COURSE DATA

DATA SUBJECT

Code: 43033
Name: Fundamentals of gene therapy
Cycle: Master's Degree / Doctorate
ECTS Credits: 3
Academic year: 2025-26

STUDY (S)

Degree	Center	Acad. year	Period
2138 - Master's Degree in Research in and Rational Use of Medicines	Facultat de Farmàcia i Ciències de L'alimentació	1	Annual

SUBJECT-MATTER

Degree	Subject-matter	Character
2138 - Master's Degree in Research in and Rational Use of Medicines	Fundamentals of gene therapy	ELECTIVES

COORDINATION

HERRERO CERVERA MARÍA JOSÉ

SUMMARY

The course aims to raise awareness of the new opportunities offered by the knowledge of the human genome in relation to therapeutic optimization, the development of new drugs and therapeutic potential of genes and / or nucleic acids used as drugs. Discusses the basics of pharmacology integrated with knowledge of Molecular Biology. It aims to introduce the basics of one side of Pharmacogenetics and Pharmacogenomics in relation to the rational use of drugs and as a pillar of interest in individualized therapy. On the other hand, introduces the concepts needed to understand the gene transfer procedures using appropriate physical or contest viral and nonviral vectors. It deals with gene therapy clinical projection in all its dimensions, by using somatic cells (differentiated or stem) or genetically modified cells. It delves into the therapeutic potential of these new procedures, as well as the effectiveness and risks of these and the ethical issues by their use.

ot;\"\"presentation\"\"\"">risks of these and the ethical issues by their use.

PREVIOUS KNOWLEDGE

RELATIONSHIP TO OTHER SUBJECTS OF THE SAME DEGREE

There are no specified enrollment restrictions with other subjects of the curriculum.

OTHER REQUIREMENTS

Interest in new therapeutic strategies. Eagerness to know the impact of the knowledge of the genome on health and disease. Aspiring to know the basis of the development of nucleic acids as a source of new drugs. Want to understand the dialogue between drugs gene and genome of the individual in order to repair and / or restore lost functions or introduce new therapeutic purposes. Want to know what the current trends on advanced therapies and what legal and ethical issues it generates.

COMPETENCES / LEARNING OUTCOMES

2138 - Master's Degree in Research in and Rational Use of Medicines

Be able to access the information required (databases, scientific articles, etc.) and to interpret and use it sensibly.

Be able to access to information tools in other areas of knowledge and use them properly.

Dominar la comunicación científica. Poseer habilidades sociales y comunicativas en la práctica asistencial.

Know how to write and prepare presentations to present and defend them later.

Manejar adecuadamente las fuentes de información biomédica y poseer la habilidad de hacer una valoración crítica de las mismas integrando la información para aportar conocimientos a grupos asistenciales multidisciplinares

Resolver de dilemas éticos derivados del empleo de medicamentos.

Select and manage available resources (instrumental and human) to optimise research outcomes.

Students should communicate conclusions and underlying knowledge clearly and unambiguously to both specialized and non-specialized audiences.

Students should demonstrate self-directed learning skills for continued academic growth.

Students should possess and understand foundational knowledge that enables original thinking and research in the field.

DESCRIPTION OF CONTENTS

1. Molecular Basis of Gene Therapy

This unit aims to introduce and develop the impact that knowledge of the genome on health and disease.
It introduces the concepts of pharmacogenetics and pharmacogenomics and values the importance of optimizing the patient's response to conventional drugs based on the genetic characteristics of the individual. It also introduces the concept of the use of nucleic acids as drugs and the molecular basis of gene therapy based on the design and construction of tracer and therapeutic genes.

2. Vectors, strategies and methods of gene therapy

This unit NAS pretend to give special characteristics that have to be engineered nucleic acids as drugs and in this sense, using vectors ell is an important requirement for nucleic acids can reach their therapeutic target effectively. Are analyzed and discussed the different types of available vectors (viral and nonviral) and the advantages and disadvantages of each, in order to have a criterion for selecting the most appropriate based on each disease or objective that predte . Were defined and shows the main types of design and procedures utilzación in vivo or ex vivo gene therapy and its safety profile.

3. Efficacy of gene therapy

This unit shows the therapeutic value of gene therapy, using experimental models. On one hand, assesses the significance and implications in diseases having inherited the introduction of new genes into cells to reestablish lost functions and as such where loss of function is compensated by the new gene, the cell can recover its normal. It is shown that the introduction of new functions in normal cells may contribute to achieve systemic therapeutic targets. On the other hand, assesses the importance of gene silencing as a method of gene therapy aimed at blocking the function of a mutated gene, whose expression is responsible for the pathology. Finally it addresses the procedures to repair mutations in the genome, with the advantage that the gene may play its role repaired with high efficiency and safety.

4. Projection of gene therapy clinical

This unit aims to show genetic drugs already on the market and the current state of clinical trials in humans. In these cases, we evaluate its effectiveness and safety in use in different diseases hereditary or acquired, and their interest in the development of genetic vaccines or vaccines, DNA vaccines and genetically modified cells.

5. Ethical and legal aspects of gene therapy.

The modification and / or genetic manipulation of cells has social implications of important ethical and legal. Examining issues related to confidentiality of genetic information collected, the right to know and not know the patient, the limits of genetic manipulation of somatic or germ cells, as well as the use of embryonic cells, reproductive or therapeutic cloning, the inducible production of stem cells, hybrids, etc.

WORKLOAD

PRESENCIAL ACTIVITIES

Activity	Hours
Theory	17,50
Seminar	3,50
Total hours	**21,00**

NON PRESENCIAL ACTIVITIES

Activity	Hours
Attendance at other activities	0,00
Individual or group project	0,00
Independent study and work	0,00
Preparation of lessons	0,00
Preparation for assessment activities	0,00
Resolution of case studies	0,00
Total hours	**0,00**

TEACHING METHODOLOGY

1) Theory based participatory lecture by posing questions
2) Seminars presented by students on topics offered by the teacher, under the guidance and supervision of the same
3) Discussion of clinical trials and / or papers, moderated by professor
4) Debates on sensitive issues, ethical or legal derived human gene therapy
5) Expert Conferences
6) Resolution of issues raised on-line

To complete the classroom hours, the materials provided for face-to-face teaching will be adapted, so that the student can access them at any time. Use of the virtual classroom forum to answer questions.
For the practical sessions of the theoretical content, the use of videoconferences and / or the completion of the exercises proposed would be combined using the "Task" option in the virtual classroom.

During the activities, both theoretical and practical, the applications of the contents of the subject in relation to the Sustainable Development Goals (SDG) will be indicated. This is intended to provide knowledge, skills and motivation to understand and address these SDGs, while promoting reflection and criticism.

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EVALUATION

1) Avaluació formativa: Problemes i qüestions: 40%
2) Avaluació final: Examen teòric: 40%
3) Treball: 20 %

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REFERENCES

- - Artículos y revisiones en revistas especializadas en el tema: - Gene Therapy - Human Gene Therapy - Cancer Gene Therapy - Journal of Gene Medicine - Molecular Therapy