Genetic engineering

Since genetic engineering (also known as recombinant DNA technology or genetic modification) was first developed in the 1970s, scientists have discovered more and more ways in which the technology can be used in human medicine. Now techniques, including the gene editing tool known as CRISPR-Cas9, are opening up even more possibilities for us to change the DNA in the cells of bacteria, animals and plants – and potentially change medicine for ever.

Pharming

Microorganisms, animals and plants can be genetically modified to produce medically useful products. These transgenic organisms are already used regularly to produce substances such as human insulin, human growth hormone and blood clotting factors for haemophiliacs.

CRISPR

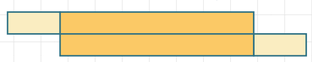
CRISPR-Cas9 is a genome editing tool which has revolutionised the world of genetic engineering. It enables scientists to directly remove, add or change sections of the DNA sequence in a living cell. CRISPR-Cas9 is much faster, much cheaper and much more accurate than previous traditional ways of editing DNA. The technology has great potential for treating any diseases which involve the genome, including cancers, heart disease and even the high cholesterol levels which are a risk factor for heart disease.

The basic steps in traditional genetic engineering of a bacterium

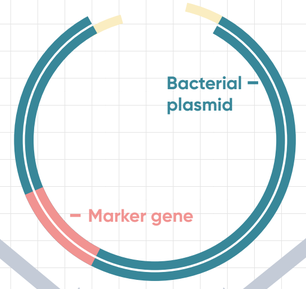
The required gene is EITHER cut from the DNA of an organism using enzymes called restriction endonucleases which leave the gene with overlapping sections called sticky ends...

or

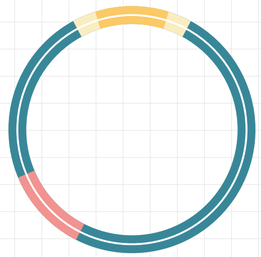
...synthesised specifically



A vector molecule (often a bacterial plasmid, a small circular strand of DNA sometimes found in bacteria) is opened up, also using restriction enzymes and leaving sticky ends.

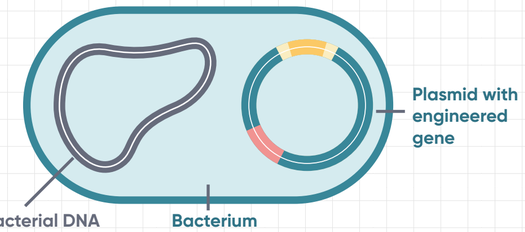


The new gene is annealed (joined into the plasmid using the sticky ends) and sealed in place using another enzyme known as DNA ligase.



Transformation

The recombinant plasmid is inserted into a bacterial cell in a process known as transformation. It is then replicated whenever the bacterial cell replicates, and brings about the manufacture of a new protein.



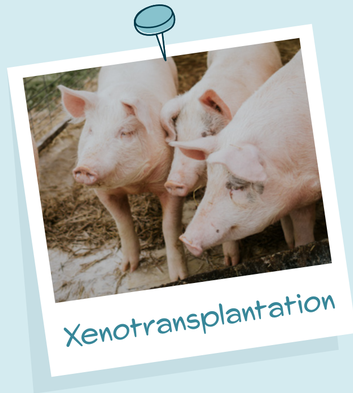
Gene therapy is still an experimental technique. It involves modifying human DNA either to repair or replace a faulty gene. The idea of gene therapy is to overcome the effects of a mutation which cause a genetic disease or tendency to a disease. There has been some success in treating some inherited genetic diseases and also acquired disorders such as leukaemia. The speed and precision of CRISPR-Cas9 gene editing technology gives scientists further hope for using this technique.

Gene therapy

Vaccines

Some vaccines are very dangerous to make using conventional methods. Genetically engineered microbes can be used to produce the antigens needed for vaccine production, in a safe and controllable way. The use of genetically modified yeast cells to produce a vaccine against the hepatitis B virus has been a major success story.

The DNA of pigs has been modified using recombinant DNA technology so that their cells develop without certain genes which trigger the human immune response. Other genes can be added which express human antigens. Work in this area requires significant ethical and safety considerations. To date, successes include German scientists using CRISPR Cas9 to deliver multiple gene modifications in pigs, greatly reducing the human immune response to the pig cells.



Xenotransplantation

Use QR code to find out more about genetic engineering on the ABPI Schools website

www.abpischools.org.uk

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