

METHODS OF GENE DELIVERY

PHYSICAL METHODS

Gene Gun

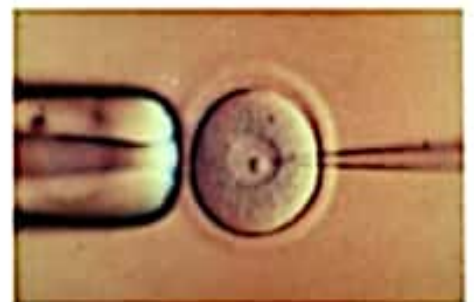
- Employs a high-pressure delivery system to shoot tissue with gold or tungsten particles that are coated with DNA



Gene Gun

Microinjection

- Process of using a glass **micropipette** to insert microscopic substances into a single living cell.
- Normally performed under a specialized optical microscope setup called a **micromanipulator**.



CHEMICAL METHODS

- **USING DETERGENT MIXTURES**

- Certain charged chemical compounds like Calcium phosphates are mixed with functional cDNA of desired function.
- The mixture is introduced near the vicinity of recipient cells.
- The chemicals disturb the cell membrane, widen the pore size and allow cDNA to pass through the cell.

- **LIPOFECTION**

- It is a technique used to inject genetic materials into a cell by means of liposomes.
- Liposomes are **artificial phospholipid vesicles** used to deliver a variety of molecules including **DNA** into the cells.

OTHER TYPES OF GENE THERAPY

GENE AUGMENTATION THERAPY

- Most common form of gene therapy
- Foreign gene replaces missing or defective gene.
- Eg. Replacement of defective **p53 gene** by a normal one in liver cancer.

GENE INHIBITION THERAPY

- Done to block the overproduction of some proteins.
- 2 types – Antigene and antisense therapy.
 - **Antigene** – blocks transcription using antigene oligonucleotide
 - **Antisense** – blocks translation using antisense oligonucleotide.

DISADVANTAGES

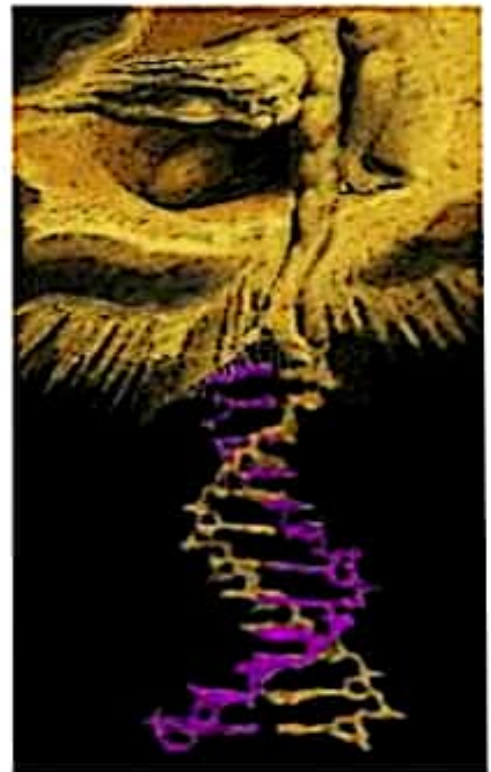
- Long lasting therapy is not achieved by gene therapy; Due to rapid dividing of cells benefits of gene therapy is **short lived**.
- **Immune response** to the transferred gene stimulates a potential risk to gene therapy.
- **Viruses** used as vectors for gene transfer may cause **toxicity**, immune responses, and inflammatory reactions in the host.
- **Disorders** caused by defects in **multiple genes** cannot be treated effectively using gene therapy.

ADVANTAGES

- Gene therapy has the potential to eliminate and prevent hereditary diseases such as cystic fibrosis, ADA- SCID etc.
- It is a possible cure for heart disease, AIDS and cancer.
- It gives someone born with a genetic disease a chance to life.
- It can be used to eradicate diseases from the future generations.

ETHICAL ISSUES

- **Who will have access to therapy?**
- **Is it interfering with God's plan?**
- **Should people be allowed to use gene therapy to enhance basic human traits such as height, intelligence etc.?**
- **Is it alright to use the therapy in the prenatal stage of development in babies?**



CONCLUSION

- **Theoretically, gene therapy is the permanent solution for genetic diseases.**
- **But it has several complexities. At its current stage, it is not accessible to most people due to its huge cost.**
- **A breakthrough may come anytime and a day may come when almost every disease will have a gene therapy**
- **Gene therapy have the potential to revolutionize the practice of medicine.**

RECENT DEVELOPMENTS

- In a new gene therapy method developed by University of Florida in Jan 2012, researchers found **treatment for a common form of blindness (X-linked retinitis pigmentosa)** that strikes both youngsters and adults.
- A gene therapy called **NLX-P101** dramatically reduces movement impairment in Parkinson's patients, according to results of a Phase 2 study published on March, 2011 in the journal *Lancet Neurology*.