

**COURSE DATA****DATA SUBJECT****Code:** 34510**Name:** Genetic and cellular therapy**Cycle:** Undergraduate Studies**ECTS Credits:** 4.5**Academic year:** 2025-26**STUDY (S)**

Degree	Center	Acad. year	Period
1204 - Degree in Medicine	Facultat de Medicina i Odontologia	3	Second quarter

SUBJECT-MATTER

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

COORDINATION

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SUMMARY

The objective of this subject is to develop knowledge and the ability to work and communicate in the field of analysis of updated information in the different aspects of therapy. The incorporation of new information, communication and bibliographic search technologies will contribute to these objectives. The training activities will include aspects related to the development of cells and genes as therapeutic tools, as well as interpretation of the effects of these procedures and special seminars aimed at the study of specific therapeutic aspects.

Gene and Cellular Therapy is currently in a phase of important translational development and has demonstrated its therapeutic interest in a significant number of serious pathologies, for which there are no alternative curative therapies.

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 / LEARNING OUTCOMES

1204 - Degree in Medicine

Students must have acquired knowledge and understanding in a specific field of study, on the basis of general secondary education and at a level that includes mainly knowledge drawn from advanced textbooks, but also some cutting-edge knowledge in their field of study.

DESCRIPTION OF CONTENTS

1. THEORETICAL UNITS

- 1) Background and current status of gene and cell therapy (GCT)
- 2) Somatic gene therapy
- 3) Cell therapy. Cell types
- 4) Nuclear transfer. Regenerative medicine
- 5) Mitochondrial replacement
- 6) Therapeutic nucleic acids
- 7) Gene silencing: ASO, siRNA
- 8) Gene implementation/addition
- 9) Gene editing/repair
- 10) RNA viral vectors for gene therapy
- 11) DNA viral vectors for gene therapy
- 12) Non-viral vectors for gene therapy
- 13) Selectivity of the therapeutic vector
- 14) GCT – Hereditary diseases
- 15) Virotherapy and gene virotherapy
- 16) GCT – Hematological diseases
- 17) GCT – Oncological diseases
- 18) Gene immunotherapy
- 19) GCT – Acquired and degenerative diseases
- 20) Challenges and perspectives of GCT

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.
6. Gene silencing.
7. Hereditary diseases.
8. Hematological diseases.
9. Oncological diseases.

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

PRESENCIAL ACTIVITIES

Activity	Hours
Theory	19,00
Seminar	26,00
Total hours	45,00

NON PRESENCIAL ACTIVITIES

Activity	Hours
Attendance at other activities	0,00
Individual or group project	10,00
Independent study and work	30,00
Preparation of lessons	10,00
Preparation for assessment activities	8,00
Resolution of case studies	9,50
Total hours	67,50

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, the respect for diversity, 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.

Attendance at practical activities is mandatory. The student is considered to meet this requirement if he or she has attended a minimum of 80% of these activities and has adequately justified the impossibility of attending the remaining sessions due to the occurrence of a cause of force majeure. It will be essential to comply with this requirement to pass the subject.

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



degree subjects.

REFERENCES

BÁSICA

- 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>
- Recursos-e Salut:
 - . ClinicalKey Student Medicina, Odontologia y Enfermería [<https://uv-es.libguides.com/RecursosSalut>]
 - . Acces Medicina [https://uv-es.libguides.com/Access_Medicina]
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En el comienzo de curso se suministrará información o documentación actualizada (revisiones o artículos) sobre las diferentes partes de la asignatura.