UC San Diego
SCHOOL OF MEDICINE

SYLLABUS

Stem Cell Translation Course

Course Co-Director
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Overview
This course will consist of twenty hours of face-to-face interaction with lecture and exercises. An overarching assignment to design a cell base therapy translational study will be turned in at the end of the course. A typical lecture will teach concepts that are linked to practical applications in translational stem cell therapeutics. Lectures will provide context through expert guest speakers. The grade of the course will come from participation, homework assignment and a final exam.

Course Purpose
This course, focused on the practical application of the principles of translating Stem Cell based therapies that focuses on the early development and phase 1 studies required to translate these interventions from the bench to the bedside. This course will highlight the unique aspects of stem cell based therapies compared to drug development.

Learning Objectives
By the end of this course, the student should be able to:

1. Understanding to bench to bedside concept
2. Understanding the process of development of new stem cell based therapies
3. Determining the preclinical work needed to support first-in-human studies
4. Articulate the rationale and objectives for a stem cell study
5. Identify and describe the appropriate study design and methods
6. Select a study population and methods for their recruitment and selection of study subjects for a stem cell study
7. Select outcome measures for safety and efficacy
8. Design a monitoring plan to protect both participants and the scientific and ethical integrity of the study
9. Determine methods of data collection, sample size and analysis plan

Course Structure
This course will have 10 weekly 2 hour sessions. Each session will have assigned background reading. Each session will have a 1 hour lecture and 1 hour of class exercises. Each lecture will be given by an expert in the field who has direct experience with developing stem cell therapeutics from basic science to clinical trials.

Course Materials/Textbooks
There is no required textbook for this course as there is no existing textbook for stem cell based clinical trials. Materials will be provided by the lecturer from current literature and guidance documents in the field of stem cell therapy.

Course Evaluation
Grades will be determined by participation in classes (10%), a written assignment (40%) and a final exam (50%). Grades will be scaled according to the class performance for A+,A,A-, B+,B,B-,C+C,C-,D,F.

Assignment

Design a first –in-human clinical trail for a cell therapy, cell targeted therapy, or gene therapy. A well-designed protocol should include the following.

- safety and proof-of-concept information to justify the risks of the proposed trial;
- choice of study population;
- study design
- dose(s) to be administered and justification for dosing;
- clinical issues related to any invasive administration procedures;
- the treatment plan for the control group, if one is proposed;
- safety monitoring plan, including long-term follow-up and any special safety assessments;
- stopping rules;
- trial endpoints.