

Genetically Modified Animals and Gene Therapy

Code: 104081
ECTS Credits: 3

Degree	Type	Year	Semester
2502445 Veterinary Medicine	OT	5	0

Contact

Name: Maria Fátima Bosch Tubert
Email: fatima.bosch@uab.cat

Use of Languages

Principal working language: catalan (cat)
Some groups entirely in English: No
Some groups entirely in Catalan: No
Some groups entirely in Spanish: No

Teachers

Anna Maria Pujol Altarriba
Miguel García Martínez
Ivet Elias Puigdomenech
Verónica Jimenez Cenzano

Prerequisites

There are no prerequisites for taking this subject. However, to achieve the proposed learning outcomes it is recommended to the student to have previous knowledge of Cell Biology, Genetics, Basic and Advanced Instrumental Techniques, Molecular Biology and Genetics and Recombinant DNA Technology.

It is also recommended to the students to have a basic knowledge of English in order to be able to use information sources, which are basically in this language.

Objectives and Contextualisation

The objectives of the course will focus on:

- 1- To provide the student with knowledge in animal transgenesis and associated technologies including the description, the types of existing transgenic animals and their function.
- 2- To provide the student with knowledge in the gene manipulation of cells *in vitro* and *in vivo* for therapeutic purposes and associated technologies.

Competences

- Comunicar la informació obtinguda durant l'exercici professional de manera fluïda, oralment i per escrit, amb altres col·legues, autoritats i la societat en general.
- Demonstrate generic knowledge of animals, their behaviour and the bases of their identification.
- Demonstrate knowledge and understanding of standards and laws in the veterinary field and regulations on animals and their trade.

- Demonstrate knowledge and understanding of structural and functional disorders of the animal organism.
- Demonstrate knowledge and understanding of the aspects of organisation, finance and management in all fields of the veterinary profession.
- Demonstrate knowledge and understanding of the physical, chemical and molecular bases of the main processes in the animal organism.
- Recognise ethical obligations in the exercise of responsibilities in terms of the profession and society.
- Treat and handle animals in a safe and humanitarian manner, and instruct other people to properly employ these techniques.

Learning Outcomes

1. Adequately manipulate laboratory animals during experimental procedures.
2. Communicate information obtained during professional exercise in a fluid manner, orally and in writing, with other colleagues, authorities and society in general.
3. Describe applicable legislation on the utilisation of laboratory animals.
4. Distinguish the factors that influence the welfare of laboratory animals, both during maintenance and during use in experimental procedures.
5. Enumerate techniques for cloning experimental animals.
6. Explain the molecular and physiological bases of the pathologies of greatest interest in experimental animals.
7. Identify the functions of the Ethical Committee in animal experimentation.
8. Identify the methodologies for obtaining transgenic animals.
9. Morphologically characterise genetically modified mice.
10. Recognise personal limitations and know when to ask for professional advice and help.
11. Use assisted reproduction techniques in experimental animals.
12. Use molecular biology techniques.
13. Use refinement in experimental surgery performed in a conventional laboratory.
14. Use suitable techniques for in vivo and ex vivo functional studies.
15. Use techniques for obtaining and maintaining cell cultures and characterising cells in cultures.
16. Use the techniques employed in the structural and functional characterisation of proteins.

Content

THEORY PROGRAM

- 1- Generation of transgenic animals by pronuclear microinjection. Applications.
- 2- Generation of constitutive or conditional Knockout / in animals (tissue-specific and / or inducible) by Gene Targeting in embryonic stem cells. Applications.
- 3- Genomic Editing using ZFNs, TALENs and CRISPR / Cas9 for the generation of Knockout / in animals.
- 4- Clonal animals by nuclear transfer. Applications.
- 5- Conceptual bases of Gene Therapy.
- 6- *In vivo* gene therapy: adenoviral and adeno-associated vectors. Applications
- 7- *Ex vivo* gene therapy: retroviral and lentiviral vectors. Applications.
- 8- Genomic editing in gene therapy
- 9- Non-viral vectors. RNA therapeutics
- 10- Applications of gene therapy for the treatment of hereditary diseases

11- Applications of gene therapy in oncology

12- Applications of gene therapy for the treatment of high prevalence diseases.

SEMINAR PROGRAM

Students will prepare and perform an oral presentation, in front of the class, of a current research work, related to the course topics and published in international scientific journals. The preparation of this exhibition will be in groups of students and will be tutored. The oral presentation will be 10 minutes, distributed equally among the members of the group, plus 5 minutes for questions (total 15 minutes). The aim of the seminars is for students to become used to the search for scientific publications, their reading and interpretation, the critical vision of graphs, tables and results, in a guided way. On the other hand, students will also deep into current applications of transgenic animal technology and gene therapy..

CLASSROOM PRACTICES

Classroom practices will consist of the visualization of audiovisual material related to the technologies of obtaining genetically modified animals, as well as the presentation and discussion of different phenotypes of animal models and cases of gene therapy.

Methodology

The course consists of theoretical classes, practical classes, and oral presentations of tutored work. The formative activities of the subject complement each other.

Theory classes:

The content of the theory program will be taught mainly by the teacher in the form of master classes with audiovisual support. The presentations used in class by the teacher will be available to students on the Virtual Campus of the subject. These expository sessions will be the most important part of the theory section. It is highly recommended for students to regularly consult the books and links in the Bibliography section of this teaching guide and in the Virtual Campus in order to consolidate and clarify, if necessary, the contents explained in class.

Classroom practices:

Classroom practices will consist of the visualization of audiovisual material related to the technologies of obtaining genetically modified animals, as well as the presentation and discussion of different phenotypes of animal models and cases of gene therapy.

Classroom practices consist on 2 sessions of 4 hours each. The dates of the different groups of practices and the classrooms will be able to consult with the sufficient advance in the Virtual Campus of the course. Attendance at practical classes is mandatory.

Oral presentations of the students (seminars):

Students will prepare and perform an oral presentation, in front of the class, of a current research work, related to the course topics and published in international scientific journals. The preparation of this exhibition will be in groups of students and will be tutored. The oral presentation will be 10 minutes, distributed equally among the members of the group, plus 5 minutes for questions (total 15 minutes). The aim of the seminars is for students to become used to the search for scientific publications, their reading and interpretation, the critical vision of graphs, tables and results, in a guided way. On the other hand, students will also deep into current applications of transgenic animal technology and gene therapy..

Tutorials:

Oral presentations of papers are tutored. In addition, at the request of students, individual tutorials will be conducted throughout the course. The aim of these sessions will be to resolve doubts, review basic concepts and guide on the sources of information consulted and on how to make a scientific presentation in public.

UAB Surveys

15 minutes of one class will be allocated for the response of the UAB institutional survey.

Annotation: Within the schedule set by the centre or degree programme, 15 minutes of one class will be reserved for students to evaluate their lecturers and their courses or modules through questionnaires.

Activities

Title	Hours	ECTS	Learning Outcomes
Type: Directed			
Practical lessons	8	0.32	9, 2, 4, 6, 8, 1, 10, 13, 14, 12, 16
Theoretical lessons	16	0.64	9, 2, 3, 4, 5, 6, 7, 8, 10, 14, 11, 16
Type: Supervised			
Preparation of oral presentation	10	0.4	9, 2, 3, 4, 5, 6, 7, 8, 1, 10, 13, 14, 12, 11, 16, 15
Study and bibliographic research	28.75	1.15	9, 2, 3, 4, 5, 6, 7, 8, 1, 10, 13, 14, 12, 11, 16, 15
Tutorials	10	0.4	2, 10

Assessment

Description:

To approve the subject it will be essential to obtain a final grade of the subject equal to or greater than 5 points (out of 10) and to have attended the practices. The scheduled evaluation activities are:

A theoretical exam

It will count 90% of the final grade. It consists of a final exam, with test-type questions (true or false), referring to the subject taught in the theoretical classes and in the practical classes.

To approve the course, attendance at practical classes is mandatory.

There will be a theoretical exam for the recovery of the subject, with the same characteristics as the final theoretical exam, for students who have not approved it.

Oral presentation of the students

It will count for 10% of the final grade. The student will be evaluated individually for its oral presentation of the research work as well as the audiovisual support document that they have prepared in groups. Oral expression and the use of scientific language, aptitude and appropriate content will be valued.

A student will obtain the grade of Non-Assessable if the number of assessment activities carried out has been less than 50% of those scheduled for the subject.

Assessment Activities

Title	Weighting	Hours	ECTS	Learning Outcomes
Exam of practical and theoretical lessons	2	2	0.08	9, 2, 3, 4, 5, 6, 7, 8, 1, 10, 13, 14, 12, 11, 16, 15
Oral Presentation of Research Papers (Seminars)	15	0.25	0.01	9, 2, 3, 4, 5, 6, 7, 8, 1, 10, 13, 14, 12, 11, 16, 15

Bibliography

Bibliografia Animals Modificats Genèticament:

- Transgenic animals. Generation and use. L.M. Houdebine. Harwood Academic Publishers 1997.
- Mouse Genetics and Transgenics. A practical approach. Edited by: I.J. Jackson and C.M. Abbott. Oxford University Press. 2000. (www.oup.co.uk/PAS)
- Gene Targeting. A practical approach. Edited by: A.L. Joyner. Oxford University Press. 2000. (www.oup.co.uk/PAS)
- Manipulating the Mouse Embryo. A laboratory manual. (3rd Edition) Edited by: Andras Nagy et al. Cold Spring Harbor Laboratory Press. 2003.
- Transgenesis Techniques. Principles and Protocols. Edited by: Alan R. Clarke. Humana Press. 2002. (2nd Edition).
- Gene Knock-out Protocols. Edited by: Martin J. Tymms and Ismail Kola. Humana Press. 2001.
- Embryonic Stem Cells. Methods and Protocols. Edited by: Kursad Turksen. Humana Press. 2002.
- Human Molecular Genetics 2. T. Strachan i A.P. Read. John Wiley & Sons, Inc., Publication. 1999.
- Advanced Protocols for Animal Transgenesis. An ISTT Manual. Shirley Pease & Tomas L. Saunders (Editors). Springer. 2011.

Adreces d'interés:

<http://www.transtechsociety.org/>

<http://www.emmanet.org/>

<http://www.mousephenotype.org/>

Bibliografia Teràpia Gènica

- Gene and Cell Therapy. Therapeutic Mechanisms and Strategies. 2nd Edition. Edited by Nancy Smyth Templeton. Marcel Dekker, Inc. 2004.
- Gene Therapy technologies, applications and regulations. From Laboratory to Clinic. Edited by Anthony Meager. John Wiley & Sons, LTD. 1999.
- Gene Therapy Protocols. 2nd Edition. Edited by Jeffrey R. Morgan. Humana Press. 2002.
- Human Molecular Genetics 2. T. Strachan i A.P. Read. John Wiley & Sons, Inc., Publication. 1999.

- Cell Therapy. D. Garcia-Olmo, J.M. Garcia-Verdugo, J. Alemany, J.A. Gutierrez-Fuentes. McGraw-Hill Interamericana. 2008.

- Gene and Cell Therapy. Therapeutic Mechanisms and Strategies. Second edition, Revised and Expanded. N.S. Templeton. Marcel Dekker, Inc. 2004.

Software

Not applicable