Gene Therapy- Science method

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Gene therapy is an experimental procedure to treat or prevent disease. It would allow doctors to insert a gene into a patient's cells to treat their medical issue instead of relying on surgery or drugs. There are three ways to use this technique, replacing a mutated gene that causes disease with a healthy copy of the gene, or inactivating or knocking out mutated gene that is not functioning correctly.

Despite being a promising technique, more research needs to be conducted to better understand this technique.

Currently, scientists are using this method to treat diseases that have no other cure. If successful, gene therapy has the potential to save lives.

Unfortunately, outside factors such as money may interfere in the process of a child being cured.

Gene therapy has the potential to revolutionize modern medicine. It has already proven to be successful for certain diseases.

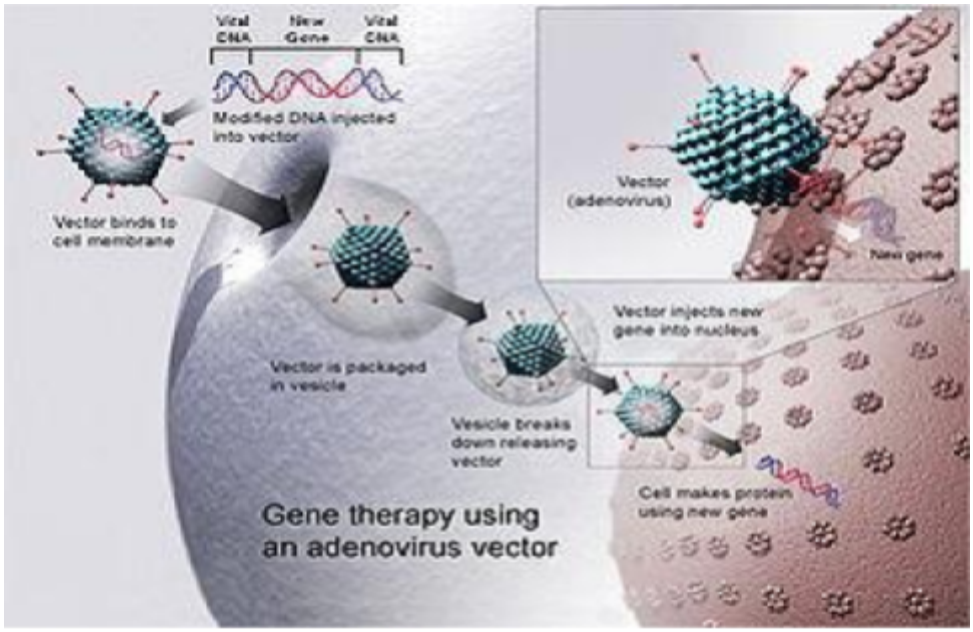
For example, doctors used gene therapy to avoid a fatal degenerative brain disease, something experts thought was impossible.

This brain disease known as adrenoleukodystrophy (ALD) causes nerve cells in the brain to die, causing children to lose the ability to talk or walk in a few years.

Therefore, these children are unable to eat without a feeding tube, see, hear or think. Sadly, they die within five years of the diagnosis.

Before gene therapy, the only treatment for ALD was a bone marrow transplant or a transplant with cord blood. However, these procedures are extremely risky with mortality rates being as high as 20 percent, and those who survive have other lifelong disabilities.

A study was conducted by the New England Journal of Medicine reported that gene therapy can cure ADL with no side effects.



The study included 17 boys from age 4 to 13 because the disease affects mostly males. Two years later 15 of the boys were functioning normally without any side effects.

Of the two that remained, one's disease progressed too fast for the gene therapy to work.

The other dropped out of the experiment to receive a bone marrow transplant, but died due to complications during surgery.

Gene therapy pioneers like Dr. Theodore Friedman stated, "The study opens new avenues for using gene therapy to treat brain diseases. Many think the central nervous system is intractable and unapproachable," he said.

This study proves them wrong." Other pioneers such as Dr. Amber Salzman, began the research for gene therapy when her nephew was diagnosed with ALD in 2000.

With the help of Dr. Tachi Yamada, Dr. Salzman found that a disabled form of HIV can be used to safely insert genes into human cells.

For another example, gene therapy is used to treat the blood cancer. Blood cancer affects the production and function of blood cells.

Most of these cancers start in bone marrow, where blood is made. The treatment for these cancers is chemotherapy and radiology. But chemotherapy and radiotherapy drugs only kill the cells present in the body, when the drugs cleared by the body the drugs cannot kill the cancer cells.

The professor Emma Morris, professor of Clinical Cell and Gene therapy, talk about the treatment of blood cancer by using gene therapy.

They use patient immune cells, by genetic engineering they find the cancer cells and killed them.

Despite the progress, treatment via gene therapy may not be an option for many due to the high price of the procedure.

Dr. David A. Williams, chief scientific officer at Boston Children's Hospital and a principal investigator of the new study, expects the price to be similar to the hundreds of thousands of dollars it costs for a bone-marrow transplant.

He states, "The expected prices are absolutely crazy." In fact, the price may be the highest for any treatment.

For example, the price for an approved therapy procedure for spinal muscular atrophy known as Zolgensma can cost $2.1 million.

Spinal muscular atrophy affects the motor nerve cells in the spinal cord, causing the body to lose physical strength and eventually robbing people of their ability to walk, talk, swallow and breathe. To make the treatment more affordable, Novartis, the company the procedures the gene therapy, announced that it would allow insurers to pay $425,000 yearly for five years.

Despite the high price, David Lennon, the President of AveXis, the Novartis unit developing Zolgensma, said the company set the price based on a 50 percent discount off what Novartis believed it is worth.

In an interview, he stated that “We believe this is a balanced approach. We do have high hopes that this is an extremely durable product and really does have the potential to provide a lifetime of benefit.”

Tina and Torence Anderson’s baby, Malachi, was diagnosed with spinal muscular atrophy when he was nearly four months old.

They enrolled their child in a clinical trial for Zolgensma. Malachi is now four years old and is able to be active and eat on his own. Ms. Anderson said that Malachi is “like a normal toddler, he just can’t stand or walk.”

She also discussed how many parents are hopefully of their child getting better because of the treatment, but worry about how they will pay for it.

Hence, the real question becomes who is going to be for all the cost if may parent won’t be able to pay for it.

Even if the treatment can be paid for, it is not guaranteed that gene therapy will cure the child. Researchers at St. Elizabeth's Medical Center here failed to report the death of a patient in gene therapy experiments.

The Food and Drug Administration reported that the hospital must stop the experiment and address the issues that it is causing among patients.

Jack Cumming, the President of the company that is sponsoring the research, finally acknowledged that there has been problems in the gene therapy experiment.

In the experiment, a gene that makes a substance called V.E.G.F. (for vascular endothelial growth factor) was injected directly into the hearts of patients with blocked heart vessels.

The gene would then hopefully create the natural substance to make blood vessels grow.

However, the FDA found that researchers violated many of the regulations. For example, one patient died two months after receiving the gene therapy, but researchers failed to report the death to the agency.

In another situation, a patient was included in the trial despite not meeting the criteria for it. The hospital and researchers have only 15 days to inform the FDA of how they will resolve the problems.

If they are unable to do so, then the FDA can ban the researchers from conducting future experiments with federal money.

Gene therapy is a promising technique to treat genetic diseases. It has the potential to save lives and has lower complications compared to current available treatments.

Despite this, the cost of gene therapy can prevent children from getting treated. The price is too high for parents to pay and insurers might not be willing to pay for it either. Moreover, more research still needs to be done within the field.

Researchers still need to find cures for other genetic disorders. In addition, gene therapy has not been successful for everyone which means it does not guarantee that the patient will be cured. Even the clinical trials are risky as patients have died from improper regulations.

Therefore, in order to save the lives of children, researchers and manufacturers have to come together and make a product that has minimum side effects and is reasonable in price.

It is the only way to ensure that these children have the option to live a healthy life.