What Are Gene and Cell Therapies?

According to the U.S. Food & Drug Administration (FDA), human gene and cell therapies seek to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. Gene and cell therapies fall into two main categories: replacement or inactivation of a disease-causing gene; or introduction of a new or [1]. Cell therapy products include cellular immunotherapies, cancer vaccines, and other types of autologous and allogeneic cells for certain indications, including hematopoietic stem cells and adult and embryonic stem cells.[2]

How Are Gene and Cell Therapies Delivered?

The several techniques and mechanisms for delivery of these new therapies can generally be categorized as either , where the / person's body and the cells then transplanted back into the body, or intravenously or conveyed directly to the affected tissue or organ in the body.[4] New genetic material can be transported to cells via engineered nanoparticles or adeno-associated virus vectors. The latter method can be problematic, as up to 50% of people have pre-existing immunity to these adenoviruses which excludes them from treatment. Research to circumvent this issue continues.[5]

Oligonucleotide therapies, which use synthesized nucleic acid polymers to treat or manage a wide range of diseases, are considered gene therapies for the purposes of this article but are often considered a separate treatment category in the medical literature.

What Diseases Have Approved Gene and Cell Therapies?

Several gene and cell therapies, including oligonucleotide therapies, have been approved for use, but this differs depending on the country. The U.S. FDA, for example, has approved gene and cell therapies for several diseases,[3, 9] including: