

Educational Objectives:

1. This course gives an overview of the field of human gene therapy. It covers the fundamentals of using gene delivery for treating human diseases, and details the viral vectors that are used for gene delivery, including their generation and use, the non-viral approaches that can be used to deliver therapeutic genes, and the methods that are used for controlling therapeutic gene expression. Students completing this course should be able to understand the concept of gene therapy for therapeutic purposes.

Course Outcomes:

2. After studying this course student should understand the types of disease that might be treatable by gene therapy. The basic principles of genetic manipulation and the differences between somatic and germline gene therapy and some of the problems involved in these potential treatments.

3. **Course Contents:**

- a. Introduction to gene therapy
- b. Delivery of Therapeutic Genes
- c. Gene Therapy Targets
- d. Delivery Modes
- e. Steps in Gene Therapy
- f. Gene Therapy Non Viral vectors
- g. Liposomes
- h. Polycation conjugates
- i. Cell bases therapies
- j. Cancer gene therapy
- k. Gene therapy in plants
- l. Ethical issues in gene therapy
- m. Therapeutic Gene Regulation
- n. Control of therapeutic gene expression
- o. Prospects for gene therapy

4. **Recommended Books:**

- a. **Understanding Gene Therapy** by NR Lemoine (1999) Springer-Verlag Singapore Pte Ltd.
- b. **Gene Therapy and Gene Delivery Systems** (Advances in Biochemical Engineering / Biotechnology) By: David V. Schaffer, Weichang Zhou (2006) Springer.
- c. **The Ethics of Human Gene Therapy** by LeRoy Walters, Julie Gage Palmer (1996) Oxford University Press, USA.
- d. **Gene Therapy: Treating Disease by Repairing Genes** by Joseph, Ph.D. Panno (2004) Facts on File.
- e. **Gene Therapy (Healthcare and Medical Issues Today)** By Evelyn B. Kelly (2007) Greenwood Press.
- f. **Stem Cell and Gene-Based Therapy: Frontiers in Regenerative Medicine** by Alexander Battler, Jonathan Leor (2005) Springer
- g. **Gene and Cell Therapy: Therapeutic Mechanisms and Strategies**, Second Edition By: Nancy Smyth Templeton (2003) CRC