Module: Genomes and gene editing

Module coordinator: Axel Schambach

Module outline:
This module will focus on current state-of-the-art technologies employed in basic research and used to develop gene and cell therapy applications. The different scientific methods to transfer genetic information into target cells will be explained. This will provide students with the necessary background for teaching the various gene therapy strategies that have been tested pre-clinically and clinically, including control of gene expression and available approaches for targeted genome editing.

Topics:
Gene Transfer: Overview of the appropriate use of non-viral and viral gene transfer methods, including discussion of the strengths and weaknesses of each approach. Systems commonly used in pre-clinical and clinical studies will be presented, such as electroporation, Sleeping Beauty transposon, adeno-associated virus (AAV) and retroviruses (lentiviral, alpha- and gammaretroviral systems).

Gene Expression: Genetic and pharmacological mechanisms to control gene expression will be presented, including antisense oligonucleotides, inhibitory RNAs (e.g. short-hairpin RNA) and microRNA.

Gene Therapy: Students will learn the history of gene therapy and the principal concepts behind gene therapy applications. We will cover the early gene therapy failures and the important lessons learned from these studies, which led to modified strategies based upon improved understanding of genetic components and how this knowledge led to successful translation for the treatment of human diseases like genetic disorders and cancer.

Genome Editing: Students will be introduced to genome editing technologies, including transcription activator-like effector nucleases (TALENs), zinc-finger nucleases (ZFNs) and clustered regularly interspaced short palindromic repeats (CRISPR)-associated nuclease Cas9.

Bioinformatics: Students will get an overview of bioinformatics resources for analysis of protein sequences. They will be introduced to immunomic and infectivity databases and learn how to deal with immunogenicity analysis.

Learning: 5 ECTS
Lectures: 32h
Seminars: 32h
Independent work: 86h

Assessment:
50% Oral presentation (scientific paper)
50% Written review of a research paper