SYLLABUS **Spring 2026**

CAMB 633 – *Advanced Seminar in Cell and Gene Therapy*

Perelman SCHOOL OF MEDICINE
University of Pennsylvania

Time	Thursdays 3.30 PM – 5.00 PM
Dates	2026, Jan 22 – April 16
Location	BRB 1403

Course Director

Peter Kurre, MD | Department of Pediatrics, Division of Hematology)
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Co-Directors

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COURSE GOALS:

The course provides students with a conceptual framework for the critical appraisal of current cell and gene therapy landscape through review of the literature and seminar presentations. The course will critically review select articles from the scientific literature, exploring key aspects of experimental design and data interpretation, scientific rigor and reproducibility. There are several specific goals for this course. One is to introduce students to current approaches in the field of gene therapy, with emphasis on key techniques for delivery as well as laboratory and translational endpoint metrics. A second goal is to review the relevant disease physiology and translational challenges in matching treatment approach and disease context. Throughout, students will learn to consider both technical limitations and ethical boundaries of these novel approaches. A final goal is to convene with experts to better understand the role of intellectual property protection, industry partnerships and the requirements to bring a novel drug to the FDA for approval. These goals will be achieved through paper reviews, lectures and class discussions.

COURSE DESCRIPTION:

Prerequisites: CAMB 633 is open to students at all levels, but students will benefit from foundational knowledge in the molecular basis of gene therapy and basic immunology.

Structure of the course: The course comprises a mix of student-led Journal Club classes and Expert Seminar lectures.
Class: Students will be responsible for leading a group discussion of assigned scientific manuscripts in the field of cell and gene therapy selected by course faculty. At the beginning of the course, students select from faculty selected primary research papers, with each student co-leading between 2 and 4 Journal Clubs, depending on the number of enrolled students. Student co-leads will collaboratively prepare slides covering background and paper figures. They

will each cover a portion of the article result section with corresponding figure (-s). Emphasis in review will be placed on technical rigor and reproducibility, as well as the broader scientific context and disease pathophysiology. Each class will last 90 minutes, including presentation and discussion of the manuscripts with Q&A. Each class will cover one manuscript, with all paper presentations led by a given student contributing in aggregate to the student grade (50%).

Lectures: 3-4 lectures are spread throughout the semester. During each lecture, a faculty member or external speaker will lecture for ~45-60 minutes followed by ~30 minute discussion. Students are expected to ask questions during or at the end of the lecture. Course faculty will moderate lecture and discussion.

Evaluation:

Students are expected to actively participate in all aspects of the course and come prepared for class. Together, the lead student and faculty will guide and moderate the discussion of papers, their impacts on the field of gene therapy, including potential future outcomes. Class grades will be based on: 50% on paper presentations and 50% on discussion participation (in class and seminars). Absences should be cleared with course directors ahead of time. More than two absences will impact the attendance grade portion.

SELECTION OF ARTICLES BY FACULTY (PROVISONAL)

Peter Kurre (Perelman SOM, Department of Pediatrics, Division of Hematology)

- In vivo macrophage engineering reshapes the tumor microenvironment leading to eradication of liver metastases. Kerzel et al., Cancer Cell 2023; **PMID: 37863068**
- Suprachoroidal gene transfer with nonviral nanoparticles in large animal eyes; **PMID: 38457512**
- Durable and efficient gene silencing in vivo by hit-and-run epigenome editing:; **PMID: 38418872**
- Intra-tumoral administration of CHST15 siRNA remodels tumor microenvironment and augments tumor-infiltrating T cells in pancreatic cancer. **PMID: 38799652**

Norbert Pardi (Perelman SOM, Department of Medicine, Division of Infectious Diseases)

- IL-1 and IL-1ra are key regulators of the inflammatory response to RNA vaccines; **PMID: 35332327**
- Modified mRNA Vaccines Protect against Zika Virus Infection; **PMID: 28222903**
- mRNA A phase 1 trial of lipid-encapsulated mRNA encoding a monoclonal antibody with neutralizing activity against Chikungunya virus; **PMID: 34887572**
- Biocompatible, Purified VEGF-A mRNA Improves Cardiac Function after Intracardiac Injection 1 Week Post-myocardial Infarction in Swine; **PMID: 30038937**
- CAR T cells produced in vivo to treat cardiac injury; **PMID: 34990237**

Stefano Rivella (Perelman SOM, Department of Pediatrics, Division of Hematology)

- In vivo haemopoietic stem cell gene therapy enabled by postnatal trafficking. **PMID: 40437086**

- A differentiated β-globin gene replacement strategy uses heterologous introns to restore physiological expression. **PMID: 40022449**
- Correcting a pathogenic mitochondrial DNA mutation by base editing in mice. **PMID: 39879319**

Rebecca Ahrens-Nicklas (Perelman SOM, Department of Pediatrics, Division of Human Genetics and Metabolism)

- Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy. **PMID: 40419681**;.
- Lentiviral Gene Therapy for Cerebral Adrenoleukodystrophy. **PMID: 39383459**.
- Hematologic Cancer after Gene Therapy for Cerebral Adrenoleukodystrophy. **PMID: 39383458**.
- An AAV variant selected through NHP screens robustly transduces the brain and drives secreted protein expression in NHPs and mice. **PMID: 40367194**.

LECTURES (Provisional, July 2024)

Development and Translation for Gene Therapy Products
Speaker: Kathy High

From 2004 to 2014, Dr. High was a Professor at the Perelman School of Medicine at the University of Pennsylvania, an investigator at Howard Hughes Medical Institute, and the Director of the Center for Cellular and Molecular Therapeutics at the Children's Hospital of Philadelphia. She completed a five-year term from 2000 to 2005 on the U.S. Food and Drug Administration Advisory Committee on Cell, Tissue, and Gene Therapies and is a past president of the American Society of Gene & Cell Therapy. Dr. High was instrumental in the development of Luxturna gene therapy and went on to co-found Spark Therapeutics, Inc., where she served as President and board member from September 2014 to December 2019. Since December 2024, Dr. High has served as Chief Executive Officer and a board member of RhyGaze AG, a private biotech start-up. Prior to that, from January 2021 to December 2022, she served as President, Therapeutics of Asklepios BioPharmaceutical, Inc. (AskBio), a subsidiary of Bayer AG (BAYRY), and also served on AskBio's board of directors.

Gene Therapy and Ethical Challenges
Speaker: Steven Joffe, PhD, (UPenn Medical Ethics and Health Policy)

Dr. Steven Joffe is a pediatric oncologist and bioethicist who is currently the Art and Ilene Penn Professor of Medical Ethics & Health Policy and Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine. He is also the Director of the Penn Postdoctoral Training Program in the Ethical, Legal and Social Implications (ELSI) of Genetics and Genomics. His research addresses ethical challenges arising in the conduct of clinical and translational investigation and in genomic medicine and science with particular focus on the integration of whole-exome sequencing technologies into the clinical care of cancer patients and strategies for diagnosis of germline risk among young adults with cancer.

From Bench to Bedside: The FDA Regulatory Pathway to IND Submission for Cell and Gene Therapy products.
Speaker: Nancy Robinson Garvin, PhD

Dr. Garvin Robinsomn is the **Associate Director of Regulatory Affairs at Children's Hospital of Philadelphia**. Her lecture will focus on the FDA governing body for cell and gene therapy products (CBER), the various types of FDA

meetings available to researchers and how to request each meeting type, data needed to support the request, and timeline from submission to approval/clinical trial. The lecture will also review the various types of FDA applications focusing primarily on the IND submission. The lecture will conclude with a discussion of the new four distinct FDA approaches (Priority Review, Breakthrough Therapy, Accelerated Approval, & Fast Track) for new breakthrough/first in human therapies and how this can apply to novel cell and gene therapy drug products and the timelines for each.

Technology Transfer, Business Development and Licensing of Gene Therapy Products
Speaker: Tom Wilton, MA, MS
As Senior Vice President, Tom Wilton leads CHOP Innovation Ventures, an integrated team of licensing, business development, and investment professionals who collectively serve as the primary gateway for researchers seeking to commercialize their discoveries. His experience as a biopharmaceutical executive with more than 20 years of leadership experience spans corporate strategy, business development, commercialization, and R&D operations. He has a particular interest in advanced cutting-edge therapeutic platforms, with a focus on oncology, rare diseases, cell and gene therapies, and AI-enabled technologies.