MTR/REG 621/CAMB 707: Cell and Gene Therapy  
Spring 2018  
Wednesdays 9:00am-12:00pm  
Location: SPE 08-100

Course Director:  
Michael Milone, MD, PhD  
Associate Professor of Pathology and Laboratory Medicine  
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Assistant Course Directors:  
Elizabeth Hexner, MD, MTR  
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Course Coordinator:  
Anna Greene  
acgreene@upenn.edu  
215-662-4619

Description:  
This course will provide students with a general overview of translational research in the area of gene and cell therapy. This includes technical considerations, translating preclinical investigation into therapeutics, the execution of gene and cell therapies clinical trials, and key regulatory issues. Entrepreneurial considerations will be discussed as well. By the end of this course, students will understand the basic technologies employed for gene and cell therapy along with approaches and pitfalls to translating these therapies into clinical applications including regulatory and commercial aspects of this emerging area.

Evaluation and Due Dates:  
Your grade in this course is based on your attendance and participation in class (30%), presentation of a paper for discussion (30%) and two group project presentations (25% each). The following table serves as a quick reference for each of these components and includes due dates for your exams.

<table>
<thead>
<tr>
<th>Assignment</th>
<th>Due Date</th>
<th>Evaluation %</th>
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</thead>
<tbody>
<tr>
<td>Attendance &amp; Class Participation</td>
<td>Ongoing</td>
<td>30%</td>
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<tr>
<td>Paper discussion</td>
<td></td>
<td>20%</td>
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<tr>
<td>Project Presentation #1</td>
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<td>25%</td>
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<tr>
<td>Project Presentation #2</td>
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Attendance & Participation (30%)
Attendance:
Students are expected to attend and participate in all classes. If for any reason a student will not be in class, they should contact Anna Greene (acgreene@upenn.edu) or Dr. Milone (milone@upenn.edu) prior to class to alert them of the absence and make arrangements to make up course content. One absence is allowed during the course which will not affect the attendance grade. All absences require students to make up content.

Participation:
Prior to each class materials will be posted online to the Canvas course site. Students are expected to read the materials and come to class prepared with two points of discussion. Each class will involve discussion in which you are expected to participate. Your engagement and participation are important not only for your own learning but also for the learning of others.

Paper discussion (20%):
Each student will be responsible for preparing and leading at least one discussion on a selected research paper. The format of the paper discussion will be a 10-15-minute introduction of the key background and underlying questions to be presented by the student, followed by an in-depth analysis of the paper by all members of the class. Students will be graded based on their introductory presentation and active participation in the paper discussions.

Project (50% divided equally over two presentations)
The students will be assigned to a group of 3-4 students. As a group, the students will develop a plan to translate a product based upon a gene and/or cell therapy technology from the basic preclinical phase into a phase I clinical trial. The group will be required to prepare two (2) presentations. The first presentation will focus on assessing the basic science around the technology, the adequacy of the preclinical testing as it relates to safety and efficacy prior to entering a human clinical trial and critically evaluating approaches to manufacturing the product appropriate for use in humans. The second presentation will focus on the regulatory and ethical aspects to translating the technology into the clinic. This will include proposing a clinical trial strategy for a first-in-human study with a critical evaluation of the pitfalls and challenges associated with the proposed strategy.

Course Policies and Procedures

Academic Policies:
As a student at The University of Pennsylvania, you are required to uphold the Code of Academic Integrity. Specifically, this means that materials that you submit either online or in person should be independent works created by you that uphold all tenets of academic integrity (i.e. do not cheat, fabricate, or plagiarize, amongst others). We encourage you to reach out to the course director or coordinator if you are not clear on what potential violations are.

Canvas:
All course materials (ppts, announcements, lecture recordings) and assignments will be posted on Canvas at https://canvas.upenn.edu (log in with your Pennkey). Contact the course coordinator with questions. We recommend that you choose the “Notify me right away” option for your most frequently
checked email address in the “Announcements” area of the “Notification Preferences” page: https://canvas.upenn.edu/profile/communication.

**Course Evaluations:**
Course & Lecture evaluations are completed via OASIS throughout the semester. These are a required part of course participation. Students can access evaluation forms with their Pennkey and password and will also receive emails when forms are available: http://gme-evals.med.upenn.edu/

**Student Disabilities Services:**
The University of Pennsylvania, provides reasonable accommodations to students with disabilities who have self-identified and been approved by the office of Student Disabilities Services (SDS). Please make an appointment to meet with me as soon as possible in order to discuss your accommodations and your needs. If you have not yet contacted SDS, and would like to request accommodations or have questions, you can make an appointment by calling SDS 215.573.9235. The office is located in the Weingarten Learning Resources Center at Stouffer Commons 3702 Spruce Street, Suite 300. All services are confidential.