



Cooperative European Medicines  
Development Course

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## MODULE 7

**Place:** Semmelweis University, Department of Pharmacology and Pharmacotherapy  
Floor 4, Knoll (formerly Issekutz) Room – No. 404  
1089 Budapest, Nagyváradi tér 4.

**Date:** February 07-10, 2019

### MODULE 7: BIOLOGICAL MEDICINAL PRODUCTS AND ADVANCED THERAPIES

**Module Leaders:** Dr. Sándor Kerpel-Fronius and Dr. István Antal

LEARNING OUTCOMES
<b>At the end of this Module the student should be able to:</b>
1. Demonstrate an understanding of the scientific, regulatory, ethical and legal issues that are peculiar to biological and advanced therapies.
2. Demonstrate an understanding of the challenges presented in constructing a package of non-clinical data to support the clinical development and marketing of biological and advanced therapies.
3. Recommend a clinical trial plan that is appropriate for the different types of products and technologies represented by biological and advanced therapies.
4. Demonstrate an understanding of the technical and manufacturing issues that are peculiar to biological and advanced therapies.
5. Critically review the non-clinical and clinical scientific knowledge underlying the development of new or prospective biological or advanced therapies.
6. Describe the new technologies now available and those in development; describe the therapeutic opportunities that might arise from the technology.
7. Critically analyse the differences between natural and modified protein constructs.
8. Describe the global need for new and improved vaccines and the barriers to their development.
9. Discuss the history and future prospects for gene therapy, and the technical difficulties developing a gene therapy product.
10. Describe the concept of stem cell therapy, what opportunities it might present, and the ethical issues that are unique to this technology.
11. Describe the particular ethical and regulatory issues of advanced therapies and how The Advanced Therapy Directive is addressing these.
12. Demonstrate the understanding of the basic principles of the development of medical devices. Assess the scientific and regulatory issues relevant to the clinical development of medical device-drug combinations.



**Day 1** - February 07, 2019 - Thursday

<b>Time</b>	<b>Name of Lecturers Titles and topics of the lectures and case discussions</b>	<b>Syllabus reference numbers</b>	<b>Learning outcomes reference number</b>
9:00-9:50	<b>Molnár-Érsek, Barbara</b> Introduction to immunology (1): humoral and cellular immunity, adaptive immune response	1.2, 1.3, 1.4, 1.6, 1.7, 4.1	1, 2, 4, 5, 6, 7
10:00-10:50	<b>Molnár-Érsek, Barbara</b> Introduction to immunology: humoral and cellular immunity, adaptive immune response	1.2, 1.3, 1.4, 1.6, 1.7, 4.1	1, 2, 4, 5, 6, 7
11:00-11:50	<b>Molnár-Érsek, Barbara</b> The principles of the development of monoclonal antibodies (mAb) and modified protein constructs. The effector functions of mAbs	1.2, 1.3, 1.4, 1.6, 1.7, 4.1	1, 2, 4, 5, 6, 7
12:00-13:00	<b>Kerpel-Fronius, Sándor</b> Biological medicinal agents: general characteristics, principles of production and clinical development	1.4, 1.9, 3.2, 7.1	1, 2, 3, 4, 5
13:00-14:30	<b>Lunch</b>		
14:30-15:15	<b>Mikus, Endre</b> Translational medicine, special issues to be addressed in the non-clinical development of biological medicinal agents	1.7, 1.8, 1.9, 3.2, 4.1	2, 4, 5
15:30-16:15	<b>Mikus, Endre</b> Case presentation: translational medicine of biological medicinal agents	1.7, 1.8, 1.9, 3.2, 4.1	2, 4, 5
16:30-17:15	<b>Zelkó, Romána</b> Biopharmaceutical formulation of biological medicinal agents	4.2, 4.3	1, 2, 5



**Day 2** - February 08, 2019 - Friday

<b>Time</b>	<b>Name of Lecturers Titles and topics of the lectures and case discussions</b>	<b>Syllabus reference numbers</b>	<b>Learning outcomes reference number</b>
9:00-9:50	<b>Boyd, Alan</b> Introduction to the principles of gene therapy. Delivery of DNA into cells, viral vectors and other methods. Gene editing	1.7, 4.1, 4.2, 4.4, 4.5, 5.3, 5.4, 5.13, 5.14, 7.1, 10.6,	1, 2, 3, 6, 9
10:00-10:50	<b>Boyd, Alan</b> Challenges for the clinical development of gene therapy, safety and ethical issues. Regulatory approach. Case presentation of the development of gene therapy	1.7, 4.1, 4.2, 4.4, 4.5, 5.3, 5.4, 5.13, 5.14, 7.1, 10.6,	1, 2, 3, 6, 9
11:00-11:50	<b>Boyd, Alan</b> RNA-based therapeutics: inhibitors of mRNA translation (antisense), the agents of RNA interference (RNAi), catalytically active RNA molecules (ribozymes), RNAs that bind proteins and other molecular ligands (aptamers)	1.7, 4.1, 4.2, 4.4, 4.5, 5.3, 5.4, 5.13, 5.14, 7.1, 10.6,	1, 2, 3, 6, 9
12:00-13:00	<b>Kerpel-Fronius, Sándor</b> Pharmacokinetics of biological medicinal products	5.5, 5.6, 5.7	1, 2, 5
13:00-14:30	<b>Lunch</b>		
14:30-15:15	<b>Fodor, Krisztián</b> Various types of advanced therapies	5.1, 5.2, 5.3, 5.4, 10.8	1, 2, 3, 4, 5
15:30-16:15	<b>Fodor, Krisztián</b> Challenges for the clinical development of advanced therapies, safety and ethical issues. Regulatory approach	5.1, 5.2, 5.3, 5.4, 10.8	2, 3, 4, 5, 12
16:30-17:15	<b>Fodor, Eszter</b> Basic principles of the development of medical devices. Evaluation of efficacy and safety	5.1, 5.2, 5.3, 5.4, 7.1, 10.1, 10.18	12
17:30-18:15	<b>Páros, Andrea</b> Regulatory and quality aspects of the clinical development of the combination of medicines and medical devices	5.1, 5.2, 5.3, 5.4, 7.1, 10.1, 10.1	12



**Day 3** - February 09, 2019 - Saturday

<b>Time</b>	<b>Name of Lecturers Titles and topics of the lectures and case discussions</b>	<b>Syllabus reference numbers</b>	<b>Learning outcomes reference number</b>
9:00-9:50	<b>Kistner, Otfried</b> Immunological background of vaccination. The global need of vaccines. Principles of vaccines production	4.1, 4.2, 4.3 4.5, 5.3, 5.4, 5.13, 5.14, 10.6	1, 2, 3, 8
10:00-10:50	<b>Kistner, Otfried</b> Clinical Development and regulation of human vaccines	4.1, 4.2, 4.3 4.5, 5.3, 5.4, 5.13, 5.14, 10.6	1, 2, 3, 8
11:00-11:50	<b>Kistner, Otfried</b> Clinical development of vaccines	4.1, 4.2, 4.3 4.5,	1, 2, 3, 8
12:00-13:00	<b>Kistner, Otfried</b> Regulation of vaccines	10.1, 10.8	1, 2
13:00-14:00	<b>Lunch</b>		
14:00-14:50	<b>Mészner, Zsófia</b> Clinical significance of vaccination	14.1, 14.2	1, 8
15:00-15:50	<b>Peták, István</b> The development, regulation and marketing of in vitro companion diagnostics	5.1, 5.2, 5.3, 5.4, 7.1, 10.1, 10.1, 14.1, 14.2, 14.5	3, 5, 7, 12
16:00-16:50	<b>Telekes, András</b> Biological therapy of tumors with mAbs	14.1, 14.2	3, 5
17:00-17:50	<b>Kerpel-Fronius, Sándor</b> Biological Immune therapy of tumors with mAbs, tumor vaccines	14.1, 14.2	3, 5

**Day 4** - February 10, 2019 - Sunday

<b>Time</b>	<b>Name of Lecturers Titles and topics of the lectures and case discussions</b>	<b>Syllabus reference numbers</b>	<b>Learning outcomes reference number</b>
9:00-10:50	<b>MCQ examination</b>		
11:00-11:50	<b>Kerpel-Fronius, Sándor</b> Ethical aspects of clinical trials with complex therapies performed by multidisciplinary clinical development teams	8.1, 8.2, 8.3, 8.8, 8.14	1, 12