YEDITEPE UNIVERSITY
FACULTY OF ENGINEERING

COURSE SYLLABUS
2016/2017-SPRING

Course Code-Name	GBE 435 Gene Therapy

Instructor	Assist. Prof. Dr. Fatih Kocabaş Genetics and Bioengineering Department, Room B504 0-216-578 0618 fatih.kocabas@yeditepe.edu.tr
Course Schedule	Mon 09:00 - 10:50, Thursday 17:00 - 17:50 @ B317
Office Hours	Tuesday 11:00 – 12:50 *by appointment only*
Course Description	Gene Therapy
Course Objectives	Recent technologies that are already in use or may be used in future to correct genetic defects in human; human somatic cell gene therapy; viral and nonviral gene transfer techniques; gene therapy applications in hereditary and acquired diseases; ethical issues in genetic modification of humans; case studies: gene therapy for cystic fibrosis, gene therapy for ADA deficiency. In addition, recent breakthroughs in gene editing based on CRISPR/CAS9 system will be discussed.
Required Textbook & Supplementary Materials	**Gene and Cell Therapy Therapeutic Mechanisms and Strategies**, Second-Third or Fourth Edition, Edited by Nancy Smyth **Templeton**. CRC Press. **CRISPR 101**:A Desktop Resource. Created and Compiled by Addgene January 2016 (1st Edition) **Gene Therapy Protocols Volume I & II**. Joseph M. Le Doux. Humana Press.
Grading	Quizzes/Presentation: **30%** Mid-Semester Exam: **30%** Final Exam: **40%** TOTAL: **100%** **If you achieve less than 50% overall in the class, you will automatically get an "F".**
Make-up Exams	There is no planned make-up for any missing examination. You must demonstrate a valid excuse to re-take a missed exam. In addition, the school policies will be taken into account in cases when you miss a scheduled examination.
Homework / Quizzes	It is highly recommended that you read the relevant chapters in the textbook as the course progresses and study for at least one hour for every one hour of lecture. A short quiz will be given every week based on the previous week's reading assignments. . *Each student is required to give one presentations related to recent developments in gene therapy and gene editing technologies.*
Attendance	If you fail to attend less than **80%** of the lectures from the beginning of the semester, you will get "FA" in the course and have no right to take **BÜTÜNLEME** exam. In addition, tardiness to class may incur a penalty of loss of marks.
Academic Integrity	Adherence to the University Academic Integrity policy is expected. No breach of this policy will be tolerated. Any offenders, explicit or complicit, will be dealt with in accordance with the established University procedures.

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Exam Schedule

Mid-Semester Exam	Wed, March 27th	09:00-10:50
Final Exam	May 15-28, 2017	Exact time and date will be announced later.

Course Outline

Week	Chapters	Topics
Jan 30-Feb 3	-	First meeting
Feb 6-10	Part I Templeton	Gene Delivery Systems and Therapeutic Strategies for Gene Therapy
Feb 13-17	Part II Templeton	Therapeutic Strategies for Gene Therapy
Feb 20-24	Part III Templeton	Gene Expression and Detection in Gene Therapy
Feb 27-Mar 3	Part IV Templeton	Disease Targets and Therapeutic Strategies in Gene Therapy
Mar 6-10	Part V Templeton	Gene Therapy Clinical Trials and Regulatory Issues
Mar 13-17	-	***No planned lectures (Out of town due to Congress)***
Mar 20-24	Part VI Templeton	Other Cell-based Therapies and Trials
Mar 27-31	-	**Midterm (No lecture on March 30th).**
Apr 3-7	CRISPR 101	*Introduction to Genome and Gene Editing* *History of CRISPR*
Apr 10-14	CRISPR 101	*Components of CRISPR/CAS9 system* *Using CRISPR/CAS9 system*
Apr 17-21	CRISPR 101	*Editing with homology directed repair* *The Crıspr Software Matchmaker and Validation of Gene Edits*
Apr 24-28	CRISPR 101	*Genome-wide Screening and Regulation of Gene Expression Usıng Crispr/Cas9* *CRISPR Purification, enCHIP, and Multiplexible Crispr Expression Systems*
May 1-5	CRISPR 101	*Mammalian Expression Systems, Delivery Methods and Therapeutic Applications of Crispr*
May 8-12	-	Review lectures and presentations

Active Student Participation: This class emphasizes on active student participation. You are supposed to define a very specific research topic within the field of gene therapy and gene editing technologies and perform an exhaustive literature search. You will present the specific topic in as a presentation.

Research Paper Presentation: You will be given research papers related to gene therapy and gene editing technologies, prepare a powerpoint presentation and explain this paper along with relevant studies in detail in class. One or two slides should indicate the general theme of the paper. All used methods and potentially unknown terminology should be explained in detail. All results should then be explained and critically evaluated. The presentation should take **approximately 5 minutes, followed by 2 minutes of discussion**.

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Actual length of presentations and number of presented papers will depend on total number of participating students. There is no makeup for missed presentations.

Powerpoint presentation regarding the research paper will be submitted to the instructor via email. This is due by the date of your presentation date. Please email to fatih.kocabas@yeditepe.edu.tr

Disclaimer: This syllabus provides a general plan and subject to change. The instructor reserves the right to make modifications in content and schedules as necessary to promote the best education possible within the prevailing conditions affecting this course. It is the student's responsibility to note the changes that may occur during the semester