



DEPARTMENT OF LABORATORY MEDICINE

H5F6056, From Research to ATMP Treatment - Process Development, Quality Assurance, Regulatory Affairs and Logistics, 3 credits (hec)

Från forskning till ATMP terapi - processutveckling, kvalitetssäkring, regulatoriska krav och logistik, 3 högskolepoäng

Third-cycle level / Forskarnivå

Approval

This syllabus was approved by the The Committee for Doctoral Education on 2025-02-22, and is valid from autumn semester 2025.

Responsible department

Department of Laboratory Medicine, Faculty of Medicine

Contributing department/s

Department of Clinical Science, Intervention and Technology

Prerequisite courses, or equivalent

None

Purpose & Intended learning outcomes

Purpose

The aim of the course is to provide a comprehensive understanding of all steps in process development and production of cell and gene therapy products.

During the course students will gain insight into establishing standard operating procedures (SOPs) for the production of cell and gene therapy products, and how to validate product purity and function. They will learn to understand differences between process development within pre-GMP and GMP and transfer of SOP from pre-GMP to GMP. They will also learn to understand differences/similarities between pre-GMP and GMP and the regulatory

requirements that exist within ATMP. The students will also obtain an overview of both regulatory regulations and legal aspects of ATMP products.

Upon successful completion of the course, students will have acquired basic knowledge of how to set up production of a cell or gene therapy product according to good manufacturing practice (GMP).

Intended learning outcomes

On completion of the course the student should be able to:

1. Describe basis of ATMP, GMP, Quality Control/Quality Assurance, tissue establishment (vävnadsinrättning)
2. Describe routines and procedures how to work in a classified cleanroom
3. Successfully gown-up for cleanroom, aseptic gowning
4. Perform selected steps of genetic modification of cell production.

Course content

The course will cover basic techniques in the production of ATMP. To give the students a good knowledge of the complexity of all steps we will use real world examples of the development of various ATMP products such as; Vecura, Nextcell, KITM, Procella, Hospital pharmacy, (radiopharmacy) preparation, AFERES (KITM)

The fundamental differences between research, process development and clinical production will be the focus of the course. Lectures by experienced researchers will showcase the development of a product from research, process development, GMP production and clinical trials. Lectures will cover topics such as: pre-GMP and GMP, Process development/SOP, ATMP/JACIE, GLP, GDP, GMP, QC/QA, Gene therapy, CAR T-cells and more.

In laboratory sessions the students will produce and formulate mRNA, genetically modified cells in the closed bioreactor system. The students will gain practical experience in working in a GMP-like environment.

Forms of teaching and learning

The course is 2 weeks long on site at ANA Futura at KI Campus South. Lectures, seminars, workshops and study visits are held Monday to Friday in the morning. Laboratory work will be carried out in the afternoons in our state-of-the-art pre-GMP laboratory. The students will visit several ATMP production facilities including Karolinska GMP facilities, clinics using cell and gene therapy and local ATMP SMEs.

Language of instruction

The course is given in English

Grading scale

Pass (G) /Fail (U)

Compulsory components & forms of assessment

Compulsory components

All lectures, seminars and labs are compulsory. To compensate for absence, students may be required to submit a written summary on the topics discussed during the missed sessions.

Forms of assessment

Knowledge will be assessed in a written examination at the end of the course. The written examination will cover lectures, seminars, site visits and laboratory procedures.

The student will have to successfully perform practical procedures such as gowning and cleaning.

The final assessment of pass or fail will include written examination and practical procedures.

Course literature

Recommended course literature will consist of

- Selected scientific articles
- Regulatory guidelines for GMP
- Legal framework and directives for ATMP
- Selected handbooks i.e. bioreactor, formulation