COURSE DESCRIPTION (SYLLABUS)

1.	Course: **Genetic manipulation and selected aspects of gene therapies**
2.	Language of instruction: **English**
3.	Faculty: **Faculty of Biotechnology**
4.	Course/module code: **29-BT-S2-E4-GMSAGT**
5.	Course/module type (*mandatory* or *elective*): **mandatory**
6.	Programme: **Medical Biotechnology**
7.	Study cycle: **2nd cycle**
8.	Year: **2nd**
9.	Semester *(autumn* or *spring):* **spring**
10.	Form of tuition and number of hours: Lecture: **15 h**
11.	Name, Surname, academic title: **Ryszard RZEPECKI, Prof.**
12.	Initial requirements (knowledge, skills, social competences) regarding the course/module and its completion: **Knowledge of nucleic acid structure and functions, genomic organization in *Eucaryotes*; molecular genetics, metabolism of nucleic acids, replication transcription, splicing, transport and translation of mRNA, cell biology, regulation of cell cycle including regulation of nucleus re-assembly. Basic knowledge of histology, embryology and regulation of development of animals.**
13.	Objectives: **The main objectives are to present and make students familiar with:** • **genetic manipulations in molecular biology laboratories;** • **basic methods of genetic engineering on cellular and animal model systems;** • **methods of manipulation on expression level of particular gene and protein;** • **novel methods of genome editing as well as methods for detection of protein-nucleic acid interactions *in vitro* and *in vivo;*** • **historical strategies of gene therapies and currently performed treatment**

	strategies in model organisms and in humans; • **typical, well developed strategies for monogenic disorders;** • **most frequent gene therapy strategies for cancer;** • **future trends for gene therapy treatment strategies.**	
14.	Content: **First group of lectures about genetic manipulations in molecular biology laboratories will serve as introductory course to gene therapy. Students will get familiar with basic methods of genetic engineering on cellular and animal model systems They will learn about methods of manipulation of expression level of particular gene and protein. Novel methods of genome editing as well as methods for detection of protein-nucleic acid interactions *in vitro* and *in vivo* will be described. The second part of the lecture will focus on description of gene therapy historical strategies and currently performed treatment strategies in model organisms and in humans. Typical, well developed strategies for monogenic disorders will be described and analysed. The most frequent gene therapy strategies for cancer will be also be described. At the last lecture an existing, gene therapy treatment strategies will be discussed and future trends will be presented.**	
15.	Learning outcomes: • **make a qualitative and quantitative description of the basic biological phenomena and processes** • **be able to link theoretical knowledge of biochemistry, biotechnology, molecular biology and microbiology with its practical application in industry, health care and environmental protection** • **make the synthesis of information from various sources and be capable of correct conclusions based on them** • **use proper scientific language and terminology in discussions with specialists in the field of biotechnology** • **understand the need for continuing education throughout the whole life, including broadening knowledge in biotechnology** • **understand the need for careful planning of tasks and scientific experiments** • **possess the ability to plan experiments using molecular biology methods** • **knows basic safety procedures and ergonomics in modern molecular and cellular laboratory, knows the procedures and ethical issues associated with work with GMO and GMM** • **Is able to perform critical analyses of techniques and laboratory procedures used for genetic engineering and gene therapy procedures** • **Is able to critically analyse and interpret provided experimental data/experimental papers and is able to draw independent and proper conclusions** • **Is able to work as a team member in planning and**	Outcome symbols: K2_W01, K2_W02, K2_W07, K2_W09, K2_U01, K2_U06, K2_K02

<table>
<tr><td></td><td colspan="2">solving experimental procedures.</td></tr>
<tr><td>16.</td><td colspan="2">Recommended literature:
Current review literature, current experimental papers with new discoveries in genetic engineering and gene therapy OMIM, FDA and EMA databases.</td></tr>
<tr><td>17.</td><td colspan="2">Methods of verification of the assumed learning outcomes:
• written examination report and oral presentation of examination report in front of entire class.</td></tr>
<tr><td>18.</td><td colspan="2">Conditions of earning credits:
• participation in lectures, active participation in discussions and answering control questions during lectures;
• written examination report and oral presentation.</td></tr>
<tr><td rowspan="6">19.</td><td colspan="2">Student’s workload:</td></tr>
<tr><td>Activity</td><td>Number of hours for the activity</td></tr>
<tr><td>Hours of instruction (as stipulated in study programme) :
• lecture: 15 h</td><td>15 h</td></tr>
<tr><td>Student’s own work:
• reading the literature
• preparation for the exam and presentation</td><td>15 h</td></tr>
<tr><td>Total number of hours:</td><td>30 h</td></tr>
<tr><td>Number of ECTS:</td><td>2 ECTS</td></tr>
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