



Information about the subject

Degree: Bachelor of Science Degree in Biotechnology

Faculty: Faculty of Veterinary Medicine and Experimental Sciences

Code: 1102024 **Name:** Gene Therapy

Credits: 6,00 **ECTS Year:** 0, 4 **Semester:** 1

Module: Elective Courses

Subject Matter: Gene Therapy **Type:** Elective

Department: Biotechnology

Type of learning: Classroom-based learning

Languages in which it is taught: Spanish

Lecturer/-s:

OPB11 Mercedes Cervera Alamar (**Responsible Lecturer**)

mercedes.cervera@ucv.es

David Millan Esteban

david.millan@ucv.es



Module organization

Elective Courses

| Subject Matter | ECTS | Subject | ECTS | Year/semester |
|--|------|--|------|---|
| Marine Biotechnology | 6,00 | Marine Biotechnology | 6,00 | 0, 3, 4/1 |
| Pharmacology and Toxicology | 6,00 | Pharmacology and Toxicology | 6,00 | 0, 3/1 |
| R&D in Marine Sciences | 6,00 | R&D in Marine Sciences | 6,00 | 3, 4/1 |
| Sea Food Technology | 6,00 | Sea Food Technology | 6,00 | 3, 4/1 |
| Instrumental Techniques of Marine Analysis | 6,00 | Instrumental Techniques of Marine Analysis | 6,00 | 4/1 |
| Genetic Techniques Applied to the Marine Environment | 6,00 | Genetic Techniques Applied to the Marine Environment | 6,00 | This elective is not offered in the academic year 25/26 |
| Principles of Food Biotechnology | 6,00 | Food Biotechnology | 6,00 | 0, 3, 4/1 |
| Plant Tissue and Cell Culture | 6,00 | Plant Tissue and Cell Culture | 6,00 | This elective is not offered in the academic year 25/26 |
| Molecular Phytopathology | 6,00 | Molecular Phytopathology | 6,00 | 3, 4/1 |
| Agricultural Plant Breeding | 6,00 | Agricultural Plant Breeding | 6,00 | 0/1 |
| Seed Physiology and Molecular Biology | 6,00 | Seed Physiology and Molecular Biology | 6,00 | This elective is not offered in the academic year 25/26 |



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|--|------|--|------|---|
| Biocontrol for Crop Protection | 6,00 | Biocontrol for Crop Protection | 6,00 | This elective is not offered in the academic year 25/26 |
| Agrigenomics | 6,00 | Agrigenomics | 6,00 | This elective is not offered in the academic year 25/26 |
| Food Microbiology and Toxicology | 6,00 | Food Microbiology and Toxicology | 6,00 | 0/1 |
| Biomolecular Modeling | 6,00 | Biomolecular Modeling | 6,00 | 0/1 |
| Pharmaceutical Engineering and Drug Design | 6,00 | Pharmaceutical Engineering and Drug Design | 6,00 | 0, 4/1 |
| Gene Therapy | 6,00 | Gene Therapy | 6,00 | 0, 4/1 |
| Molecular Pathology | 6,00 | Molecular Pathology | 6,00 | 0, 4/1 |
| Clinical Biotechnology | 6,00 | Clinical Biotechnology | 6,00 | 0/1 |
| Immunology | 6,00 | Immunology | 6,00 | 0, 3/1 |
| Principles of Environmental Biotechnology | 6,00 | Environmental Biotechnology | 6,00 | 4/1 |
| Biosensors | 6,00 | Biosensors | 6,00 | This elective is not offered in the academic year 25/26 |
| Environmental Engineering | 6,00 | Environmental Engineering | 6,00 | This elective is not offered in the academic year 25/26 |
| Bioremediation | 6,00 | Bioremediation | 6,00 | This elective is not offered in the academic year 25/26 |



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|--------------------------|------|--------------------------|------|---|
| Environmental Toxicology | 6,00 | Environmental Toxicology | 6,00 | This elective is not offered in the academic year 25/26 |
| Bioindicadores | 6,00 | Bioindicators | 6,00 | 0, 3, 4/1 |

Learning outcomes

At the end of the course, the student must be able to prove that he/she has acquired the following learning outcomes:

- R1 The student has understood and assimilated the contents of the subject.
- R2 The student is able to solve problems or case studies related to the subject contents, by using different resources (bibliographic, IT, etc.)
- R3 The student is able to work in a laboratory, carrying out basic operations correctly and taking into account the corresponding safety standards. He/she understands the planning, development and purpose of the experience, and is able to contrast and validate the obtained results.
- R4 The student is able to write an intelligible and organized text on different aspects of the subject.
- R5 The student is able to present and defend his/her work adequately.
- R6 The student seeks bibliographic information from different sources and can analyze it with a critical and constructive spirit.
- R7 The student collaborates with the teacher and his/her peers throughout the learning process; he/she works in a team; treats everyone with respects, is proactive and fulfills the organization rules of the course.



Competencies

Depending on the learning outcomes, the competencies to which the subject contributes are (please score from 1 to 4, being 4 the highest score):

| BASIC | | Weighting | | | |
|----------|---|-----------|---|---|---|
| | | 1 | 2 | 3 | 4 |
| CB1 | Students acquire and understand knowledge in their field of study based on general secondary education but usually reaching a level that, although supported on advanced text books, also includes aspects involving state-of-the-art knowledge specific to their area. | X | | | |
| CB2 | Students are able to apply knowledge to their work in a professional way and have the competences enabling them to state and defend views and opinions as well as perform problem-solving tasks in their field of study. | | X | | |
| CB3 | Students are able to collect and interpret relevant data (generally in their field of study) and give opinions that involve reflection on relevant social, scientific or ethical issues. | | | X | |
| CB4 | Students can communicate information, ideas, problems and solutions to a specialized or non-specialized audience. | | X | | |
| CB5 | Students develop the necessary learning skills to undertake further studies with a high level of autonomy. | | | X | |
| GENERAL | | Weighting | | | |
| | | 1 | 2 | 3 | 4 |
| CG01 | Capacity to analyze and synthesize. | | X | | |
| SPECIFIC | | Weighting | | | |
| | | 1 | 2 | 3 | 4 |
| CE22 | Knowing and understanding contents, principles and theories related to biotechnology. | | X | | |



| | | | | | |
|------|---|---|--|---|---|
| CE23 | Knowing how to use laboratory equipment and to carry out basic operations for each discipline including: safety measures, handling, waste disposal and activity register. | | | X | |
| CE24 | Knowing basic and instrument laboratory techniques in the different areas of biotechnology. | | | X | |
| CE25 | Knowing how to analyze and understand scientific data related to biotechnology. | | | X | |
| CE26 | To understand and identify the mechanisms that influence genetic inheritance | | | | X |
| CE27 | Knowing and applying action plans and assessment criteria of biotechnology processes. | X | | | |
| CE28 | Integrating life science and Engineering into processes of development of biotechnological products and applications. | | | | X |
| CE29 | Contrasting and checking results of biotechnological experimentation. | | | | X |
| CE30 | Solving and analyzing problems posed by biotechnology. | | | X | |
| CE31 | Describing and calculating important variables of processes and experiments. | | | X | |
| CE32 | Knowing how to use different specific operating systems and software packages designed for Biotechnology. | | | X | |
| CE33 | Knowing and complying with legislation and ethics of biotechnological processes and applications. | | | X | |
| CE34 | Knowing main characteristics of Molecular biosciences and biotechnology communication. | X | | | |

TRANSVERSAL

Weighting

| | 1 | 2 | 3 | 4 |
|--|---|---|---|---|
| CT02 Capacity to organize and plan. | | X | | |
| CT03 Mastering Spanish oral and written communication. | X | | | |
| CT05 Knowing and applying Basic ITC skills related to Biotechnology. | | | X | |
| CT06 Capacity to manage information (capacity to look for and analyze information coming from different types of sources). | | | X | |



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|------|---|---|---|---|
| CT07 | Problem solving. | | | X |
| CT08 | Decision making | | X | |
| CT09 | Capacity to work in interdisciplinary and multidisciplinary team. | | | X |
| CT10 | Interpersonal skills. | | X | |
| CT11 | Understanding multicultural and diverse environment | X | | |
| CT12 | Critical and self-critical capacity. | | X | |
| CT13 | Ethics. | | X | |
| CT14 | Capacity to learn | | X | |
| CT15 | Capacity to adapt to new situations | | X | |
| CT16 | Capacity to produce new ideas (creativity) | | | X |
| CT19 | Capacity to apply theoretical knowledge | | | X |
| CT20 | Research skills | | X | |
| CT21 | Sensitivity to environmental issues | | X | |



Assessment system for the acquisition of competencies and grading system

| Assessed learning outcomes | Granted percentage | Assessment method |
|----------------------------|--------------------|----------------------|
| R1, R2, R5 | 65,00% | Written test |
| R1, R4, R5, R6, R7 | 30,00% | Submission of papers |
| R1, R2, R3, R7 | 5,00% | Laboratory test |

Observations

This course is not eligible for single evaluation. According to the general evaluation and qualification regulations, the preferred evaluation system will be continuous evaluation:

The final written exam may contain multiple choice questions and short open-ended questions.

The paper submission (30% of the final grade) will consist of:

1. A group project on: "Design of a therapeutic strategy based on CRISPR/Cas" (15%). Students will be able to submit a draft that will be corrected by the professor (without grade) before the final submission.
2. A group project on a topic from a list proposed by the professor (10%) that will be presented in class.
3. Continuous evaluation (5%). Continuous evaluation is designed to ensure a progressive understanding of key concepts, as well as to encourage the practical application of the knowledge acquired. Students will participate in a variety of activities including completion of in-class assignments and participation in discussions.

The laboratory test (5%) will be a written exam held at the end of the lab sessions and will include short-answer questions. Attendance at laboratory sessions is mandatory.

To pass the course, students must obtain at least a 5 (out of 10) in the written exam, the lab test, and the assignments.

*The use of artificial intelligence (AI)-based tools is subject to the discretion of the teacher, who may establish specific limits or conditions depending on the training or assessment activity.



MENTION OF DISTINCTION:

In accordance with the regulations governing the assessment and grading of subjects in force at UCV, the distinction of "Matrícula de Honor" (Honours with Distinction) may be awarded to students who have achieved a grade of 9.0 or higher. The number of "Matrículas de Honor" (Honours with Distinction) may not exceed five percent of the students enrolled in the group for the corresponding academic year, unless the number of enrolled students is fewer than 20, in which case a single "Matrícula de Honor" (Honours with 9 Distinction) may be awarded. Exceptionally, these distinctions may be assigned globally across different groups of the same subject. Nevertheless, the total number of distinctions awarded will be the same as if they were assigned by group, but they may be distributed among all students based on a common criterion, regardless of the group to which they belong. The criteria for awarding "Matrícula de Honor" (Honours with Distinction) will be determined according to the guidelines stipulated by the professor responsible for the course, as detailed in the "Observations" section of the evaluation system in the course guide.

Learning activities

The following methodologies will be used so that the students can achieve the learning outcomes of the subject:

- M1 Teacher presentation of contents, analysis of competences, explanation and in-class display of skills, abilities and knowledge.
- M2 Group work sessions supervised by the professor. Case studies, diagnostic tests, problems, field work, computer room, visits, data search, libraries, on-line, Internet, etc. Meaningful construction of knowledge through interaction and student activity.
- M3 Activities carried out in spaces with specialized equipment.
- M4 Supervised monographic sessions with shared participation..
- M5 Application of multidisciplinary knowledge.
- M6 Personalized and small group attention. Period of instruction and/or guidance carried out by a tutor to review and discuss materials and topics presented in classes, seminars, readings, papers, etc.
- M7 Set of oral and/or written tests used in initial, formative or additive assessment of the student
- M8 Group preparation of readings, essays, problem-solving, seminars, papers, reports, etc. to be presented or submitted in theoretical , practical and/or small-group tutoring sessions. Work done on the university e-learning.



- M9 Student's study: Individual preparation of readings, essays, problem-solving, seminars, papers, reports, etc. to be presented or submitted in theoretical, practical and/or small-group tutoring sessions. Work done on the university e-learning platform.

IN-CLASS LEARNING ACTIVITIES

| | LEARNING OUTCOMES | HOURS | ECTS |
|---|----------------------------|--------------|-------------|
| ON-CAMPUS CLASS M1 | R1, R2, R7 | 29,30 | 1,17 |
| PRACTICAL CLASSES M2 | R1, R2, R6, R7 | 11,30 | 0,45 |
| LABORATORY M3 | R3 | 2,60 | 0,10 |
| SEMINAR M4 | R1, R7 | 1,70 | 0,07 |
| GROUP PRESENTATION OF ASSIGNMENTS M5 | R1, R4, R5 | 10,90 | 0,44 |
| TUTORIAL M6 | R1 | 2,50 | 0,10 |
| ASSESSMENT M7 | R1, R2, R3, R4, R5, R6, R7 | 1,70 | 0,07 |
| TOTAL | | 60,00 | 2,40 |

LEARNING ACTIVITIES OF AUTONOMOUS WORK

| | LEARNING OUTCOMES | HOURS | ECTS |
|----------------------------------|----------------------------|--------------|-------------|
| AUTONOMOUS GROUP WORK M8 | R1, R2, R3, R4, R5, R6, R7 | 17,10 | 0,68 |
| AUTONOMOUS INDIVIDUAL WORK M9 | R1, R2, R3, R4, R5, R6 | 72,90 | 2,92 |
| TOTAL | | 90,00 | 3,60 |



Description of the contents

Description of the necessary contents to acquire the learning outcomes.

Theoretical contents:

| Content block | Contents |
|--|---|
| Section 1: Molecular bases of Gene Therapy | Unit 1: Basic Definitions and Principles of Gene Therapy Unit 2: History of Gene Therapy Unit 3: Molecular Basis of Gene Therapy. Genome Organization and Functions |
| Section 2: Gene Therapy Delivery Systems | Unit 4: Viral Vectors Unit 5: Non-viral Vectors Unit 6: Barriers to Gene Transfer |
| Section 3: Therapeutic Strategies in Gene Therapy | Unit 7: Gene Suppression Unit 8: Gene Editing Unit 9: Other Therapeutic Strategies |
| Section 4: Therapeutic Potential | Unit 10: Application of Gene Therapy for the Treatment of Monogenic Diseases Unit 11: Ocular Gene Therapy Unit 12: Gene Therapy in Viral Infections Treatment Unit 13: Gene Therapy in Neurodegenerative Disorders Unit 14: Gene Therapy and Cancer |
| Section 5: Ethical and Legal Aspects of Gene Therapy | Unit 15: Ethical and Legal Aspects of Gene Therapy |
| Section 6: Clinical Development of Gene Therapy | Unit 16: Clinical Trials and Approved Products in Gene Therapy |



Organization of the practical activities:

| | Content | Place | Hours |
|------|----------------------|--------------|-------|
| PR1. | LABORATORY PRACTICES | Laboratory | 2,00 |
| PR2. | CLASSROOM PRACTICES | Lecture room | 12,00 |

Temporary organization of learning:

| Block of content | Number of sessions | Hours |
|--|--------------------|-------|
| Section 1: Molecular bases of Gene Therapy | 4,00 | 8,00 |
| Section 2: Gene Therapy Delivery Systems | 6,00 | 12,00 |
| Section 3: Therapeutic Strategies in Gene Therapy | 7,00 | 14,00 |
| Section 4: Therapeutic Potential | 7,00 | 14,00 |
| Section 5: Ethical and Legal Aspects of Gene Therapy | 2,00 | 4,00 |
| Section 6: Clinical Development of Gene Therapy | 4,00 | 8,00 |

References

SCHERMAN D, Advanced textbook on gene transfer, gene therapy and genetic pharmacology. Principles, delivery and pharmacological and biomedical applications of nucleotide-based therapies, Imperial College Press, 2014. TEMPLETON NS, Gene and Cell Therapy. Therapeutic Mechanisms and Strategies. CRC Press; 4th Edition, 2015. RODRÍGUEZ A, RODRÍGUEZ GASCÓN A, SOLINIS ASPIAZU MA. Terapia Génica. Editorial: Síntesis, 2018.