



UNIVERSITÀ  
DEGLI STUDI DI MILANO-BICOCCA

## SYLLABUS DEL CORSO

### Farmacologia Applicata

2122-1-F0802Q059

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#### Aims

The course aim to discuss the principles and the practices of two pharmacological disciplines: pharmacogenetic and gene therapy. The course provides the knowledges of how some polymorphism can affect individual responses to drugs, both in terms of therapeutic effect as well as adverse effects, thus leading the clinicians to the application of the personalized medicine. The second part of the course aim to details the actual protocol of gene therapy through various examples of the use of nucleic acids as alternative to conventional drugs for the treatment of different human pathologies.

Knowledge and understanding.

At the end of the course the student will have acquired the knowledge about the relationship between genetic and drug response and regarding the application of nucleic acid as drugs;

Applying knowledge and understanding.

The student will be able to propose pharmacological strategies applied to personalized medicine and to propose gene therapy protocols.

Making judgements.

The student will be able to propose alternatives to the current pharmacological strategies on the basis of the knowledges acquired during the course.

Communication skills.

At the end of the course the student will have acquired adequate pharmacological language through which he'll be able to describe the knowledge.

Learning skills.

the student will have expertise useful to be applied in other pharmacological studies or in research project.

## Contents

1) Pharmacogenetics: the genetic basis of drug responses. Polymorphisms of genes encoding proteins involved in pharmacokinetics and gene variants of genes encoding the molecular target of drugs.

2) Gene therapy: use of nucleic acids as drugs. Therapeutic genes, antisense oligonucleotides, ribozymes and aptamers. Additive therapy and ablative therapy with various application examples.

## Detailed program

### 1) Pharmacogenetics

Polymorphisms in the genes encoding for the biotransformation enzymes of phase I and II: cytochrome P450 (unexpected pharmacological responses to NSAIDs, codeine, warfarin), thiopurine methyltransferase (the example of thiopurine antitumor drugs). Genetic polymorphisms in the genes encoding for the primary therapeutic targets of drug action: the beta-adrenergic receptor and the failure in the treatment of bronchial asthma, the serotonin transporter and the example of antidepressant drugs. The genetic polymorphism in the phenomenon of addiction.

### 2) Gene therapy

The treatment of hereditary diseases through conventional therapy and through additive gene therapy: SCID, cystic fibrosis, hemophilia, muscular dystrophies. Ischemic diseases: conventional preventive and surgical therapy and innovative therapy through the administration of VEGF. Gene therapy in the treatment of neurodegenerative diseases (Alzheimer, Parkinson). Infectious diseases: the example of AIDS (antiviral drugs and ablative strategies of viral gene expression). Conventional cytotoxic anticancer drugs and antineoplastic immunotherapy and gene therapy applications (suicide gene therapy, p53 as a therapeutic gene).

## Prerequisites

Background. Basic knowledge of general pharmacology and molecular biology.

Prerequisites. None

## Teaching form

Classroom lectures including the analysis and discussion of literature and team work concerning simulation of gene therapy protocols.

Teaching language: Italian.

## Textbook and teaching resource

Slides. Available at the e-learning platform of the course.

Bibliography. Reviews and research papers available at the e-learning platform of the course.

## Semester

Second semester

### **Assessment method**

Oral examination. The questions aim to assess the acquisition of the basic knowledge and to evaluate the concepts comprehension, the ability to connect the different issues and the ability to discuss about a pharmacological problem. One question regarding pharmacogenetics and one question regarding gene therapy

### **Office hours**

Contact: on demand by mail to the lecturer.

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