



DEPARTMENT OF LABORATORY MEDICINE

H5F5737, Nucleic Acid Chemistry and Therapy, 3 credits (hec)

Nukleinsyrekemi och terapi, 3 högskolepoäng

Third-cycle level / Forskarnivå

Approval

This syllabus is approved by the The Committee for Doctoral Education on 2023-12-27, and is valid from Spring semester 2024.

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

The purpose of the course is to enable participants to acquire good and up to date knowledge of nucleic acid chemistry and nucleic acid therapy. The intention is to increase general knowledge about nucleic acids and how these can undergo different reactions as well as how oligonucleotides are chemically synthesized and modified for therapeutic or other use. We expect that this will stimulate and inspire the students in their own research whether they work on nucleic acid biology, therapy, analyse nucleic acids or use nucleic acids/oligonucleotides as tools for investigations. It is an intention to increase understanding of nucleic acids, how these molecules work and how they can be used in therapy. In addition, knowledge on how oligonucleotides are synthesized and how these can be modified will enhance the insight and enable the students to improve their use of oligonucleotides as tools or potential therapeutics.

Intended learning outcomes

At the end of the course the students

- should be able to explain the underlying chemistry of nucleic acids and how these can react at

different parts of the structure.

- should be able to explain how oligonucleotides can be synthesized and modified and why currently used modifications and conjugations are done.
- should understand the different modalities of Nucleic acid therapies, a field that boomed in the last decade.
- should be able to make selections of modifications and to design oligonucleotides/nucleic acids, for use as potential therapeutics.

Course content

Introduction to Nucleic acid chemistry and nomenclature
 Reactions at nucleic acid bases, ribose and deoxyribose sugars
 Reactions at phosphates and phosphate modifications
 Methods for synthesis of native and modified oligonucleotides
 Common modifications used for oligonucleotide therapy
 Introduction to Oligonucleotide therapy
 Antisense, pre-mRNA Splice-switching, siRNA, mRNA and DNA-targeting ON therapy
 mRNA and Crispr/Cas as potential therapeutic agents
 The problem of oligonucleotide delivery in therapy

Forms of teaching and learning

The course consist of lectures by experts and seminars in the presence of experts on different aspects of nucleic acid chemistry and oligonucleotide/nucleic acid therapy as well as workshop activity including presentations by the students. Additional home work combined with individual and group activities in the class room will be a part of the learning.

Language of instruction

The course is given in English

Grading scale

Pass (G) /Fail (U)

Compulsory components & forms of assessment

Compulsory components

The lectures, seminars and workshop activities with student presentations will be compulsory. Absence will be compensated by extra assignments. The student will also submit reports from a workshop in written form for review and approval, in connection to the student presentations.

Forms of assessment

Oral presentations on workshops as well as a written account with specific course related questions.

Course literature

Lectures and handouts from the lectures as well as articles found during the workshops.

Recommended books:

- Nucleic Acids in Chemistry and Biology. Editors: G M Blackburn, M J Gait, D Loakes, D M Williams. RSC Publishing

- Advanced Organic Chemistry of Nucleic acids. Z. Shabarova, A Bogdanov, VCH Verlagsgesellschaft.

In addition a couple of the most up to date review articles may be recommended to registered students by lecturers for the respective topics.