Trichromatic West Inc.

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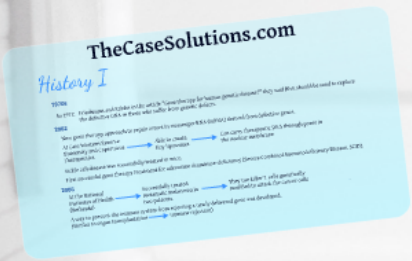

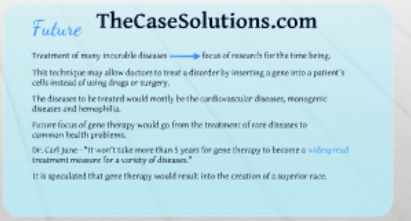

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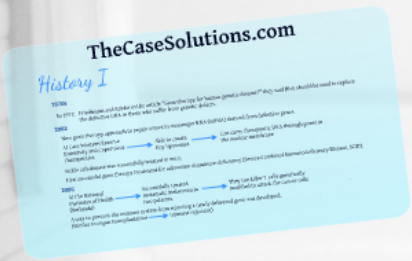

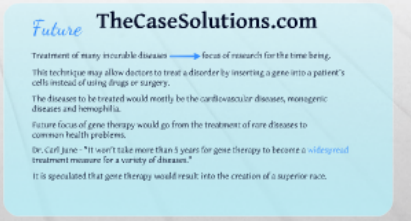

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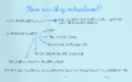
What is Gene Therapy?

Experimental technique
- Uses genes to treat or prevent disease.
- Approaches being tested
  - Replacing a defective gene with a healthy copy of it.
  - Inactivating genes that are functioning improperly.
  - Introducing new genes to help fight a disease.



Gene therapy involves
- Insertion of a functioning gene into cells to correct a cellular dysfunction
- To provide a new cellular function.

Especially successful in
- Treatment of combined immunodeficiency syndromes
- Showing lasting
- Remarkable therapeutic benefit

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History I

1970s

In 1972: Friedmann and Roblin in the article "Gene therapy for human genetic disease?" they said DNA should be used to replace the defective DNA in those who suffer from genetic defects.

2002

New gene therapy approach to repair errors in messenger RNA (mRNA) derived from defective genes.

At Case Western Reserve University and Copernicus Therapeutics → Able to create tiny liposomes → Can carry therapeutic DNA through pores in the nuclear membrane

Sickle cell disease was successfully treated in mice.

First successful gene therapy treatment for adenosine deaminase-deficiency (Severe Combined Immunodeficiency Disease, SCID).

2006

At the National Institutes of Health (Bethesda) → Successfully treated metastatic melanoma in two patients. → They use killer T cells genetically modified to attack the cancer cells

A way to prevent the immune system from rejecting a newly delivered gene was developed.
(Similar to organ transplantation → immune rejection)

Types

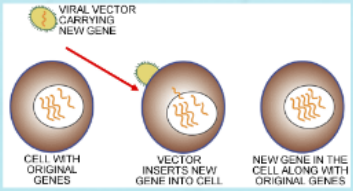
There are two different techniques:

Germ line gene therapy

In this type, germ cells are modified by the introduction of functional genes, which integrate into their genome. Because of this, it can be passed down to future generations because it would be hereditary. It could be very effective in curing hereditary diseases, but there are a lot of legal restrictions for the use in humans.

Somatic gene therapy

In this type, genes are transferred to a patient, into its somatic cells. This type isn't hereditary, and only affects to the patient. This option is available for those who want to avail the benefits of gene therapy but don't want the genetic changes to pass on to the next generations.



Future TheCaseSolutions.com

Treatment of many incurable diseases → focus of research for the time being.

This technique may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery.

The diseases to be treated would mostly be the cardiovascular diseases, monogenic diseases and hemophilia.

Future focus of gene therapy would go from the treatment of rare diseases to common health problems.

Dr. Carl June - "It won't take more than 5 years for gene therapy to become a widespread treatment measure for a variety of diseases."

It is speculated that gene therapy would result into the creation of a superior race.

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Applications

In 2011, they had already treated more than 5000 patients in 12 years worldwide.

- Severe Combined Immunodeficiency Disease (SCID) Adenosine Deaminase Deficiency (ADA). Patients die if untreated.
- Ornithine transcarbamylase (OTC) deficiency Urea cycle disorder → ammonia accumulates in the blood and travels to the brain (coma, brain damage or death).
- Cystic fibrosis
- AIDS
- Familial Hypercholesterolemia (defective cholersterol)
- Cancer
- Blindness
- Parkinson

SUCCESS →

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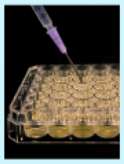
Problems

Scientific Issues

There is no guarantee that the viral enzyme that is responsible for this step will be able to introduce the correct gene at the specific point in the host chromosome. In case, there is an error in this process, it would result in error in the genetic makeup of the cell and can result in serious disorders.

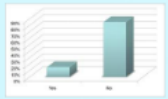
Moreover, the body's immune system may destroy the vector as it may perceive the carrier as a foreign body. Due to this reason a patient may need to undergo multiple therapy treatment processes. But once the immune system is triggered by a foreign body, it attacks the foreign body more aggressively when it invades the body next time.



Ethical Issues

"Gene therapy will make the rich, richer and the poor, poorer."

Given the technology involved, it is obvious that gene therapy treatment will be expensive. It will be just the rich who would be able to afford its benefits.



Religious Issues

Manipulating genetic makeup of man is absolutely unacceptable by those with strong religious beliefs. It's like questioning God's will or in other words, 'playing God', so it is perceived as sinful.

The therapeutic advantage of gene therapy is blessing for mankind. However, unless the techniques of gene therapy are perfected, gene therapy pros and cons will keep fueling the controversy.

Should we deny mankind the revolutionary scientific development that would bring an end to numerous incurable diseases?

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