BME3122: FUNDAMENTAL GENE THERAPY

Effective Term
Semester B 2024/25

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 Biomedicine (BD)

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
BME2103 Medical Biotechnology; 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	Apply 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.

Learning and Teaching Activities (LTAs)

	LTAs	Brief Description	CILO No.	Hours/week (if applicable)
1	Lecture	Students will develop an understanding of key concepts, applications and explanations about the methodology and ideology.	1, 2, 3, 4, 5	3 hrs/week as average
2	Lab Module	Students will gain hands-on experience and 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.