

# **BIO 48006 / 58006 Gene Regulation and Disease Syllabus**

## **FALL 2022**

**Instructor: Cavit Agca**

Nowadays, medicine is in a transformation stage where classical drug treatments are slowly getting replaced by approaches like personalized medicine and gene therapy. Therefore, therapies are requiring more and more in depth knowledge of molecular biology and to be able make connections with more advanced techniques and the etiology of the disease that is studied. This course will provide advanced level of training and understanding of the molecular mechanisms of diseases by focusing on gene regulatory mechanisms like chromatin remodeling and mRNA transcription, RNA splicing, as well as post-transcriptional and post-translational mechanisms. The course will also focus on gene regulatory and gene correction tools for therapeutic approaches like dCas9, TALE, Zinc finger, CasRX, Cas13a and base editing. The course will complement the understanding of basic research findings and their outcomes in animal models and thus their consequences on the whole organism.

### **Readings:**

Primary articles given in advance for lectures. Copies will be available on SUcourse.

### **Supporting Material:**

-Molecular Biology of the Cell (6th Ed)

Alberts, B., Johnson, A., Lewis, J., Raff, M., Roberts, K. and Walter, P.

Garland Publishing Inc. (2015) ISBN 978-0-8153-4464-3

[www.homerbooks.com](http://www.homerbooks.com)

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| Week 1: | First Meeting and introduction to the course / Discussion of future plans.                                       |
| Week 2: | Gene regulatory tools I / Article discussions  |
| Week 3: | Gene regulatory tools I / Article discussions  |
| Week 4: | Gene regulatory tool applications / Article discussions  |
| Week 5: | Gene therapies (Retina) and article discussions / Student presentations  |
| Week 6: | Gene regulatory tools II Antisense oligonucleotides / Article discussion / Student presentations                 |
| Week 7: | Deadline for disease conditions selection / 10-15 min presentations of proposal plans                            |
| Week 8: | AAV VECTORS and article discussions / Student presentations / 10-15 min presentation of proposal plan continues. |
| Week 9: | Gene regulatory tools III – Protein delivery / Article discussions / Proposal discussions /                      |

Week 10:	Age-related Macular Degeneration and gene therapies / Article discussion / Student presentations / Proposal discussions /
Week 11:	Optogenetics and gene therapies / Article discussions / Student presentations / / Proposal discussions
Week 12:	Gene regulation related diseases and gene therapies / Article discussion / Student presentations / Proposal discussions
Week 13:	Base editing, Prime editing and Cas13 / Article discussion / Student presentations / Proposal discussions
Week 14:	Extracellular nanovesicles, exosomes and gene therapies / Article discussion / Student presentations / Proposal deadline

### **Grading:**

**40% An essay / proposal will be written covering the etiology, diagnostics and ongoing or possible therapeutic approaches of a given disease. Students will decide disease condition / gene therapy at 7th week of the course. Details of the format is attached.**

**40% Article presentations are expected from each student. Details for presentation are given as a separate sheet. Assignments will be coherent with the subject of the week. Graduate students will be assigned first.**

**20% Participation to classroom discussions.**

### **Contact:**

#### **Instructor:**

**Cavit Agca:** L025. Office hour: Open

#### **TA:**

**Mehri Ahmadian,** [mehriahmadian@sabanciuniv.edu](mailto:mehriahmadian@sabanciuniv.edu)

Office hrs: Open

**Lectures:** Thursday 8:40-11:30, FENS, L030

Zoom Meeting

<https://sabanciuniv.zoom.us/j/7556177895?pwd=cTViMFhwWk9tZGN1MFJ4WDIzUi94Zz09>

Meeting ID: 755 617 7895

Passcode: Bio332