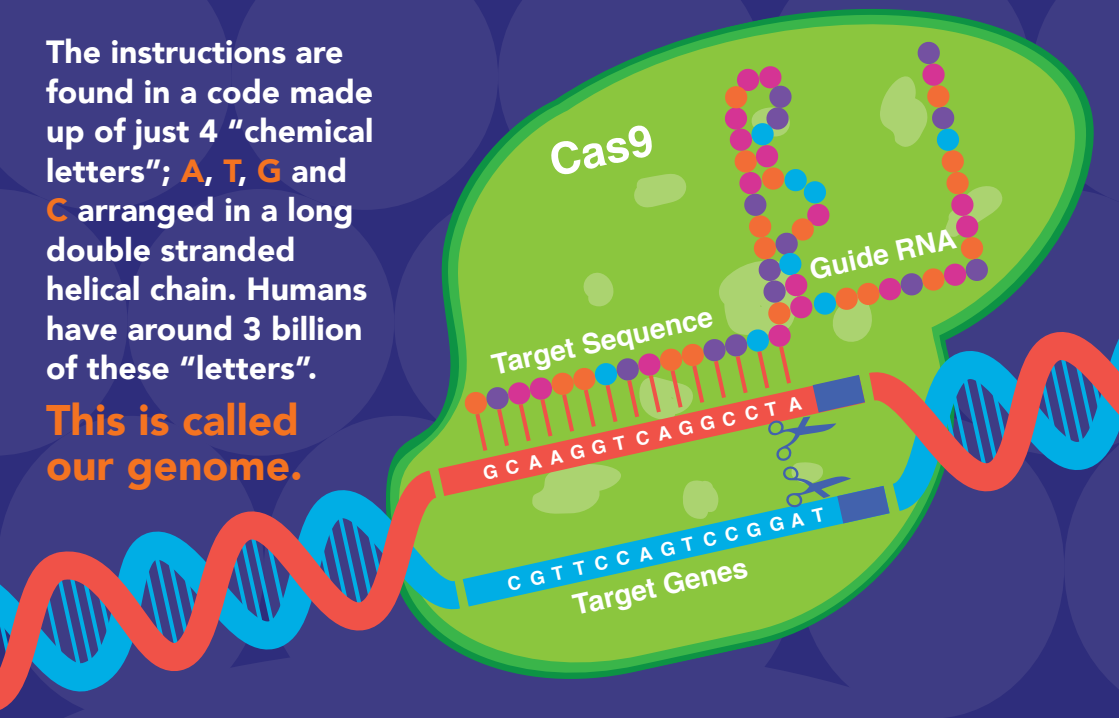
DNA is a set of instructions for how to build and run a living thing – including you!

SCIENTIFIC SCISSORS

The instructions are found in a code made up of just 4 "chemical letters"; A, T, G and C arranged in a long double stranded helical chain. Humans have around 3 billion of these "letters".

This is called our genome.



Genome editing is a way to change a DNA code. It uses specialised "molecular scissors" called nucleases to cut the DNA chain at a specific site. One of the methods of doing this is known as CRISPR-Cas9, but there are others.

We can guide these "molecular scissors" to cut at a specific part of the DNA using a short molecule called RNA that we can design in the lab. RNA is very similar to DNA, but it has only one strand. An RNA molecule can find and stick to a piece of DNA if they share the same sequence of chemical letters.

Now that we have read the whole human genome and know all of its DNA letters, we can choose exactly where to cut.

After cutting the DNA, we can decide what happens to it:

We can mend it with another piece of DNA so that we can correct mutations (mistakes in the DNA code which can cause disease) or put in a new piece of DNA.

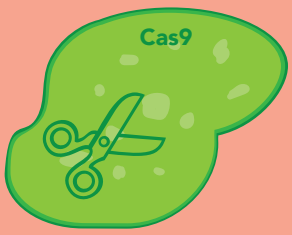
We can deliberately break the DNA strand to see what happens if that particular part of the DNA instruction manual is broken or missing.

British Society for Gene & Cell Therapy

BIOCHEMICAL SOCIETY

New genome editing methods are so specific that from the 3 billion letters in the human genome, they can choose to cut in any given 20-letter region.

CRISPR stands for
Clustered
Regularly
Interspaced
Short **P**alindromic
Repeats.



Cas9 is a CRISPR associated system protein – and there's more than one of them. It's the Cas9 protein that does the DNA cutting.

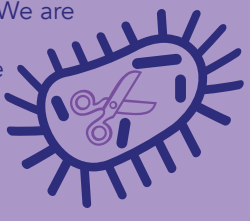
Some diseases are caused by 'spelling' mistakes in the DNA code. Genome editing offers the chance to correct these 'spelling' mistakes.

Genome editing has already been carried out in bacteria, plants, animals and human cells. It is currently illegal in the UK to allow edited human embryos to develop for longer than 14 days.

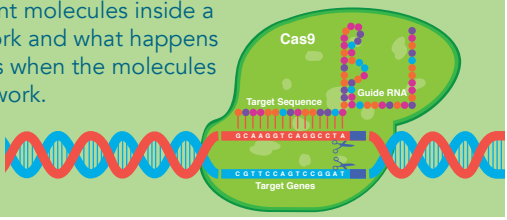
Genome editing is not the only way to change genomes. Humans have been selectively breeding and cross-breeding plants and animals for thousands of years. However, genome editing is more tightly controlled and often quicker and cheaper.

There are two ways to edit human genomes. **Somatic editing** is not passed on to the next generation and may not affect every cell. **Germline editing** can affect all cells and any changes will be passed on to offspring. This raises ethical issues about consent and unforeseen consequences.

Bacteria first developed these "molecular scissors" as a defence mechanism to fight against viruses. For millions of years, these nucleases were used to cut the DNA of invading viruses so that it is inactivated and saves the bacteria from infection. We are now using the same technique to study DNA.



Genome editing can be used to study human cells in a lab so that we understand how the different molecules inside a cell work and what happens to cells when the molecules don't work.



In 2015, genome editing was used to cure a young girl of leukaemia. It is hoped that it will offer treatment options for many types of cancers and genetic diseases.