Gene therapy

Gene is a hereditary unit which is transferred from parent to off spring and is responsible for determination of some characteristics of offspring's. The technique was first developed in 1972 but has, so far, had limited success in treating human diseases. Gene therapy is a technique which uses genes to prevent or treat the disease. This is an experimental technique, which in future, is expected to allow doctors to treat disease/disorder by introducing a gene into the cell of patient instead of drugs and surgery. Gene is made up of DNA. When a new DNA is introduced into the cell of patient to treat a genetic disorders the technique is called gene therapy. Diseases are mostly caused by disease-causing mutated genes, usually new DNA contains a functional gene to correct the effect of mutation caused by mutated gene. The DNA is selected carefully to correct the disease causing gene.

Gene therapy is a topic of interest now a days, as many research approaches are testing including; replacing a disease causing gene with healthy copy of genes, inactivating a mutated gene which is functioning improperly, introducing a new into the body to help fight a disease. Although it is an upbeat treatment option for many disease, the technique is still under study for its effectiveness and safety so remains risky. Currently this technique is being tested only for diseases/disorders that have no other cure like muscular dystrophy, cystic fibrosis, cancer etc.

How this technique works

Gene therapy is made to introduce new genetic material into cells to compensate for the abnormal genes or to make a beneficial protein. If a mutated gene cause a necessary protein to be missing or faulty, gene therapy is the technique which may be able to introduce a normal copy of the gene to restore the function of the protein.

A gene which is inserted directly into a cell usually does not function. Instead, gene is delivered through a genetically engineered carrier called vector. Oftenly viruses are used as vectors because they can deliver desired genes by infecting target cell. These vectors are modified so that they cannot cause disease when used inside human body. There are some viruses such as retroviruses, which are able to integrate their genetic material (which also includes new gene) into a chromosome in the human cell. Some other viruses like adenoviruses are able to introduce their DNA into the nucleus of cell, but it is not integrated into the chromosomes. The vector (vehicle) can be injected or can be given intravenously(IV) directly into the specific tissue(target tissue) in the body, where it is taken up by cells individually. Alternately, a sample of patients target cell is being removd from body and exposed to the vector in laboratory setting(invitro). The cells containing the vector (vehicle for gene) is inserted inside the body of patient. If the treatment is successful, the new gene will make the functioning protein.

There are two different types of gene therapy depending upon types of cells being treated.

1. ***Somatic gene therapy***: somatic cells are those cells of body which are not able to form sperm or egg, in other words, somatic cells are diploid cells of human body which are present all over the body e.g. bone cells, brain cells(nerve cells), muscle cells, skin cells etc. somatic gene therapy is transfer of DNA or a section of DNA to any cell of body which produce egg or sperm. In this process, effect of gene therapy are not passed from patient to children.

2. ***Germline gene therapy***: Germ cells are those cells of body which are able to reproduce. Germline gene therapy is a process which include transfer of DNA or section of DNA to cells which can produce egg or sperm. The effect of those genes will be passed from patient to children and their subsequent generations.

Gene therapy is an important topic in terms of competitive exams and in almost every exam, questions have been asked from this topic. Exams like UPSC, HAS, PCS, SSC, CDS, NDA, NAVY, Air Force, and all civil services exams include questions regarding gene therapy.

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