

# Genetic Diagnostics and Gene Therapy

**Genetic Diagnostics** is an assumption made about genetic defects before the action and process of genetic testing is performed



**Genetic Testing** must be performed before genetic therapy because they first must diagnose genetic mutation or genetic flaws. Genetic testing is the next step where they take a sample of the person's blood, hair skin cells, etc. The samples are taken with a cotton swab and are then observed by technicians in a lab looking for changes in the DNA or proteins.

**Gene Therapy** is a way to fix a genetic problem, it is an experimental method that uses genes to treat or prevent disease. Gene therapy is when you make a correct copy of a defective gene and re-inject it into a person. The gene is transmitted through a vector which is a way to insert the new genes into the patient.

## Gene Therapy: Vectors

When delivering copy genes to the defective gene, the new gene must be delivered in a vector which is used as a delivery vehicle. Vectors must be customized for each specific disorder.

Although there are different kinds of vectors, scientists like to use commonly use viruses, they modify the virus, to ensure that injecting it won't make people sick. Viruses are good at targeting cells but can only carry a limited amount of genetic information.



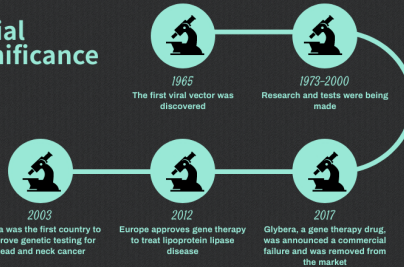
**Gene Delivery**, there are two ways to deliver a vector into the patient's body. First is *in vivo*, when the vector is injected directly into the patient. Second is *ex vivo*, which is when scientists remove the cells from the patient and then inject the vector. Letting them activate outside of the body which is less likely to trigger an immune response then reinjecting the new cells into the patient.



## History and Development

In 1972 a seminal paper was published proposing whether genetic therapy could cure genetic diseases, a few US scientists looked into what measures they would have to take and found a few roadblock due to a lack of scientific understanding. In the year 1990, they had done enough research to launch a human trial. The first viral vector was extremely successful and was a huge milestone for their research and development. In 1999 one of the experiments resulted in a research participant's death due to the virus affecting their immune system. Scientists realized that viral vectors can have more harmful effects and decided to proceed with more caution for further research.

## Social Significance



## Conclusion

Some of the **Greatest Advancements in Gene Therapy** was in 1972 when an article was published suggesting that viruses can be used to carry genetic information. Another was in the 1980s when the retrovirus was discovered and proved to be important in treating leukemia. Then, in 1990 when the first-ever patient was successfully treated for SCID by gene therapy. Most recently, in 2015 the first successful use of gene therapy treated and prevented baby dying from leukemia.

**Some of the Best Uses for Gene Therapy** is for treating immune deficiencies, hereditary blindness, Hemophilia, Blood disease, Fat metabolism disorder, Cancer and Parkinson's disease. Every disease has a genetic factor; however, these diseases have been researched and have had successful results.

**Gene therapy is Continuing to Change the Future** by providing cures for diseases that used to be viewed as untreatable. Every year scientist are continuing to expand their knowledge and data on gene therapy. The goal for gene therapy is to fix problems that occur when the virus affects the patient negatively. In conclusion, gene therapy is slowly making its way to changing the medical field forever.

