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A Treatment: Explore a New life (Gene Therapy)

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Abstract: *Gene therapy is a treatment modality, which is used to correct the genetic disorder by replacement of correct gene. Gene therapy should be administered for both kind of cell, such as somatic and germinal cell. The viral and non viral methods are used to insert the therapeutic gene. It includes Insertion of therapeutic gene, inhibition of destructive activity or kill the target gene. By gene therapy, one can prevent or recover the various type of diseases such as HIV, cancer, Cystic fibrosis. Even this can prevent the future transmission of disease to the next generation. The technique remains risky and still study is going on to make it safe and effective in future. This therapy is currently being tested only for diseases that have no other cures.*
Key Word: *Gene, Gene Therapy, Nucleotide, Viral, Non Viral*

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I. Introduction

Gene is a unit which contains sequences of nucleotides, part of chromosome. It transmits the information from parent to offspring. Gene therapy could be additional way to cure the genetic problems. It is a transfer of nucleic acid or section of DNA into patient's cell to treat or prevention of disease. Gene therapy helps defective tissue and organ to function properly by insertion of corrected copy of gene.

Gene therapy was once considered a fantasy. In 1980, Martin Cline tried for the first time for modification of Human DNA, but first success in nuclear gene transfer was approved in 1989. However, thousands of individuals have already undergone human clinical trials. A great leap in medical science has taken place on 14th September 1990, when a girl suffering from adenosine deaminase deficiency (severe immunodeficiency) was treated by transferring the normal gene for adenosine deaminase.

II. Definition

Gene therapy is an experimental treatment designated to modify the expression of an individual's gene or to correct abnormal gene to prevent disease.

Gene therapy is an experimental technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into patient's cells instead of using drugs or surgery.

III. Approach

There are several approaches used by the researcher in gene therapy:

1. Replacing the abnormal mutated gene with Healthy Gene copy.
2. Insertion of Functionally normal gene at different location of chromosome.
3. Inactivation of muted gene that function abnormally.

IV. Types of Gene Therapy

On the basis of cell selection, there are two different types of gene therapy:

- ***Somatic cell Gene Therapy:*** This therapy includes transfer of gene into the any body cell except reproductive cell e.g: Bone Marrow cell, blood cell, Skin cell. The effect of gene therapy will not be inherited to offspring. At present, all the researchers directed to correct genetic defects in somatic cells.

- ***Germline Gene Therapy:*** This therapy includes transfer of gene into Germ cells which produce sperm and Ovum. The effect of gene therapy will be inherited to offspring and subsequent generations. For safety, ethical and technical reasons it is not being attempted at present.

On the basis of process/ or delivery of gene there are two type of gene therapy.

1. Ex vivo Gene Therapy:

a) Defective gene are isolated from the patients and grown into culture.
b) The therapeutic gene is then introduced into cultured cell.

c) Select genetically corrected cells and grow. These modified cells are transplanted into same individual.

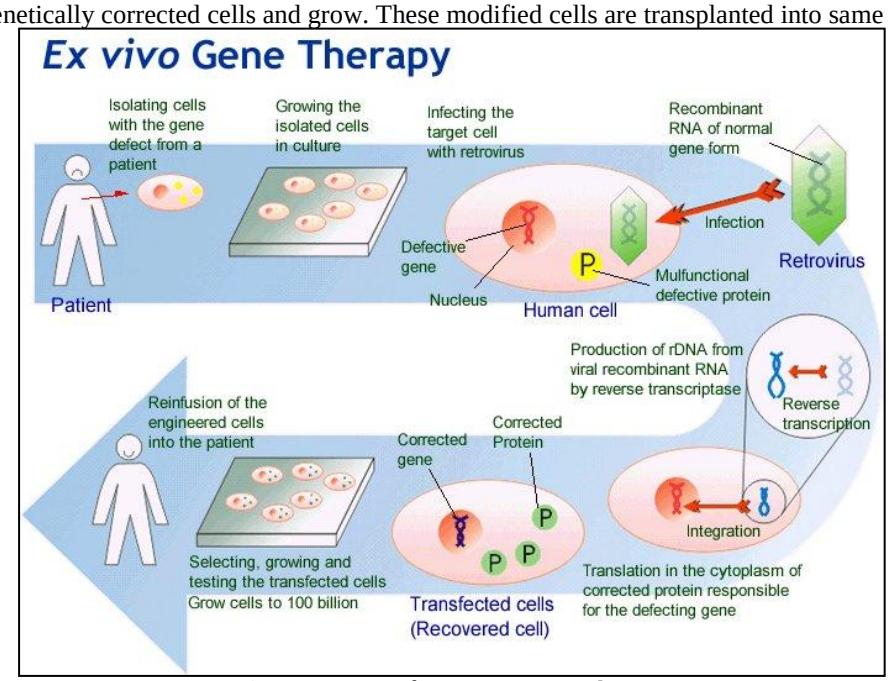

Fig.1: Process of Ex Vivo Gene Therapy

2. In vivo Gene Therapy:

a) Direct insertion of therapeutic gene into the target cell of patient's body. It can be carried out by vector and non vector system.

b) It can be the possible option in where in individual cells cannot be cultured in vitro in sufficient number or cultured cells cannot be reinserted in patient effectively.

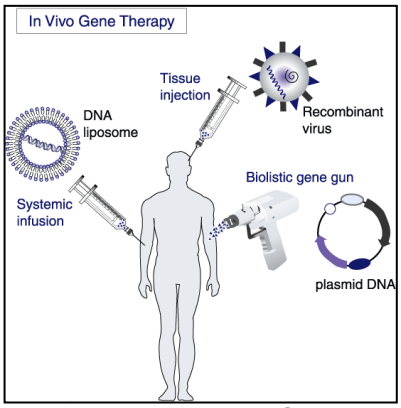

Fig.2: In Vivo Gene Therapy

V. Vectors In Gene Therapy

A carrier is required to be transferred therapeutic gene into target cell. Such carrier of gene transfer is known as vectors. There are main two types of vector: Viral Vectors and Non Viral vectors.

- ***Viral Vectors:*** Viruses have evolved a way of encapsulating and transferred their genes to human cells to remove disease causing genes and insert therapeutic gene. Virus binds to their hosts and introduce their genetic material into host cell. There are various viruses are used as a vector such as, Retrovirus, Adenosine Virus, Adeno Associated Virus, Herpes Simplex Virus.

- ***Non Viral Vector:*** Physical, chemical and other methods are used to transfer therapeutic genes to target cell. Such as liposomes, Naked Gene, Gene Gun, Electroporation, Sonoporation, Magnetofection, Hydrodynemic delivery, oligonucleotide, Hybrid Methods.

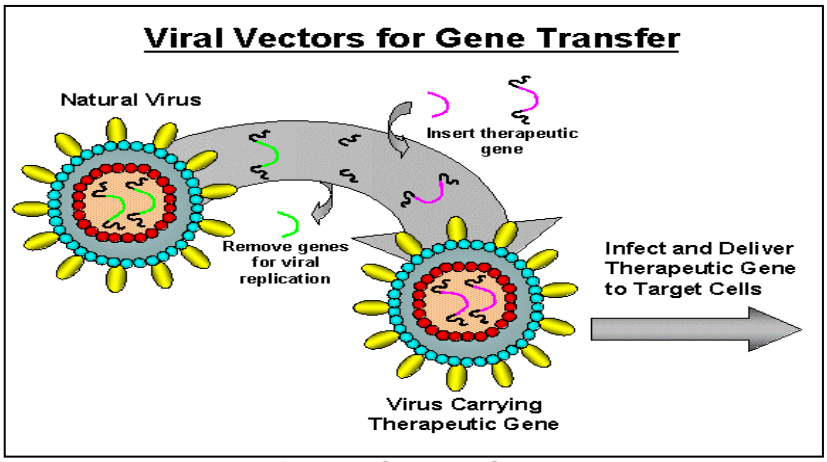

Fig.3: Viral Vector Therapy

TECHNIQUES OF GENE THERAPY

There are various therapy used in gene therapy. This include following:

1. ***Gene Augmentation Therapy:*** This is the most common technique of gene therapy. This is used to treat the disease caused by mutation that stops a gene from producing a functioning product, Such as protein. The functional gene replace the defective or nonfunctional or missing gene, So it will produce sufficient amount of protein. This is the only successful if the effects of disease are reversible or have not resulted in lasting damage to the body. E.g: cystic fibrosis, Deficiency of ADA, replacement of defective P53 gene.

2. ***Gene Inhibition Therapy:*** This therapy is appropriate for the treatment of infectious diseases, cancer and inherited diseases caused by inappropriate gene activity. The expression of gene blocked at the DNA/RNA/protein level.

3. ***Killing of specific cells:*** The aim is to insert DNA into diseased cell that causes that cell to die. This can be achieved by two ways:

a) The inserted DNA contains suicide gene that produces a highly toxic product which kills the diseased cell.

b) The inserted DNA causes expression of protein that marks the cells so that the diseased cells are attacked by the body's natural immune system.

c)

VI. Disadvantages Of Gene Therapy

1. ***Short lived nature of gene therapy:*** Some cells are rapidly divided due to that long term benefits of gene therapy is not achieved.
2. ***Immune Response:*** Transfer of therapeutic gene may increase the risk of stimulating immune response which ultimately affect the gene therapy.
3. ***Problem with viral vector:*** Virus vector used in gene therapy may lead to toxicity, inflammatory reaction in the patient.
4. ***Multiple Gene Disorder:*** Disorder that arise from single gene mutation is best candidate for gene therapy. Genetic disorder may occur because of multiple gene defects which can not be effectively treated by gene therapy.

VII. Advantages Of Gene Therapy

1. It has potentially to eliminate and prevent hereditary diseases such as cystic fibrosis.
2. It is possible cure for heart disease, AIDS and cancer.
3. It can be used to eradicate the diseases from the future generation.

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