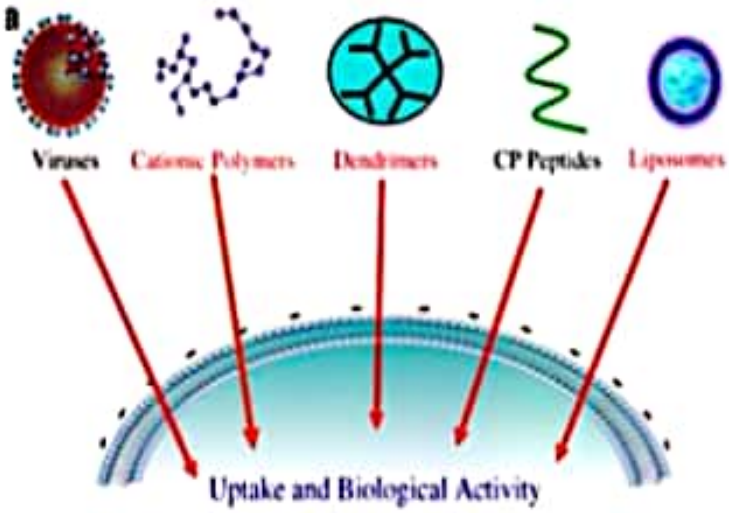
VECTORS IN GENE THERAPY

- To transfer the desired gene into a target cell, a carrier is required. Such vehicles of gene delivery are known as **vectors.**

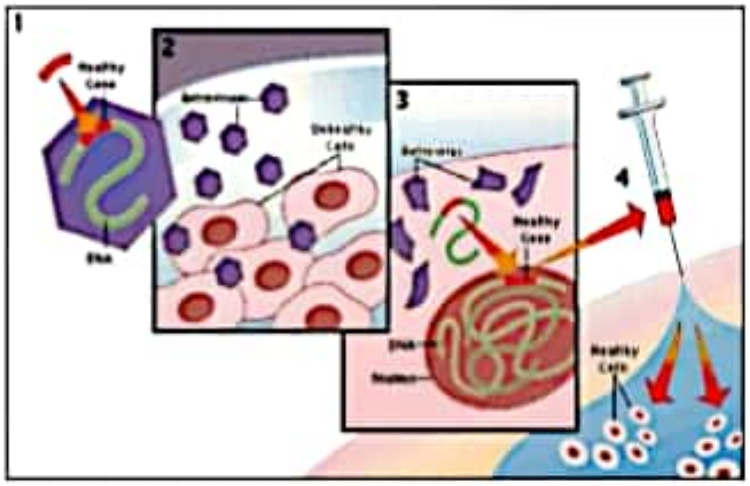
- 2 main classes
  - **Viral** vectors
  - **Non viral** vectors



VIRAL VECTORS

1) RETROVIRUS VECTOR SYSTEM

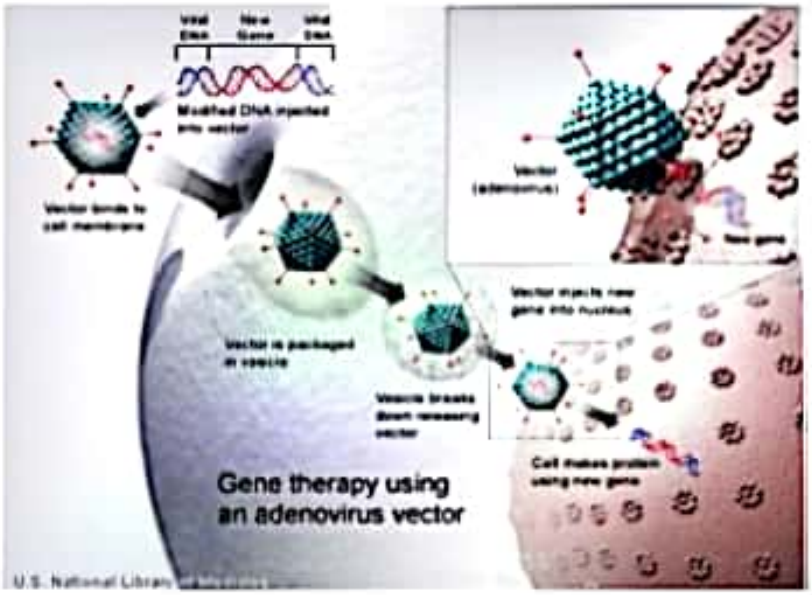
- The recombinant retroviruses have the ability to integrate into the host genome in a stable fashion.
- Can carry a DNA of size – **less than 3.4kb**
- Replication defective virus particles
- Target cell - dividing



VIRAL VECTORS

2) ADENO VIRUS VECTOR SYSTEM

- Adeno virus with a DNA genome – good vectors.
- Target- **non dividing human cell.**
- Eg. Common cold adenovirus.



VIRAL VECTORS

3) ADENO ASSOCIATED VIRUS VECTOR

- It is a human virus that can integrate into chromosome 19.
- It is a single stranded, non pathogenic small DNA virus.
- AAV enters host cell, becomes double stranded and gets integrated into chromosome.

4) HERPEX SIMPLEX VIRUS VECTOR

- Viruses which have natural tendency to infect a particular type of cell.
- They infect and persist in **nervous cells**.

NON VIRAL VECTOR SYSTEM

1. **PURE DNA CONSTRUCT**

   - Direct introduction of pure DNA construct into target tissue .
   - Efficiency of DNA uptake by cells and expression rather low.
   - Consequently, large quantities of DNA have to be injected periodically.

2. **LIPOPLEXES**

   - Lipid DNA complexes; DNA construct surrounded by artificial lipid layer.
   - Most of it gets degraded by lysosomes.

NON VIRAL VECTORS

3) DNA MOLECULAR CONJUGATES

- Commonly used synthetic conjugate is poly- L- lysine bound to specific target cell receptor.
- Therapeutic DNA is then made to combine with the conjugate to form a complex.
- It avoids lysosomal breakdown of DNA.

4) HUMAN ARTIFICIAL CHROMOSOME

- Can carry a large DNA ie, with one or more therapeutic genes with regulatory elements.