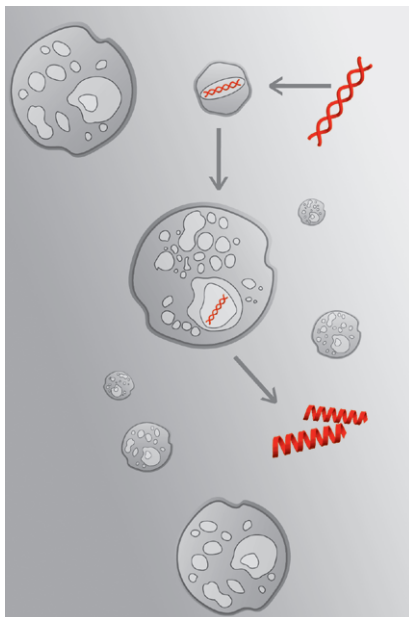




Gene therapy overview

Genes are specific areas of DNA that encode the proteins that maintain a human body. The aim of gene therapy is to correct genetic alterations that cause disease. Gene therapy is a relatively new therapeutic approach that is currently targeted only at somatic cells, i.e. the cells that make up the body and not at egg or sperm cells that would be passed on to children.



The most common form of gene therapy involves inserting normal, healthy genes into the patient so that the healthy gene can replace or supplement the activity of a damaged gene. To ensure that the functional gene is delivered inside cells where the protein can be made, it is carried by a 'vector' that can enter the cell without harming it. Vectors are normally derived from viruses, as viruses have evolved a way of packaging and delivering their own genes into human cells so that they can replicate. Scientists have taken advantage of the virus's way of introducing genes into cells by replacing the viral genes with therapeutic genes.

A common, harmless type of virus, known as an adeno-associated virus (AAV), is often used for developing gene therapy. It is generally considered a safe vector and one that most people have already been exposed to in the course of their normal lives. When used for gene therapy, the AAV vector, which contains the therapeutic gene, and the vector is altered so that it cannot infect or replicate.

In general, gene therapy is easier to carry out when the disease is caused by mutations in only one gene, as in LPLD, as one gene is easier to introduce into a cell than several genes.

Gene therapy can also be used to prevent faulty parts of a gene being used (gene silencing) and it is possible to switch genes "on" and "off". Until now, gene therapy research has mainly focused on hereditary diseases and cancers. More recently, scientists have started to look into using gene therapy for other diseases, such as cardiovascular diseases, liver diseases and CNS disorders.