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What is Gene Therapy?

Experimental techniques

- The gene is viral or plasmid based
- A specific targeting vector
- Producing a defective gene and the healthy copy of it
- Replacing genes that are functioning improperly
- Introducing new genes to help fight a disease

Gene therapy involves

- Insertion of a functional gene into cells to correct a cellular dysfunction
- It provides a new cellular function
- Production of a normal protein molecule to replace a missing or defective protein

Especially successful in

- Hematology
- Neurobiology
- Immunology



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Problems

Health Issues

• Gene therapy is still in its early stages and many of the diseases it is being used to treat are rare and difficult to study.

Ethical Issues

• Gene therapy is still in its early stages and many of the diseases it is being used to treat are rare and difficult to study.

Cost Issues

• Gene therapy is still in its early stages and many of the diseases it is being used to treat are rare and difficult to study.

Bibliography

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1. [Gene Therapy: A New Approach to Treating Disease](#)

2. [The History of Gene Therapy](#)

3. [Gene Therapy: A New Approach to Treating Disease](#)

4. [Gene Therapy: A New Approach to Treating Disease](#)

5. [Gene Therapy: A New Approach to Treating Disease](#)

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History I

1970s

• The first gene therapy trial was conducted in 1970. It was a failed attempt to treat a patient with a rare form of cancer.

1980s

• The first successful gene therapy trial was conducted in 1980. It was a failed attempt to treat a patient with a rare form of cancer.

1990s

• The first successful gene therapy trial was conducted in 1990. It was a failed attempt to treat a patient with a rare form of cancer.

2000s

• The first successful gene therapy trial was conducted in 2000. It was a failed attempt to treat a patient with a rare form of cancer.

2010s

• The first successful gene therapy trial was conducted in 2010. It was a failed attempt to treat a patient with a rare form of cancer.

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Applications

In 2015, they had already treated more than 5000 patients in 31 years worldwide.

- Severe Combined Immunodeficiency Disease (SCID)
- Adenosine Deaminase Deficiency (ADA)
- Hemophilia
- Sickle Cell Anemia
- Cystic Fibrosis
- HIV
- Leukemia
- Cancer
- Parkinson

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Types

There are two different techniques:

Gene gene therapy

In this type, gene cells are modified by the introduction of functional genes, which insert into their genomes. Because of this, it can be passed down to future generations instead of needing to be replaced. It can be very effective against harmful viral diseases, but there are a lot of legal restrictions for its use in humans.

Somatic gene therapy

In this type, gene cells are transferred to somatic cells in the body. This type is temporary and only affects the patient. This option is available for those who want to avoid the benefits of gene therapy but don't want the genetic changes to pass on to their generations.




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Future

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 disease, monogenic diseases and hemophilia.

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

Dr. Cliff 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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What is Gene Therapy?

Experimental techniques

- The gene is viral or plasmid based
- A specific target is used
- Producing a defective gene inside the cell itself
- Replacing genes that are functioning improperly
- Introducing new genes to help with a disease

Gene therapy involves

- Insertion of a functional gene into cells to correct a cellular dysfunction
- It provides a new cellular function
- Production of cultured stem cells to carry out a process

Especially successful in

- Hematology
- Immunology
- Gene therapy for inherited blindness



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Problems

Health Issues

• Gene therapy is still in its early stages and is not yet widely available.

• It is expensive and can be risky.

Ethical Issues

• Gene therapy raises ethical concerns, particularly around the potential for genetic enhancement.

• There are concerns about the safety and efficacy of the procedure.

Technical Issues

• Delivering the gene to the target cells is a major challenge.

• The immune system can attack the viral vector used to deliver the gene.

Bibliography

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History I

1970s

• The first gene therapy trial was conducted in 1970, using a retrovirus to deliver a functional gene to a patient with a rare form of blindness.

1980s

• The first gene therapy trial for a genetic disease was conducted in 1980, using a retrovirus to deliver a functional gene to a patient with a rare form of blindness.

1990s

• The first gene therapy trial for a common disease was conducted in 1990, using a retrovirus to deliver a functional gene to a patient with a rare form of blindness.

2000s

• The first gene therapy trial for a common disease was conducted in 2000, using a retrovirus to deliver a functional gene to a patient with a rare form of blindness.

2010s

• The first gene therapy trial for a common disease was conducted in 2010, using a retrovirus to deliver a functional gene to a patient with a rare form of blindness.

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Applications

In 2015, they had already treated more than 5000 patients in 21 years worldwide.

- Severe Combined Immunodeficiency (SCID)
- Adenosine Deaminase Deficiency (ADA)
- Hemophilia A
- Sickle Cell Disease
- Cystic Fibrosis
- HIV
- Leukemia
- Cancer
- Parkinson

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Types

There are two different techniques:

Gene gene therapy

In this type, gene cells are modified by the introduction of functional genes, which are inserted into their genomes. Because of the difficulty of introducing genes into their genomes, it is not yet possible to use this technique for the treatment of many common diseases, but there are a lot of legal restrictions for its use in humans.

Somatic gene therapy

In this type, gene cells are modified by the introduction of functional genes, which are inserted into their genomes. Because of the difficulty of introducing genes into their genomes, it is not yet possible to use this technique for the treatment of many common diseases, but there are a lot of legal restrictions for its use in humans.




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Future

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 disease, monogenic diseases and hemophilia.

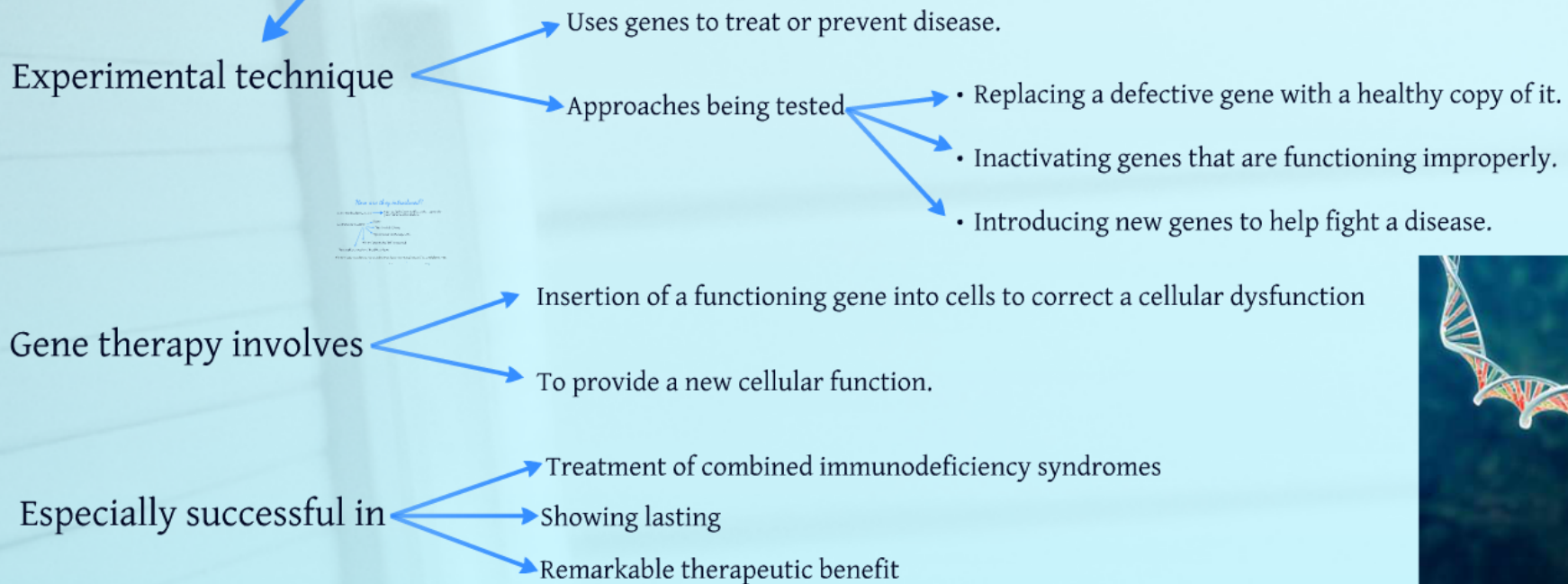
Future focus of gene therapy would go from the treatment of rare diseases to common health problems.

Dr. Cliff June: "It won't take more than 5 years for gene therapy to become a widely used 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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What is Gene Therapy?



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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

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There are two different techniques:

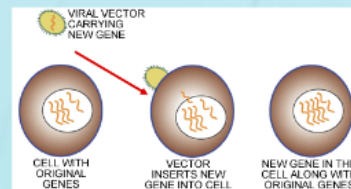
Germ line gene therapy

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 cholesterol)
- Cancer
- Blindness
- Parkinson

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Advantages & Disadvantages



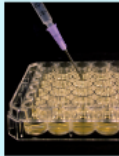
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Problems

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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