Gene Therapy

Similar to GM crops, we can transfer a gene from human DNA or from anywhere to everywhere. Like X could be a virus or animal and Y could be bacteria or virus. Gene Therapy is a technique in which a faulty gene is removed and a new gene is added.

Definition of Gene Therapy:

- Gene therapy is based on genetic engineering in which the genome of a human being is modified either by removing a faulty gene or by replacing a faulty gene to treat a disease.
- The first experiment of gene therapy was done in 1990 by Dr French Anderson (USA). He has successfully treated SCID or bubble boy syndrome.

Additional Information

- **SCID (Severe Combined Immuno-deficiency Disease):** It is a disease in which the immunity system collapses and the boy is kept in a bubble as a bubble boy.
- **Genetic Engineering:** It is the artificial manipulation, modification, and recombination of DNA or other nucleic acid molecules in order to modify an organism or population of organisms.

Types of Gene Therapy:

(A) Based on Cell Type:

1. **Somatic Cell Gene Therapy:** In this gene therapy, genes are replaced or removed in body cells. The effect of this therapy will be limited to the person only, it will not pass on to the next generation.
2. **Germ Cell Gene Therapy:** Here, germ cell means reproductive cells. In this therapy, genes are replaced or removed in germ cells like a sperm, ovum or zygote.

- This method can give treatment to future generations.
- This is controversial and banned all over the world. (because risk is high and success rate is less).
- This is done on the experiment level.

Note: Somatic cell gene therapy is allowed, but germ cell gene therapy is not allowed.

(B) Based on Method and Approach:

- **Knockdown Gene Therapy:** In this type of gene therapy, a faulty gene is silenced. This therapy is also known as gene silencing. Example: RNA interference and antisense technology.
- **Knockout Gene Therapy:** In this type of gene therapy, the faulty gene is removed. This therapy is also known as gene removal/gene termination. Example: CRISPR Cas-9 and Transcription Activator-like Effector Nucleases (TALENs).

About CRISPR Cas-9:

- It is a genome editing tool.

Components of this tool are:

- **CRISPR Cas-9:**
  - **CRISPR:** It's a segment of DNA.
  - It stands for Clustered Regularly Interspaced Short Palindromic Repeats.

- **Cas-9 CRISPR Associated Protein-9:** It is a restriction of endonuclease enzymes.
- **g-RNA:** It is a guide RNA.

How does CRISPR Cas-9 work?

- Consider E Coli bacteria having DNA in them. When this bacteria is attacked by a bacteriophage or virus having viral DNA in it, it breaks the bacteria layer and sends viral DNA into the body of bacteria and starts infecting it.
- Then naturally, bacteria should adopt a method to kill the pathogen. Then bacterial DNA starts this process to save bacteria from the virus.
- It was discovered in 1986 that Bacterial DNA is having CRISPR cas-9. Here the viral DNA is converted to guide RNA or g-RNA through the process of transcription.
- This transcripted g-RNA is having a complementary sequence of viral DNA which has been a bacterial body and has scissor cas-9 around the sequence.
- This cas-9 attaches the viral DNA and complementary RNA joined with viral DNA and breaks down each chain.
- If the viral DNA contains a sequence of CTAAGTCCT, then g- RNA forms a sequence called GAUUCAGGA.
- Each chain breaks down like A G T is broken down as U C A.
- Here Adenine joins Thymine and Guanine joins with Cytosine but in RNA, Thymine is replaced by Uracil, hence U in the g- RNA sequence.
- Guide RNA is a guide in which the address is written and it will go to deliver it to the address hence the name guide RNA.

More about CRISPR cas-9:

- CRISPR cas-9 was first discovered in bacteria in 1986-87.
- It was thoroughly studied in 2007 and in 2012, it was used for human genome editing for the first time.
- According to the world-famous journal The Science, it was the biggest scientific achievement of the decade.
- The genetic sequence of g-RNA can be modified and changed so that it can target the faulty gene in the human genome and this can be used to remove the faulty gene. This CRISPR cas-9 technique is so important that even a noble award has been given for its discovery.
- CRISPR cas-9 is the most advanced and most accurate technology to modify or edit the genome of an organism.
- CRISPR cas-9 is naturally present in bacteria as an immunity system against bacteriophage.

Benefits of CRISPR Cas-9:

- CRISPR cas-9 will give a major boost to gene therapy because of its accuracy.
- In 2017, American doctors used this technology to edit the genome of an embryo for the treatment of the heart disease Hypertrophic Cardiomyopathy which is the thickening of the heart muscles.
- In 2018, a Chinese scientist Janikui modified the genome of an embryo with CRISPR cas-9 and made it HIV-resistant. And twin babies were born out of this experiment. This was highly controversial and heavily criticized all over the world.
- CRISPR cas-9 can also be used for genome editing in crop plants for the development of GM crops.
- Recently, USA based Sherlock Bioscience has developed a COVID test kit based on CRISPR cas-9.

Additional Benefits can be:

- Many scientists are considering that CRISPR cas-9 can be used as a tool to achieve immunity against viral diseases such as COVID-19 or HIV.
- CRISPR cas-9 can change the genome of microorganisms to make them more useful for the production of biofuels and bio-chemicals like enzymes and hormones.

Issues Related to CRISPR Cas-9:

- **Promotes the idea of a designer body.**
- **Cost issues** - available only to rich people.