

# **BME 1550H: Cellular and Gene Therapy-Science, Manufacturing and Regulations**

## **Description of the Course**

This seminar/reading and conference course is an interactive course designed to provide graduate students a basic understanding of latest developments in cellular, gene-modified cells and gene therapy, articulating basic science concepts and challenges in process development, scalability, quality control, safety, potency and meeting evolving regulatory requirements. Each week, we will invite a leading guest speaker to present a seminar on their respective field of research related to cellular and gene therapy, science, manufacturing and regulation. The course is divided into **4 sub-categories: MSCs, iPSCs, Immunotherapy, GMP/Regulatory considerations** with 3 lectures on each subcategory. An Invited Speaker will present a seminar on his/her research for 45-min followed by an in-depth discussion on two papers assigned by the Guest Speaker. In addition to discussing the papers, the speaker and students will discuss the 2 written questions that each student submitted prior to lecture time.

A total of two hours will be dedicated to each session, which will include the seminar by the Guest Speaker and a detailed discussion of an assigned paper and submitted questions, led by the Guest Speaker. There will be 13 lectures (2 hours each for a total of 26 hours).

## **Course Specifications**

### **Readings:**

Two research articles (corresponding with the weekly Guest Speakers) will be circulated for students to critically review and prepare questions for submission for a discussion led by the Guest Lecturer during the second half of the lecture. **Students are required to provide 2 written questions by 11:59 every Tuesday before the next day's lecture** and participate in the discussion on the paper that follows the guest lecture.

### **Research seminars presented by Guest Speakers:**

*45-minute seminar and remaining time for discussion*

The Guest Speaker will present a research seminar and follow this with an in-depth discussion on selected papers that speak to the state-of-the-art on MSCs, iPSCs, Immunotherapy, GMP/Regulations. **Students are required to attend these seminars and provide 2 written questions and participate in the discussion on the paper that follows the lecture.**

## **Discussion Questions and Class Participation**

Students are required to read the assigned readings and come prepared for a detailed discussion. The students are required to submit or ask 1-2 questions during **class**. All students are expected to **contribute to the discussion of the paper during class**. All students will be marked on their participation in class by the guest lecturer. 5 marks are allotted per class for a total of 60 marks for class participation (10% of total) and includes both the written and oral component.

### **Presentation of Student-Selected Papers**

Students will choose one research article related to one of the topics presented by the guest lecturers on MSCs, iPSCs, Immunotherapy, GMP/Regulatory. Different topics will need to be selected each for student oral presentation vs. student term paper. Student Oral presentation: Breakdown: 12-15 minutes for presentation, 10 minutes for questions/discussion. Students will select one publication (provided by speaker) prior to the introductory session and will be required to prepare an oral presentation for approximately 15 minutes. This will include an introduction, results, and discussion of results of the paper, followed by a 15-minute discussion session.

All students are expected to contribute to the discussion of papers. All students will be marked on their participation in each class, whether as presenter or discussant, or by asking one question during discussion session. Questions asked/answered by students at the end of seminars are included in this mark. Marking of each student will be performed by course coordinators using the attached rubric; the oral presentation is worth 40% of the total mark. Detailed guidelines for presentations are included in the course description and in the attached rubric. The topic will be different from the one for the student's term paper. Students will have access to a computer and multimedia projector for the presentation; plan on approximately 10 slides in total (1 per minute). The professors and fellow students will provide constructive comments about your presentation, in particular with respect to clarity, content, and your ability to critically discuss and answer questions. The comments will be provided to you with the mark for your presentation.

## Term Paper

*Maximum 7-8 pages double-spaced (page limit does not include references)*

Students will be expected to write a review (critique) of a research topic, selected by them on one of the lectures presented. The term paper topic should not be the same area as their oral presentation. The research review should be a critical appraisal of the current area of research, working beyond the two papers presented. The term paper is due on **April 12th 2023 at 11:59 pm**. Please note that no extensions will be given for the submission of the term paper. Term paper will be marked out of 50 – please see breakdown under evaluation criteria and in the attached rubric

### **Term Paper Specifications:**

Paper must be typed, double-spaced, 12-point font size, preferably Arial or Times New Roman font with 1 inch margins all around. All pages should be numbered. Inserting figures/tables is not mandatory, but if you do, it should be included within the limit of 7-8 pages. Please do not insert more than 2 figures or tables. References should be included on an additional page, with a maximum of 25. The term paper should include the following sections: Abstract, Introduction, Methods, Results, Discussion, and References.

### **Abstract:**

The abstract consists of 200-250 words in which you summarize the topic, the specific subject of the research problems, the major findings, and the conclusions.

### **Introduction:**

Provide background on the research topic, summarize the present state of knowledge of the topic, and provide a clear description of the nature of the research problem that will be investigated in the Results section. Be selective with the background information that you include, as you are not writing a textbook. This section should be about 1-1½ pages long.

### **Methods:**

Briefly, describe methods used in the current studies using subheadings. This section should be about 1-1½ pages long. Indicate the methodology used for the experiments, but do not go into the details of how the experiments were done. You can use subheadings for the different topics discussed.

### **Results:**

Present in your own words main relevant results on the research topic. You can use subheadings for the different topics discussed. Max. 2 pages.

### **Discussion:**

Summarize the major new findings, discuss their relevance to the current literature, and draw the major conclusions. You should discuss similarities, discordant, or complementary results from the articles, and your own literature review of the field. You may find including your own table or schematic diagram helpful with your discussion. You should also briefly propose future studies relevant to the findings and or discuss limitations/challenges.

### **References:**

References should be numbered (1, 2, 3...) as they appear in the text – limit to 25 references.

Use the following format:

Viswanathan, S, Shi, Y, Galipeau, J, Krampera, M, Leblanc K, Martin, I, Nolte, J D G Phinney, D.G, Sensebe, L 9: **Mesenchymal stem versus stromal cells: International Society for Cell & Gene Therapy (ISCT®) Mesenchymal Stromal Cell committee position statement on nomenclature.** *Cytotherapy* 2019, 21(10):1019-1024

If you are using reference management software, use the “American Journal of Pathology” format. If textbooks are used as references, first list all the authors as shown in the example above followed by the chapter title, title of the textbook, edition, year of publication and page numbers used.

### **Evaluation Criteria**

1) 10% - Attendance and Class Participation

Total – 60 marks for 12 classes

Students are required to submit at least 2 written questions by 11:59 the night before a given lecture.

2) 40% Presentation

➤ Total-40 marks

- Presentation layout: 10
- Presentation style: 10
- Interpretation of results and critique: 20

3) 50% - Term Paper

➤ **Due date: April 12<sup>th</sup>, 2023**

➤ Total - 50 marks

- Discussion including references: 25 marks
- Results and Methodology: 15 marks
- Language and references: 10 marks

## Course Schedule with Speakers + Topics

<b>Date</b>	<b>Time</b>	<b>Speakers</b>	<b>Topic</b>
Week 1 – Jan 11	Room: XYZ 14:00- 16:00	Dr. Sara Nunes Vasconcelos And Dr. Sowmya Viswanathan	Introductory Lecture
Week 2 – Jan 18	14:00- 16:00	Dr. John Davies	MSC Introduction
Week 3 – Jan 25	14:00- 16:00	Dr. Sowmya Viswanathan	MSC Regenerative Properties vs. MSC Immunomodulatory Properties
Week 4 – Feb 1	14:00- 16:00	Dr. Sara Nunes Vasconcelos	PSC Overview
Week 5 – Feb 8	14:00- 16:00	Dr. Stephanie Protze	hPSC and cardiac conduction system regeneration
Week 6 – Feb 15	14:00- 16:00	Dr. Michael Laflamme	Challenges in hPSC translation: large scale cell production
<b>Week 7 – Feb 22</b>			<b>READING WEEK: No lecture</b>
Week 8 – Mar 1	14:00- 16:00	Dr. Rohin Iyer	Immunotherapy Overview with a focus on CAR-T/NK
Week 9 – Mar 8	14:00- 16:00	Dr. Linh Nguyen	Use of TILS in clinical trials
Week 10 – Mar 15	14:00- 16:00	Dr. Sonya MacParland	Manipulation of the liver immune environment to promote regeneration
Week 11 – Mar 22	14:00- 16:00	Dr. Jason Dowd	GMP Overview
Week 12 – Mar 29	14:00- 16:00	Dr. Nick Timmins	CMC: To and through the clinic
Week 13 – April 5	14:00- 16:00	Dr. Sowmya Viswanathan	Regulatory Overview - Health Canada vs. FDA/EMA regulations for cell and gene therapies
<b>Week 14 – April 12th</b>		<b>TERM PAPER DUE AT 11:59 PM</b>	