

## SYLLABUS DEL CORSO

### Editing di Geni e Genomi

2223-1-F0802Q078

---

#### Aims

In the last years, we are witnesses of an explosion of genome editing techniques which are continuously improving and finding important applications, both in the industrial and in the health field. Knowledge of these topics is essential for our students and for their future work.

This course aims at teaching in details specific, up-to-date and widely used techniques for genome editing and their applications. Specific cases of applications to address different biological questions will be analyzed in detail. Technical and experimental details will be addressed as well, in order to help the student to understand genome editing techniques.

- Knowledge and understanding: at the end of this course students will be aware of the several different techniques for genome editing and of the applications that they can be used for, including their limitations.
- Applying knowledge and understanding: by analysing different cases and applications to both industrial and research processes, students will gain the ability to choose the most suitable technique for a specific biological problem.
- Making judgment: the student will be able to elaborate what he has learned and to apply it to several biological problems; moreover, she/he will be able to interconnect the new knowledge with others offered in this master degree course.
- Communication skills: at the end of the course the student will be able to express himself with appropriate scientific language properties.
- Learning skills: students will be autonomous and they will be able to look for, to apply, to integrate and to connect the acquired knowledge.

#### Contents

The course is focused on the most important and up-to-date systems widely used for genome editing approaches. Deep analyses of their pros and cons, applications (industry and/or research), limitations (including consequences for genome stability), future applications and optimizations will be discussed.

An important part of the course is focused on genome editing applications, both in the industrial field (e.g. manipulation of microorganisms for better production, biological drugs production and synthetic biology), as well as in the health field, (e.g. genomic screenings, animal models of specific diseases, diagnosis and therapy approaches).

## Detailed program

- An introduction to Genome Editing : homologous recombination, gene targeting, knock-in, knock-out.
- Site-specific nucleases : Meganucleases, Zinc-finger nucleases (ZFN) and TALEN nucleases; mechanism of action through DNA double-strand breaks formation and repair; applications (knock-out, knock-in, transcriptional regulation). Analysis of specific scientific papers.
- CRISPR-Cas: RNA-guided specific nucleases. The original system in bacteria and archaea.
- CRISPR-Cas for genome editing: knock-out, knock-in, base editing and gene correction, regulation of gene expression, inducible CRISPR-Cas systems. Considerations about different Cas proteins widely used so far. Direct examples of several different application from basic research to pre-clinical studies through the analysis of scientific papers.
- CRISPR-Cas for genome editing: applications, versatility, limitations, future perspectives. Analysis of scientific papers.
- Transposons-based genome editing. PiggyBac and Sleeping Beauty transposons. Applications and pros/cons.
- Genomic screening and genes function assesment by genome editing. Application to the understanding of pathogenesis processes and to the identification of new targets. CRISPR-based genome-wide screening. Analysis of specific scientific papers.
- Genome editing in organisms and industrial applications: bacteria, yeast, mammals. Industrial applications for microorganisms improvement : biological pathways reconstitution and synthetic biology
- Genome editing and biological drugs: e.g. vaccines and monoclonal antibodies production
- Genome editing for generation of animal models of diseases : some examples about cancer, metabolic and cardiovascular diseases.
- Genome editing for therapeutic applications. Recent examples about clinical trials

## Prerequisites

Genetics, molecular biology and industrial microbiology theoretical bases.

Prerequisites: none

## Teaching form

Frontal teaching : slides and analysis of scientific papers.

Different group work on scientific papers and subsequent oral presentations by the students.

Teaching language: italian

## Textbook and teaching resource

Slides, review articles and research articles available at the e-learning web page.  
Teaching resources are mainly in english.

## **Semester**

FIRST semester

## **Assessment method**

ORAL EXAM at the end of the course.

No intermediate evaluations/partial exams.

The exam will assess, by questions and discussions, all the topics addressed during the course and in detail:

- a) basic knowledge
- b) understanding and critical reading of scientific papers
- c) knowledge of specific technical methods

## **Office hours**

make an appointment with the professor by e-mail

## **Sustainable Development Goals**

GOOD HEALTH AND WELL-BEING | QUALITY EDUCATION

---