**GENERAL FEATURES**

<table>
<thead>
<tr>
<th>Type:</th>
<th>☐ Basic training, ☐ Compulsory, ☑ Elective</th>
</tr>
</thead>
<tbody>
<tr>
<td>Duration:</td>
<td>☐ Final Degree Project, ☐ Practicum</td>
</tr>
<tr>
<td>Semester/s</td>
<td>Semester / s: 8</td>
</tr>
<tr>
<td>Number of ECTS credits:</td>
<td>5</td>
</tr>
<tr>
<td>Language / s:</td>
<td>☑ English</td>
</tr>
</tbody>
</table>

**DESCRIPTION**

**SHORT DESCRIPTION AND JUSTIFICATION** (of the meaning of the course in relation to the studies. Between 100 and 200 words)

Transgenic animal technology enables but is not limited to, the generation of human disease animal models to test pharmaceuticals and the production of recombinant molecules. Conversely, gene therapy allows to manipulate genes with a therapeutic goal and, for specific conditions, can be the only possible path to achieve a cure. Hence, knowledge on the broad range of applications of transgenic animal technology and gene therapy are of great interest in the context of Biotechnology for Health.

The course “Transgenic animals and Gene therapy” will focus in providing the students with knowledge on the different kinds of transgenic animals and gene therapy strategies currently available, together with the technology associated with the generation of transgenic animals and the design of gene therapy strategies (i.e. methods for gene delivery and gene modification). In addition to presenting the different strategies, specific applications will be discussed.

**COMPETENCES** (of the course placed in relation to the pre-assigned competences in the subject matter)

- That students develop those learning skills necessary to undertake further studies with a high degree of autonomy (CB5)
- Be able to assess the impact of their professional activity on the sustainable development of society (T3)
- Be able to incorporate contemporary aspects related to the exercise of their profession (T5)
- Be able to understand and apply advanced knowledge of Biosciences and Engineering to the field of Biotechnology (E3).
- Be able to integrate the knowledge and tools of biotechnology for their application to different industrial sectors that use, develop or produce biotechnological products or processes (E6).

* These features should not be modified without the approval of the academic board (subject matter, module and / or studies program).
COURSE: TRANSGENIC ANIMALS AND GENE THERAPY
SUBJECT MATTER: Biotechnology for Health
MODULE: Optional subjects
PROGRAM: Degree in Biotechnology

PREVIOUS REQUIREMENTS * (modules, subject matters, courses or knowledge necessary for the follow-up of the subject. State previous courses required to be completed)

Students should have previously acquired the knowledge of the biotechnology degree related with the courses of “Cell biology and Genetics” and “Animal and Plant Biology”, belonging to the basic module. Moreover, students should have taken the following 2nd year subjects of the Biotechnology degree: “Molecular Biology”, “Recombinant DNA Technology”, “Structure and function of Biomolecules” and “Metabolism and Regulation”. Finally, taking the optional courses “Molecular Physiopathology” and “Tissue Engineering” is also recommended.

CONTENTS (List the content of the course, with up to two level detail)

0. Course introduction

Part I. ANIMAL TRANSGENESIS

1. Introduction to animal transgenesis - The mouse as a model for biomedical applications
2. Additive transgenesis: generation of transgenic mice and design of chimeric/transgenic genes
3. Approaches for the generation of transgenic animals
4. Embryonic Stem Cell (ESC) technology and gene targeting
5. New technologies: transposons and genome editing
6. Generation of transgenic farm animals
7. Other transgenic laboratory animals
8. Other aplications of transgenesis: gene trap, conditional overexpression mediated by Cre/LoxP .
9. Applications of transgenic animals in medicine, agriculture and industry. Biopharming

Part II. GENE THERAPY

11. Therapeutic nucleic acids
12. Delivery methods: biological
   12.1. Virus as gene delivery vectors: retrovirus, lentivirus, adenovirus and adeno-associated vectors
13. Delivery methods: chemical and physical
14. Transposons and endonucleases in gene therapy
15. Gene therapy applications

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METHODOLOGY

LEARNING ACTIVITIES * (Complete the table relating activities, workload in ECTS credits, and competences.)

<table>
<thead>
<tr>
<th>Learning Activities</th>
<th>ECTS Credits</th>
<th>Competences</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lectures</td>
<td>1.3</td>
<td>T3, T5, E3, E6</td>
</tr>
<tr>
<td>Case and Problem-Solving Sessions</td>
<td>0.1</td>
<td>CB5, T3, T5, E3, E6</td>
</tr>
<tr>
<td>Seminars</td>
<td>0.1</td>
<td>CB5, T3, T5, E3, E6</td>
</tr>
<tr>
<td>Practical and Lab Work</td>
<td>-</td>
<td></td>
</tr>
<tr>
<td>Presentations</td>
<td>0.1</td>
<td>CB5, T5</td>
</tr>
<tr>
<td>Personal Study</td>
<td>3.3</td>
<td>CB5, T3, T5, E3, E6</td>
</tr>
<tr>
<td>Assessment Tasks (Exams, Continuous Assessment...)</td>
<td>0.1</td>
<td>CB5, T3, T5, E3, E6</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>5.0</strong></td>
<td><strong>CB5, T3, T5, E3, E6</strong></td>
</tr>
</tbody>
</table>

TEACHING METHODOLOGY (justify the teaching methodology in relation to the competences and course contents. Between 100 and 200 words)

The subject, taught on a face-to-face basis, will consist mainly of sessions of exposition of concepts by the teacher (i.e. lectures). These sessions will be combined with case and problem-solving sessions, exercises and discussion of specific examples drawn from specialized journals. In addition, seminar-type sessions will be held to resolve doubts. Furthermore, the students, in groups, will prepare a report, based on bibliographic research, that will be presented to the rest of the class and presented in written form for its evaluation. The methodology used is described in detail below:

- **Lectures** - Presentation and explanation of contents by a teacher (possibly including demonstrations), using powerpoint as teaching support, or the blackboard as needed.
- **Case and Problem-Solving Sessions** - Resolution of exercises and problems, and exposition / discussion of cases (e.g. published papers) by a teacher with the active participation of students. The cases and exercises can be presented and solved collectively in class, or be given to the students as a task to be solved individually at home.
- **Seminars** - Period of instruction carried out by a teacher with the aim of reviewing, discussing and resolving doubts about the materials and topics presented in the lectures and in the case and problem-solving sessions. Short sessions will be scheduled to guide and solve doubts regarding the bibliographic research report.
- **Presentations** - Oral presentation by a student to the teacher and all the other students. The presentation will be a work prepared by students (in groups) through searches in published bibliography or other online resources.
- **Personal study activities** - Personal work of the student necessary to acquire the competences of each subject matter, and to assimilate the knowledge exposed in lectures and case and problem-solving sessions, using the recommended reference

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COURSE: TRANSGENIC ANIMALS AND GENE THERAPY

SUBJECT MATTER: Biotechnology for Health
MODULE: Optional subjects
PROGRAM: Degree in Biotechnology

ASSESSMENT

ASSESSMENT METHODS * (Complete the table relating assessment methods, competences, and weight percentage in the course qualification)

<table>
<thead>
<tr>
<th>Assessment methods</th>
<th>Weight</th>
<th>Competences</th>
</tr>
</thead>
<tbody>
<tr>
<td>Final Exam</td>
<td>35%</td>
<td>CB5, T3, T5, E3, E6</td>
</tr>
<tr>
<td>Midterm Exam/s</td>
<td>-</td>
<td></td>
</tr>
<tr>
<td>Continuous Assessment Activities</td>
<td>40%</td>
<td>T5, E3, E6</td>
</tr>
<tr>
<td>Reports and Presentations</td>
<td>20%</td>
<td>T5, E3, E6</td>
</tr>
<tr>
<td>Lab or Field Work</td>
<td>-</td>
<td>T5, E3, E6</td>
</tr>
<tr>
<td>Projects</td>
<td>-</td>
<td></td>
</tr>
<tr>
<td>Host Student Evaluation</td>
<td>-</td>
<td></td>
</tr>
<tr>
<td>Participation</td>
<td>5%</td>
<td>T5, E3, E6</td>
</tr>
</tbody>
</table>

LEARNING OUTCOMES (Explanation of the student's achievements that allow the assessment of competences, relating them to the competences and the assessment methods)

- The student must demonstrate a degree of learning and autonomy that allows him/her to undertake studies and subsequent professional activities (CB5).
- The student must understand the importance and usefulness of transgenic animals and gene therapy in biotechnology for current and future health, in order to be able to assess the impact of their professional activity on the sustainable development of society (T3).
- The student must demonstrate his/her ability to design strategies to generate transgenic animals for specific applications. Likewise, the student must be able to identify and design the most appropriate gene therapy strategy for the treatment of specific diseases. The student must be able to understand the tools currently available to incorporate these aspects in the exercise of his/her profession (T5).

- The student must know the tools available to generate transgenic animals and design gene therapy strategies. Must understand the advantages and limitations of each of the systems and methods worked in the course to be able to apply this advanced knowledge of Biosciences and Engineering in activities in the field of biotechnology (E3).
- The student must be able to integrate the acquired knowledge of the subject in the framework of biotechnology, to be able to apply it to the different industrial sectors that use, develop or produce products with and application in biotechnology for health (E6).

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QUALIFICATION

The evaluation of the subject (final grade, FG) will consider the grades obtained in the final and midterm exams (FE and MTE, respectively), the follow-up activities (FA), the report and presentation (RP), and the participation (P). Each of these activities will be graded on a 1-10 scale.

The qualification of the follow-up activities (FA) and of the report and presentation (RP) will be calculated as a weighted average of the different activities carried out. The qualification of the participation (P, 5% of the final grade) is awarded by the teacher at the end of the subject based on the level of participation of the student in the overall of the activities of the subject throughout the course.

The aim of the final exam (FE, 35% of the final grade) is to evaluate the course synthesis.

In order to pass the subject, the mark of the final exam (FE) must be at least 4.5. In case the FE mark is lower than 4.5, this mark will be the final grade (FG) of the subject.

If the FE mark is equal or higher than 4.5, then the final grade (FG) of the subject is calculated as the weighted average of the marks obtained in the final exam (FE, 35%), midterm exam (MTE, 15%), follow-up activities (FA, 25%), the report and presentation (RP, 20%) and the participation (P, 5%), using the following formula:

\[ FG = 0.35 \text{FE} + 0.15 \text{MTE} + 0.25 \text{FA} + 0.20 \text{RP} + 0.05 P \]

Only if this final grade (FG) is equal to or greater than 5 will the subject be approved.

ASSESSMENT OF THE COMPETENCES

To evaluate the competences CB5 and T3, the FE and MTE marks will be used as indicators. To evaluate the competences T5, E3 and E6, the FE and MTE marks, the follow-up activities (FA), the report and presentation (RP) and participation (P) marks will be used as indicators.

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6. Papers and reviews published in specialized journals.

**DOCUMENT HISTORY**

**PREVIOUS REVISIONS** (Indicate date and author / s, first the most recent one)

**CURRENT REVISION** (Indicate date and author / s)

March 25th 2019, Dr. Sara Cervantes

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