

Genomic Medicine: Understanding Genome Engineering, a Focus on Genome Editing



There was a time when it was believed that the human genome inherited from our parents – the body's complete set of genetic instructions – could not be changed. There was little hope of effective treatments for someone born with a disorder caused by a mutation in a gene.

Many conditions including cancer, certain blood disorders, metabolic disorders and diseases of the nervous system have a genetic basis. Genomic medicine is a field of research that involves the study of our genes (DNA) and their interaction with our health, leading to new ways to treat disease by addressing the underlying mistakes in DNA that lead to some genetic diseases.

Genome engineering technologies create permanent change to the genetic code of a cell by correcting, disabling, removing, or modifying a person's DNA. Genome editing tools are the specific applications scientists and doctors use to modify the genomic DNA within the cells of an individual.

One primary method of genome editing works by deploying enzymatic scissors called "engineered nucleases" to a targeted region of DNA. Once the engineered nuclease has been directed to the precise spot, it binds to and cuts the targeted gene. At this point, the nuclease makes permanent changes to the genetic code of a cell by revising, removing or replacing the defective gene. Depending on the type of cell, the genomic edit may remain in the patient's body temporarily or permanently during the life.

There are three major technologies in use today to make these targeted genomic edits: Zinc Finger Nucleases (ZFN), Transcription Activator-like Effector Nucleases (TALEN) and Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR). While there are differences, all engineered nucleases are designed to do the same thing: to bind the DNA at a precise spot in the genome to remove, add, or replace DNA.

Genome editing tools including the Zinc Finger Nuclease can be designed to precisely target the affected gene. By packaging the ZFN into a small virus called an adeno-associated virus (AAV) that can enter human cells without causing diseases, clinicians can deliver the ZFNs to specific cells in the body where they are needed.



Using Genome Editing to Treat Disease

The goal of genome editing is to provide a long-term therapeutic solution for genetic diseases. Using genome editing, scientists and clinicians may not only treat the symptoms of a disease but address its underlying root cause at the genetic level.

In the type of genome editing that is currently being developed by pharmaceutical companies, only somatic cells are targeted for treatment. Somatic cells are all cells in the body excluding reproductive cells. Therefore, any changes to the genes will only impact the cells of the individual's body who has received the treatment. Consequently, it is still possible for the defective gene to be passed along to future children.

Clinical trials are underway to study genome editing as a safe and effective treatment for some diseases that currently have no cure. Through the application of genome editing technologies, physicians might eventually be able to prescribe targeted therapies to make corrections to patient genomes and prevent, stop, or reverse disease.

Learn More

For more information on genomic medicines, including genome editing, visit the websites below. If you or a family member have a genetic-related condition, your physician or a genetics professional can help you understand if a genomic medicine approach is right for you, or help you find an appropriate clinical trial.

U.S. National Library of Medicine, Your Guide to Understanding Genetic Conditions | ghr.nlm.nih.gov

National Institutes of Health, Human Genome Research Institute | [Genome.gov](https://www.genome.gov)

Alliance for Regenerative Medicine | alliancerm.org

