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Gene Therapy

It is a collection of methods that allows correction of a gene defect that has been diagnosed in a child or embryo. This method is applied in a person with a hereditary disease. In this method, genes are inserted into a person's cells and tissues to treat a disease.

- The correction of gene defect involves delivery of a normal gene into the individual or embryo to take over the function of and compensate for non-functional gene.
- The first clinical gene therapy was done in 1990 to a 4 year old girl with adenosine deaminase (ADA) deficiency. This disorder is caused due to the deletion of the gene for adenosine deaminase that is essential for immune system to function. This defect can be treated by enzyme replacement therapy in which functional ADA is given to the patient by injection or bone marrow transplant.
- In gene therapy method lymphocytes from the blood of the patient are grown in culture medium outside the body. A functional ADA cDNA is then introduced into these lymphocystes and returned to the patient. In this method periodic infusion of such genetically engineered lymphocytes is needed. If gene isolated from bone marrow cells producing ADA is introduced into cells at early embryonic stages, it could be a permanent cure.

Molecular Diagnosis

Conventional method of diagnosis such as serum or urine analysis is not able to early detection of disease causing pathogens or virus. Following methods can be used to diagnosed earlier-

I. Recombinant DNA technology

II. Polymerase Chain Reaction (PCR)

III. Enzyme Linked Immuno-sorbent Assay (ELISA).

- Symptoms of disease appear only when the concentration of pathogen get increased significantly. Low concentration of bacteria and virus can be detected by amplification of nucleic acid by PCR. It detects the mutation in the gene in cancer patient. PCR is routinely used to detect the HIV in suspected AIDS patients. Genetic disorder can be also detected by using PCR technique.

- A single stranded DNA or RNA having radioactive molecule is allowed to hybridise to its complementary DNA in a clone of cells followed by detection using autoradiography. The clone having the mutated gene will not appear on the photographic film.
- ELISA is based on the principle of antigen-antibody interaction. Infection by pathogen can be detected by the presence of antigens like proteins, glycoproteins etc. or by detecting the antibodies synthesised against the pathogen.