CMT Research Foundation

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Gene Therapies Demystified: Terms to Know

DNA — The complete instruction manual inside nearly every cell in your body. It contains the directions for building and running every part of you, written in a chemical code.

Gene — One single instruction within your DNA. Each gene tells the body how to make a specific protein. You have roughly 20,000 genes in total.

mRNA (messenger RNA) — A temporary copy of a gene's instructions that gets carried out of the cell's nucleus to where proteins are built. Think of it as a work order issued from the DNA library.

Protein — The actual workers of the cell, built using the instructions in a gene. Proteins carry out nearly every function in the body, from transmitting nerve signals to repairing tissue.

Gene Replacement Therapy — A treatment that delivers a healthy, working copy of a broken or missing gene into the body's cells. The faulty gene stays in place — this approach simply adds a working version alongside it.

Gene Silencing — A treatment that blocks a gene's message from being carried out, preventing a harmful protein from being made. The DNA itself is not changed. Because the effect wears off over time, silencing therapies typically require repeat doses.

Gene Editing — A treatment that makes a permanent change directly to the DNA itself, correcting or disabling a faulty gene. Because the edit is written into the genetic code, a single treatment can have a lasting effect.

ASO (antisense oligonucleotide) — A short, synthetic piece of genetic material designed to stick to a specific mRNA message and block it from being read. One of the main tools used in gene silencing therapies.

siRNA (small interfering RNA) — Another gene silencing tool, similar to an ASO but working through a slightly different mechanism. Both ASOs and siRNAs intercept and destroy a gene's mRNA message before it can be turned into a protein.

CRISPR-Cas9 — A precision gene editing tool that acts like molecular scissors. A guide molecule directs it to the exact spot in the DNA that needs to be changed, and the Cas9 protein makes the cut. The cell's own repair system then fixes or alters the DNA at that location.

AAV (adeno-associated virus) — A small, naturally occurring virus that has been stripped of everything that makes it harmful and repurposed as a delivery vehicle. Scientists load it with a therapeutic gene and use it to ferry that gene into the body's cells.

Lipid Nanoparticle (LNP) — A tiny fat-based bubble used to package and deliver genetic medicine into cells. LNPs can be engineered to target specific tissues in the body.

I**ntrathecal Injection** — An injection delivered directly into the fluid-filled space surrounding the spinal cord, allowing a therapy to reach the spinal cord and nearby nerve roots more efficiently than traveling through the bloodstream.