



Brahmanand College Kanpur

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Human Gene Therapy

A Way To Disease
free society

Made
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What is Gene Therapy?

Gene therapy is an experimental technique that uses genes to treat or prevent diseases by –

- 1) Replacing a mutated gene that causes disease with a healthy copy of gene.
- 2) Inactivating or 'knocking out' a mutated gene that is functioning improperly.
1. Introducing a new gene into the body to help fight a disease.

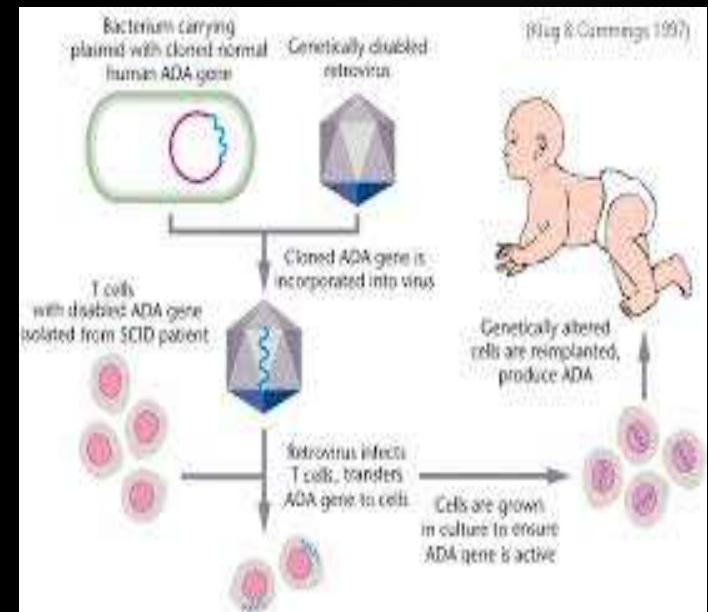
Gene therapy is a promising treatment option for a number of diseases including inherited disorders , some type of cancers , certain viral infections and many more but it remains risky and is still under study to make sure that it will be safe and effective or not !!



Types of Gene therapy

Somatic cell Gene therapy

- Transfer of therapeutic genes into somatic cell.
Ex :- Introduction of gene into bone marrow cell , blood cell, skin cell etc.
- It will not be inherited in later generations.
- At present all researches are directed to correct genetic defect in somatic cell.



Schematic representation of somatic gene therapy

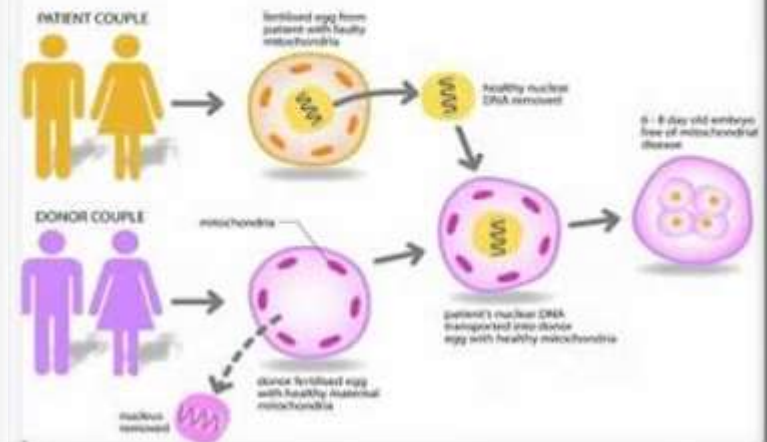
Types Of Gene Therapy

- Gene therapy in germ line cells has the potential to affect not only the individual, but also their children as well.
- Any genetic changes in the reproductive cells or changes made to the embryo before the stage of differentiation, would affect all future offspring of that person. This makes an vital distinction, affecting major ethical issues

Germ Line Gene Therapy

Simple Model of Germ-line Gene Therapy

Pronuclear transfer in human embryos



Types of somatic gene therapy

**Ex-
vivo/In-
vitro**

Cells are modified outside the body and then transplanted again.
Ex. Treatment of SCID.

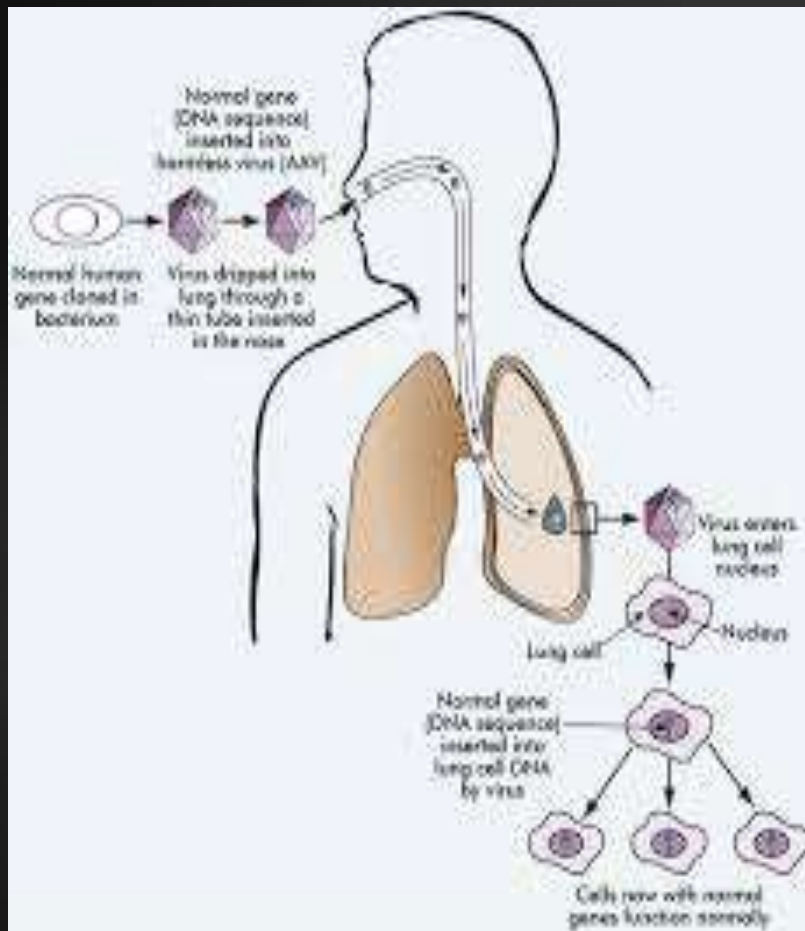
Called as ex-vivo because the therapeutic genes are transferred and manufactured outside the human body i.e. in laboratory.

In-Vivo

Genes are changed or treated in the cells .When the cells are still in body.
Ex. Treatment of cystic fibrosis

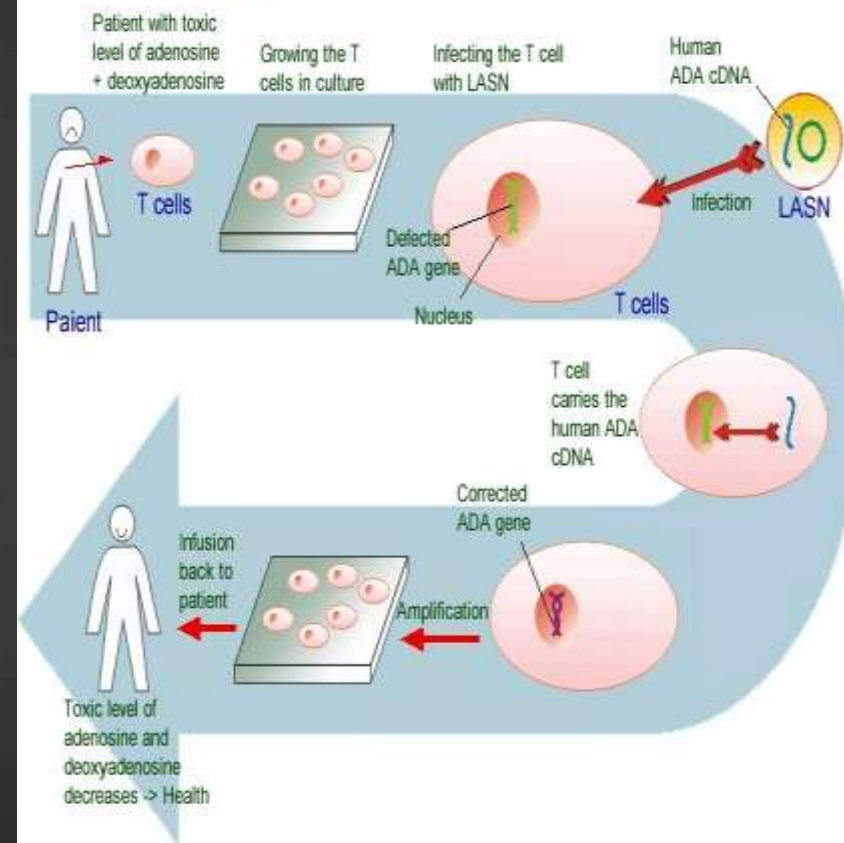
Called as in-vivo because the therapeutic genes are manufactured and transferred inside the patient's body'

EXAMPLES OF IN-VIVO AND EX-VIVO GENE THERAPY



Gene therapy of cystic fibrosis by ex-vivo gene therapy

Gene Therapy for ADA-SCID



Gene therapy of severe combined immune deficiency (SCID)

Advantages and Disadvantages

• Advantages

- 1.It has the ability to replace defective genes that cause diseases.
- 2.It has a great therapeutic potential.
- 3.It can eradicate diseases like Parkinson's diseases, Huntington's disease, Alzheimer's disease' etc.

Disadvantages

- 1.It can damage the gene pool.
- 2.It will certainly modify human capabilities'
- 3.It has the potential of giving rise a new disorder.

Conclusions

It is a fact that gene therapy is in a way a boon for human beings because theoretically it is possible to almost eliminate some disorders like **Sickle Cell Anemia and, many forms of cancers** and many more diseases through gene therapy but there are two sides of this human ingenuity . This is the bright side of the technology but on the other hand gene therapy will also open ethically questionable ways that is it good to **play with nature or play with God !!!**

Also the misuse of gene therapy is hazardous and certainly any kind of misuse would lead the human beings to its end .

“It’s like a sword ,you have to use it to save yourself ,not to cut fruits” .

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Thank you

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