|SCIENCE|

Fighting serious disease through cell and gene therapy

Jaw dropping research is leading to treatments modifying DNA.

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IN THIS CRAZY world of science fiction, inserting a gene into the DNA of human cells to treat or cure disease is nothing we haven't heard about. Now, thanks to decades of research by academic researchers, the gene therapy that once was in futuristic stories is becoming a reality.

Gene therapy is when a normal gene is inserted into the genome to replace an abnormal gene responsible for causing a certain disease. With many challenges involved in the process, one of the most significant is the difficulty in releasing the gene into the stem cell. So, a molecular carrier called a vector is used to release the gene. It needs to display efficiency in the release of one or more genes of the sizes necessary for clinical applications and not be recognized by the immune system. Like I said, With gene therapy, the treatment involves adding, removing, or altering a gene. In cell therapy, cells are taken either from the patient themselves or

a donor, are genetically altered to treat disease, then injected back into the patient. These treatments If togther are often referred to together as cell and gene therapy because they generally involve genetically modifying cells and DNA inside or outside of the body.

IMAGE FROM GENENGNEWS.COM

Genes, which are made up of DNA, are like the set of instructions the body follows to make each living organism unique and different. According to the National Institutes of Health, while most human genes are identical among people, a small amount (less than one percent) differ in many ways. Though small, these differences have a big difference because it affects everything from physical traits to psychological characteristics.

Some people were born to develop certain diseases, such as diabetes, heart disease, and specific cancers, and other people have a more direct connection between genes and disease. Hemophilia, cystic fibrosis, and certain forms of blindness are the diseases caused by problems with just genes.

Image from American Gene Technologies (AGT)

In the 1980s scientists first began researching gene therapy to cure genetic disorders. Scientists now have a much better understanding of genes and the role they play in causing or preventing disease. They learned how to edit, remove, or return genes in a person's cells. One examples can be replacing a mutated gene with a functional copy or introducing a new or modified gene to help treat a disease. Many new cell and gene therapies that can treat conditions, such as cancer, cardiovascular disease, and eye disorders are in clinical trials or awaiting review by the FDA.

Editing genes in the lab is thought to sound easy but modifying a patient's genes really is much more challenging. What if the patient's body won't accept the treatment? Well today's gene therapies are sometimes packed in a deactivated virus, to target the correct cells delivering good genetic material into them. Once the new material combines with the existing genes, affected cells can begin producing the correct protein which fights/ cures the disease. With the potential impact on diseases, Companies are committed to making possibly life changing gene therapies a reality for more patients. With patients like Lindsay needs this. She was diagnosed with Canavan disease. Doctors didn't know much and did not know how to treat it. After "years of fustration, in 2001 a clinical trial" was conducted. It gave them hope and that is what this can do for many other kids and people.