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Gene Therapy And Uses

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Introduction

Gene therapy is an experimental technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patients cells instead of using drugs or surgery.

Researchers are testing several approaches to gene therapy, including:

1. Replacing a mutated gene that causes disease with a healthy copy of the gene.
2. Inactivating, or “knocking out” a mutated gene that is functioning improperly.

Introducing a new gene into the body to help fight a disease. (1) figure 1

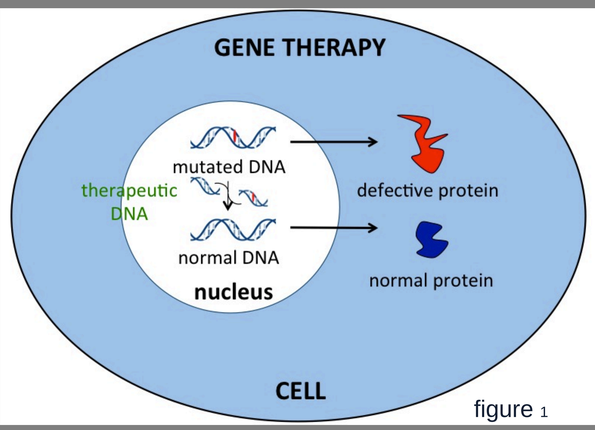


figure 1

How does work

In out-of-the-body : gene therapy, researchers take blood or bone marrow from a patient and separate out immature cells. They then add a gene to those cells and inject them into the bloodstream of the patient; the cells travel to the bone marrow, mature and multiply rapidly, eventually replacing all of the defective cells.

In the body : by a carrier called a vector is genetically engineered to deliver the gene. Certain viruses are often used as vectors because they can deliver the new gene by infecting the cell. The viruses are modified so they can't cause disease when used in people.

Some types of virus, such as retroviruses and adenoviruses.

The vector can be injected or given intravenously by (IV) directly into a specific tissue in the body, where it is taken up by individual cells. (2) figure 3

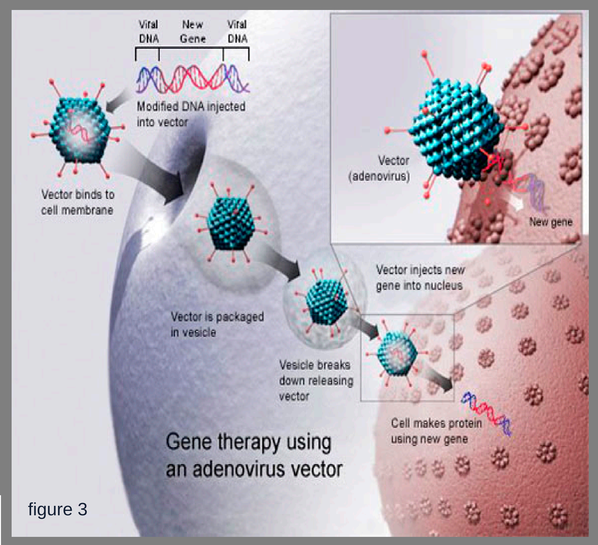


figure 3

Types

There are two basic types of gene therapy: germline therapy and somatic gene therapy.

Germline therapy

This therapy involves the modification of the genes inside germ cells (sperm or ova).
During reproduction, these gamete cells fuse to form a zygote, which would divide and pass on the modified gene into all other cells of the body during the development of offspring.
In this way, the therapy alters the genome of future generations to come. (2) figure 2

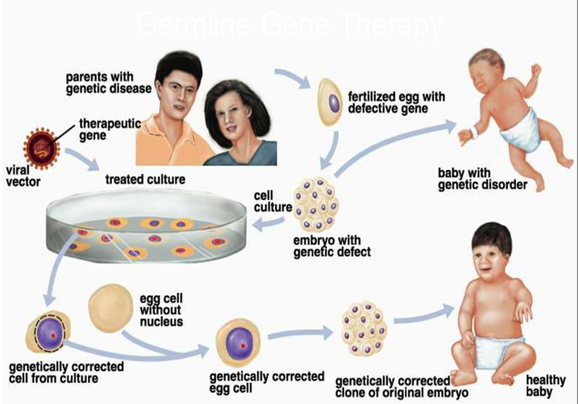


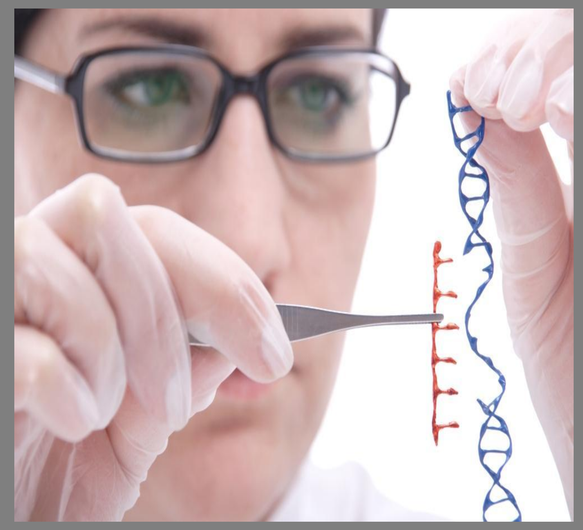
figure 2

Somatic gene therapy

Unlike germline therapy, somatic gene therapy only involves the insertion of therapeutic DNA into body cells and not the germ cells or gametes.
This means any effects of the therapy are confined to the individual being treated and are not inherited by future offspring. (2)

Example

Gene therapy techniques in cancer treatment



Harnessing the immune response

Some forms of gene therapy are designed to strengthen the body’s existing ability to target and kill cancer cells.
The role of certain cells of the immune system is to recognize and kill these cells.
Adding certain genes to a patient’s immune cells can improve their ability to find or kill certain forms of cancer.
These techniques are currently being tested in a few trials across the UK. (1)

Blocking the protection of cancer cells

Certain processes cancer cells use to survive can be blocked using gene therapy.
For example, one process called apoptosis (programmed cell death)
In cancer cells, apoptosis is stopped and the cells divide to form new cells that also contain the damage DNA.
Some gene therapy techniques are designed to prevent this inhibition of apoptosis to ensure that the cancer cells do in fact die rather than survive. (1)

Pro-drug gene therapy

Certain gene therapy techniques insert genes into cancer cells that allow conversion of an inactive drug called a pro-drug into the active form. The converting gene is given in the form of a tablet or capsule and the pro-drug is then administered. The pro-drug does not harm normal cells and only reaches cancer cells, where it is activated by the gene to become destructive.(1)

Conclusions

Gene therapy has been considered a promising technique to treat diseases but also been regarded as a risky experimentation.

If successful, this will be a medical breakthrough to cure most of the medical conditions that are not incurable or have unknown origins. It offers a lot of potential when it comes to medicine but it also is expensive.

Gene Therapy poses some downsides but overall I think it could really revolutionize the world of genetic disorders and medicine.

Reference

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2_FAQs. American Society of Gene & Cell Therapy. http://www.asgct.org/general-public/educational-resources/faqs. Accessed July 22, 2016