

**COURSE DATA****Data Subject**

Code	34510
Name	Genetic and cellular therapy
Cycle	Grade
ECTS Credits	4.5
Academic year	2023 - 2024

Study (s)

Degree	Center	Acad. year	Period
1204 - Degree in Medicine	Faculty of Medicine and Odontology	5	Second term

Subject-matter

Degree	Subject-matter	Character
1204 - Degree in Medicine	18 - Optional subjects	Optional

Coordination

Name	Department
ALIÑO PELLICER, SALVADOR FRANCISC	135 - Pharmacology

SUMMARY**English version is not available**

El objetivo de esta materia es desarrollar el conocimiento y la capacidad de trabajo y comunicación en el ámbito del análisis de la información actualizada en los diferentes aspectos de la terapéutica. La incorporación de las nuevas tecnologías de la información, comunicación y búsqueda bibliográfica contribuirán a dichos objetivos. Entre las actividades formativas se incluirán aspectos relacionados con el desarrollo de las células y genes como herramientas terapéuticas, así como interpretación de los efectos de estos procedimientos y seminarios especiales destinados al estudio de aspectos terapéuticos puntuales.

La terapia Génica y Celular se encuentra en la actualidad en una fase de importante desarrollo traslacional y ha demostrado su interés terapéutico en un número significativo de patologías graves, para las que no existen terapias curativas alternativas.



PREVIOUS KNOWLEDGE

Relationship to other subjects of the same degree

There are no specified enrollment restrictions with other subjects of the curriculum.

Other requirements

It is recommended to have completed the following subjects: Anatomy, Biology, Biochemistry, Physiology, General Pharmacology and General Pathology

OUTCOMES

LEARNING OUTCOMES

Upon completion of this course the student should be able to:

- 1) Know the scientific bases on which gene and cell therapy is based.
- 2) Understand the biological aspects of genes and cells in their interaction with the human body.
- 3) Reason the influence of genes and cells on the organism.
- 4) Understand the bases of the action of genes and cells on the physiopathology of the human being.
- 5) Reason what effects it will have in a hypothetical therapeutic application and which ones will be interpreted as

Adverse reactions, depending on the patient.

- 6) Know the bases of possible interactions between different strategies in the organism, with the objective of its provision in medical practice

DESCRIPTION OF CONTENTS

1. THEORETICAL UNITS

1. Advanced therapies. Background and current status.
2. Cell types for transplantation. Adult and embryonic stem cells.
3. Nuclear transfer. Regenerative medicine.
4. Mitochondrial transfer: therapeutic interest.
5. Therapeutic nucleic acids.
6. Gene silencing and therapeutic strategies.



7. Gene editing and repair.
8. Gene implementation: strategies and vectors.
9. Viral gene therapy vectors.
10. Non-viral gene therapy vectors.
11. Vector targeting and conditional gene expression.
12. Genetic vaccines and immune tolerance.
13. Gene and cell therapy of hereditary diseases.
14. Gene and cell therapy of hematological diseases.
15. Cell and gene therapy for oncological diseases.
16. Gene and cell therapy of acquired diseases.
17. Gene and cell therapy for degenerative diseases.

2. PRACTICES

SEMINARS:

1. Organization and functions of the genome.
2. Gene drugs. Development.
3. Genes and cells as drugs: mitochondrial therapy
4. Ethical aspects of gene and cell therapy.
5. Gene Implementation and Clinical Use
6. Gene silencing and clinical use
7. Hereditary diseases and clinical use
8. Hematological diseases and clinical use
9. Oncological diseases and clinical use
- 10 Immunotherapy, Acquired Diseases, and Clinical Use

NOTE: In each seminar, the issues proposed in the theoretical classes taught previously will be discussed. Timely current examples of diseases with their corresponding gene therapy approved for clinical use by the EMA (European Medicines Agency) will be included.

WORKLOAD

ACTIVITY	Hours	% To be attended
Seminars	26,00	100
Theory classes	19,00	100
TOTAL	45,00	

TEACHING METHODOLOGY

The teaching methodology contemplates the clinical translation of the theoretical foundations of the subject in two sets of activities:



Theoretical Classes: the teacher introduces the main guidelines of the subject in three large well-differentiated blocks: a) The scope of application (units 1-6), contemplated within the framework of the so-called advanced therapies, aims to establish its potential spectrum of action; b) the scientific foundations and the available tools (topics 7-13) that allow evaluating the benefit/risk of its use for therapeutic purposes; c) The clinical application of gene therapy and

cell in relevant diseases (topics 14-19) by their frequency or severity.

The class begins by exposing its main objectives and ends by establishing a series of questions that students must answer briefly in writing and submit in the next class. This stimulates the critical follow-up of the class by the student and favors their active participation during it, in order to resolve conceptual doubts.

Seminars: at the beginning of the course, the articles (8-10) that must be read, summarized and delivered are delivered in the Virtual Classroom, according to an established agenda and the volunteers who wish to carry out the presentation of the work in class are chosen. , for later discussion.

The seminars are intended to achieve several objectives: a) recover and qualify the student's previous basic knowledge in cell biology, molecular biology and pharmacology to better understand the basis of the design of genes and cells as medicines; b) understand that the development of these new therapeutic strategies can generate important ethical conflicts that the student must know how to identify, explain and analyze objectively; c) that the student acquires the ability and skill to read, understand, present and/or critically discuss published clinical trials on gene and cell therapy.

The development of the seminar contemplates: a) knowing the answers given by the students (chosen by the teacher) to the questions formulated in the theoretical class and agreeing on the answers with the rest of the class; b) raise by the students any other question or doubt and try to solve them by the students themselves, if possible; c) Acquire habits and updating skills in the translational advancement of gene and cell therapy in medical practice.

The gender perspective and the sustainable development goals (SDGs) will be incorporated into teaching, whenever possible.

EVALUATION

THEORY: in the qualification, it represents 50% of the grade. It will be evaluated: either by means of the Final Official Exam, which consists of 15 short questions and 2 topics with limited space on the face of a sheet of paper for each topic, or through Continuous Evaluation, which consists of 3-4 exams that include 5-8 questions. short and can also include a theme, in some of them. The score is: up to 2 points for each short question and up to 5 points for each topic. The grades will be notified simultaneously after the Official Exam.

PRACTICES: 1) They are compulsory; 2) They represent 50% of the final grade; 3) The student will be evaluated continuously: attendance, participation, questionnaires, article summaries, ...



It is a requirement to access the advance call for this subject that the student has completed all of their internships. Attendance at practices will be compulsory.

Students are reminded of the importance of carrying out evaluation surveys on all the teaching staff of the degree subjects.

REFERENCES

Basic

- A guide to human gene therapy. Eds R.W. Herzog, S Zolotukhin. World Scientific Publishing Co. 2010
- Gene Transfer, Gene Therapy and Genetic Pharmacology. Ed. D Scherman. Imperial College Press 2014
- Advances in genetics. Non viral vectors for gene therapy. Physical methods and medical translation. Ed. L. Huang, D. Liu, E.Wagner. Elsevier Academic Press 2015
- CRISPR 101. Ed. Addgene 2021 www.addgene.org
- Antisense RNA design, delivery and analysis. Eds. V. Aarechavala-Gomez, A. Garanto. Humana Press 2022
- Oligonucleotide, Therapy, and Applications. Ed. S.F. Aliño and L. Sendra. IJMS. MDPI 2022 ISBN 978-3-0365- <https://www.mdpi.com/books/pdfview/book/4951>

Additional

- Recursos-e Salut: ClínicaKey Student. Elsevier (Scopus, ScienceDirect). uv-es.libguides.com/RecursosSalut/BibliotecaSalut
- En el comienzo de curso se suministrará información o documentación actualizada (revisiones o artículos) sobre las diferentes partes de la asignatura.