safescimet course 1.2 - Safety and Efficacy of Cell Therapies

Key Questions Addressed

- Basic biology of cells used as therapies, including stem cells
- Therapeutic treatment of diseases via novel therapeutics including cell therapies and extracellular vesicles
- Drug safety aspects of novel therapeutic approaches – the key issues
- Regulatory requirements for cell therapies
- Safety assessment of cell therapies

Feedback from the Previous Course

"Course provided practical case lectures that helped me better understand the theory."

"Quality and closeness of teachers were great! They were available for questions and discussions of any course topic."

For more experiences listen to an interview from one of our students on www.youtube.com

Moreover, our home assignment will be published as a multi-author scientific review - see last review published:
Scarfe et al., Nature Regenerative Medicine 2017, https://doi.org/10.1038/s41536-017-0029-9

About safescimet

safescimet is a unique pan-European network of academia and pharmaceutical industry, which have joined forces to establish a comprehensive modular education and training in Safety Sciences for medicines. The programme covers all aspects of efficacy and safety of the use of cell therapies and stem cells, in order to fulfill the needs of drug safety scientists in the pharmaceutical industry, regulatory authorities and academia. The aim of the programme is to bridge crucial gaps in the education and training of scientists evaluating the safety and efficacy of cell therapies and regenerative medicine therapies and to ensure that European regenerative medicine therapy scientists are at the forefront of their field.

The course *Safety and Efficacy of Cell Therapies* and the other single courses of the safescimet programme provide new opportunities for Continuous Professional Development (CPD) purposes and are part of European Master for Advanced Safety Sciences for Medicines degree at the University of Konstanz. The individual courses are clustered within five separate domains. Each domain deals with one or more specialised topics and contains from two to six single courses. Please visit www.safescimet.eu for details of the full course programme and confirmation of course dates.

Course Objectives

This will introduce the emerging area of the safety science of the therapeutic application of stem cells, and will address all the key issues that need to be considered by developers of these therapies, including academics and the pharmaceutical/biotech industry, as well as the important area of regulation. Other new areas of therapy will also be considered, such as extracellular vesicles as regenerative medicine therapies. The use of stem cells in the screening of conventional drugs for efficacy and particularly safety will also be investigated. UK and European leaders in these fields, from Industry, Academia and the Regulatory Agencies, will contribute teaching sessions in a small, informal and interactive learning environment.

Key Subjects Covered by the Course

- Basic biology of cells used as cell therapies, including mesenchymal stromal cells of various origins, and stem cells
- Key issues: efficacy and safety of using cell therapies
- Considerations for preclinical and clinical applications of mesenchymal stromal cells/stem cells
- Insight into progress in preclinical and clinical studies using cell therapies
- Introduction to immune system and importance in regenerative medicine therapies
- Use of stem cells in safety assessment – where we are
- New nanoparticles and reporters for imaging and tracking cell therapies
- Regulatory requirements for cell therapies and stem cells and as well as extracellular vesicles

Target Audience

safescimet courses are open to all scientists and students from industry, academia and regulatory authorities, who need a broad comprehensive understanding of the use of cell therapies and stem cells with particular emphasis on safety and legal frame works. The applicant will normally possess an MSc degree in a Life Science discipline or equivalent. In addition, applicants are expected to have an at least one year working experience in a related discipline.

Why Join the Course

The on-site training of the course during the first week consists of five days with lectures, practical exercises, group work and discussions. This setup offers an intense and broad training with leading experts in their field and ample opportunities for lecturer-student interactions. The balance of academic, industry and regulatory teachers provides knowledge directly available to considerations of the use of cell therapies and stem cells, including dataset discussions from real case studies.

Learning Outcomes

On successful completion of the course, participants will have acquired an understanding of the relevance of regenerative medicine therapeutics for preclinical and clinical applications. More specifically, participants will be able to

- gain an understanding of where the science has reached
- appreciate the novelty of these areas and the concomitant challenges to therapy developers and the regulators
- understand that there are still many gaps in our knowledge – i.e. concept of an evolving area
- understand the basic biology that underpins this field and that is being translated into man
- appreciate the utility and possible drawbacks of different preclinical models for assessment of safety
- overview the state of the art in the application of stem cells in safety assessment of conventional compounds

Course Programme

The first week of the course consists lectures, practical exercises, group work and discussions, provided by specialists from academia, pharmaceutical industry and regulatory authorities with diverse backgrounds on drug safety aspects.

On-site Training

Day 1	Working with cell therapies: tools and techniques
Day 2	Workshop regenerative medicine therapies for kidney disease
Day 3	Workshop: application of stem cells in safety assessment – what is required of model systems and are we there yet?
Day 4	Legal aspects of cell therapies – regulations and risks

Individual Home Assignments

After the week of on-site training, students receive an individual 38 hours home assignment consisting of written questions and a case study. These individual assignments will be completed through a frequent exchange with the safescimet teachers using distance learning approach. Written individual assignments are to be completed and submitted via Moodle within 6 weeks after the on-site training and will then be approved by the course leader.

Course Credits

In order to receive the full 3 ECTS credits for a successfully completed course, participants need to pass the written exam of the on-site training week as well as to have successfully completed the home assignment.

Participants attending the on-site training week only, without completing the exam and/or the home assignment, will be given a certificate of attendance confirming completion of a Continuing Professional Development (CPD) course.

Syllabus

The syllabus contains lecture hand outs, list of abbreviations and list of reading materials and will be provided by the course leaders 14 days prior to the course. The information and the material for the individual home assignment will be provided during the first week of the course.

Assessment

The assessment is based on a every day 30 minutes assessment and on the evaluation of the home assignment.

Type	- The purpose of the examination is to test that the examinee has aquired a broad knowledge and comprehension of the course subjects during the lectures. - The percentage of items on the test devoted to a particular topic will roughly correspond to the emphasis given on the topic during the course. - Stem cells: 40% - Other novel therapeutics: 20% - Pre-clinical application: 20% - Regulatory guidelines: 20%
Assessors	Course leaders
Exam aids	All written exam aids are allowed, especially the provided material: lecture hand outs, list of abbreviations and case studies.

Contact Person

Professor Daniel Dietrich

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Course Leaders

Prof. Daniel Dietrich - Professor of Human and Environmental Toxicology, Faculty of Biology, University of Konstanz, Germany
Prof Christopher Goldring - MRC Centre for Drug Safety Science, University of Liverpool, UK
Dr Bettina Wilm - MRC Centre for Drug Safety Science, University of Liverpool, UK

Practical Information

Course credits	3 ECTS credits
Level	Master's level (second cycle higher education)
Location	Human and Environmental Toxicology, University of Konstanz.
Teaching methods	Lectures, case reports, demonstrations and home assignments.
Student workload	- Preparation: 12 hours - Course: 38 hours - Assignment: 38 hours - Examination: 2 hours - Total: 90 hours
Course fee	Please visit on www.safescimet.eu **Fees** and **How to apply** for more information.
Course capacity	20 participants
Language	- The official language of the course is English. - No simultaneous translations will be provided.
Course notes	Complete course notes, except for the textbook, will be available for all the participants.
Course accreditation	- safescimet courses meet the criteria for Continuous Professional Development (CPD) diplomas, and are part of Master programme Advanced Safety for Medicines. - When registering for one or several "Stand Alone"courses of our programme, this course provides CPD credits for your individual CPD portfolio. Each course is credited with 38 contact hours. - The course setup is already consistent with the requirements of the Bologna process. Tha full Master programme is undergoing evaluation for being accredited and certified as a regular postgraduate MSc degree at the University of Konstanz/Germany.

Accommodation

We generally room our participants in the Hotel Hirschen (www.hirschenkonstanz.de/html/eng/). Please contact the safescimet Office (safescimet@uni-konstanz.de) for information on special booking codes.

Transport

Konstanz can be reached in approximately 1 hour by train from Zürich Airport, Switzerland or in 2.5 hours from Stuttgart Airport, Germany.
More details, also for parking in case you are coming by car, will follow in the Welcome letter for registered participants.

Registration

To apply please visit www.safescimet.eu. Your application needs your registration on www.safescimet.eu.

Cancellation

For our cancellation policy please visit **How to Apply/Terms and Conditions** on www.safescimet.eu.