

Medical Biotechnology

Learning Objectives

1. Know how genetic diseases are generated and how genes are candidates for gene therapy.
2. List and define the methods used to deliver genes into cells.
3. Know the difference between *ex vivo* and *in vivo* gene therapy.
4. List diseases that have been the subject of gene therapy research, the causes of those diseases, and how gene therapy attempts to treat them.
5. Know the types of clinical trials and the steps of the clinical trial process required for gene therapy.
6. List and define new approaches to gene therapy, such as spliceosome-mediated RNA trans-splicing, triplex-helix-forming oligonucleotide therapy, antisense therapy, and ribozyme therapy.
7. Be familiar with the potential of stem cells, the types of stem cells used in research, and their applications. What are the issues surrounding the use of stem cells?
8. Know the potential applications of biotechnology regarding vaccines, tissue engineering, xenotransplantation, drug delivery, and biosensors.