

## **RNA – Unusual Properties and Applications in Science and Medicine** (lecture 15h, ETCS 2, test)

The goal of the lecture is the presentation of RNA – macromolecule playing indispensable role in a cell, its growth and proliferation. RNA is important not only in translation of genetic code from the nucleotide sequence to amino acid sequence in proteins, but also in metabolism regulation on the level of transcription and translation. RNAs act as gene expression regulators in the process of RNA interference, as riboswitches, or through their catalytic activities. Students will learn about the connections between the RNA structure and functions, mechanisms of gene expression regulation and the potential of RNA application in diagnostics and medicine (gene therapies). The methods of therapeutic RNA delivery to target cells will also be presented.

### 1. RNA structure (1 h)

#### 1.1. Comparison of RNA and DNA

1.2. Properties of RNA resulting from its structure – flexibility, tertiary structures, motives, intra and intermolecular interactions

### 2. Mechanisms of RNA interference (4 h)

2.2. siRNA and miRNA – two pathways leading to gene silencing

2.3. The most important protein involved in RNAi: DICER, AGO

2.4. Applications of RNAi in gene function identification, cancer diagnosis and therapy, antiviral therapy and neurodegenerative diseases therapies

2.5. Methods for silencing RNA delivery to target cell

### 3. Riboswitches as molecular targets in therapy (4 h)

3.1. Types of riboswitches and their structures

3.2. Mechanisms of riboswitch action

3.3. The potential of riboswitches in antibacterial therapies

### 4. Ribozymes in therapy (3 h)

4.1. Catalytic properties of RNA

4.2. Types of ribozymes

4.3. „RNA World” hypothesis

4.4. Therapeutic applications of ribozymes

### 4. Viral vectors in gene therapy (2 h)

4.1. Gene therapy clinical trials – statistics: number of trials in the world, geography, diseases treated with gene therapy, therapeutic genes

4.2. The most often used viral vectors and methods for their preparation

4.3. Advantages, limitations and dangers connected with introducing of viral vector into human organism

The completion of the subject is written. The test consists of 5 open questions, each scored in the range of 0-4 points (10.1-12 points - 3.0; 12.1-14 points - 3.5; 14.1-16 points - 4.0; 16.1-18 points - 4.5; 18.1-20 points - 5.0). The condition for passing the course is passing the colloquium, i.e. obtaining a grade of at least 3.0. In the event of failure to pass the colloquium, a make-up colloquium is planned on the agreed date. The subject can be repeated the following year if it is conducted.