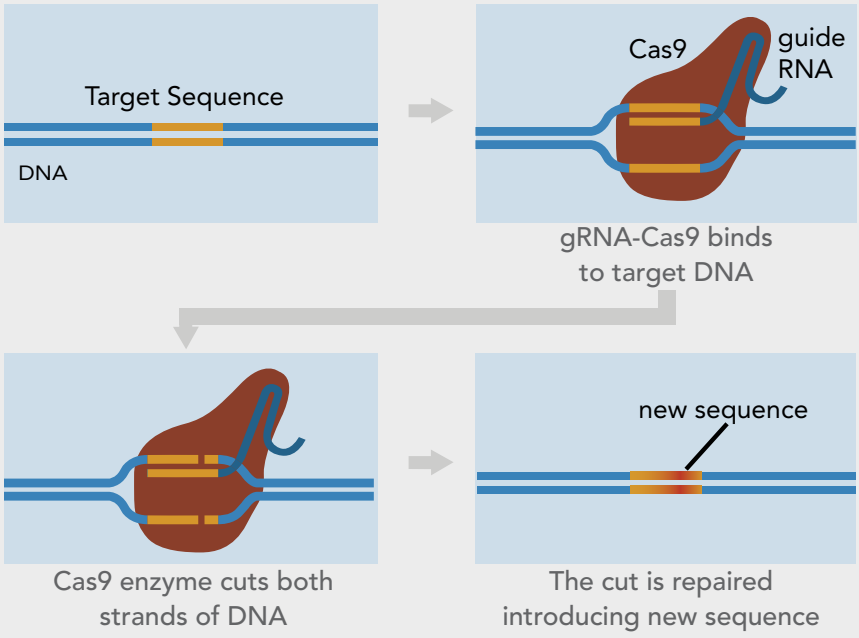
ASHG
American Society of Human Genetics

# Success Stories
## in Human Genetics and Genomics Research

# GENE EDITING WITH CRISPR

**In the past decade, scientists have found a way to make specific, targeted changes to DNA much more quickly and efficiently than ever before. This is made possible by a revolutionary gene editing tool called CRISPR, discovered through federally funded basic research investigating the immune systems of bacteria. Scientists are now harnessing CRISPR as a research tool to study the human genome. Clinical trials are also underway, examining its therapeutic utility for a variety of genetic defects and disorders, as well as for cancer treatment and rapid diagnostics for infectious diseases.**

## How it Works




In nature, CRISPR is used by bacteria as an immune system to kill invading viruses. The scientists who developed CRISPR as a scientific tool recognized that they could harness its ability to "remember" specific genetic sequences and cut them with a special protein called Cas9.[1] Now, in hundreds of laboratories all over the world, scientists are routinely using CRISPR-based tools as molecular scissors to precisely snip out genes and insert other desired genes in their place.

## CRISPR and Human Health

Many diseases have a genetic basis, from single-gene disorders like cystic fibrosis to more complex disorders like cancers and mental illnesses. Scientists are studying the utility of CRISPR for treating a variety of diseases. For instance, it is being used to treat patients with rare blood disorders such as sickle cell anemia. In these cases, doctors remove cells from patients' bodies, edit genes in the cells using CRISPR, and then infuse the cells back into the patients.[2] The results have been promising so far, and as more research is conducted, we will be able to better and fully understand its efficacy.

## Other recent developments include:

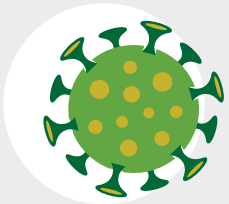
### Leber's Congenital Amaurosis

In March 2020, scientists reported that for the first time, they used CRISPR to edit a gene while it was still inside an individual's body. The treatment was part of a landmark clinical trial to cure blindness caused by a rare genetic disorder. Doctors injected the components of CRISPR directly into a patient's eye, where it is hoped the gene-editing tool will fix the genetic defect causing blindness. If this attempt proves safe, doctors plan to test it on more patients.[3]

### Cancer

Another recent clinical trial demonstrated the safety of using CRISPR to combat cancer. Researchers removed immune cells from three cancer patients, used CRISPR to make genetic modifications to improve antitumor immunity, and then administered the engineered cells back into the patients, where they were well tolerated. While this treatment at best kept cancers stable, it paves the way for future trials of CRISPR-engineered cancer immunotherapies.[4]

### Infectious Diseases



Scientists are using CRISPR's ability to identify when certain viral components are present to develop new rapid diagnostic tests for cancers and infectious diseases, including the first-ever FDA approved CRISPR test for COVID-19.[5,6]

### *Basic Research is Essential*

Basic research which explores the structure and function of the human genome is essential for understanding the mechanisms of disease and discovering new therapeutic targets. Novel techniques developed in the lab are first typically used to discover new information and understand human disease in model organisms before adapting it for clinical use. Editing the genomes of organisms such as zebrafish and mice has led to countless discoveries about the connections of genes to physical traits and disease.[7]

### *How Can Congress Support Research?*

Researchers need robust, predictable, and sustained NIH funding. Congress can support the next genetics and genomics advances by continuing to fund basic biomedical research. CRISPR-based gene therapies can continue to advance with sustained NIH funding.

**Additional Resources**

Genome Editing: www.genome.gov/about-genomics/policy-issues/what-is-Genome-Editing

What are genome editing and CRISPR-Cas9: *https://ghr.nlm.nih.gov/primer/genomicresearch/genomeediting*

SCIENCE & TECH SPOTLIGHT: CRISPR Gene Editing *gao.gov/products/GAO-20-478SP*

References: *ashg.org/advocacy/fact-sheets/*

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