Course code	BUM103				
Course title	GENE THERA	PY			
General information					
Study programme	Graduate study "Biotechnology in medicine"			Academic	
				year	
Lecturer	Prof. Dr. Sc. Sandra Kraljević Pavelić				
Status		Required		Elective	
ECTS system		<u>-</u>			3
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Course objectives

Intensive biomedical research in the last few decades has unravelled the molecular mechanisms of a number of diseases. These developments were followed by dramatic improvements in gene manipulation in animal models. Together, both achievements have enabled foundation for development of therapies based on gene manipulation.

During this course student will obtain insight into the most recent advances in gene therapy, including methodology, effectiveness as well as potential hazards and ethical issues related to use of gene therapy.

Course description

- 1. definition of gene therapy and overview of pathological conditions to be treated with gene therapy
- 2. in vitro manipulation of genes for application in gene therapy
- **3.** regulation of gene expression in gene therapy
- **4.** preparation of vectors (viral and non-viral)
- 5. in vivo, ex vivo and in situ gene therapy
- **6.** gene silencing in therapy
- 7. accomplishments and failures of gene therapy
- **8.** technical problems with current gene therapy trials, required technical advances, potentials of gene therapy in future
- **9.** ethical and socio-economical aspects of gene therapy

Learning outcomes

- 1. knowledge in basic principles of gene therapy and newest accomplishments in the field: A1, A3, A6, A7
- 2. knowledge in current technical obstacles and ways of their solving in future: C1, C2,
- **3.** capability to present a topic and critically comment of ethical and socio-economical consequences of gene therapy: A2.1, A2.2, A2.3, A8, B1