# Project/Teaching guide of the subject

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|---|---|---|---|
| **Subject** | Gene Therapy | | |
| **Area** | Advanced Therapies | | |
| **Módule** | Advanced Therapies | | |
| **Title** | Degree in Biomedicine and Advanced Therapies | | |
| **Plan** | 710 | **Code** | 47916 |
| **Delivery period** | 2nd Semester | **Type/Character** | Mandatory/In-person |
| **Level/Cycle** | Degree | **Course** | third |
| **ECTS credits** | 5 | | |
| **Language** | English | | |
| **Responsible teacher/s** | Dra. Beatriz Merino Antolín (Coordinator)<br>Dña. Nadia Galindo Cabello<br>Dra. Sara Galindo de la Rosa<br>Dr. Ricardo Usategui Martín<br>Dña. Maria Jesús Esteban Amo | | |
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| **Department** | Cell Biology, Genetics, Histology and Pharmacology | | |
| **Review date by Title Committee** | July 4, 2024 | | |

## 1. Situation/Sense of the Subject

### 1.1 Contextualization

This subject will cover the fundamentals of gene therapy and its main clinical applications. Gene therapy is based on the transfer or modification of a patient's genetic material in an attempt to cure their diseases. During the course, the fundamentals of gene therapy will be covered in detail, as well as the tools necessary to carry it out, with special emphasis on the main types of vectors used to transfer genes, as well as the molecular tools that allow the correction or modification of genetic information. An important part of the course will be devoted to the clinical applications of gene therapy in different types of pathology. Finally, regulatory and ethical aspects of gene therapy will be addressed.

### 1.2 Relationship with other subjects

This subject is related to the rest of the subjects in the block as well as the rest of the teaching plan so that the student has a comprehensive knowledge.

### 1.3 Prerrequisitos

A background in medical biology, biochemistry and genetics is recommended.

## 2. Competences

### 2.1 General

GC1 – To know how to analyze and synthesize basic problems related to Biomedicine and Advanced Therapies, solve them using the scientific method and communicate them efficiently.

GC2 - To know the scientific and technical bases of Biomedicine and Advanced Therapies, in order to facilitate the learning of new methods and technologies, as well as the development of great versatility to adapt to new situations.

GC3 – To acquire the ability to solve problems with initiative and creativity, as well as to communicate and transmit knowledge, skills and abilities, understanding the ethical, social and professional responsibility of biomedical activity.

GC5 – To acquire, analyze, interpret and manage information.

GC6 – To prepare reports and make judgments based on a critical analysis of reality.

GC7 – To know the rules, regulations and legislation in force, to develop the ability to define and draw up regulations specific to the area.

GC8 – To understand the social, technological and economic changes that condition professional practice.

GC9 – To draft, represent and interpret scientific-technical documentation.

### 2.2 Specific

SC4 - To understand the organization and functions of the genome, the mechanisms of transmission and expression of genetic information and the molecular and cellular bases of genetic analysis.

SC5 - To apply genetic and molecular methods to the study of genetic diseases, their diagnosis and management.

SC21 - To acquire a broad vision of the new personalized therapies. Development, design and application.

SC23 - To explain the bases and different modalities of cell, gene and tissue therapy and to identify which human pathological alterations can be treated with these advanced therapies.

## 3. Objectives

At the end of the course, the student should:

1. To understand the conceptual framework of gene therapies in the current context, their potential and possible future development.

2. To interpret the current situation of the different applications in specific indications.

3. To know the current methodology for specifically modifying the genome of a cell.

4. To be able to carry out and interpret the techniques commonly used in this area.

5. To know the main current gene therapy strategies, their applications and their limitations.

6. To know concrete examples of specific applications.

## 4. Contents and/or thematic blocks

### Block 1: “Gene Therapy”

**Workload in ECTS credits:** 5

### a. Contextualization and justification

Previously described in point 1

### b. Learning objectives

Previously described in point 3

### c. Contents

- Advanced therapies. Gene therapy concept. Gene therapy history. Types of gene therapy (in-vivo vs. ex-vivo).
- Review of basics of genetics. Structure of genetic material. Gene structure. Concept of transgene. Gene information flow.
- PCR. DNA electrophoresis Sequencing
- Restriction enzymes. DNA modifying enzymes (nucleases, polymerases, DNA end-modifying enzymes). DNA ligase.
- Cloning strategies
- Transformation and transfection
- Synthetic nucleotides. Interfering RNA. Genome modification: addiction and editing techniques.
- Retroviral and lentiviral vectors.
- Adenoviral vectors
- Adeno-associated vectors
- HSV-1 (herpes simplex) vectors.
- Non-viral vectors.
- Delivery techniques (mainly non-viral vectors): macromolecule conjugates, lipid systems, DNA injections directly into the vasculature, electroporation.
- Cancer. DNA vaccines in immunotherapy.
- Cancer. CART-T.
- Severe combined immunodeficiency (SCID).
- B-hemoglobinopathies.
- Hemophilia.
- Ocular pathology.
- Neurology.
- Muscular pathology: Duchenne dystrophy.
- Applications in regenerative medicine.
- Ethical aspects associated with gene therapy.

### d. Teaching methods

Lectures

Seminars

Guided laboratory sessions

Individual or group tutorships

Resources and materials on the virtual campus

Autonomous and personal work of the student

### e. Work plan

**Lectures**: 4 hours of theory lessons per week. Within the timetable assigned and the spaces provided by the Faculty of Medicine.

**Seminars**: once a block of subjects has been completed and at the timetable established by the teaching staff, within the timetable assigned.

**Guided experimental laboratory sessions**: Once the block of subjects has been completed, experimental laboratory sessions will be held in order to learn some of the techniques explained through experience. Within the timetable assigned and the spaces provided by the Faculty of Medicine.

**Resources and materials on the virtual campus**: Students will have the resources of the subject available on the virtual campus, as well as forums, notices and calendars of activities.

**Autonomous and personal work of the student:** The student will have to dedicate an average of 1.5 hours of personal work outside the classroom for each classroom hour.

### f. Evaluation

There will be a single final exam of the whole course, which will consist of a multiple-choice exam on the theoretical content of the course (value 60%) and on the content of the seminars (value 20%). The remaining 20% will correspond to continuous assessment, the requirements of which will be specified throughout the course. In order to obtain the continuous assessment, it will be necessary to obtain 4 out of 8 in the final exam. The sum of both marks must be 5 out of 10 to pass.

### g Teaching material

*It is essential that the references provided for this course are up to date and complete. Teachers have access to the Leganto platform in the Library to update their recommended bibliography ("Reading lists"). If they have already done so, they can put the permanent link to Leganto in the teaching guide and on the Virtual Campus.*

*The Library uses the bibliography recommended in the Teaching Guide to adapt its collection to the teaching and learning needs of the degree programmes.*

*If you need to update your bibliography, the link is https://buc-uva.alma.exlibrisgroup.com/leganto/login?auth=SAML (access using your UVa passwords). This link sends you to the authentication page of the UVa directory, which redirects you to Leganto. Once there, you will see, by default, the reading lists corresponding to the different subjects you teach ("instructor" in Leganto / Alma terminology). From here you could add new titles to the existing lists, create sections within them or, on the other hand, create new lists of recommended bibliography.*

*At the top right of each reading list there is a button with the omission sign "□□□" (ellipsis points), which displays a menu that, among other options, allows you to "Create a shareable link" that can be directed either to the specific reading list or to the "Course" (subject). This link can be indicated both in the section "g. Teaching materials" (and sub-sections) of the Teaching Guide as well as in the Bibliography section corresponding to the subject in the Uva Virtual Campus.*

*If you have any doubts, you can consult the library of your centre. Teacher's Guide*

### g.1 Bibliography

https://buc-uva.alma.exlibrisgroup.com/leganto/readinglist/searchlists/7282271450005774

- Principles of Gene Manipulation and Genomics. S.B. Primrose and R.M. Twyman. Seventh Edition.

- Advanced Textbook on Gene Transfer, Gene Therapy and Genetic Pharmacology: Principles, Delivery and Pharmacological and Biomedical Applications of Nucleotide-Based Therapies. Scherman. 2nd Edition.

- Gene Therapy: Therapeutic Mechanisms and Strategies. Marvin Walker

### g.2 Complementary bibliography Comple

It will be available through virtual Campus

### g.3 Other telematics resources

### h. Basic resources

Work environment on the Moodle platform located on the UVa Virtual Campus.

- Access to scientific and technical journals related to the subject.

### i. Timing

| ECTS LOAD | EXPECTED DEVELOPMENT PERIOD |
|---|---|
| 5 | Second term |
| | |
| | |

## 5. Teaching methods and methodological principles

- Lectures
- Seminars
- Guided laboratory sessions
- Individual or group tutorships.
- Work and oral presentation of assignments
- Resources, materials and assignments on the virtual campus
- Personalized student work with material available on the campus.

## 6. Table of student dedication

| ON-SITE OR REMOTE ACTIVITIES(1) | HOURS | NON-PRESENTIAL ACTIVITIES | HOURS |
|---|---|---|---|
| Lectures | 30 | Autonomous work on lectures | 55 |
| Seminars | 15 | Autonomous work on seminars | 22.5 |
| Laboratory sessions | 5 | Autonomous work on laboratory sessions content | 7.5 |
| Total ON-SITE | **50** | Total NON-PRESENTIAL | **85** |
| | | TOTAL N-SITE+NON-PRESENTIAL | **135** |

(1) Remote activity is when a group follows a videoconference synchronously to the class given by the teacher.

## 7. Evaluation system and characteristics

| INSTRUMENT/PROCEDURE | FINAL NOTE LOAD | OBSERVATIONS |
|---|---|---|
| Lectures exam content | 60% | Test type and/or short questions |
| Seminars exam content | 20% | Test type and/or short questions |
| continuous assessment | 20% | To specify during the course |
| | | |

**EVALUATION CRITERIA**

- **Ordinary call:**
    - Sum of the marks in the summary table. It will be necessary to obtain 4 out of 8 in the final exam to add the continuous assessment mark. The sum of both marks must be 5 out of 10 to pass the course.
- **Extra-ordinary call(*):**
    - Sum of the marks in the summary table. It will be necessary to obtain 4 out of 8 in the final exam to add the continuous assessment mark. The sum of both marks must be 5 out of 10 to pass the course

**(*)Extraordinary call is understood to be the second call.**
**Art 35.4 of the ROA 35.4. Participation in the extraordinary call will not be subject to class attendance or to the presence in previous tests, except in the cases of external practices, laboratories or other activities whose evaluation would not be possible without the prior completion of the aforementioned tests.**https://secretariageneral.uva.es/wp-content/uploads/VII.2.-Reglamento-de-Ordenacion-Academica.pdf

## 8. Final considerations