

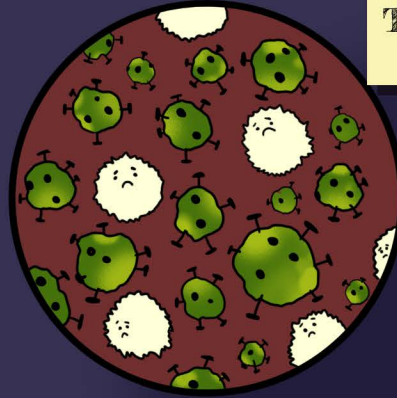
What is Gene Therapy?

written by Zoe Simonson
Illustrated by Monica Keszler

Gene therapy is a technique used to treat genetic diseases. In this process, new genes are added to a patient's cells to replace a missing or malfunctioning gene.



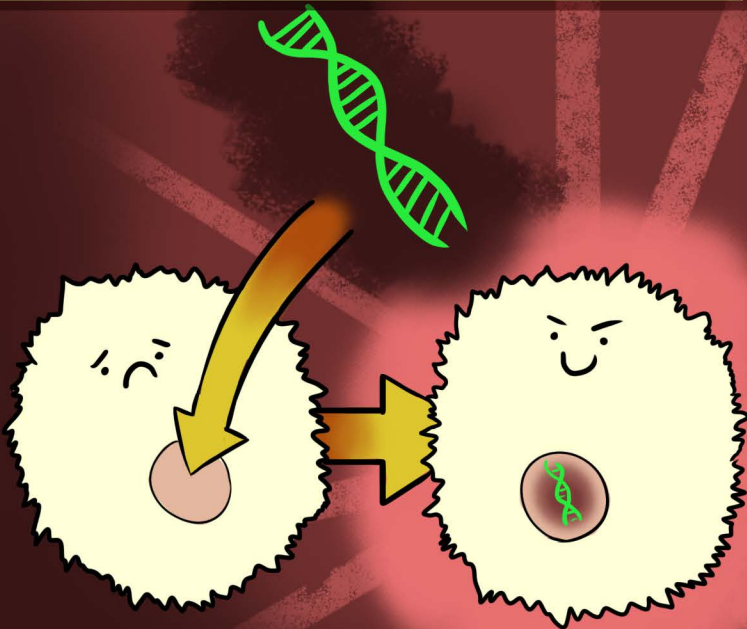
The first trial of gene therapy was in 1990.



A 4-year old girl had ADA, a genetic disease that affected her white blood cells and made it hard for her to fight off infections.



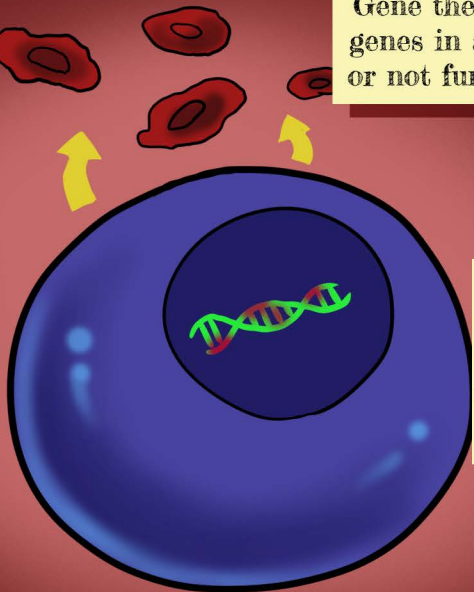
In treatments, her white blood cells were given a new gene that allowed them to function properly.



The treatments were successful and she is now leading a normal, healthy life.

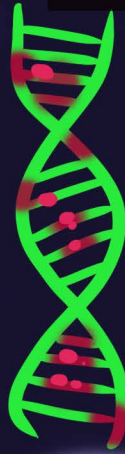


Gene therapy is used when genes in a cell are missing, or not functioning properly.



Missing or faulty genes can cause genetic diseases, such as Parkinson's or hemophilia.

In gene therapy, cells receive a new, functioning gene

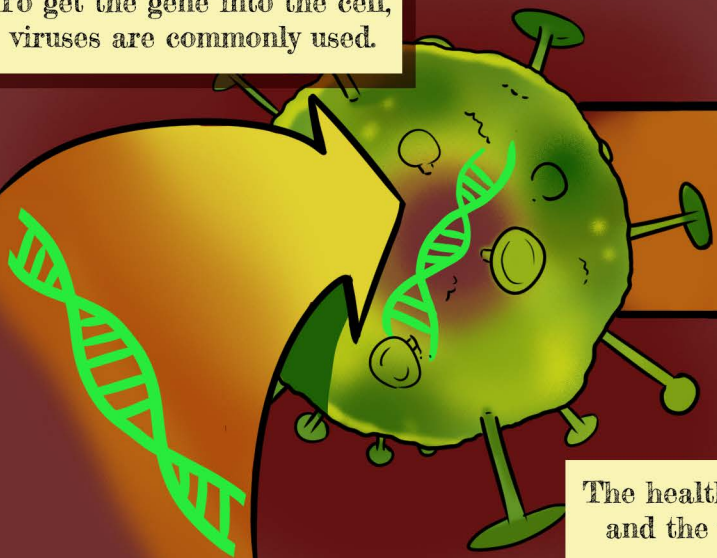


OLD

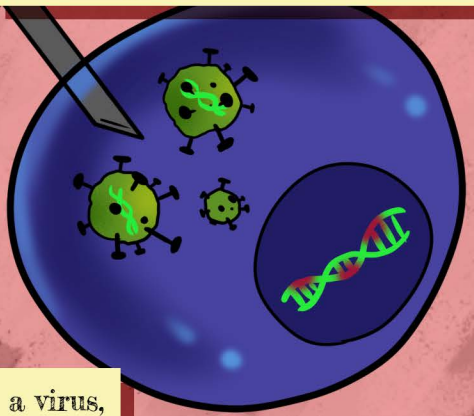


NEW

To get the gene into the cell, viruses are commonly used.

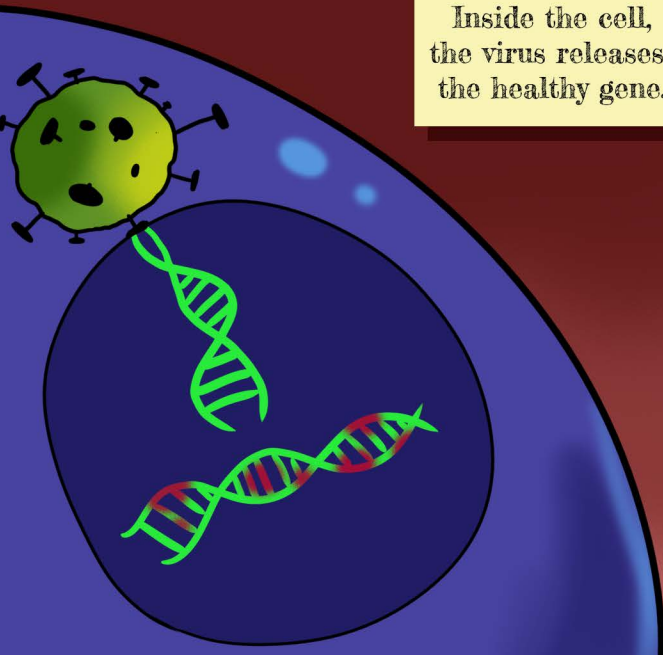


Viruses work well because they can recognize specific cells and deliver genetic materials to them

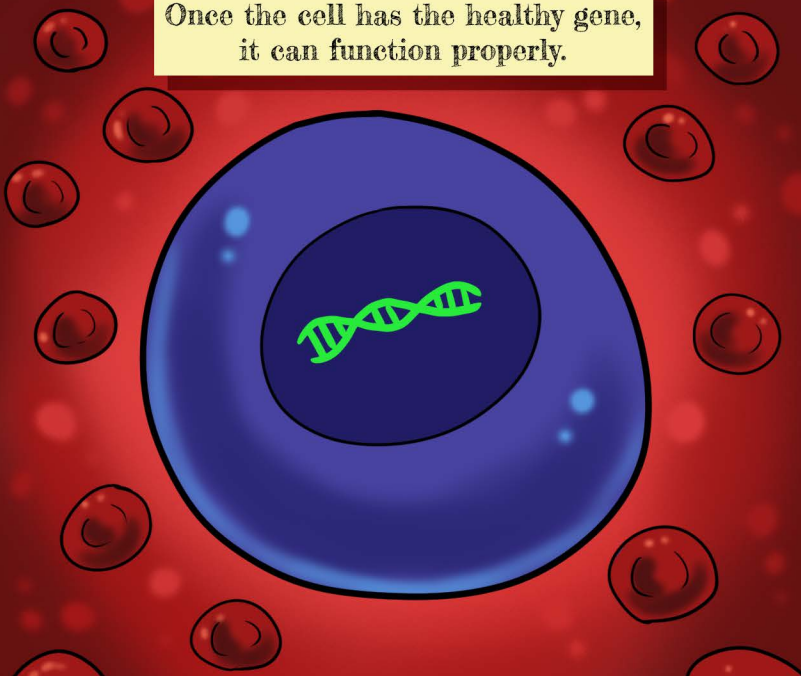


The healthy gene is injected into a virus, and the virus is injected into the cell.

Inside the cell, the virus releases the healthy gene.



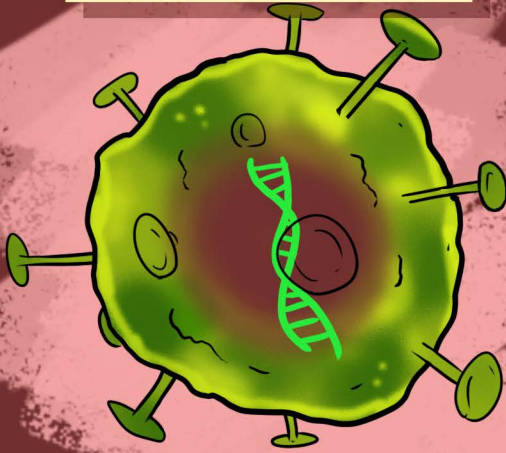
Once the cell has the healthy gene, it can function properly.



There are two methods of gene therapy.

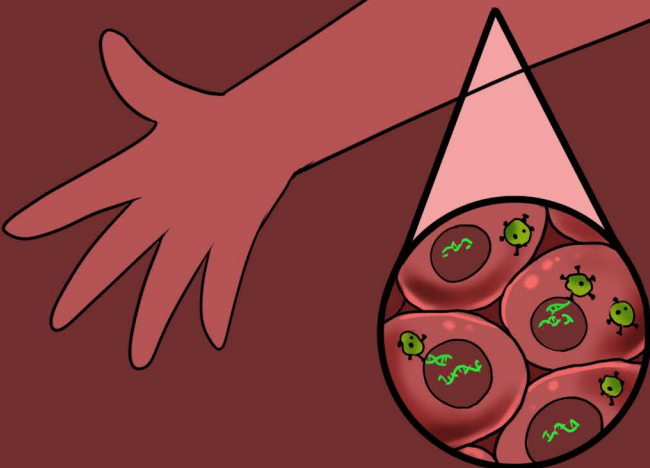
IN VIVO

The first method happens inside of the body.



The healthy gene is placed inside of a virus.

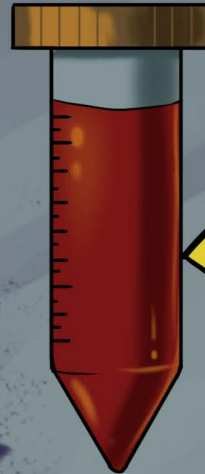
Then, the virus is injected directly into the part of the body that contains the defective cells.



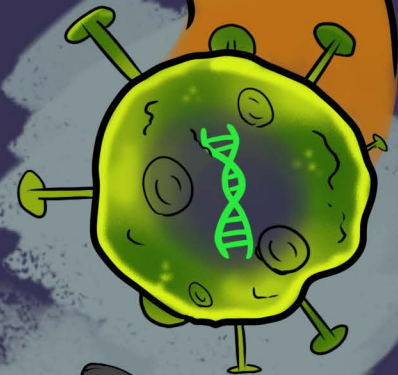
EX VIVO

The second method happens outside of the body.

Blood or bone marrow is taken outside of the body.



Then, a virus is used to transfer the healthy gene into the cells.



Finally, the cells with the new, healthy gene are injected back into the body.

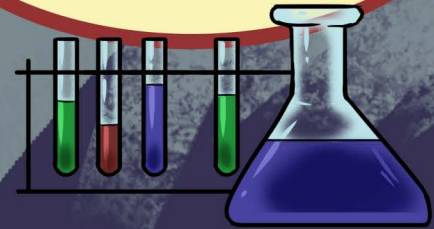


This method was used in the very first trials for gene therapy in 1990!

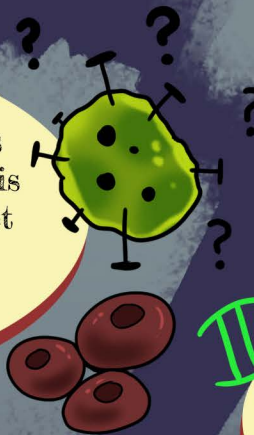
Scientists are looking to gene therapy as a possible cure for many genetic conditions like cystic fibrosis, hemophilia, cancer, and heart failure.



While these treatments are not currently available to the public, there have been many successful trials and scientists are continuing to research the topic.



One of the biggest concerns with gene therapy right now is that viruses sometimes target the wrong cells, which can create a bigger issue.



While it hasn't been perfected, gene therapy is projected to be available as a treatment for many previously incurable diseases in the near future!

