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english 1101

Imitation article

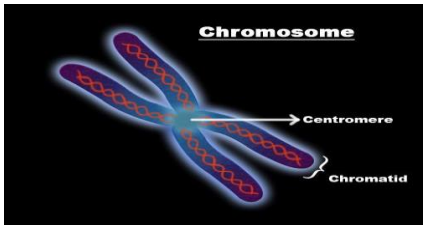
fall semester

1. What is a gene?

GENES ARE SEGMENTS OF DNA

The main point of genes is to give the steps for making **protein**. Proteins are the building blocks of the body and make major functions like tissue repair and helping blood to clot.

The **DNA** is like the language used in your genetic order. DNA is made up of components called **nucleotide bases** that are to be compared to the letters of a word. You need to have the right nucleotide bases in the correct format for the gene to do what it needs to do, which is to regulate protein. The 4 nucleotide bases in charge for gene construction are adenine (A), guanine (G), cytosine (C), and thymine (T). These nucleotides pair up with each other, A with T and C with G.



A chromosome

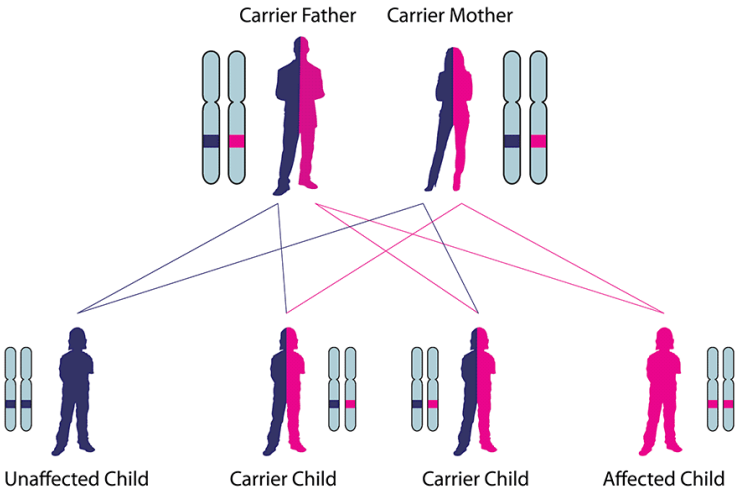
They can be found in our **chromosomes**, which contain tens of thousands of known genes. Your chromosomes lie deep within a structure called the **nucleus**, which acts as the command center of the **cells** that make up your body.

A gene

2. What causes genetic conditions?

GENETIC CONDITIONS ARE THE RESULT OF MUTATIONS

Genetic conditions are the result of mutations or diseases. These mutations are usually passed down from biological parents but can sometimes happen randomly. **High cholesterol** and some **cancers** are examples of genetic conditions.



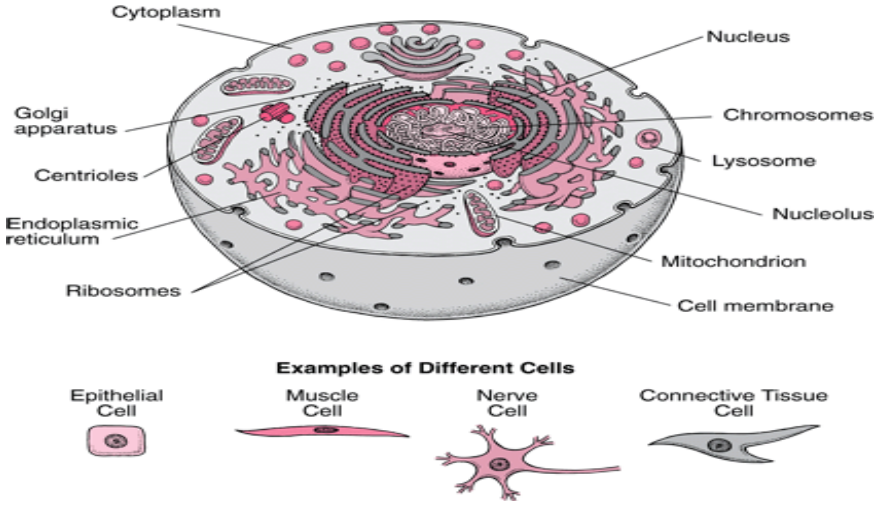
For example, In high cholesterol, If you have a close family member who has high cholesterol you're at a higher risk of having it yourself. This is usually from the passing of genes from the parents which indicates higher cholesterol levels, such as a gene that's found as a defective receptor. This is known as, **Familial hypercholesterolemia.**

Familial hypercholesterolemia is a form of passed down high cholesterol. People with this condition generally have higher cholesterol levels than people without this condition, despite if they are healthy or not. That's because people with this condition aren't able

to regulate cholesterol levels as efficiently as other people. People with familial hypercholesterolemia don't have control their levels of it even with healthy eating and exercise. Medication for this specific condition is needed to keep levels from getting too high. Having a genetic risk for high cholesterol doesn't mean you will have it too, it just means you have an increased risk.

3. What is gene therapy?

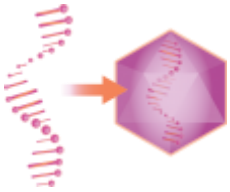
GENE THERAPY SEEKS TO ADDRESS GENETIC MUTATIONS



Inside a cell

gene therapy is being researched in clinical studies as a novel method that attempts to use **genes** to treat or prevent disease.

METHODS OF GENE THERAPY BEING EXPLORED



Gene transfer therapy

There are a couple of different things being tested for gene therapy. Gene therapy is trying to attempt to fix or change mutated genes. Getting rid of dysfunctional gene or replacing with a functional gene to help the body produce the affected protein.

Ex vivo gene therapy

- A functioning gene is inserted into a cell with the hope that it will operate in place of the mutated gene using the gene therapy method called gene transfer.Viral, chemical and physical methods for moving genes are being investigated. Upon systemic injection, the transition of the new gene occurs inside the body (in vivo), often using an IV infusion. The original genetic material is meant to remain unchanged in the chromosomes. It means that the mutated gene still exists and can still be transferred to the offspring of an individual

- The process takes place outside the body in ex vivo gene therapy, a form of cell-based therapy. First, through a biopsy, the infected cells are removed from the body. Functional genetic material is injected into the cells in the laboratory and then returned to the body of the human.

Gene editing

- The idea is to make changes to the original DNA in gene editing. This technique allows restoring the original DNA or adding new DNA at a particular location. Zinc finger nucleases and CRISPR (regularly clustered short palindromic repeats) are experimental methods for gene editing.

4. How is gene therapy designed to work?

GENE TRANSFER

This gene therapy technique, currently undergoing clinical trials under many different conditions, including hemophilia A and B, aims at inserting a working gene that can instruct the body to produce the protein required.

BUILDING A TRANSPORT VEHICLE

It is now essential to bring the functional gene into the body. A transportation vehicle is created from a neutralized virus to protect the gene and allow it to be incorporated into the body.

The vehicle of transport produced from a neutralized virus is called a vector of therapy. The neutralized virus is produced by extracting the viral material inside a laboratory, leaving behind an empty shell of protein.

Adenoviruses, adeno-associated viruses (AAVs), and lentiviruses are the viruses used in gene transfer.

DELIVERING THE FUNCTIONAL GENE

Inside the transport vehicle, a functional gene is put and large numbers are supplied through an intravenous infusion. Having a working gene inside the transport vehicle, it is considered a therapeutic vector. The therapeutic vector is designed to target a suitable tissue for the active gene. The liver is the focus in hemophilia A and B because it can make the proteins required for blood clot.

When the functional gene is inside the AAV, additional DNA is included to allow it to function and promote protein production only within the targeted cells. To what extent the AAV will transfer the functional gene to the other tissues of the body, work is ongoing.

5. What are the goals of gene therapy research?

Typically we take medicine to help us feel better, physically or mentally. Clinical trials are underway for gene therapy to determine the risk and whether gene therapy could reduce or eliminate the need for ongoing treatment and chronic disease burdens for some people. It is important to remember that gene therapy's long-term effects, including length of effect, are also being studied and have not yet been determined.

SO, WHAT DO WE HOPE FOR FROM GENE THERAPY RESEARCH?

Genes are the instructions for the body

In an individual's genetic instructions, gene therapy seeks to address specific mutations, allowing the body to generate the proteins it requires.

PRODUCING PROTEIN

One aim of gene therapy is to insert functional genes into the body to try to eliminate the mutations that are responsible for genetic conditions.

POTENTIALLY REDUCING THE NEED FOR TREATMENT

Work on whether gene therapy will help the body produce the proteins it needs is continuing.

POTENTIALLY ELIMINATING OR REDUCING SYMPTOMS

Clinical trials evaluate whether gene therapy can eliminate or decrease routine management of disease. This in turn could potentially reduce a disease's physical, psychological, and emotional burden. Although gene therapy may not be able to address pre-existing damage, it may be able to mitigate progression of any existing damage. All of these priorities as well as the risks of human clinical trials are currently being assessed.

6. What are the risks of gene therapy?

Most types of gene therapy are being tested only in adults, at least initially, and in people with certain antibodies or other pre-existing conditions, certain gene therapies will not work. Gene therapy is also fraught with dangers. In many fields, current clinical trials are performed across people to determine the potential risks of gene therapy treatment.

With any virus, the immune system of the body will react as if it were an intruder to the newly introduced therapeutic vector. An immune system reaction can result in inflammation and other serious risks An immune reaction could also reduce the effectiveness of gene therapy. That's why patients are often tested for prospective gene therapy to see if they have antibodies to a particular virus.

While the purpose of using a particular vector is to direct the new gene to a specific type of tissue, viruses that affect other non-target cells, potentially causing damage or additional infection or disease.

Vector particles can be released from the body of the recipient after delivery of the gene therapy. This can occur by feces, urine, saliva, and other excreted body fluids, called vector shedding. Shedding raises the possibility of passing on the remaining materials (through close contact) to untreated individuals. The importance of gene therapy is currently being tested in clinical trials

If gene therapy may adversely affect the health of the targeted organ or tissue is tested with long-term studies Gene therapy may lead to too much protein being produced. The result of this overproduction, or overexpression, can vary depending on the type of protein being produced. Gene therapy may not function at all for some patients.