MODELLO SCHEDA INSEGNAMENTO

Corso di LM	**BIOLOGIA**
Denominazione insegnamento:	**GENE THERAPY AND STEM CELL**
Numero di Crediti:	**6**
Anno	**I**
Semestre:	**II**
Docente Titolare:	**AMBROSINO CONCETTA**
Dottorandi/assegnisti di ricerca che svolgono attività didattica a supporto del corso:	**-**
Orario di ricevimento:	**11-13, MARTEDI'**
Indirizzo:	**VIA PORT'ARSA, 11**

PRESENTAZIONE DEL CORSO:

The course has the primary purpose to remind and deepen the knowledge of different cellular processes and their regulation as basic tools for the development of new therapeutic approaches such as gene and cellular therapy. The main goal of the course is to provide students with the knowledge tools necessary to understand the theoretical/practical mechanisms that are under the development of the modern techniques of gene and cell therapy. Strong emphasis will be placed on the molecular therapeutic protocol chosen in relation to the chosen gene transfer systems, the adopted cellular systems and the pathological alterations discussed.

GLI OBIETTIVI FORMATIVI

The main goal of the course is to offer to the student the possibility to build a conceptual framework to critically evaluate the new scientific concepts at the basis of new therapeutic approaches for mono-factorial (cystic fibrosis, etc.) and poly-factorials (cancer, etc.) genetic diseases and on biotechnological approaches that can be used to this purpose.

Aim 1: Acquisition of knowledge of the relevant methods, instrumentation and basic and avant-garde procedures used for experimentation in the field. Understanding of the biological bases of gene therapy, the importance of biology in the development of new gene transfer methods and the regulation of transgene expression.

Aim 2: Understanding mechanisms of cell-cell and cell-microenvironment communication. Particular attention will be paid to emphasize how the relationship between cells and microenvironmental

factors essential for a correct function of the cell itself, its differentiation and its use in cell therapy.

Aim 3: To equip students with the knowledge tools needed to understand the main current biological problems and the modern methods in biology.

PREREQUISITI RICHIESTI

Basics aspect of biology.

FREQUENZA DELLE LEZIONI

Although not obligatory, it is advisable to attend the course in order to be routed in the study of a vast and heterogeneous subject for which it is not possible to identify a single textbook. The course is structured to guide the student through different topics by placing them in different physio-pathological contexts.

CONTENUTI DEL CORSO

• General aspects of cellular and molecular therapy. Characterization of the tools required for gene eand cellular therapy: vectors, transgenes and cell cultures (definition and characterization of primary and line cell cultures and molecular aspects of the cell immortalization process).

• Recombinant DNA and gene transfer systems: Applications of naked plasmids, adenoviral vectors, AAV vectors, retroviral and lentiviral vectors.

• Cellular models and gene transfer in: gene therapy of metabolic diseases, congenital immunodeficiency gene therapy, gene therapy of solid tumours.

• Cellular and regenerative therapy: stem cells, symmetrical and asymmetric cell division, definition of the characteristics of embryonic and adult stem cells and their use in cell therapy, hematopoietic stem cell, mesenchymal stem cell.

METODI DIDATTICI

Frontal lesson.

TESTI DI RIFERIMENTO

Giacca- Gene Therapy- Springer Verlag (2010)

Alberts – Biologia Molecolare della Cellula VI edizione - Zanichelli

ESAME DI PROFITTO

The final exam will consist of an oral test evaluated considering the quality of the content and the relevance of the answers. Particular attention will be paid to the ability to link the different topics and to the technical language skill of the student.

Intercourse test II° week of may (II semester)

CALENDARIO ESAMI

Go to the link

PRENOTAZIONE ESAMI

Go to the link

SYLLABUS

MODELLO SYLLABUS

Argomenti	Ore	Riferimenti bibliografici	Tipologia di lezione
General aspects of cellular and molecular therapy. Characterization of its basic tools for its realization: vectors, transgenes and cell cultures	6	**Giacca- Gene Therapy- Springer Verlag (2010)**	Frontal lesson
Recombinant DNA and gene transfer systems: Applications of naked plasmids, adenoviral vectors, AAV vectors, retrovirus and lentivirus.	16	**Giacca- Gene Therapy- Springer Verlag (2010)**	Frontal lesson
Terapia genica di malattie metaboliche, di immunodeficienze congenite e di tumori solidi	8	**Giacca- Gene Therapy- Springer Verlag (2010)**	Frontal lesson
Gene therapy of metabolic diseases, congenital immunodeficiency and solid tumours	8	**Alberts – Biologia Molecolare della Cellula VI edizione e materiale fornito al corso**	Frontal lesson
Stem cells and their therapeutic use in regenerative medicine	10	**Alberts – Biologia Molecolare della Cellula VI edizione e materiale fornito al corso**	Frontal lecture