Definition: **gene therapy** from *The Penguin Dictionary of Science*

The treatment of disease by the introduction of a functional gene which can be inserted into an embryo or into mature somatic cells where a defect has been identified. For example, treatment is being developed for sufferers of cystic fibrosis, who have a defective gene encoding a chloride ion channel, in which a DNA preparation is inhaled and becomes incorporated into the cells lining the respiratory tract, restoring some degree of normal function to affected cells.

Summary Article: **gene therapy** from *The Hutchinson Unabridged Encyclopedia with Atlas and Weather Guide*

Medical technique that alters genes inside the body's cells to treat or prevent disease. The main strategies employed in gene therapy are replacing defective genes with healthy versions, rendering defective genes inactive, or introducing new genes into the body to treat disease. Gene therapy researchers aim to develop treatments for genetic diseases such as cystic fibrosis, other diseases such as AIDS, and genetic disorders such as haemophilia. Although gene therapy may cure a patient, it cannot prevent the genetic defect being passed on to any children; germ-line gene therapy is a theoretical modification that would allow the treatment to be hereditary, by modifying genes in eggs and sperm.

Gene therapy was first proposed as a method of curing human genetic diseases in 1972. The first human to undergo gene therapy, in 1990, was Ashanti Desilva, a four-year-old US girl suffering from a rare enzyme (adenosine deaminase, or ADA) deficiency that cripples the immune system. Unable to fight off infection, such children are nursed in a germ-free bubble, and their life expectancy is low, with death in childhood common. Researchers engineered a virus to carry functioning ADA genes, combined these with white blood cells collected from Ashanti's blood, and injected them into her. This treatment, combined with regular injections of ADA, allowed Ashanti to live outside an isolation bubble.

The first gene therapy treatment approved for medical use (rather than just clinical trials) was Glybera in 2012. This restores the functionality of lipoprotein lipase (LPL) enzymes in patients suffering from LPL deficiency. However, the licence, issued by the European Medicines Agency, restricts its use to patients who have failed to respond to conventional treatment and who are suffering from severe pancreatitis despite restricting their dietary fat intake. Although as of 2012 there had been over 1,800 clinical trials of gene therapy treatments in 13 countries worldwide, there are currently no gene therapy products available as standard medical treatments.

The biggest challenge for gene therapy is to ensure that the replacement gene can enter the patient’s cells and insert itself into the genome without disrupting the other genes and causing unwanted side effects. Retroviruses are being used as vectors but as these cannot be used to target specific chromosomal sites experiments are being conducted using both adenovirus and adeno-associated virus (AAV). Using adenovirus, the cystic fibrosis defect in humans has been successfully corrected within the nasal cavity. AAV is particularly advantageous because it integrates its gene cargo at specific
sites, and causes no known human illnesses.

Some biotechnology firms are avoiding the use of viruses altogether, for example by basing research on the fact that DNA injected directly into the body can be taken up by some cells and expressed. The use of liposome vectors to deliver new or altered DNA to cells is safe but imprecise, as they deliver their cargo to target cells and nontarget cells alike. Disabled virus vectors are more precise but there is a danger they will be reactivated and are therefore unsafe. Research began in 1995 into a safe and precise vector involving a cluster of genes called a locus control region (LCR), whereby the therapeutic gene is attached to the LCR and is only activated by the LCR when it encounters proteins unique to the target cell type.

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