

WHAT IS GENE THERAPY?

WHAT IS A GENE?

Genes are regions of DNA that code for proteins. Failure to produce normal levels of functional protein due to a defective gene can result in disorders such as hemophilia B, cystic fibrosis, lysosomal storage disorders, lipoprotein lipase deficiency (LPLD) and some cancers.^{1,2}

WHAT IS GENE THERAPY?

Gene therapy is a technique that uses genetic material (a piece of DNA) for the long-term treatment of genetic disorders.^{1,2} This may involve delivering a copy of a healthy or therapeutic gene, repairing a faulty gene, and/or altering the degree to which a gene is turned 'on' and 'off'.

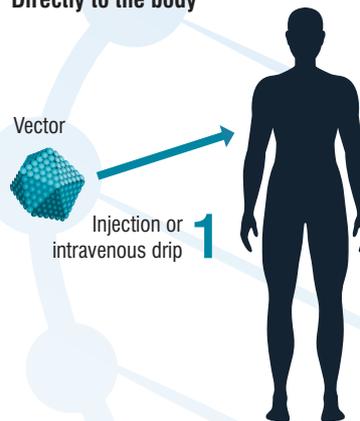
WHAT ARE THE POTENTIAL ADVANTAGES OF GENE THERAPY?

Gene therapy can potentially be used to treat genetic disorders with single or few administrations rather than frequent dosing, improving quality of life and reducing the need for physician visits. Gene therapy also offers the potential to specifically target the affected tissues within the body.^{1,3}

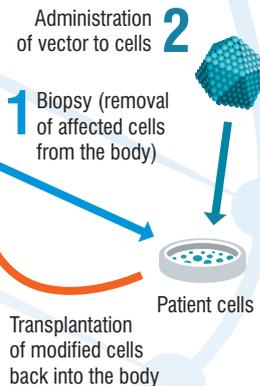
HOW DOES GENE THERAPY WORK?

A healthy gene is inserted into a carrier, called a vector, and transferred to the affected cells, either inside or outside the body.¹⁻³

Method A: Directly to the body

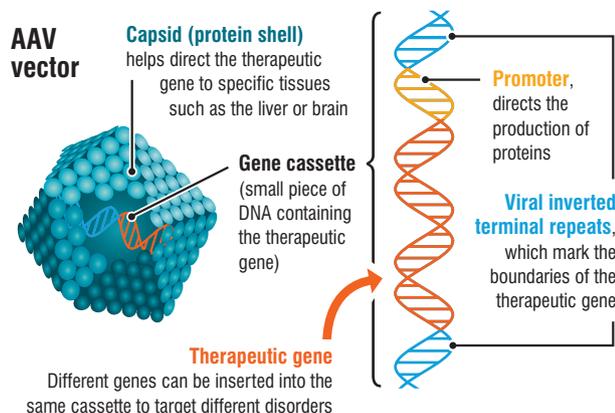


Method B: Outside the body

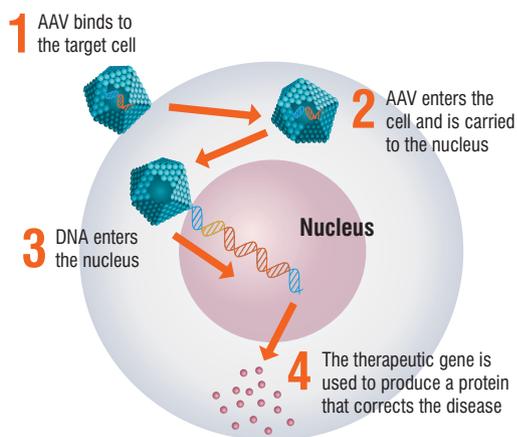


The transfer of therapeutic genes to the targeted cells is described on the reverse side.

The most common gene therapy vectors are viruses (e.g. adeno-associated virus [AAV]) that have been modified to replace their disease-causing genetic material with a therapeutic gene; however, non-viral vectors are also available.^{2,3} Different vectors target different cell types.



Once inside the cell, the healthy gene is carried to the nucleus, where the cell uses it to produce the therapeutic protein to improve or correct the disorder.



IS GENE THERAPY SAFE?

Gene therapy is primarily an experimental technology and, as such, is highly regulated and carefully monitored to maximize patient safety. There have been considerable advances in the field of gene therapy over recent years including approval of 3 gene therapies (Glybera® [uniQure, B.V.] – LPLD; Imlygic® [Amgen] – melanoma; Strimvelis™ [GlaxoSmithKline] – adenosine deaminase severe combined immunodeficiency) as well as numerous clinical studies showing promising safety profiles.⁴⁻⁶ Depending on the type of gene therapy used, potential risks can include unwanted immune reactions and the formation of tumors. The effects of current gene therapy approaches are limited to the treated patient's cells. Modified genes are not passed on from one generation to the next.²

Gene Therapy: Terms to Know

Capsid	Protein shell surrounding viral DNA that helps target the genetic material to specific cell types
Cell	The smallest structural and functional unit of an organism
DNA	A molecule that carries genetic instructions
Gene cassette	A small piece of DNA containing the therapeutic gene and instructions for the cell on how to use the gene
Nucleus	The central part of a cell; contains the DNA used to make proteins
Protein	Essential structural and functional components of all living organisms, e.g. body tissues (muscle, hair, etc.), enzymes and antibodies
Vector	May refer to the DNA molecule itself or to the carrier construct containing the DNA to be delivered to a cell

References

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