



Course Title: Human Genetics and Gene Therapy

Course Code:

Credit Units: 03

L	T	P/S	SW/F W	TOTAL CREDIT UNITS
3	-	-	-	03

Course Objectives:

The course is designed to give students an understanding of various facets of human genetics, including nature of genetic diseases. The students will be familiarized with various approaches of gene therapy using different gene therapy vectors and therapeutic approaches for various human diseases.

Pre-requisites: Basics of genetics, Biochemistry, Molecular biology, Immunology and Recombinant DNA Technology

Student Learning Outcomes: By the end of the course, students will be able to:

- Describe and comprehend the fundamental concepts of human genetics.
- Describe the basic concepts of gene therapy and its applications.

Course Contents/Syllabus- Theory:

	Weightage (%)
Module I Introduction to Human Genetics	20%
Historical aspects of Genetics in Health and Diseases, Human genetics and its scope, Pedigree analysis and Inheritance patterns, Monogenic versus multi-factorial inheritance.	
Module II Chromosomal and Genetics Disorders	20%
Chromosomes and genes: autosomes and sex-chromosomes, sex determination, Chromosomal abnormalities and syndromes. Genetic diseases: involvement of single versus multiple genes, Diagnosis parameters.	
Module III Human Genome	20%
Human Genome: Organization of genes within genome, Genetic variability and single nucleotide polymorphisms (SNPs). Human Genome project and its impact in Health Sciences	

Mitochondrial genome and genes, mitochondrial myopathies.	
Module IV Principles of Gene Therapy	10%
Definition, approaches and types of gene therapy, optimal disease targets, history and evolution of gene therapy.	
Module V Vectors for Gene Therapy	20%
Viral Vectors: Adenovirus, adeno-associated virus (AAV), retrovirus, and lentivirus for gene therapy. Physical and chemical methods of gene therapy. RNAi technology for gene therapy, Nano Medicine, Immune response to gene therapy vectors, strategies to overcome vector immunity.	
Module VI Gene Therapy: Promises and Challenges	
Present status of gene therapy, Gene therapy trials: challenges and opportunities, Ethical issues in gene therapy, Genetic counseling.	10%

Pedagogy for Course Delivery:

Lectures: 39
 Tutorial: 0
 Test: 2
 Presentation/ Seminar: 4
 Total: 45

Assessment/ Examination Scheme:

Theory L/T (%)	Lab/Practical/Studio (%)	End Term Examination
100	0	100

Theory Assessment:

Continuous Assessment/Internal Assessment					End Term Examination
Components (Drop down)	Class Test 1	Class Test 2	Home Assignment	Attendance	
Weightage (%)	10	10	5	5	70

Text Books and References:

- Human Molecular Genetics by Tom Strachan and Andrew Read. Garland Science, 4th Edition, 2011. ISBN 1-85996-202-5
- Gene and Cell Therapy (3rd edition): Therapeutic Mechanisms & Strategies. Editor: Nancy Smyth Templeton. Publishers: CRC Press, 2008. ISBN 9780849387685