Gene Therapy: Cure for Cancer

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What is Gene Therapy?

Human gene therapy seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. Gene therapy is a technique that modifies a person's genes to treat or cure disease. Gene therapies can work by several mechanisms:

- Replacing a disease-causing gene with a healthy copy of the gene.
- Inactivating a disease-causing gene that is not functioning properly.
- Introducing a new or modified gene into the body to help treat disease.

Gene therapy products are being studied to treat diseases including cancer, genetic diseases, and infectious diseases.

There are a variety of types of gene therapy products, including:

- Plasmid DNA: Circular DNA molecules can be genetically engineered to carry therapeutic genes into human cells.
- Viral vectors: Viruses have a natural ability to deliver genetic material into cells, and therefore some gene therapy products are derived from viruses. Once viruses have been modified to remove their ability to cause infectious disease, these modified viruses can be used as vectors (vehicles) to carry therapeutic genes into human cells.
- Bacterial vectors: Bacteria can be modified to prevent them from causing infectious disease and then used as vectors (vehicles) to carry therapeutic genes into human tissues.
- Human gene editing technology: The goals of gene editing are to disrupt harmful genes or to repair mutated genes.
- Patient-derived cellular gene therapy products: Cells are removed from the patient, genetically modified (often using a viral vector) and then returned to the patient.

How could gene therapy cure cancer?

Gene therapy is a way of treating or preventing disease by altering the genetic instructions within an individual's cells. Genes are responsible for virtually every aspect of cell life: they hold the code for proteins that enable cells to grow, function, and divide. When a gene is defective, it can give rise to proteins that are unable to do their job. When a gene is missing, or is overactive, important bodily functions may be impaired. The goal of gene therapy is to correct such problems by fixing them at the source. Gene therapy can involve replacing abnormal or absent genes with healthy ones that enable cells to produce useful proteins. It also can involve changing the way genes are regulated, so that under- or overactive genes operate properly. Finally, gene therapy can be used to express entirely foreign genes in cells that alter their function and/or survival.

A variety of efforts are underway to apply gene therapy to cancer treatment. Most are in early, exploratory stages, where they're being studied in the laboratory or in clinical research trials. One approach, however, known as CAR T-cell therapy, has received approval from the U.S. Food and Drug Administration for use as a therapy in certain groups of patients and is expected to receive additional approvals in the near future. Research in gene therapy for cancer is currently focused in multiple areas, including genetically engineered viruses that directly kill cancer cells,

gene transfer to alter the abnormal functioning of cancer cells, and immunotherapy (which includes CAR T-cell therapy), which helps the immune system better find and kill tumor cells.

How does 'base editing' help in curing cancerous T-cells?

Allogeneic chimeric antigen receptor T-cell (CART) therapies require multiple gene edits to be clinically tractable. Most allogeneic CARTs have been created using gene editing techniques that induce DNA double-stranded breaks (DSBs), resulting in unintended on-target editing outcomes with potentially unforeseen consequences. Cytosine base editors (CBEs) install C•G to T•A point mutations in T cells, with between 90% and 99% efficiency to silence gene expression without creating DSBs, greatly reducing or eliminating undesired editing outcomes following multiplexed editing as compared with clustered regularly interspaced short palindromic repeats (CRISPR)/CRISPR-associated protein 9 (Cas9).

What is CRISPR Technique? How does the CRISPR-cas9 system work?

Genome editing (also called gene editing) is a group of technologies that give scientists the ability to change an organism's DNA. These technologies allow genetic material to be added, removed, or altered at particular locations in the genome. Advanced research has allowed scientists to develop the highly effective Clustered Regularly Interspaced Palindromic Repeat (CRISPR) -associated proteins based systems. This system allows for targeted intervention at the genome sequence. This tool has opened up various possibilities in plant breeding. Using this tool, agricultural scientists can now edit the genome to insert specific traits in the gene sequence.

CRISPR-Cas9 is a unique technology that enables geneticists and medical researchers to edit parts of the genome by removing, adding or altering sections of the DNA sequence.