UNIVERSITÀ
DEGLI STUDI DI MILANO-BICOCCA

COURSE SYLLABUS

Genome Editing

2526-1-F0803Q078

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.

1. 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.
2. 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.
3. 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. This ability will be built upon the analysis of examples of different applications in basic research and in the industry.
4. Communication skills: at the end of the course the student will be able to express himself with appropriate scientific language properties.
5. 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. From yeasts to mammals.
- Site-specific nucleases : how they revolutionize genome editing, working principles, generation of knock-outs and knock-ins, available technologies.
- Meganucleases, Zinc-finger nucleases (ZFN) and TALEN nucleases: detailed analyses about what they are and how they work, their applications and limitations. Analysis of specific scientific papers. Real examples of applications in the pipeline of different pharma companies.
- CRISPR-Cas: RNA-guided specific nucleases. The original system in bacteria and archea.
- 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 anlaysis of scientific papers.
- CRISPR-Cas for genome editing: applications, versatility, limitations, future perspectives. Analysis of scientific papers.
- Upgrade of CRISPR-Cas systems: Base editors and Prime editors. Working principles, novelty, applications, limitations.
- Transposons-based genome editing. PiggyBac and Sleeping Beauty transposons. Applications and pros/cons
- Genomic screening and genes function assessment 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
- Genome editing and biological drugs: e.g. 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

18 2-hours lessons composed by **face-to-face** delivered didactics (Didattica erogativa, DE) focused on the presentation-illustration of contents and theoretical principles by the lecturer (30 hours) as well as 6 hours of interative teaching including teaching interventions supplementary to delivered didactic activities, exploration and

active discussion of practical applications (e.g. journal club, case analyses).

3 2-hours **face-to-face** lessons given by external experts and supervised by the Professor composed by 3 hours of delivered didactics (Didattica erogativa, DE) and 3 hours of interactive teaching (Didattica Interattiva, DI) including teaching interventions supplementary to delivered didactic activities, exploration and active discussion of practical applications.

Teaching laguage: english in case non italian students are enrolled in this course

Textbook and teaching resource

Slides, review articles and research articles available at the e-learning web page. The exam will focus on concepts explained in the slides.
Teaching resources are mainly in english.

Lessons will be recorded and available for students

Semester

SECOND semester (March-June)

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 of genome editing systems
c) understanding the meaning of specific scientific approaches
c) knowledge of different examples of real applications

Office hours

make an appointment with the professor by e-mail

Sustainable Development Goals

GOOD HEALTH AND WELL-BEING | QUALITY EDUCATION