MTR/REG 6210 Cell & Gene Therapy

Spring 2023

## Time: Wednesdays 10:15 AM-12:15PM

## Dates: Jan 18 – Apr 19

## Location: Smilow Research Center, SCTR 8-146 AB (8th Floor)

# Instructor InformationI

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| Course Director |  |  |
| **Michael Milone, MD, PhD**  Associate Professor of Pathology and Laboratory Medicine  University of Pennsylvania  **milone@pennmedicine.upenn.edu** |  |  |
| Assistant Course Director **Elizabeth Hexner, MD, MSTR**  Associate Professor of Medicine  University of Pennsylvania  **hexnere@pennmedicine.upenn.edu** |  |  |
| **Course Coordinator** |  |  |
| **Megan Maxwell**  **Associate Director, ITMAT Education**  **267-408-9585, mmaxwell@upenn.edu** |  |  |

# 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

30% – Paper Presentation

20% – Project Presentation #1

20% - 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](mailto:milone@pennmedicine.upenn.edu)) or Dr. Hexner ([hexnere@pennmedicine.upenn.edu](mailto:hexnere@pennmedicine.upenn.edu)) prior to class and copy [mmaxwell@upenn.edu](mailto:mmaxwell@upenn.edu) to alert them of the absence and make arrangements to make up course content. Two excused absences are allowed during the course which will not affect the attendance grade. Absences in excess of two will result in 1 point deducted from the attendance portion of the grade.

**Participation:**

Prior to each class, materials will be posted online to the Canvas course site. Students are expected to watch and read the materials and **come to class prepared with to discussion the material**. 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 (30%):**

Students will be responsible for reading the assigned papers prior to class. The format of the paper discussion will be a brief introduction of the key background and underlying question(s) followed by an in-depth analysis of the paper by all members of the class. Each student should be prepared to present the brief background if called upon. This background should include:

* What question did the study set out to address?
* What is the significance of the question to the field?
* What was the overall approach used to address the question?

In addition, the students should be able to explain each figure of the assigned paper if called upon during the discussion. Students will be graded based on their introductory presentation and active participation in the figure discussions.

**Project (40% 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](https://provost.upenn.edu/policies/pennbook/2013/02/13/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 Blue 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.

**Weingarten Center:**

The Weingarten Center offers a variety of resources to support all Penn students in reaching their academic goals. All services are free and confidential. To contact the Weingarten Center, call 215-573-9235. The office is located in Hamilton Village at 220 S. 40th Street, Suite 260.

**Academic Support:**

Learning consultations and learning strategies workshops support students in developing more efficient and effective study skills and learning strategies. Learning specialists work with students to address time and project management, academic reading and writing, note-taking, problem-solving, exam preparation, test-taking, self-regulation, and flexibility.

**Disability Services:**

The University of Pennsylvania provides reasonable accommodations to students with disabilities who have self-identified and received approval from Disability Services. Students can contact Disability Services and make appointments to discuss and/or request accommodations by calling 215-573-9235.

# Tentative Course Schedule

| Week | Topic | Lecturer |
| --- | --- | --- |
| Jan 18 | Overview of Gene and Cell Therapy Field & Orientation to Course  Paper discussions (Instructor Lead)Horowitz 1990 HSCT and T cells | Michael Milone  Elizabeth Hexner |
| Jan 25 | Gene Therapy Vectors  Paper Discussions (Student Lead) | Michael Milone |
| Feb 01 | Gene Editing  Paper Discussions (Student Lead) NYCE T cell paper | Michael Milone |
| Feb 08 | Stem Cells & Tissue Engineering  Paper Discussions (Student Lead) | Elizabeth Hexner Roddy O’Connor |
| Feb 15 | **MAGE-A3 TCR toxicity**  Correlative Sciences and its Importance to Successful Clinical Trials – Tet2 story  Paper Discussions (Student Lead) | Michael Milone |
| Feb 22 | Pre-clinical Safety Testing of Vector-based Gene Therapies  Paper Discussions (Student Lead) | TBD |
| Mar 01 | Manufacturing of Complex Cell-based Therapeutics  Apheresis/Tour of South Tower cGMP Facility | Bruce Levine  Andrew Fesnak  Han Van Der loo |
| Mar 08 | **No class Spring Break** |  |
| Mar 15 | **Project Presentations – Part 1** |  |
| Mar 22 | Patenting Gene & Cell Therapies | Kathryn Doyle, PhD, JD  Saul-Ewing |
| Mar 29 | 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 | Julie Jadlowsky |
| Apr 5 | Ethical Considerations in Gene and Cell Therapy Clinical Trials | Megan Kasimatis Singleton (JHMS) |
| Apr 12 | Translating Gene & Cell Therapies into Viable Commercial Products | Arun Das |
| Apr 19 | **Final Project Presentations**  **Final Class Wrap up** |  |