GUJARAT TECHNOLOGICAL UNIVERSITY
Integrated Master of Science (Biotechnology)
Semester: 8
Subject Name: Advanced Therapeutics
Subject Code: 1380408

Prerequisite: Prerequisites for Advanced Therapeutics: mRNA, RNAi, and Antisense Therapy typically include a solid foundation in molecular biology, genetics, and biochemistry. Understanding RNA biology, gene expression regulation, nucleic acid chemistry, and cell signaling is crucial. Familiarity with biotechnology techniques like PCR, qRT-PCR, gene silencing, and CRISPR is beneficial. Basic knowledge of pharmacology and drug delivery systems can also help in grasping the therapeutic applications.

Rationale: Advanced therapeutics like mRNA, RNA interference (RNAi), and antisense therapy offer revolutionary approaches to treating genetic disorders, infectious diseases, and cancers by targeting gene expression at the molecular level. mRNA-based therapies, such as vaccines, harness the body's own machinery to produce therapeutic proteins, while RNAi and antisense oligonucleotides selectively silence or modify gene expression to correct disease-causing mutations. These technologies provide precision medicine solutions with high specificity, rapid adaptability, and reduced side effects compared to traditional treatments, making them a promising frontier in modern biotechnology and healthcare.

Course Scheme:

Teaching Scheme			Total Credits	Assessment Pattern and Marks				Total Marks
				Theory		**Practical**		
L	**T**	**PR**	**C**	ESE (E)	PA(M)	ESE (V)	PA (I)	
4	0	0	4	70	30	0	0	100

Course Content:

Sr. No.	Course Content	No. of Hours	% of Weightage

1	**Unit 1: Fundamentals of RNA-Based Therapeutics**
	• **Introduction to RNA Biology and Therapeutics**
	  ○ Types of RNA: mRNA, siRNA, miRNA, lncRNA
	  ○ RNA Stability, Modifications, and Half-life Regulation
	• **RNA Interference (RNAi) and Gene Silencing**
	  ○ siRNA and miRNA Mechanisms in Post-Transcriptional Gene Regulation
	  ○ Design and Delivery of RNAi-Based Therapeutics
	• **Antisense Oligonucleotides (ASOs) in Gene Therapy**
	  ○ Mechanism of Action: RNase H-mediated and Splice Modulation
	  ○ FDA-Approved ASO Therapies (Spinraza, Exondys 51)
	• **Challenges and Limitations of RNA-Based Drugs**
		15	**25**

2	**Unit 2: mRNA-Based Vaccines and Therapeutics**
	• **mRNA Therapeutics and Vaccine Technology**
	  ○ In Vitro Transcription of mRNA and Codon Optimization
	  ○ mRNA Vaccine Mechanism: Translational Activation and Immune Response
	• **mRNA Delivery Strategies and Nanocarriers**
	  ○ Lipid Nanoparticles (LNPs), Polymers, Exosomes, and Viral Vectors
	• **Clinical Applications of mRNA Vaccines and Therapeutics**
	  ○ COVID-19 Vaccines (Pfizer-BioNTech, Moderna)
	  ○ Cancer mRNA Vaccines and Autoimmune Disease Treatments
	• **Regulatory Considerations and Safety in mRNA Therapeutics**
		15	**25**

3	**Unit 3: CRISPR and RNA-Based Gene Editing**
	• **CRISPR-Cas Systems for RNA Targeting and Editing**
	  ○ CRISPR-Cas13 and RNA-Specific Editing Mechanisms
	  ○ Base Editing and Prime Editing for Genetic Diseases
	• **Applications of RNA-Targeting CRISPR in Therapy**
	  ○ Targeting Viral Infections (HIV, Hepatitis B, Influenza)
	  ○ CRISPR-Based Therapeutics in Neurological and Metabolic Disorders
	• **Challenges in CRISPR-Based RNA Editing**
	  ○ Off-Target Effects, Delivery Barriers, and Ethical Concerns
		15	**25**

4	**Unit 4: Future Trends and Clinical Applications of RNA Therapeutics**
	• **Emerging RNA Therapeutics**
	  ○ Self-Amplifying RNA (saRNA), Circular RNA (circRNA), and mRNA-Encoded Antibodies
	• **AI and Computational Biology in RNA Drug Design**
	  ○ In Silico Modeling of RNA Structure and Function
	• **Personalized RNA Medicine and Precision Therapeutics**
	  ○ Individualized RNA Therapy Based on Genomic Variability
	• **Future Prospects and Commercialization of RNA-Based Drugs**
	  ○ Industry Trends and Investment in RNA Therapeutics
		15	**25**

Reference Books:

1. RNA-Based Therapeutics – Kevin V. Morris
2. mRNA Therapeutics: Methods and Protocols – Katalin Karikó
3. Gene Silencing by RNA Interference – Muhammad Sohail
4. CRISPR-Cas Systems: RNA-Mediated Adaptive Immunity – Rodolphe Barrangou
5. Antisense Therapy: Concepts and Clinical Applications – Paul M. Tulkens

List of Experiments:

1. Isolation of RNA from different human source
2. cDNA library prearation and qRT-PCR
3. Design and Synthesis of siRNA and ASOs for Gene Knockdown
4. In Silico Prediction of RNA Secondary Structure for Drug Design
5. Differential expression analysis of miRNA in human from Publically available dataset
6. Analysis of miRNA in human blood sample

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