Gene Therapy

Introduction:

Gene therapy is an experimental technique that uses genes to treat or prevent disease. This can be used to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery. The approaches to gene therapy currently tested are:

-Replacing a mutated gene that causes disease with a healthy copy of the gene.

-Inactivating, or "knocking out," a mutated gene that is functioning improperly.

-Introducing a new gene into the body to help fight a disease.

This technique has the potential to cure Cancer, Central Nervous system disorders and immune system diseases.

Risks:

- **Unwanted immune system reaction.** Your body's immune system may see the newly introduced viruses as intruders and attack them. This may cause inflammation and, in severe cases, organ failure.
- **Targeting the wrong cells.** Because viruses can affect more than one type of cells, it's possible that the altered viruses may infect additional cells — not just the targeted cells containing mutated genes. If this happens, healthy cells may be damaged, causing other illness or diseases, such as cancer.
- **Infection caused by the virus.** It's possible that once introduced into the body, the viruses may recover their original ability to cause disease.
- **Possibility of causing a tumour.** If the new genes get inserted in the wrong spot in your DNA, there is a chance that the insertion might lead to tumour formation.

Cases where it went wrong:

Gene Therapy Trial for OTC deficiency (**Ornithine transcarbamylase deficiency**). **Ornithine transcarbamylase deficiency** is when toxic levels of ammonia build up in the body. Jesse Gelsinger had a mild form of this disease so he voluntarily participated in the trial. The after effects severe, his immune system rejected the therapy and there were multiple organ failures. He had died 4 days after being injected.

Similarly, Jolee Mohr had died from a gene therapy trial for Arthritis. She acquired a fungal infection during the trial. The level of immune system suppression provided by the gene therapy heavily impacted her dead but so did her arthritis drug Adalimumab Humira. For this reason officials had decided to continue the trials.

Conclusion:

The treatment is highly effective or at least has the potential to be using evidence from clinical trials. However, we should tread carefully with advancements to prevent unfortunate cases like the Thalidomide drug. The fear of long term effects causing more harm than good could hinder our species evolutionary development. If this technique fails it could put us as a species at a disadvantage.

How does it work?

Genes that are inserted directly into the cell do not function because of our immune system's response. So a vector is engineered to deliver the gene to the cell and not cause a disease. An example would be an adenovirus vector. They introduce their DNA into the nucleus of the cell but do not integrate it into the chromosome. However a retrovirus can integrate their DNA containing the new gene into a chromosome of the human cell.

To test the methods effectiveness it can be injected into a sample of the patients cells and

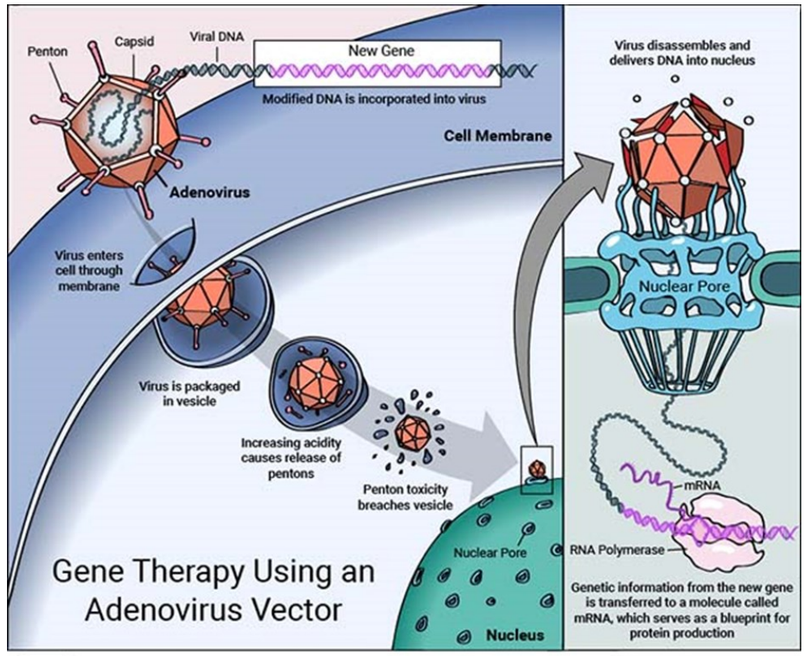
Penton protein capsid containing viral DNA with new gene enters cell via endocytosis.

Vesicle forms around capsid

Acidity of cell breaks down structure and releases penton

Penton breaks vesicle down.

Virus travels to nuclear pore and releases DNA into the nucleus for transcription.



Other uses for Gene Therapy:

Could plastic surgery evolve?

There is a possibility that gene therapy could change our phenotype. Although it will be most likely expensive this could be a safer option than undergoing surgery to change appearance. It also has its benefits for well being by boosting individuals self esteem and confidence

A starting stage could be using gene therapy as a means for speeding up healing post surgery. Recovery can be a lot shorter if mitosis is sped up but controlled.

Could we direct our own evolution?

If we become successful in editing phenotype and genotypes, could this then be inherited by our children? Genetic diseases could be removed completely if that were the case. Our chances of survival as a race would increase. But since there is no substantial long term evidence, this technique seems too risky to delve into just yet. I think with all the regulations and safety precautions, advancement in gene therapy will be very limited and controlled.