA and Histone methylation as therapeutic targets
10. The RNA interference targeting strategy
11. High Throughput Sequencing applied to neoplastic diseases

## **Contents**

The course aims to provide students with a review of selected topics relating to the most relevant biotechnological applications resulting in innovative therapies in the oncology-hematology field. The main pillars consist of the description of the development of therapies with somatic and/or genetically modified cells, molecular therapies and targeting strategies using "small molecules". Furthermore, students will be able to learn the importance of the relationship between targets and neoplastic transformation.

## **Detailed program**

The extended course program is contained in the individual modules of Cell and Gene Therapy and Molecular and Oncological Therapy.

## **Prerequisites**

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

## **Teaching form**

Teaching form

In-person learning and interactive lessons.

The course is divided into 12 in-person learning lessons of 2 hours each and 4 in-person interactive lessons of 2 hours each for the Cellular and Gene Therapy Course and into 6 in-person learning lessons of 2 hours each and 2 in-person interactive lessons of 2 hours each for the Molecular and Oncological Therapy Course.

## **Textbook and teaching resource**

For each topic, updated reviews will be indicated in class on which to orient your study.  
Some relevant publications and slides (in PDF format) of the lessons will be uploaded on the course page.

## **Semester**

First semester

## **Assessment method**

Written exam: two open-ended questions (a question related to the Cellular and Gene Therapy Course and a question related to the Molecular and Oncological Therapy Course).

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 (email address: [marta.serafini@unimib.it](mailto:marta.serafini@unimib.it) and [rocco.piazza@unimib.it](mailto:rocco.piazza@unimib.it))

## **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING

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