

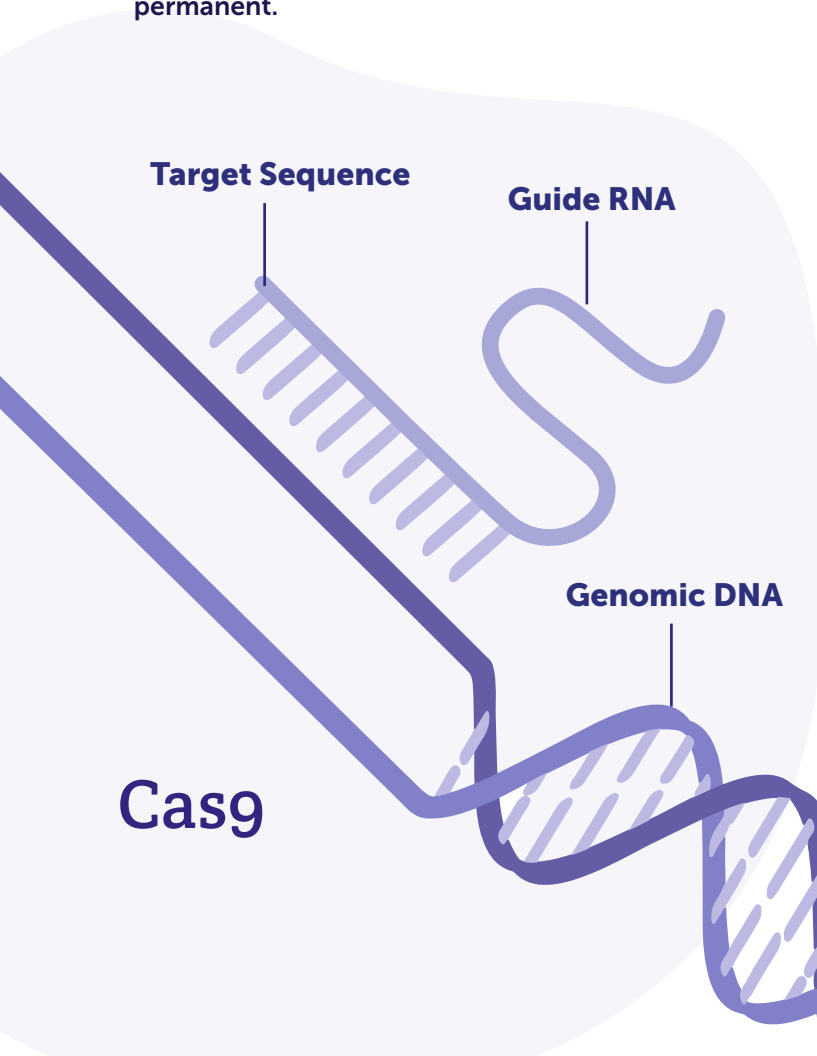
How Does Gene Editing Work?

Gene Editing is a Type of Gene Therapy

Gene therapy is the use of genetic material to treat or prevent disease. Some gene therapy approaches work by delivering a working gene into cells to provide the cell with new instructions to function properly. Gene editing is a type of gene therapy approach that directly edits pieces of DNA within the cell. This changes the instructions that the DNA encodes.

How Gene Editing Works

There are various gene editing approaches currently being researched, all of which work a little differently. For an example, we'll discuss CRISPR Cas9, which uses two core components. The first is a small piece of Guide RNA (gRNA) that finds the sequence of DNA that will be edited. The second component is a type of protein called a Cas9 enzyme or nucleus that is able to make the edit at the target DNA location that is found by the gRNA. Once the edit occurs, the cell's natural repair process occurs, making the DNA change permanent.



How Gene Editing is Administered

Gene editing technologies can be delivered into cells using *in vivo* or *ex vivo* methods. *In vivo* means the gene editing components are delivered directly into the body to make edits within the cells. *Ex vivo* means the cells are first removed from the patient, edited in a specialized laboratory and then returned back to the patient's body.

Gene Editing Research

The potential risks, benefits and outcomes are being carefully researched in preclinical studies and clinical trials. These are required parts of the research process that determine if the therapy is safe and effective. Preclinical studies test the therapy in cell and animal models. Then if the treatment being studied is showing the potential to be safe and effective, then it moves into clinical trials with humans.

Cas9