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Gene Therapy-A New Technology to Cure Genetic Disorders

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What are genes?:

Our bodies are made up of millions of tiny structures called cells. Inside each cell is an area called the nucleus, which contains 23 pairs of chromosomes. Chromosomes are made up of thousands of genes. Genes are tiny chemical structures. They organize the repair of damaged cells and tissues.

We have at least 30,000 different genes. They are made up of a complex chemical called DNA (deoxyribose nucleic acid). DNA controls all the processes which take place in our bodies by producing proteins which carry out the genes' instructions. When genes are damaged they may cause the production of abnormal proteins that lead to disease. It is known that cancer can occur due to changes in particular genes.

How genes cause cancer?:

The cells in our bodies are constantly dividing to make new cells to replace those that are damaged or worn out. This process is controlled by particular genes. They make sure that exactly the right number and type of new cells are made to meet our needs.

If a gene is damaged, cells may start to divide in an uncontrolled way. This may eventually lead to a cancer. The damage to the genes is called a mutation. As we learn more about genes and cancer, it seems likely that almost all cancers might be caused by faulty or damaged genes.

It is likely that several changes or mutations have to happen in the genes before a cell starts to divide abnormally and multiply out of control. This series of changes may be brought about by various factors including cigarette-smoking, environmental factors or other causes that we are not yet aware of. The faulty genes may lead to cancer in the following ways:

– The damaged gene can trigger cancerous changes in the cells.

– Some genes that can do this have been identified and are called oncogenes

– Some normal genes reduce the risk of a cancer developing, by repairing damage to other genes.

– These are called tumour-suppressing genes.

– If tumour-suppressor genes are damaged or mutated, so that they do not work, they may then allow a cancer to develop.

What is gene therapy?:

Gene therapy is the insertion of genes into an individual's cells and tissues to treat a disease and hereditary diseases in which a defective mutant allele is replaced with a functional one. Antisense therapy is not strictly a form of gene therapy, but is a genetically-mediated therapy and is often considered together with other methods.

Gene therapy also means putting genetic material (DNA) into cells so that the cells can produce proteins which they do not usually produce. These proteins will help to fight disease. Research so far has shown the following:

– Single genes can be taken from human cells and grown (cloned) in the laboratory, outside the body.

– These cloned genes can be altered to make them work differently.

– The altered genes can be put back into cells living in the body. This is usually done by inserting the gene into particular chemicals (liposomes) or cells (such as viruses which have been treated so that they are no longer harmful).

– The protein or cell used to deliver the altered gene into the body is known as a vector.

Sometimes, the genes themselves are introduced directly into the tissues. These are called naked genes.

Basic process:

In most gene therapy studies, a "correct copy" or "wild type" gene is provided or inserted