

Future Medicines: Gene and Cell Therapies

16 July 2018 – 20 July 2018

Course director: Dr. Ed Moret

Contact: science.fmf@uu.nl

Day 1 – 16 July 2018		
On Day 1, we will give an elaborate introduction to the topic, including both a historic overview and 'setting-the-scene'. Furthermore, we will dive into a very important aspect of this new class of drugs: the quality control.		
09.00 – 09.15	coffee	Walk in & Registration
09.15 – 09.30	opening	Welcome & Opening (Dr. Ed Moret)
09.30 – 10.15	lecture	Historical overview of drug development (Dr. Ed Moret)
10.15 – 10.30	coffee	coffee break
10.30 – 11.00	lecture	Quiz
11.00 – 12.30	lecture	Setting the scenes: Key aspects and the current landscape of cell and gene therapies (Holland Bio)
12.30 – 13.30	lunch	lunch break
13.30 – 15.00	lecture	Cell and Gene Therapies: All about quality (Dr. Pauline Meij & Dr. Annie Rietveld)
15.00 – 15.30	break	soda break
15.30 – 16.30	lecture	The story of Glybera & Stremvelis (Renske ten Ham & Delphi Coppens)
16.30 – 17.00	lecture	Introducing the case studies
17.00 – 18.30	poster	Poster session with drinks
18.30 – 20.00	dinner	dinner break

Day 2 – 17 July 2018		
During day 2, we will take a critical look at the way gene and cell therapies are administered to patients. A general overview of the drug delivery field and its challenges will be followed by lectures which will tackle more specific subjects ranging from the fundamental level (e.g. mechanisms of intracellular uptake of biopharmaceuticals) to applied formulation science in industry. At the end of this day, we will have a good grasp of the inherent challenges of designing, testing and eventually producing a successful formulation for therapeutic gene and cell delivery.		
09.00 – 11.00	lecture	Introductory lecture by Dr. Enrico Mastrobattista on the challenges of drug delivery
11.00 – 11.15	coffee	coffee break
11.15 – 12.00	lecture	Prof. Dr. Niels Geijsen will discuss the development of iTOP, a delivery system allowing delivery of DNA or RNA into cells.
12.00 – 12.45	lecture	Prof. Dr. Victor van Beusechem will discuss the use of viruses for the treatment of cancer
12.45 – 13.30	lunch	lunch break
13.30 – 14.15	lecture	Prof. Dr. Raymond Schiffelers will explore extracellular vesicles with us and discuss what we can learn from them to apply in gene therapies
14.15 – 15.00	lecture	To be announced
15.00 – 15.30	break	soda break
15.30 – 17.00	case study	working on case study
17.00 – 18.30	dinner	dinner

Day 3 – 18 July 2018

Day 3 will cover aspects of preclinical research in ATMP development. We will learn that the preclinical stage is very different for ATMPs than for classical small molecule drugs, both in the discovery phase and in animal testing.

09.00 – 09.15	lecture	Opening by Prof. Dr. Gert Folkerts
09.15 – 10.15	lecture	HLA peptide discovery for personalized cancer immunotherapy (Dr. Michal Bassani)
10.15 – 10.45	coffee	coffee break
10.45 – 11.30	lecture	Basics in gene therapy (Dr. Manoe Janssen)
11.30 – 12.30	lecture	The development of gene editing to study and potentially treat genetic disorders such as cystic fibrosis, cystinosis and atopic dermatitis (Dr. Patrick Harisson)
12.30 – 13.30	lunch	lunch break
13.30 – 14.30	lecture	To be announced
14.30 – 15.30	lecture	Stem cell therapy and the hurdles in animal studies (Dr. Bettina Wilm)
15.30 – 16.00	break	soda break
16.00 – 18.00	poster	poster session with drinks
18.00 – 20.00	dinner	dinner
20.00 – 22.00	social	movie night

Day 4 – 19 July 2018

Day 4 is devoted to extraordinary examples of ATMPs that have reached clinical trials and shown first successes in human subjects. Moreover, we will learn what requirements have to be met before ATMPs can enter the clinical stage.

09.00 – 10.15	lecture	After an introductory lecture by the day chair, Dr. Lucienne Vonk (Utrecht UMC) will present the concept of first-in-man cartilage cell transplantation
10.15 – 11.15	lecture	Dr. Henk Aanstoot (Biosafety officer from Utrecht UMC) and a clinician from Utrecht UMC will discuss requirements for gene therapies in clinical trials and present an ongoing example
11.15 – 11.30	coffee	coffee break
11.30 – 12.30	lecture	Dr. Christine Günther, CEO of apceth biopharma (Munich, Germany) will introduce the company's mission as contract development & manufacturing organization for ATMPs
12.30 – 13.30	lunch	lunch break
13.30 – 14.30	lecture	To be announced
14.30 – 15.30	lecture	Dr. Gerty Schreibelt (Nijmegen, the Netherlands) will present the development and optimization of a clinical vaccination program using dendritic cells to treat cancer patients
15.30 – 16.00	break	soda break
16.00 – 18.30	case study	Finalizing case study
18.30 – 19.30	dinner	dinner

Day 5 – 20 July 2018

The last day zooms out again for a broader perspective of ATMP development and application. After an introductory lecture, participants will present their case studies. Afterwards, there will be a broad debate with multiple stakeholders considering the opportunities and hurdles with ATMP development and marketing. Finally, an innovation expert will put the challenges in medical innovation into the broad perspective of innovation science.

09.00 – 10.30	lecture	Introduction: Regulation & HTA (Prof. Dr. Bert Leufkens)
10.30 – 11.00	coffee	coffee break
11.00 – 12.30	debate	case studies debate
12.30 – 13.30	lunch	lunch break
13.30 – 15.00	debate	Debate: Post-authorization opportunities and hurdles of gene and cell therapies
15.00 – 15.30	soda break	soda break
15.30 – 16.30	lecture	Innovation studies: broader perspective on innovation and the hurdles surrounding it (Dr. Jarno Hoekman)
16.30 – 17.00	lecture	Quiz & wrap up
17.00 – 17.30	drinks	collecting keys & closing drink