

GENE TRANSFER 101

Certain terms used in the science of gene transfer will be common throughout the special section of this issue. Below are the definitions of some terms that may be unfamiliar.

■ Gene

The functional and physical unit of heredity passed from parent to offspring. Genes are pieces of DNA, and most genes contain the information for making a specific protein. A gene occupies a specific locus on a chromosome. Each human has an estimated 100,000 separate genes.

■ Gene Transfer

Gene transfer is an experimental medical intervention that involves modifying the genetic material of living cells to fight disease. One of the goals of gene transfer is to supply cells with healthy copies of missing or altered genes.

■ Genome

A genome is an entire system of genes. Human genome refers to all genes within the 46 chromosomes.

■ Mutations

Mutations are permanent changes in DNA. These changes are sometimes passed on to offspring and may or may not be harmful.

■ Deoxyribonucleic acid (DNA)

The molecule that encodes genetic information in the nucleus of cells. One of the two types of nucleic acids found in all cells. The other is RNA. Nucleic acids are the chemical components of a gene. DNA transmits genetic information to RNA, which in turn provides instructions for making specific proteins.

■ Ribonucleic acid (RNA)

One of the two types of nucleic acids found in all cells. The other is deoxyribonucleic acid (DNA). Ribonucleic acid transmits genetic information from DNA to proteins produced by the cell.

■ Chromosome

Chromosomes are packets of genes in a cell, composed of DNA and other reinforcing molecules. Humans have 23 pairs (46 total). One member of each pair of chromosomes is inherited from the mother and the other from the father.

■ Vector

An agent, such as a virus or a small piece of DNA called a plasmid, that carries a modified or foreign gene such as DNA sequences. When used in gene therapy, a vector delivers the desired gene to a target cell.

■ Viruses

Viruses are intra-cellular parasites. Designed, through the course of evolution, to infect cells, often with great specificity for a particular cell type.

■ Adenovirus

A group of DNA-containing viruses that cause respiratory disease, including one form of the common cold. The life cycle does not normally involve integration into the host chromosome, rather replicate independently in the nucleus of the host cell; consequently there is no risk of insertional mutagenesis (production of a mutation).

■ Adeno-associated virus (AAV)

Adeno-associated viruses are DNA-containing viruses, dependant on a helper virus, usually an adenovirus, to proliferate. They are capable of infecting both dividing and nondividing cells. Scientists believe they tag along with other viruses during a common cold infection. Once the cold virus disappears, AAVs simply rest quietly within the cell, becoming active and replicating only when a new cold virus infects the cell. Dormant genes for AAV are also present in primates.

■ Retrovirus

A type of virus that contains RNA as its genetic material. The RNA of the virus is translated into DNA, which inserts itself into an infected cell's own DNA. Retroviruses can cause many diseases, including some cancers and AIDS.

■ Lentivirus

A subclass of retroviruses that are able to infect both proliferating and non-proliferating cells.

■ Recombinant protein

Proteins that are the result of genetic engineering. A regulatory part of one or more genes is combined with the structure of another gene. The protein is formed after transcription and translation of the fused gene.

■ Phase I trials

These first studies in people evaluate safety of a new drug or treatment. A phase I trial usually enrolls only a small number of patients, sometimes as few as a dozen.

■ Phase II trials

A phase II trial continues to test the safety of the drug and begins to evaluate how well the new drug works.

■ Phase III trials

These studies test a new drug or treatment in comparison with the current standard. A participant will usually be assigned to the standard group or the new group at random (called randomization). Phase III trials often enroll large numbers of people and may be conducted at many doctors' offices, clinics and centers nationwide. 

SOURCES

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