

BME3122: FUNDAMENTAL GENE THERAPY

Effective Term

Semester B 2022/23

Part I Course Overview

Course Title

Fundamental Gene Therapy

Subject Code

BME - Biomedical Engineering

Course Number

3122

Academic Unit

Biomedical Engineering (BME)

College/School

College of Engineering (EG)

Course Duration

One Semester

Credit Units

3

Level

B1, B2, B3, B4 - Bachelor's Degree

Medium of Instruction

English

Medium of Assessment

English

Prerequisites

BMS2801 Molecules and Cells/ BME2106 Introduction to Cellular and Biomolecular Engineering#

Precursors

MBE2103/BME2103 Medical Biotechnology; MBE2104/BME2104 Tissue Engineering

Equivalent Courses

Nil

Exclusive Courses

Nil

Additional Information

Prerequisites which are not part of the Major Requirement are waived for students admitted with Advanced Standing or record of receiving fundamental biology course(s).

Part II Course Details

Abstract

The course aims to teach students about fundamental knowledge and technology of gene therapy and genetic engineering. Gene therapy related research and applications have become more and more popular and important in medical and biomedical areas. This course is developed to deliver the fundamental knowledge about gene therapy and genetic engineering with the typical applications. This course is mainly designed to emphasize on basic concepts and focus on the application of them – how to translate biological sciences into biomedical engineering. This course is set up in such a way that students can understand this interdisciplinary subject with minimal background. Its major components include: Definitions of Gene Therapy and Genetic Engineering; Gene Delivery: Viral and Non-Viral Vectors; Therapeutic Antisense; Basic Gene Editing Technology; and typical clinical applications, such as CAR-T. It will be delivered to emphasize biomedical engineering perspectives, including the related instrumentation and methodologies for gene sequencing, medical/molecular imaging and so on.

Course Intended Learning Outcomes (CILOs)

CILOs	Weighting (if app.)	DEC-A1	DEC-A2	DEC-A3
1 Describe the basic principles of gene and antisense therapy and approaches of gene editing, gene delivery and transgenic treatment, and the related instrumentation and methodologies for gene sequencing, medical/molecular imaging and so on.		x		
2 Explain fundamental concepts on gene therapy and transgenic engineering.			x	
3 Assess the existing techniques to edit, deliver genes, therapies with gene and antisense, and typical applications such as CAR-T for anticancer therapy.			x	x
4 Identify the practical issues for implementation of biomedical engineering.		x	x	
5 Design a feasible and effective engineering approach to a specific gene therapy related problem.			x	x

A1: Attitude

Develop an attitude of discovery/innovation/creativity, as demonstrated by students possessing a strong sense of curiosity, asking questions actively, challenging assumptions or engaging in inquiry together with teachers.

A2: Ability

Develop the ability/skill needed to discover/innovate/create, as demonstrated by students possessing critical thinking skills to assess ideas, acquiring research skills, synthesizing knowledge across disciplines or applying academic knowledge to real-life problems.

A3: Accomplishments

Demonstrate accomplishment of discovery/innovation/creativity through producing /constructing creative works/new artefacts, effective solutions to real-life problems or new processes.

Teaching and Learning Activities (TLAs)

TLAs	Brief Description	CILO No.	Hours/week (if applicable)	
1	Lecture	Introduction of key concepts, applications and explanations about the methodology and ideology.	1, 2, 3, 4, 5	3 hrs/week as average
2	Lab Module	Practise key learning points via experiments	1, 2, 3, 4, 5	6 hrs total: two experimental modules to be conducted in two weeks, 3hrs per module per week

Assessment Tasks / Activities (ATs)

ATs	CILO No.	Weighting (%)	Remarks (e.g. Parameter for GenAI use)	
1	Mid-Term Quiz	1, 2, 3	30	
2	Lab Experiments and Reports	3, 4, 5	10	Up to 2 Lab reports

Continuous Assessment (%)

40

Examination (%)

60

Examination Duration (Hours)

2.5

Additional Information for ATs

For a student to pass the course, at least 30% of the maximum mark for both coursework and examination should be obtained.

Assessment Rubrics (AR)**Assessment Task**

Mid-Term Quiz

Criterion

Ability to explain the principles and methodology related to gene therapy and transgenic engineering.

Excellent (A+, A, A-)

High

Good (B+, B, B-)

Significant

Fair (C+, C, C-)

Moderate

Marginal (D)

Basic

Failure (F)

Not even reaching marginal levels

Assessment Task

Lab Experiments and Reports

Criterion

Ability to demonstrate the key learning points via experimental operation and collect, record and analyze the experimental outcomes.

Excellent (A+, A, A-)

High

Good (B+, B, B-)

Significant

Fair (C+, C, C-)

Moderate

Marginal (D)

Basic

Failure (F)

Not even reaching marginal levels

Assessment Task

Examination

Criterion

Ability to explain the principles and methodology related to gene therapy and transgenic engineering.

Excellent (A+, A, A-)

High

Good (B+, B, B-)

Significant

Fair (C+, C, C-)

Moderate

Marginal (D)

Basic

Failure (F)

Not even reaching marginal levels

Part III Other Information

Keyword Syllabus

- **Principles:** gene therapy; antisense treatment; gene editing; gene delivery; transgenic immunocytes therapy
- **Gene therapy:** nature of a bio/pharmaceutical approach, design and application of therapeutic transgenes
- **Gene delivery:** viral and non-viral delivery and transgene types of episomal and integrative
- **Gene editing:** CRISPR/Cas RNA-guided endonucleases system as fundamental tool
- **Antisense:** Ribozyme, ODN and RNAi
- **CAR-T:** ex vivo transgenic immune cell therapy for anticancer treatment
- **Biomedical engineering perspective:** the related instrumentation and methodologies for gene sequencing, medical/molecular imaging and so on
- **Other Issues:** ethical issues, safety of viral vectors including allergic side-effect, insertion mutation by integrative transgenes and so on

Reading List

Compulsory Readings

Title	
1	Nil

Additional Readings

Title	
1	Turksen Kursad. (2016). Genome Editing, Springer International Publishing, Switzerland.
2	K.K. Jain. (1998). Textbook of Gene Therapy, Hogrefe & Huber Publishing, Cambridge, MA, USA. ISBN: 978-0889371903.