

DEPARTMENT OF LABORATORY MEDICINE

H5F6037, Advanced Therapeutic Medicinal Products (ATMPs) Enabled by Regenerative Medicine and Gene Editing Technologies, 2 credits (hec)

Avancerade terapeutiska läkemedel (ATMP) genom regenerativ medicin och genterapiteknologier, 2 högskolepoäng

Third-cycle level / Forskarnivå

Approval

This syllabus was approved by the The Committee for Doctoral Education on 2025-03-04, and is valid from autumn semester 2025.

Responsible department

Department of Laboratory Medicine, Faculty of Medicine

Prerequisite courses, or equivalent

No prerequisite courses, or equivalent, demanded for this course.

Purpose & Intended learning outcomes

Purpose

This course aims to advance participants' knowledge of Advanced Therapeutic Medicinal Products (ATMPs) enabled by regenerative medicine and gene editing technologies through selected lectures. These innovative therapies leverage cutting-edge scientific advancements to treat, modify, or prevent diseases. They include gene therapies, somatic cell therapies, and tissue-engineered products, all designed to restore or regenerate function in damaged tissues or organs. By offering this course on ATMPs developed through gene editing technologies like CRISPR and advancements in regenerative medicine, participants will gain insight into novel advanced treatments for previously untreatable conditions.

Intended learning outcomes

By the end of this course, students will be able to:

- Demonstrate a comprehensive understanding of the foundational concepts of Advanced Therapeutic Medicinal Products (ATMPs) and their role in regenerative medicine.
- Explain gene editing technologies and their applications in modern medicine.
- Describe gene therapies, somatic cell therapies, and tissue-engineered products and their significance in treating diseases.
- Analyze the applications of ATMPs in treating, modifying, or preventing diseases.
- Understand the principles and mechanisms behind gene editing tools such as CRISPR.
- Evaluate current scientific advancements in ATMP research.
- Identify research trends and emerging technologies in the field of ATMPs.

Course content

Topics

- Stem cells technology and reprograming
- Genome engineering technologies in human cells (base editors, prime editors, nucleases, transposons)
- High-throughput cell expansion technology "Prodigy"
- Patient specific isogenic cell transplantation
- Spheroids of human autologous matrix-associated chondrocytes
- ATMPs and their categories.
- ATMP regulations and directives in Europe.
- Intellectual property in therapeutics.
- Ethical and regularory aspects
- During the course, we will go through ATMPs that are authorized by the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA).

Forms of teaching and learning

Forms of teaching and learning

The course will include lectures and seminars by internal and external experts, complemented by one or two short practical lab demonstration sessions. Additionally, group discussions will be incorporated to emphasize the course's key themes.

Language of instruction

The course is given in English

Grading scale

Pass (G) /Fail (U)

Compulsory components & forms of assessment

Compulsory components

Compulsory participation in laboratory demonstrations is required. If a student is unable to attend, they must complete an individual literature assignment to compensate for their absence.

Forms of assessment

The ILOs of this course will be assessed through a written exam consisting of open-ended and multiple-choice questions. These questions will evaluate whether students have understood the key concepts and principles of the course.

Course literature

Recommended course literature:

- Single-Use Technology in Biopharmaceutical Manufacture. ISBN:9781119477839. Single-Use Bioreactors for Manufacturing of Immune Cell Therapeutics. doi:10.1002/9781119477891.ch30
- Bioprocessing for Cell Based Therapies. ISBN:9781118743416
- Cell Therapy in Practice. doi:10.1002/9781118743362.ch8
- Bioprocessing for Cell Based Therapies. ISBN:9781118743416
- Overview of the Cell Therapy Field. doi:10.1002/9781118743362.ch1
- Practical Transfusion Medicine. ISBN:9781119665816. Regulation and Accreditation in Cellular Therapy. doi:/10.1002/9781119665885.ch41
- Stem Cells in Regenerative Medicine: Science, regulation and business strategies. ISBN:9781119971399. Regulatory and intellectual property considerations for therapeutic human stem cell-based regenerative medicine product development: a US perspective. doi:10.1002/9781118846193.ch6