Review on Contributions, limitations and prospects in gene therapy.

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Abstract

Since the gene was identified as the fundamental component of heredity, the capacity to alter the human genome at precise sites has been a goal in medicine. Gene therapy is therefore defined as the capacity to change a person's genetic makeup through the repair of altered (mutated) genes or site-specific alterations that are intended to treat a medical condition. The development of genetics and bioengineering, which allowed for the manipulation of vectors for the transfer of extra chromosomal material to target cells, made this therapy conceivable. The optimization of delivery vehicles (vectors), which are mostly plasmids, nanostructured materials, or viruses, is one of the key areas of concentration of this method. Due of their prowess at invading cells and introducing their genetic material, viruses are more frequently studied.

Keywords: Gene therapy, Vector, Induced pluripotent stem cells, Hepatic transplantation.

Introduction

Since the discovery of DNA as the fundamental building block of heredity, the goal of medicine has been to be able to locally alter the human genome. The ability to enhance genes through the repair of misplaced (mutated) genes or site-specific alterations with therapeutic treatment as the aim is known as gene therapy. Different tactics that are frequently employed for this objective are explained in the following sections. Gene therapy is now a field that mostly exists in research labs, and its applicability is still being tested. Recombinant DNA technology is one of the most frequently used techniques. In this method, a gene of interest or a healthy gene is inserted into a vector, which can be plasmodia, nanoestructurally structured, or viral. The latter is most frequently used because it is most effective at invading cells and introducing its genetic material [1].

Gene therapy procedure: Gene release

A normal gene is put into the genome during gene therapy to replace a defective gene that is responsible for a particular illness. The difficulty in releasing the gene into the stem cell is one of the most important difficulties in the procedure. In order to release the gene, a molecular carrier known as a "vector" must be very specific, show efficiency in releasing one or more genes of the sizes required for clinical applications, not be recognised by the immune system, and be purified in large quantities and high concentrations so that it can be produced and made accessible on a large scale. Once the vector has been implanted, it cannot cause allergic responses or inflammatory processes; instead, it must enhance healthy functions, make up for inadequacies, or prevent harmful behaviours. Additionally, it must be secure not just for the patient but also for the surroundings and the experts using it. The vector should also be able to express the gene generally throughout the duration of the patient's life [2, 3].

Due to their tremendous potential for lifespan and aptitude for self-renovation, hematopoietic stem cells have emerged as perfect candidates for gene transfer. The generation of gene transfer vectors for the creation of induced pluripotent stem cells (iPS), in order to create the differentiation of the iPS and offer an extra phenotype from this differentiated derived cell, would be one example of this combination of gene therapy and stem cells. Hepatic transplantation of mature hepatocytes or those generated from iPS cells may be an option for patients who need a liver transplant and have chronic liver disease and hepatitis virus infection (such as hepatitis B virus and hepatitis C virus).Since the transplanted cells are prone to re-infection by the hepatitis virus, the transfer of a vector that encodes a short hairpin RNA directed against the virus would give the transferred cells resistance or "immunity" to re-infection. Gene transfer alone may not be sufficient to transform stem cells into hepatocytes. Over time, resistant cells might repopulate the liver and bring it back to its pre-infection state [4, 5].

Conclusion

The idea of genetically altering germlines has long been the subject of contentious debate in the scientific community. When new procedures are developed, bioethics is always there to evaluate the procedure's hazards and moral ramifications. Genetic treatment in somatic cells is widely accepted in the scientific community, particularly in situations of severe diseases like cystic fibrosis and Duchenne muscular dystrophy.

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***Received:** 27-Dec-2022, Manuscript No. AATR-22-86933; **Editor assigned:** 31-Dec-2022, Pre QC No. AATR-22-86933(PQ); **Reviewed:** 11-Jan-2023, QC No. AATR-23-86933; Revised: 18-Jan-2023, Manuscript No. AATR-23-86933(R); Published: 25-Jan-2023, DOI:10.35841/aatr-7.1.131*

***Citation:** Prajwal R. Review on contributions, limitations and prospects in gene therapy. Am J Transl Res. 2023;7(1):131*

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Citation: *Prajwal R. Review on contributions, limitations and prospects in gene therapy. Am J Transl Res. 2023;7(1):131*