

# Gene Therapy - Web course

## COURSE OUTLINE

Introduction, Somatic and germ line gene therapy, Gene replacement and gene addition, In vivo, ex vivo and in vitro gene therapy, Transgenic animal models, Vehicles for gene transfer-viral vectors: retrovirus, Adenovirus, Adeno-associated virus, Lentivirus, Recombinant SV40 virus, Nonviral vectors, DNA vaccines, Liposomes and lipoplexes, Naked DNA, transposon, Cancer gene therapy, RNA-DNA chimera, Gene therapies for Criglar-Najjar syndrome I, Cystic fibrosis, Duchenne muscular dystrophy, Bleeding disorders, Tyrosinemia, Severe combined immunodeficiency syndrome (SCID), Gene therapy of non-heritable disorders, Recent advancement in Gene Therapy.

## COURSE DETAIL

S. No.	Module	Topics and Contents	No of Lectures
1.	Introduction	Introduction, Somatic and germ line gene therapy, Gene replacement and gene addition.	6
2.	In vivo gene therapy	In vivo, ex vivo and in vitro gene therapy, Transgenic animal models, Vehicles for gene transfer-viral vectors: retrovirus, Adenovirus, Adeno-associated virus.	8
3.	Viral vectors	Lentivirus, Recombinant SV40 virus, Nonviral vectors, DNA vaccines, Liposomes and lipoplexes, Naked DNA, transposon.	8
4.	Cancer gene therapy	Cancer gene therapy, RNA-DNA chimera, Gene therapies for Criglar-Najjar syndrome I.	6
5.	Gene Therapy and diseases-I	Cystic fibrosis, Duchenne muscular dystrophy, Bleeding disorders, Tyrosinemia.	6
6.	Gene Therapy and diseases-II	Severe combined immunodeficiency syndrome (SCID), Gene therapy of non-heritable disorders, Recent advancement in Gene Therapy.	6
		<b>Total</b>	<b>40</b>

## References:

- Friedman T. 1999. *The Development of Human Gene Therapy*. Cold Spring Harbor, NY: Cold Spring Harbor Lab. Press.
- Knipe DM, Howley PM, eds. 2001. *Fields Virology*. Philadelphia, PA: Lippincott Williams & Wilkins.
- Hackett NR, Crystal RG. 2000. Adenovirus vectors for gene therapy. In *Gene Therapy*, ed. NS Templeton, DD Lasic, pp.17-39. New York: Marcel Dekker



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# NPTEL

<http://nptel.iitm.ac.in>

## Biotechnology

### Additional Reading:

- Gene therapy:** Twenty-First Century Medicine. Annu. Rev. Biochem. 2005. 74:711-38
- Gene therapy:** Promises and Problems. Annu. Rev. Genomics Hum. Genet. 2001. 2:177-211

### Hyperlinks:

- <http://www.liebertpub.com/hum>
- [www.nature.com/gt/index.html](http://www.nature.com/gt/index.html)
- [www.nature.com/mt/index.html](http://www.nature.com/mt/index.html)

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