CRISPR

Clustered regularly interspaced short palindromic repeats (**CRISPR**, pronounced *crisper*) are segments of prokaryotic DNA containing short, repetitive base sequences. These play a key role in a **bacterial defence system**,and form the basis of a genome editing technology known as CRISPR-Cas9 that allows permanent modification of genes within organisms.(WIKIPEDIA)

Source: http://www.yourgenome.org/facts/what-is-crispr-cas9

CRISPR-Cas9 is a genome editing tool that is creating a buzz in the science world. It is faster, cheaper and more accurate than previous **techniques of editing DNA** and has a wide range of potential applications.

What is CRISPR-Cas9?

- CRISPR-Cas9 is a unique technology that enables geneticists and medical researchers to edit parts of the genome? by removing, adding or altering sections of the DNA sequence.
- It is currently the simplest, most versatile and precise method of genetic manipulation and is therefore causing a buzz in the science world.

How does it work?

- The CRISPR-Cas9 system consists of **two key molecules** that introduce a change (mutation) into the DNA. These are:
    - **an enzyme** called Cas9. This acts as a pair of 'molecular scissors' that can cut the two strands of DNA at a specific location in the genome so that bits of DNA can then be added or removed.
    - a piece of RNA called **guide RNA** (gRNA). This consists of a small piece of pre-designed RNA sequence (about 20 bases long) located within a longer RNA scaffold. The scaffold part binds to DNA and the pre-designed sequence 'guides' Cas9 to the right part of the genome. This makes sure that the Cas9 enzyme cuts at the right point in the genome.
- **The guide RNA is designed to find and bind to a specific sequence in the DNA**. The guide RNA has RNA bases that are complementary to those of the target DNA sequence in the genome. This means that, at least in theory, the guide RNA will only bind to the target sequence and no other regions of the genome.
- **The Cas9** follows the guide RNA to the same location in the DNA sequence and **makes a cut across both strands of the DNA.**
- At this stage the cell recognises that the DNA is damaged and tries to repair it.
- Scientists can use the DNA repair machinery to introduce changes to one or more genes in the genome of a cell of interest.

How was it developed?

•Some bacteria have a similar, built-in, gene editing system to the CRISPR-Cas9 system that they use to respond to invading pathogens like viruses,**much like an immune system.**

•Using CRISPR the bacteria **snip out parts of the virus DNA and keep a bit of it behind to help them recognise and defend against the virus next time it attacks**.

•Scientists adapted this system so that it could be used in other cells from animals, including mice and humans.

What are the applications and implications?

•CRISPR-Cas9 has a lot of potential as a tool for treating a range of **medical conditions** that have a genetic component, including cancer, hepatitis B or even high cholesterol.

•Many of the proposed applications involve editing the genomes of somatic (non-reproductive) cells but there has been a lot of interest in and debate about the potential to edit germline (reproductive) cells.

•Because any changes made in germline cells will be **passed on from generation to generation it has important ethical implications.**

•Carrying out gene editing in germline cells is **currently illegal** in the UK and most other countries.

•By contrast, the use of CRISPR-Cas9 and other gene editing technologies in somatic cells is uncontroversial. Indeed they have already been used to treat human disease on a small number of exceptional and/or life-threatening cases.

What's the future of CRISPR-Cas9?

•It is likely to be many years before CRISPR-Cas9 is used routinely in humans.

•Much research is still focusing on its use in animal models or isolated human cells, with the aim to eventually use the technology to routinely treat diseases in humans.

•There is a lot of work focusing on eliminating 'off-target' effects, where the CRISPR-Cas9 system cuts at a different gene to the one that was intended to be edit

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