For Healthcare Providers

Gene Therapy: A Clinical Overview

Gene therapy is a novel therapeutic approach that modifies a patient's genes to treat or prevent disease. It involves the introduction, correction, or replacement of genetic material within a patient's cells to restore normal function or introduce new therapeutic capabilities.

Types of Gene Therapy

There are two main types of gene therapy, with subtypes that can be broken down into the following.

- **Gene addition:** Introduces a functional copy of a gene to compensate for a defective gene
- **Genome editing:**
  - Gene correction – Repairs or modifies a faulty gene to restore normal function
  - Gene silencing – Inhibits the expression of a harmful gene[1,2]

Mechanisms of Gene Therapy

Gene therapy can be done inside the body (in vivo) or outside the body (ex vivo). The technique used may depend on the condition being addressed. In both methods, a vector must be used to deliver the therapeutic genetic material* to the cells. Vectors are most commonly made from viruses in four main types: adeno-associated viral, adenoviral, lentiviral, and retroviral.[3] Nonviral vectors include liposomes and nanoparticles.

- Ex vivo
  Step 1: A sample of blood, bone marrow, or other tissue is taken from a patient.
  Step 2: In a lab, specific cells are separated from the sample and introduced to a vector that contains the desired genetic material.
  Step 3: The modified cells are returned to the patient's body through an injection or infusion.
- In vivo
  Step 1: A vector is engineered to carry the needed genetic material to the cells of the affected organ.
  Step 2: The vector is delivered to the patient through an infusion or injection.

Gene Therapy Research

Several gene therapies have received regulatory approval, demonstrating promising clinical efficacy and safety. These therapies can treat many genetic and acquired conditions, including certain autoimmune diseases, cancers, and blood disorders.[4]

As an emerging type of treatment, gene therapy research is quickly evolving, with new discoveries and advancements happening often. Ongoing research is expanding the applications of gene therapy, bringing new hope to patients with genetic conditions that are difficult to treat.

For information about clinical research opportunities, visit ClinicalTrials.gov.

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*Genetic material may consist of manufactured deoxyribonucleic acid (DNA) or ribonucleic acid (RNA).

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Addressing Misconceptions About Gene Therapy

The amount of information available about gene therapy may become overwhelming for patients who are seeking treatment. Certain aspects have become topics of debate, and conflicting information can cause confusion.

It is important to address the presence of misinformation and to make sure patients are fully educated before making decisions regarding their health. The following are some common misconceptions about gene therapy that may be an important part of the conversation with patients.

Myth: Gene therapy is always complicated and invasive.

Reality: Many gene therapies are administered through a single injection, similar to a vaccine. Advancements in gene delivery methods are making treatments less invasive and more accessible.

Myth: All gene therapies use stem cells from embryos.

Reality: While some gene therapies can use donated embryonic stem cells, many often use stem cells from the patient's own body as a key component. These are adult stem cells and do not come from an embryo.

Myth: Gene therapy "edits" genes and changes who you are.

Reality: Gene editing, which is a type of gene therapy that does not yet have any treatments approved for use outside of research, directly changes pieces of DNA within a cell to make the existing genes work differently.[5] Most other gene therapy approaches instead only introduce a new, working gene into a cell. No gene therapies used to treat disease change a person beyond addressing their condition.

Patient Resources

For patients who would like to learn more, the following resources can provide additional information about gene therapy:

American Society of Gene and Cell Therapy – patienteducation.asgct.org

National Organization for Rare Disorders – rarediseases.org/gene-therapy

National Human Genome Research Institute – genome.gov

References
1. genome.gov/research-at-nhgri/Projects/Democratizing-Education/understanding-gene-therapy-approaches
2. who.int/health-topics/human-genome-editing
3. pubmed.ncbi.nlm.nih.gov/21590391/
4. fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products
5. https://patienteducation.asgct.org/patient-journey/ethical-issues-germline-gene-editing

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