

## GENETIC SCISSORS CRISPR-CAS9

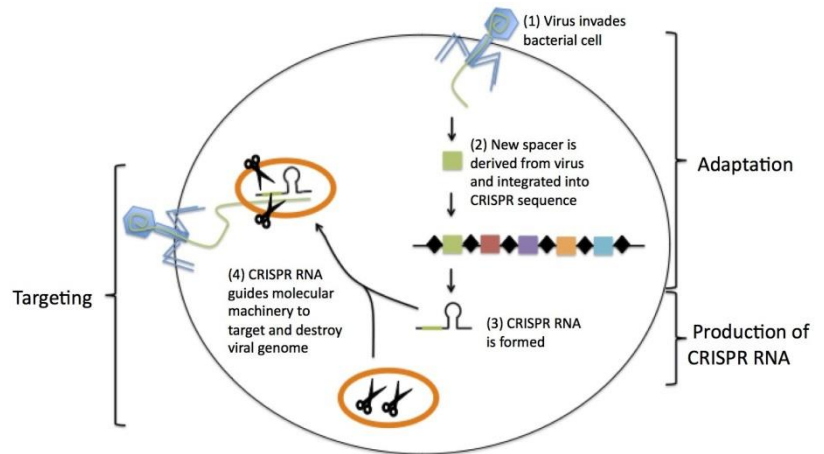
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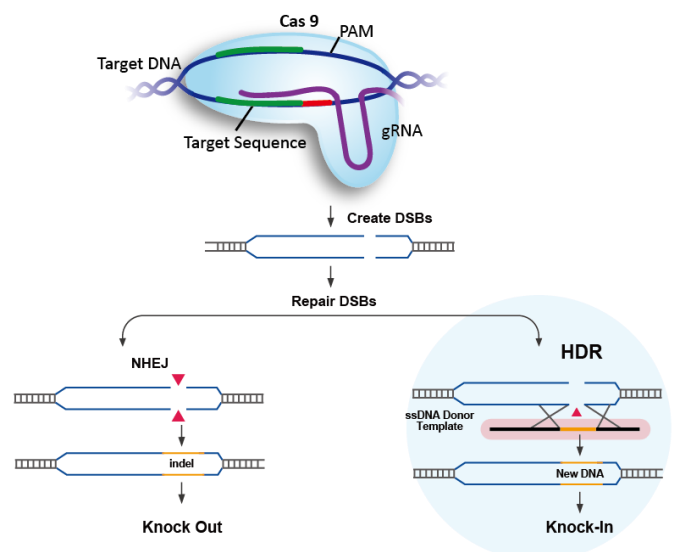
**Introduction.** Every cell in our body contains a copy of our genome, over 200 thousand genes and 3 billion of letters of DNA. Our genes shape who we are as individuals and species. Genes also profound effects on health and thanks to DNA sequencing researchers identify a thousand of genes that affect our risk of diseases. To understand how genes work researchers develop ways to control and manipulate genes in living cells, therefore recently a new method has been found which provide a genius tool to add, eliminate, and change at specific locations in the genome of any species including humans.



**Aim.** According to the researches made by the two scientists Emmanuelle Charpentier and Jennifer Doudna in 2012 CRISPR-CAS9 (Clustered Regularly Interspaced Short Palindromic Repeats) as revolutionary innovation was adapted from a natural pathway used by bacteria to protect themselves from infections by viruses. Many bacteria have short DNA sequences that are regularly repeated along their genome, called palindromes. These palindromes are interspaced with different viral DNA (spacer) forming CRISPR locus. It allows bacteria to retain the memory of a virus or bacteriophage infection to better defend themselves the next time they are infected. When an infection by virus occurs, the viral DNA within the CRISPR is copied into RNA and is associated with the Cas9 protein (for CRISPR associated). This endonuclease associated RNA attaches to the DNA of the virus and inactivates the viral DNA by cutting it, which disables the virus.

**Materials and methods.** We have received and analyzed data about this topic from literature and Internet resources.

**Results and discussion.** The engineered CRISPR-CAS9 system was introduced in the lab by creating a snippet of RNA with a short sequence named guide that binds to the desired target sequence of DNA in the genome, then the protein-9 nuclease cuts it. Once it is incised, researchers employ the cell's own DNA repair machinery to insert or remove parts of genetic material (non homology end joining), or to modify DNA by replacing an existing fragment with a customized DNA sequence (homology-directed repair). All this can be done in stem cells providing different cell types, or in the state of a fertilized egg



which allows the creation of transgenic animals with targeted mutations. CRISPR-CAS9 is characterized from other methods by the capacity to detect many genes at once, it is an open door for studying complex human diseases that are caused not only by a single mutation but by many genes acting together.

**Conclusion.** The technique is already being engaged in numerous applications in different domains. Within the context of health it could pave the way to the development of new treatments for rare metabolic disorders like Gaucher and hunter syndromes, mental diseases such as Autism and Alzheimer, and genetic maladies from Haemophilia through to Huntingdon's disease. It is also being utilized in the creation of transgenic animals to produce organs for transplants into human patients (in the case of pigs). This method was also investigated for gene therapy. Such therapy aims to insert normal genes into the cells of patients to cure various disorders. Several start-up companies have been founded to develop this technology commercially and large pharmaceutical companies are also exploring its use for drug discovery and development purposes.

## GENE MUTATION

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**Introduction.** Firstly, a gene can be defined as the basic physical and functional unit of heredity. It can also be described as a particular succession of nucleotides in DNA or RNA that is typically found on a chromosome. Genes are tasked with conveying and relaying some or all traits by the identification of structure of a certain polypeptide, especially a protein or by being in charge of the function of other genetic materials.

**Aim.** Mutation can be defined as an alteration which takes place in our DNA sequence, which is typically caused by mistakes which occur when our DNA is copied. Mutations also take place due to environmental influences. Examples of such influences include ultraviolet lights, smoke from cigarettes and other basic examples. Mutations come in different sizes and so they are capable of affecting all the other parts of the body, from one single DNA building block to a huge segment of a chromosome which consists of several genes.

**Materials and methods.** We analyzed many sources of science literature to get enough information about this problem.

**Results and discussion.** A Gene Mutation is a change or an adjustment in the succession of the DNA which makes up a gene in such a way that the succession is different from what is found in regular people. Gene mutations are permanent. They can generally be classified in two major ways:

1. Hereditary Mutations:

These mutations are the ones that are passed down from parents that tend to stay throughout a person's life. These mutations are in basically every cell in the organism. Hereditary mutations can also be referred to as Germ-Line mutations. This is due to the fact that they are present in the parent's egg or sperm cells which are called germ cells.

2. Acquired Mutations:

These mutations are also called somatic mutations. They are mutations that happen at any point in the life time of an organism. They occur only in certain cells of the body unlike hereditary mutations that occur in every cell in the body. Acquired mutations happen due to environmental factors like the sun's ultraviolet rays. They can also occur due to errors made when the DNA copies itself during the process of cell division. Acquired or Somatic mutations can not be passed on from generation to generation.

There is a genetic mutation called "De Novo" mutation. De Novo mutations can either be Hereditary or Acquired. Sometimes, this mutation happens in a person's egg or sperm cell but it won't be