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Gene therapy

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Gene therapy may be a medical field which focuses on the genetic modification of cells to supply a therapeutic effect or the treatment of disease by repairing or reconstructing defective genetic material. the primary attempt at modifying human DNA was performed in 1980 by Cline , but the primary successful nuclear gene transfer in humans, approved by the National Institutes of Health, was performed in May 1989.The first therapeutic use of gene transfer also because the first direct insertion of human DNA into the nuclear genome was performed by French Anderson during a trial starting in September 1990. it's thought to be ready to cure many genetic disorders or treat them over time.

Gene therapy techniques

There are several techniques for completing gene therapy. These include:

Gene augmentation therapy

• This is employed to treat diseases caused by a mutation that stops a gene from producing a functioning product, like a protein.
• This therapy adds DNA containing a functional version of the lost gene back to the cell.
• The new gene produces a functioning product at sufficient levels to exchange the protein that was originally missing.
• This is merely successful if the consequences of the disease are reversible or haven't resulted in lasting damage to the body.
• For example, this will be wont to treat loss of function disorders like CF by introducing a functional copy of the gene to correct the disease.

Gene inhibition therapy

• Suitable for the treatment of infectious diseases, cancer and genetic disease caused by inappropriate gene activity.
• The aim is to introduce a gene whose product either:
inhibits the expression of another gene
interferes with the activity of the merchandise of another gene.
• The basis of this therapy is to eliminate the activity of a gene that encourages the expansion of disease-related cells.
• For example, cancer is usually the results of the over-activation of an oncogene (gene which stimulates cell growth). So, by eliminating the activity of that oncogene through gene inhibition therapy, it's possible to stop further cell growth and stop the cancer in its tracks.

DNA transferring Procedure

• A section of DNA/gene containing instructions for creating a useful protein is packaged within a vector, usually an epidemic , bacterium or plasmid.
• The vector acts as a vehicle to hold the new DNA into the cells of a patient with a genetic disorder .
• Once inside the cells of the patient, the DNA/gene is expressed by the cell's normal machinery resulting in production of the therapeutic protein and treatment of the patient's disease.

Challenges of gene therapy

• Delivering the gene to the proper place and switching it on:

it is crucial that the new gene reaches the proper cell delivering a gene into the incorrect cell would be inefficient and will also cause health problems for the patient even once the proper cell has been targeted the gene has got to be turned on cells sometimes obstruct this process by shutting down genes that are showing unusual activity.

• Avoiding the immune response:

The role of the system is to repel intruders.Sometimes new genes introduced by gene therapy are considered potentially-harmful intruders. this will spark an immune reaction within the patient, that would be harmful to them.Scientists therefore have the challenge of finding how to deliver genes without the system 'noticing'.This is usually by using vectors that are less likely to trigger an immune reaction .

• Making sure the new gene doesn't disrupt the function of other genes:

Ideally, a replacement gene introduced by gene therapy will integrate itself into the genome of the patient and continue working for the remainder of their lives.There is a risk that the new gene will insert itself into the trail of another gene, disrupting its activity.

This could have damaging effects, for instance , if it interferes with a crucial gene involved in regulating cellular division , it could end in cancer.

• The cost of gene therapy:

Many genetic disorders which will be targeted with gene therapy are extremely rare.

Gene therapy therefore often requires a private , case-by-case approach. this might be effective, but can also be very expensive.

Replacing mutated genes

Some cells become diseased because certain genes work incorrectly or not work on all. Replacing the defective genes may help treat certain diseases. as an example , a gene called p53 normally prevents tumor growth. Several sorts of cancer are linked to problems with the p53 gene. If doctors could replace the defective p53 gene, which may trigger the cancer cells to die.

Fixing mutated genes

Mutated genes that cause disease might be turned off in order that they not promote disease, or healthy genes that help prevent disease might be turned on in order that they might inhibit the disease.

Making diseased cells more evident to the system

In some cases, your system doesn't attack diseased cells because it doesn't recognize them as intruders. Doctors could use gene therapy to coach your system to acknowledge the cells that are a threat.

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