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REVIEW ARTICLE

Gene Remedy: A New-Fangled Line of Attack to Pay for Sicknesses

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ABSTRACT

The average human body consists of trillions of small building blocks called cells. Within each of these cells, there are numerous genes. Genes contain the necessary genetic information to govern our bodily functions; as an example particular gene carries this unique "code" that is needed for the synthesis of this specific protein, which, in turn, exerts its defined biological role. Clinical trial of gene therapy has proven that it is capable of treating diseases such as hemophilia, leukemia, severe combined immune deficiency, and blindness caused by retinitis pigmentosa.

Keywords: DNA, Fangledline, Gene, Remedy, RNA

WHAT IS A GENE THERAPY?

The average human body consists of trillions of small building blocks called cells. Within each of these cells, there are numerous genes. Genes contain the necessary genetic information to govern our bodily functions; as an example particular gene carries this unique "code" that is needed for the synthesis of this specific protein, which, in turn, exerts its defined biological role. The code that is the specific DNA sequences of the gene can undergo alterations as a result of exposure to mutagens or due to the occurrence of spontaneous errors during replication. These alterations, also known as mutation, will often lead to cancer and various other genetic disorders that possibly harm the health of the individual. Recognizing this, scientists have been working on ways to modify and replace the defective genes to prevent treat and cure certain genetic disease instead of using conventional methods such as molecular therapeutics or surgery. The approach to gene therapy cans differ according to the genetic problem that is present. A mutated gene can either be replaced with a healthy copy of the mutated gene or its expression can be suppressed by employing RNA interference or genome editing tools. Although not yet proven in clinical trials, it is theoretically possible to correct a mutated gene in its precise location using genome editing.

VECTORS AS GENE DELIVERY VEHICLES

Inserting the modified or novel gene directly into the cell usually does not work. Therefore, a carrier called vector is used to carry the gene. An ideal vector should be able to transfer the specific genetic material to the target cell and allow the expression of the gene product without causing toxicity. Vectors can be classified as viral and non-viral. Viral vectors are viruses that are specifically modified before insertion so that they will not instigate the disease. Viral vectors can be further divided into two types, integrating and non-integrating viral vectors integrating vectors such as retroviral, lentiviral, and adeno-associated viral vectors are designed to integrate at one or more loci in the chromosomes and are

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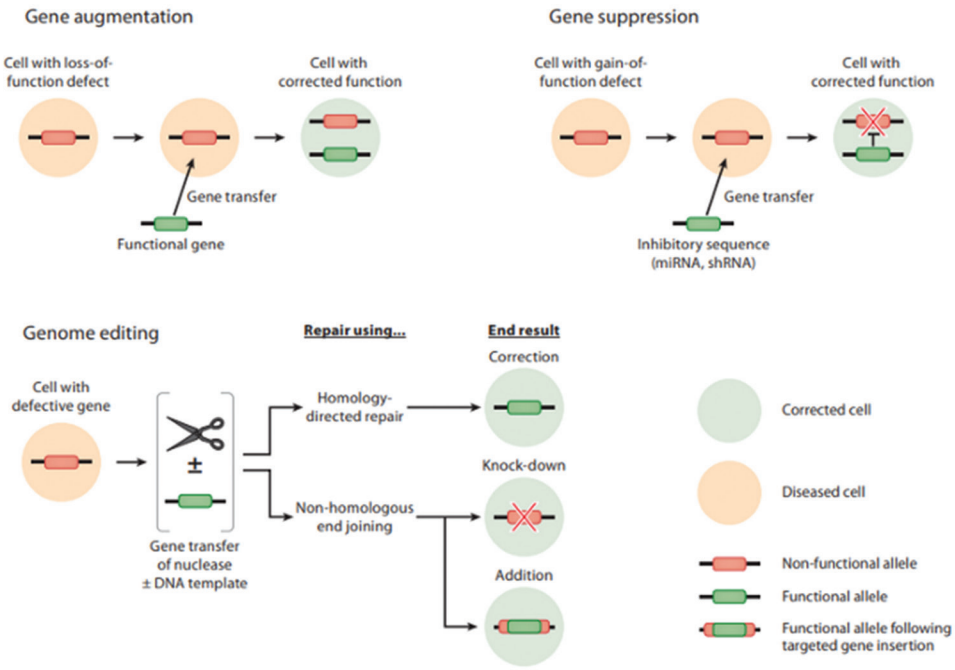


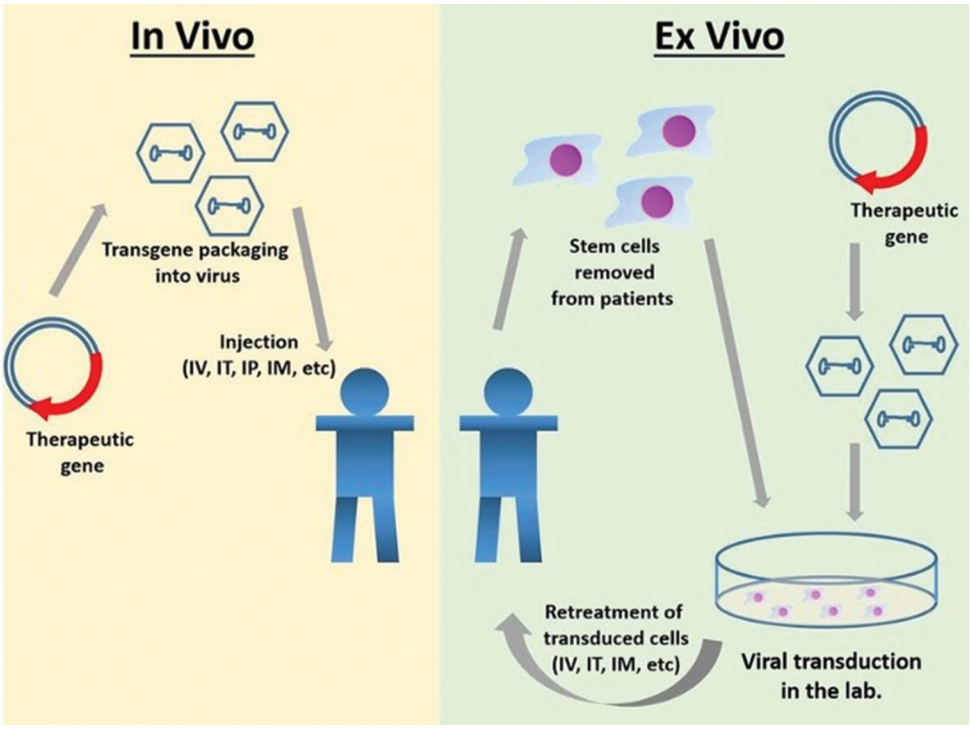
used when introducing genetic material into stem cells.

It ensures that the transferred DNA will be incorporated into the genome of the stem cells and then will be passed on to the daughter cells in subsequent cell divisions. Non-targeting vectors such as adenoviral vectors are used to deliver genes into long-lived post-mitotic cells and slowly dividing cells. Since these cells, in general, are no longer dividing, prolonged expressions of the gene can be achieved as long as the DNA is stabilized extra chromosomes. Non-viral vectors such as naked DNA and liposomes are simple and safer alternatives to viral vectors. Their relatively simple quantitative production and low-cost immunogenicity make them excellent vectors. Naked DNA vectors are usually in the form of a plasmid, which is a small circular, double strand DNA molecule.

The desire gene can be inserted into the plasmid and the recombinant plasmid can be inserted into the target cells in a variety of ways. Plasmid DNA can also be coated with lipids to construct structures such as liposomes or micelles. The use of gene guns is another novel technique for the transfer of a gene. This technique does not require the presences of complicated and potentially harmful delivery systems. Therefore, it is much safer than any kind of vector. In this, this technique micro projectiles (e.g., gold or tungsten) coated with DNA are shot into the target cell under high pressure and speed to ensure that it passes through the membrane barrier. The latest studies in gene therapy have proven that bacterial systems are capable of transferring genes to tumor cells. This technique relies on the ability of certain anaerobic bacteria to colonize and replicate in hypoxic areas (tumors), and thus, achieve expression of the desired gene in that specific area.[1-3]

IN VIVO GENE DELIVERY AND *EX VIVO* GENE DELIVERY

The major difference between *ex vivo* and *in vivo* gene therapy lies in the choice of the vector, and the way, it is inserted into the patient. In the *ex vivo* method, the cells from the patient are transduced with the gene of interested and are subsequently transplanted back. This process requires an integrating vector. On the other hand, the *in vivo* method is similar to the insertion of other pharmaceutical agents. Once the insertion is completed successfully, the transgene (or its protein product) will be stable expressed.[4-6]



SAFETY ASPECTS OF GENE THERAPY

Although gene therapy is a promising treatment option, several hurdles regarding its safety remain to be overcome before it becomes more common in clinical use. Most of the risks of integrating vector arise due to their potential for insertional mutagenesis, where it disrupts functional DNA elements in the cell such as gene. On the other hand, for vectors administered *in vivo,* the risks arise from immune response to vectors. Scientists have managed to reduce the effects of insertional mutagenesis by the production of safer (lentiviral) vectors and the effects of the immune response have been reduced through the use of adjuvant immunomodulatory drugs.

POSSIBLE THERAPY AREAS

Clinical trial of gene therapy has proven that it is capable of treating diseases such as hemophilia, leukemia, severe combined immune deficiency, and blindness caused by retinitis pigmentosa. More than 800 research studies are still underway to test gene therapy as a treatment for previously untreatable disease such as Duchene's muscular dystrophy and Huntington's diseases. At present, gene therapy is available only as part of a clinical trial. Gene therapy continues to be an active area of research as it holds the promise to revolutionize medicine and creates more options for patients suffering from incurable diseases.

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