

Titre: BIOTHERAPY MEG M2 module

Head:

Frank Griscelli

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Effective: 35

Language: English

Prerequisites:

MEG Master 1

Where:

Paris University

When:

First Semester

Evaluation:

written exam (2h): article analysis

Questions:

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Number ECTS : 3

Total number of hours: 30

Teaching format:

Conferences, 1h30 each with questions; shared pdf presentations

Teaching objectives

The MEG M2 Biotherapy UE covers two major fields: cell biotherapeutics and gene therapy. These «personalized advanced therapies» make use of custom or «à la carte» products which are specifically generated for an individual patient. The objective of the module is to understand how Biopharmaceuticals- which are complex macromolecules - are created by biotechnology. They may also involve genetic manipulation of living organisms to treat somatic and genetic diseases for which no conventional drug are currently available.

Cell therapies are either based on somatic cells or derived from stem cells, whether Embryonic or iPSCs. Cells are grown *in vitro* and then engrafted into the patient after differentiation. They can be from autologous or foreign origin. They can also be genetically engineered then fitting the definition of gene therapy. The later involve gene transfer to shuttle therapeutic transgenes to the nucleus of target cells. Gene therapy vectors can be of viral or synthetic origin; more recently strategies aiming at homologous recombination use designer nucleases such as the CRISPR-Cas system to perform targeted gene surgery.

The aim of this UE is to provide students with keys to understand «personalized advanced therapies» and prepare for career opportunities in both Academia and the rapidly developing Industry sector at the national, European, and international levels.

Teaching outline

The program covers the background and fundamental biological concepts needed to understand the various and complex biotherapy approaches. It also provides an introduction to regulatory issues, as well as manufacturing or industrial aspects involved to reach patients' bedside. Successful clinical applications are presented.