Course Code: ABS-938 CRISPR Gene Editing 3(3-0) CHs

Learning Objectives

1. Gains advanced subject and methodological knowledge regarding genome editing technology,

learns the history of CRISPR/Cas9 technology development, knows different genome editing strategies and examples of their applications

<u>Outcomes</u>

- 2. Upon finishing the course, the student should:
 - a. design sgRNA and predict its specificity
 - know the experimental methods to evaluate the CRISPR/Cas efficiency and specificity
 - c. determine applicative goals for the application of selected genome engineering methods
 - d. critically evaluate safety and ethical issues of genome editing technology

3. <u>Content</u>

- a. The history of CRISPR/Cas technology development
- b. Rapid and efficient gene deletion by CRISPR/Cas9
- c. DNA repair mechanisms
- d. CRISPR-gRNA Design
- e. Tracking CRISPR's footprints
- f. Fast and Quantitative Identification of ex vivo precise genome targetinginduced indel events by IDAA
- g. Functional evaluation of CRISPR activity by the dual-fluorescent surrogate systems
- h. Methods used in genome editing technology

CRISPR-Cas9 delivery by artificial virus (RRHPC) Production and Validation of lentiviral vectors for CRISPR/Cas9 delivery Screening of CRISPR guide RNAs (gRNAs) in cultured cells using adenoassociated viral vectors Electroporation-based CRISPR/Cas9 gene editing using Cas9 protein and

chemically

- Modifications and orthologs of Cas9 protein
- Examples of CRISPR/Cas technology applications
- Next generation sequencing

First, second and third generation sequencing technologies Ion Torrent and illumina methods

modified sqRNAs

- CRISPRi and RNA editing
- CRISPR/Cas9-mediated gene tagging
- CRISPR-based lentiviral knockout libraries for functional genomic screening and identification of phenotype-related genes
- Ethical aspects and safety of genome editing technology

g. Details of lab work (if applicable)

Not applicable

h. Recommended Readings

Text Book

Genome engineering using the CRISPR-Cas9 system. Ran FA, Hsu PD, Wright J, Agarwala V, Scott DA, Zhang F. Nat Protoc. 2013;8(11):2281-2308

Reference Books

1. A programmable dual-RNA-guided DNA endonuclease in adaptive bacterial immunity. Jinek M, Chylinski K, Fonfara I, Hauer M, Doudna JA, Charpentier E. Science. 2012;337(6096):816-21.

2. The Heroes of CRISPR. Eric S. Lander, Cell Volume 164, Issues 1-2, p18–28, 2016.

3. CRISPR interference (CRISPRi) for sequence- specific control of gene expression. Larson, M. H.; Gilbert, L. A.; Wang, X; Lim, W. A.; Weissman, J. S.; Qi, L. S. (2013) Nature Protocols. 8 (11): 2180–96.