

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

<b>Code</b>	34510
<b>Name</b>	Genetic and cellular therapy
<b>Cycle</b>	Grade
<b>ECTS Credits</b>	4.5
<b>Academic year</b>	2022 - 2023

**Study (s)**

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

**Subject-matter**

<b>Degree</b>	<b>Subject-matter</b>	<b>Character</b>
1204 - Degree in Medicine	18 - Optional subjects	Optional

**Coordination**

<b>Name</b>	<b>Department</b>
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

## COMPETENCES (RD 1393/2007) // LEARNING OUTCOMES (RD 822/2021)

### LEARNING OUTCOMES (RD 1393/2007) // NO CONTENT (RD 822/2021)

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. Genome Organization and functions.
2. Gene drugs development.
3. Characterization of genes and cells as drugs.
4. Ethical aspects of gene and cell therapy.
5. Gene Implementation
6. Gene silencing
7. Hereditary diseases
8. Hematological diseases
9. Oncological diseases

## WORKLOAD

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

## 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.

## 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](http://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](http://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.