



Proyecto/Guía docente de la asignatura

Project/Course Syllabus

Asignatura <i>Course</i>	Gene Therapy		
Materia <i>Subject area</i>	Advanced Therapies		
Módulo <i>Module</i>	Advanced Therapies		
Titulación <i>Degree Programme</i>	Degree in Biomedicine and Advanced Therapies		
Plan <i>Curriculum</i>	710	Código <i>Code</i>	47916
Periodo de impartición <i>Teaching Period</i>	2nd Semester	Tipo/Carácter <i>Type</i>	Mandatory/In-person
Nivel/Ciclo <i>Level/Cycle</i>	Degree	Curso <i>Course</i>	third
Créditos ECTS <i>ECTS credits</i>	5		
Lengua en que se imparte <i>Language of instruction</i>	English		
Profesor/es responsable/s <i>Responsible Teacher/s</i>	Dra. Beatriz Merino Antolín (Coordinator) Dra. Sara Galindo de la Rosa Dña. Nadia Galindo Cabello		
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Departamento <i>Department</i>	Cell Biology, Genetics, Histology and Pharmacology		
Fecha de revisión por el Comité de Título <i>Review date by the Degree Committee</i>	11/06/26		

En caso de guías bilingües con discrepancias, la validez será para la versión en español.
In the case of bilingual guides with discrepancies, the Spanish version will prevail.

**1. Situación / Sentido de la Asignatura****Course Context and Relevance****1.1 Contextualización****Course Context**

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 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 Relación con otras materias**Connection 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**Prerequisites**

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

2. Resultados del proceso de formación y de aprendizaje (RD 822/2021) o competencias (RD 1393/2007)

Learning outcomes (RD 822/2021) or competences (RD 1393/2007)

Para los planes de estudio al amparo del RD 822/2021 deben completarse conocimientos o contenidos, habilidades o destrezas y las competencias.

Para los planes de estudio al amparo del RD 1393/2007 deben completarse las Competencias Generales y las Competencias Específicas.

For study programmes under RD 822/2021, it is necessary to specify knowledge or content, skills or abilities, and competences.

For study programmes under RD 1393/2007, General Competences and Specific Competences must be included.

2.1 (RD1393/2007) Competencias Generales

General Competences

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 (RD1393/2007) Competencias Específicas

Specific Competences

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. Objetivos

Course 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. Contenidos y/o bloques temáticos

Course Contents and/or Modules

- 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: addition 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.

Bloque 1: "Gene Therapy"

Module 1: "Gene Therapy"

Carga de trabajo en créditos ECTS:
Workload in ECTS credits:

5

a. Contextualización y justificación

a. Context and rationale

Previously described in point 1

b. Objetivos de aprendizaje

b. Learning objectives

Previously described in point 3

c. Contenidos**c. Contents**

Previously described in point 4

d. Métodos docentes**d. Teaching and Learning 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. Plan de trabajo**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 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. Evaluación**f. Assessment**

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 and on the content of the seminars (value 60%). Other 20% will be obtained by the seminars and finally the remaining 20% will correspond to continuous assessment, the requirements of which will be specified throughout the course. To obtain the continuous assessment, it will be necessary to obtain 4 out of 10 in the final exam. The sum of both marks must be 5 out of 10 to pass.

g. Material docente



g Teaching material

Es fundamental que las referencias suministradas este curso estén actualizadas y sean completas. El profesorado tiene acceso, a la **plataforma Leganto de la Biblioteca** para actualizar su bibliografía recomendada ("Listas de Lecturas"). Si ya lo ha hecho, puede poner tanto en la guía docente como en el Campus Virtual el enlace permanente a Leganto.

La Biblioteca se basa en la bibliografía recomendada en la Guía docente para adaptar su colección a las necesidades de docencia y aprendizaje de las titulaciones.

Si tiene que actualizar su bibliografía, el enlace es el siguiente,

<https://buc-uva.alma.exlibrisgroup.com/leganto/login?auth=SAML> (acceso mediante tus claves UVA). Este enlace te envía a la página de autenticación del directorio UVA, el cual te redirige a Leganto. Una vez allí, aparecerán, por defecto, las listas de lectura correspondientes a las distintas asignaturas que imparte ("instructor" en la terminología de Leganto / Alma). Desde aquí podría añadir nuevos títulos a las listas existentes, crear secciones dentro de ellas o, por otra parte, crear nuevas listas de bibliografía recomendada.

Puede consultar las listas de lectura existentes mediante el buscador situado en el menú de arriba a la izquierda, opción "búsqueda de listas".

En la parte superior derecha de cada lista de lectura se encuentra un botón con el signo de omisión "•••" (puntos suspensivos), a través del cual se despliega un menú que, entre otras opciones, permite "Crear un enlace compartible" que puede dirigir o bien a la lista de lectura concreta o bien al "Curso" (asignatura). Este enlace se puede indicar tanto en el apartado "g. Materiales docentes" (y subapartados) de la Guía Docente como en la sección de Bibliografía correspondiente a la asignatura en el Campus Virtual Uva.

Para resolver cualquier duda puede consultar con la biblioteca de tu centro. [Guía de Ayuda al profesor](#)

It is essential that the references provided for this course are up to date and complete. Faculty members have access to the Library's Leganto platform to update their recommended reading lists. If they have already done so, they may include the permanent Leganto link both in the course syllabus and on the Virtual Campus.

The Library relies on the recommended bibliography listed in the course syllabus to adapt its collection to the teaching and learning needs of each degree programme.

To update your bibliography, please use the following link:

<https://buc-uva.alma.exlibrisgroup.com/leganto/login?auth=SAML> (access using your UVA credentials). This link takes you to the UVA directory authentication page, which will then redirect you to Leganto. Once there, the reading lists associated with the courses you teach will appear by default ("instructor" in Leganto/Alma terminology). From this platform, you can add new titles to existing lists, create sections within them, or alternatively, create new recommended reading lists.

You can browse existing reading lists using the search bar located in the top left menu, under the "Find Lists" option.

In the top right corner of each reading list, you will find a button marked with an ellipsis "•••" (three dots). Clicking it opens a menu that includes, among other options, the ability to "Create a shareable link", which can point either to a specific reading list or to the entire course. This link can be included in section "g. Teaching Materials" (and its subsections) of the Course Syllabus, as well as in the Bibliography section of the course page on the UVA Virtual Campus.

If you have any questions, please contact your faculty library. [Guía de Ayuda al profesor](#)

g.1 Bibliografía básica

Required Reading

<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 Bibliografía complementaria

Supplementary Reading

It will be available through virtual Campus



g.3 Otros recursos telemáticos (píldoras de conocimiento, blogs, videos, revistas digitales, cursos masivos (MOOC), ...)

Additional Online Resources (microlearning units, blogs, videos, digital journals, massive online courses (MOOC), etc.)

h. Recursos necesarios

Required Resources

i. Temporalización

Course Schedule

CARGA ECTS <i>ECTS LOAD</i>	PERIODO PREVISTO DE DESARROLLO <i>PLANNED TEACHING PERIOD</i>
5	Second term

5. Métodos docentes y principios metodológicos

Instructional Methods and guiding 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. Tabla de dedicación del estudiantado a la asignatura

Student Workload Table

ACTIVIDADES PRESENCIALES o PRESENCIALES o A DISTANCIA⁽¹⁾ <i>FACE-TO-FACE/ ON-SITE or ONLINE ACTIVITIES ⁽¹⁾</i>	HORAS <i>HOURS</i>	ACTIVIDADES NO PRESENCIALES <i>INDEPENDENT / OFF-CAMPUS WORK</i>	HORAS <i>HOURS</i>
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 presencial <i>Total face-to-face</i>	50	Total no presencial. <i>Total non-face-to-face</i>	85
TOTAL presencial + no presencial <i>Total</i>			135

(1) Actividad presencial a distancia es cuando un grupo sentado en un aula del campus sigue una clase por videoconferencia de forma síncrona, impartida por el profesor. *Distance face-to-face activity refers to a*



situation in which a group of students, seated in a classroom on campus, attends a class via live videoconference delivered by the instructor in real time.

7. Sistema y características de la evaluación

Assessment system and criteria

INSTRUMENTO/PROCEDIMIENTO ASSESSMENT METHOD/PROCEDURE	PESO EN LA NOTA FINAL WEIGHT IN FINAL GRADE	OBSERVACIONES REMARKS
Lectures exam content	60%	Test type and/or short questions
Seminars exam content	20%	Partial deliverables and Oral presentation
continuous assessment	20%	To specify during the course

CRITERIOS DE CALIFICACIÓN ASSESSMENT CRITERIA
• Convocatoria ordinaria. First Exam Session (Ordinary) ○ Sum of the marks in the summary table. It will be necessary to obtain 4 out of 10 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. • Convocatoria extraordinaria^(*) Second Exam Session (Extraordinary / Resit) ^(*): ○ Sum of the marks in the summary table. It will be necessary to obtain 4 out of 10 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

(*) Se entiende por convocatoria extraordinaria la segunda convocatoria.

RECORDATORIO El estudiante debe poder puntuar sobre 10 en la convocatoria extraordinaria salvo en los casos especiales indicados en el Art 35.4 del ROA 35.4. "La participación en la convocatoria extraordinaria no quedará sujeta a la asistencia a clase ni a la presencia en pruebas anteriores, salvo en los casos de prácticas externas, laboratorios u otras actividades cuya evaluación no fuera posible sin la previa realización de las mencionadas pruebas."

<https://secretariageneral.uva.es/wp-content/uploads/VII.2.-Reglamento-de-Ordenacion-Academica.pdf>

(*)The term "second exam session (extraordinary/resit)" refers to the second official examination opportunity.

REMINDER Students must be assessed on a scale of 0 to 10 in the extraordinary session, except in the special cases indicated in Article 35.4 of the ROA: "Participation in the extraordinary exam session shall not be subject to class attendance or participation in previous assessments, except in cases involving external internships, laboratory work, or other activities for which evaluation would not be possible without prior completion of the aforementioned components."

<https://secretariageneral.uva.es/wp-content/uploads/VII.2.-Reglamento-de-Ordenacion-Academica.pdf>

8. Consideraciones finales

Final remarks

