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An Overview on Advances in Gene Therapy

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Introduction

Since the identification of the gene as the basic unit of heredity, the capacity to make site-specific alterations to the human genome has been a goal in medicine. Gene therapy is defined as the ability to change one's genetic condition by correcting altered (mutated) genes or making site-specific modifications that target therapeutic treatment. This treatment was made possible by developments in genetics and bioengineering, which allowed for the manipulation of vectors to transfer extrachromosomal material to target cells.

Description

The optimization of delivery vehicles (vectors), which are primarily plasmids, nanostructured, or viruses, is one of the main foci of this technique. Viruses are studied more frequently because of their ability to invade cells and inject genetic material. However, heightened immunological responses and genetic alteration, particularly in germ line cells, are major concerns. In vivo investigations in in somatic cells produced positive results in clinical trials using approved methods. Since the discovery of DNA as the basic unit of heredity, the ability to make local changes in the human genome has been a goal of medicine. Gene therapy is defined as the ability to improve genes by correcting altered (mutated) genes or site-specific alterations with therapeutic treatment as the goal. Currently, gene therapy is primarily practiced in research facilities, and its applicability is still in its early stages.

The vast majority of trials take place in the United States, Europe, and Australia. The treatment could be utilized for diseases caused by recessive gene disorders (cystic fibrosis, hemophilia, muscular dystrophy, and sickle cell anemia), acquired genetic diseases like cancer, and viral infections like AIDS. Recombinant DNA technology is a process in which a gene of interest or a healthy gene is put into a vector, which can be plasmodial, nano e-structured, or viral; the latter is the most commonly utilized due to its efficiency in infiltrating cells and introducing genetic material [1-3].

Despite the success of some procedures, the gene therapy process remains difficult, and many approaches require further research. It is necessary to identify and reach the specific bodily cells that require treatment. There must be a means to effectively deliver gene copies to cells, and diseases and their strict genetic ties must be fully recognized. There's also the problem of gene therapy's target cell type, which is now classified into two broad categories: germline gene therapy and somatic cell gene therapy. In germline gene therapy, functional genes are introduced into the genome of stem cells, such as those found in sperm and eggs. The changes are inherited and passed on through the generations. This strategy should, in theory, be very effective in the fight against genetic and inherited disorders. Therapeutic genes are transmitted to a patient's somatic cells in somatic cell gene therapy. Any changes and consequences are unique to that patient and are not passed down to future generations.

Gene therapy involves inserting a normal gene into the genome to replace an aberrant gene that causes a disease. One of the most critical difficulties in the process is releasing the gene into the stem cell. Thus, a molecular carrier known as a "vector" is used to release the gene, which must be very specific, efficient in the release of one or more genes of the sizes required for clinical applications, immune system-free, and purified in large quantities and high concentrations in order to be produced and made available on a large scale. Once put into the patient, the vector should not cause allergic reactions or inflammation; instead, it should improve normal functioning, rectify deficits, or prevent harmful activities. It should also be safe not only for the patient but also for the surroundings and the experts that work with it [4,5].

Conclusion

Finally, the vector must be capable of expressing the gene throughout the duration of the patient's life. Because of their tremendous potential for longevity and self-renewal, hematopoietic stem cells have become ideal targets for gene transfer. The manufacture of gene transfer vectors for the generation of induced pluripotent stem cells (iPS) in order to promote differentiation of the iPS and afford an extra phenotype from this differentiated derived cell is one example of this combination of gene therapy and stem cells. Patients who require a liver transplant due to chronic liver disease and hepatitis virus infection (e.g., hepatitis B virus and hepatitis C virus) are likely to get mature hepatocytes or those generated from iPS cells.

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