MTR/REG 621 Cell & Gene Therapy

Spring 2020

Time: Wednesdays 9:00AM-12:00PM

Location: SPE 08-100

Instructor Information

Course Director

Michael Milone, MD, PhD
Associate Professor of Pathology and Laboratory Medicine
University of Pennsylvania
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Assistant Course Director

Elizabeth Hexner, MD, MSTR
Associate Professor of Medicine
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Course Coordinator

Megan Maxwell
Associate Director, ITMAT Education
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General Information

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 Methods:

Students will be graded based on class attendance, participation, a paper presentation and two group project presentations.
30% – Attendance and Class Participation
20% – Paper Presentation
25% – Project Presentation #1
25% - Project Presentation #2

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 Dr. Milone (milone@pennmedicine.upenn.edu) prior to class to alert him 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.

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 pre-clinical 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:

Academic Integrity:
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. 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 evaluations are completed via OASIS at the end of 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 at 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.

Course Schedule

Week	Topic	Lecturer
Jan 15	Overview of Gene and Cell Therapy Field & Orientation to Course	Michael Milone
	Bone Marrow Transplantation as The First Cellular Therapy	Elizabeth Hexner
Jan 22	Stem Cells & Tissue Engineering	Roddy O'Connor
	Paper 1 Discussion	

Week	Topic	Lecturer
	Paper 2 Discussion	
Jan 29	Gene Editing Paper 3 Discussion Paper 4 Discussion	Jim Riley
Feb 5	Gene Therapy Vectors Paper 5 Discussion Paper 6 Discussion	Michael Milone
Feb 12	T Cell Immunotherapies Correlative Sciences and its Importance to Successful Clinical Trials Paper 7 Discussion	Carl June Jos Melenhorst
Feb 19	Pre-clinical Safety Testing of Vector-based Gene Therapies Paper 8 Discussion Paper 9 Discussion	Valder Arruda
Feb 26	Manufacturing of Complex Cell-based Therapeutics cGMP Manufacturing Facility Design and Infrastructure Considerations Tour of South Tower cGMP Facility	Bruce Levine Han Van Derloo, Donald Siegel
Mar 4	**Project Presentations - Part 1**	
Mar 11	**No class - Spring Break**	
Mar 18	Overview of Regulatory Review for Gene and Cell Therapies FDA Guidance on Cell & Gene Therapies How-to Workshop: Writing an IND for Gene Therapy Products	Anne Chew, Gabriela Plesa
Mar 25	Patenting Gene & Cell Therapies	Jim Bowen, Kathryn Doyle PCI
Apr 1	Ethical Considerations in Gene and Cell Therapy Clinical Trials	Megan Kasimatis Singleton (JHMS)
Apr 8	Funding Development: Partnerships between Academia & Industry Managing Public Relations with High Profile Clinical Studies	Dana Hammill Holly Auer
Apr 15	Translating Gene & Cell Therapies into Viable Commercial Products	Steven Nichtberger Kush Parmar
Apr 22	**Final Project Presentations**	
Apr 29	**Final Project Presentations**	