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Review about gene therapy

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(بِسْمِ اللهِ الرَّحْمنِ الرَّحِيمِ)

﴿ قَالُوا سُبْحَانَكَ لَا عِلْمَ لَنَا إِلَّا مَا عَلَّمْتَنَا
إِنَّكَ أَنْتَ الْعَلِيمُ الْحَكِيمُ ﴾

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(الآية 32)

Supervisor Certification

I certify that this project entitled **(Review about gene therapy)** has been prepared by the students Aqeel Kamel Agel ,Muhannad Jassim Talib,Qasim Ahmed AbdAL- Kadhem, Zainab Allawi Hussein ,Maryam Haider Abbas Zahraa Ali Hussein ,under my supervision, in partial fulfilament of the requirement for the degree of bachelor's in medical laboratory technique.

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Dedication

I dedicated this study Report

To the purest heart to my role model, and my ideal in life; He is the one who taught me how to live with dignity and loftiness… My dear father

To the heaven of God on earth, to the bridge that ascends me to heaven, to my ideal… My mother

To the eyes and heartbeat You were and still my support, lean and fame in all stages of life. ...to … My brother & sisters

To anyone who helped me and was by my side throughout the study period, to everyone who was happy for my joy and prayed for me with all his heart,

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Summary

Gene therapy is a rapidly advancing field in medicine that holds great promise for treating genetic disorders and other diseases. It involves the introduction, alteration, or deletion of genetic material within a person's cells to correct or modify the underlying cause of a specific condition. While gene therapy is still an evolving technology, it has shown remarkable potential and has achieved some notable successes.

One of the key advantages of gene therapy is its ability to address the root cause of genetic diseases rather than merely treating symptoms. By targeting and modifying the faulty genes responsible for a specific condition, gene therapy aims to provide long-term or even permanent therapeutic benefits. This approach has the potential to transform the treatment landscape for various genetic disorders, such as cystic fibrosis, muscular dystrophy, and certain types of cancer.

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1. Introduction :

Gene name of which is gene therapy is a method of introducing a normal gene in an individual's genome to repair or reconstruct the mutated gene that caused the disease. A different chromosomal site from the defective allele is integrated by a normal gene when introduced into the nucleus of a mutant cell. This might restore the mutation, although a new mutation can arise if a normal gene gets integrated into an alternate functional gene [1].

The central concept of gene therapy is to treat genetic problems at their origin. If, for example a transformation caused in a particular gene comes out in the creation of a non-functional protein, then gene therapy can be used here to transport a replica of this gene without harmful mutation, and thus a functional protein can be produced. This approach is denoted as gene replacement therapy. The thought of gene therapy is confined not only to recessive ailments but is also capable of treating dominant conditions. The processes like bone marrow transplants and organ transplants cannot be called gene therapy as it is well-defined by the accuracy of intention and procedure of direct healing effect [2].

The aims of a study.

1-Assessing efficacy: One of the primary aims of gene therapy studies is to evaluate the effectiveness of the therapy in treating specific genetic disorders or diseases

2-Assessing long-term effects: Gene therapy aims to provide long-lasting or permanent therapeutic effects. Studies may aim to investigate the durability and long-term stability of gene expression and therapeutic benefits. This includes monitoring the persistence of introduced genes, potential immune responses over time, and the sustained correction of genetic abnormalities.

3-Evaluating safety: Safety is a crucial aspect of gene therapy research. Studies aim to assess the safety profile of the therapy, including the potential for adverse effects, immune reactions, or unintended consequences. The goal is to ensure that gene therapy interventions are safe for patients and do not cause harm.

4-Exploring new applications: Gene therapy has the potential to be applied to a wide range of genetic disorders and diseases. Studies may aim to explore new applications of gene therapy, identifying novel targets or expanding the understanding of gene therapies in different therapeutic areas

2. History Of Gene Therapy

During mid1960s, researchers estimated that DNA groupings could be embedded into patients' cells to treat hereditary diseases. It was Martin Cline that initially endeavored to adjust human DNA in 1980, anyway the first fruitful result of atomic quality was seen after a long stretch finally in May 1989 [3].

The main helpful use and furthermore the initial direct addition of human DNA into atomic genome was accomplished in September 1990

by French Anderson. In the year 1990, 4-year-old Ashanthi de Silva turned into the principal quality treatment example of overcoming adversity. She was brought into the world with an extreme joined immunodeficiency (SCID) because of the absence of protein adenosine deaminase (ADA). In absence of ADA, her T cells died off, making her inadequate to battle contaminations. Infusions of an engineered ADA compound aided, however just immediately. Specialists chose to convey a relatively solid ADA quality into her platelets, by the utilization of an impaired infection that can't spread in the body. The achievement they accomplished empowered more preliminaries for a similar type of SCID during the 1990s. Presently in her 30s, de Silva is loaded with life even after having an uncommon sickness. Between the hour of 1989 to December 2018, a bigger more than 2,900 clinical preliminaries were led, with the more significant part of them in the stage 1. Starting at Spark Therapeutics' Luxturna in 2017(for visual deficiency prompted by RPE65 Mutation) and Novartis' Kymriah (antigen T cell treatment of chimeric receptor) are principal quality treatments to enter the market endorsed by the FDA [4].

Since that time, medications like Alnylam's Patisiran and Novartis' Zolgensma have likewise gotten the backing of the FDA, notwithstanding other organizations' quality treatment drugs. The vast majority of these techniques use AdenoAssociated Virus (AAVs) and lentivirus for executing quality inclusions, ex-vivo and in-vivo individually [5].

3. Types Of Gene Therapy

It becomes essential to understand some of the terminologies and the types of gene therapy. Alternative, Deoxyribonucleic Acid (DNA)-based therapy and molecular therapy are often used by laymen and

biologists. Gene therapy is one of the genetic engineering tools used to alleviate suffering from hereditary diseases [6].

However, in broader terms, gic engineering not only aims to alter genes to correct genetic defects but may also be involved in modifying the genes to enhance the organism's abilities beyond what is expected. The latter is a dangerous proposition of genetic engineering. A. Gene therapy can be classified into the somatic cell and germ cell types, depending upon the type of cells that are modified by the therapeutic genes [7,8].All the gene therapies to date are directed towards somatic cells only.

3.1 Somatic Cell Gene Therapy

In this type, genetic changes are directed towards somatic cells. As these cells are non The reproductive effect is not passed into future generations, making it safer. The disadvantage is the short duration of the effects of somatic cell therapy, as most tissues will be replaced by new tissues.

3.2 Germ Cell Gene Therapy

This is the type of gene therapy where germ cells, i.e., either sperm or ova, are introduced with therapeutic gene, leading to inheritable changes, i.e., changes in the gene may affect future generations. B. Based upon the technique of delivery of vectors to the target cell, gene therapy can be further classified into ex-vivo and in-vivo therapy.

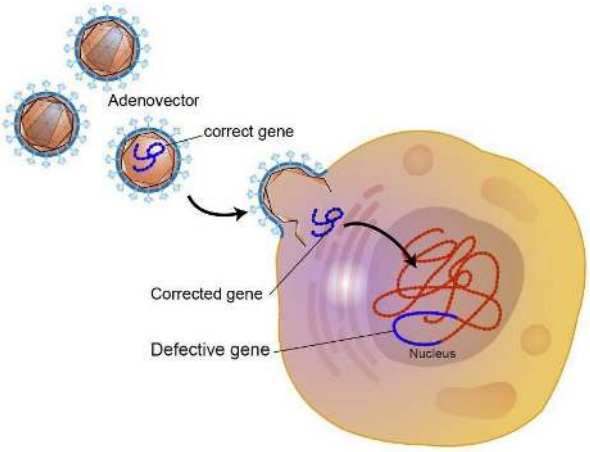


Fig. 1. The concept of gene therapy

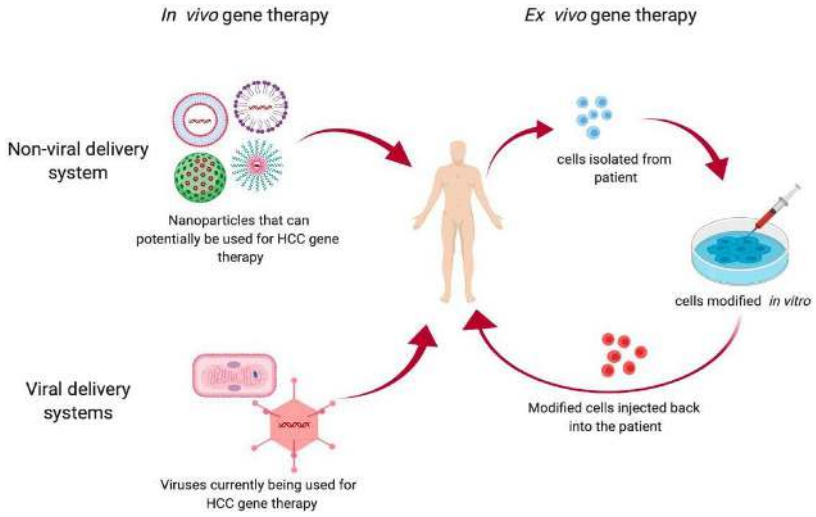


Fig. 2. Gene therapy types based on the technique of delivery

Ex -vivo gene therapy is where the defective cells are extracted from the body and targeted with a therapeutic gene. Once successfully modified, they are cultured ex-vivo and transferred back to the host, where now the corrected gene replicates.

3.3 In-vivo Gene Therapy

In this modality, a vector capable of carrying the therapeutic gene injects host cells with a normal genes. C. The type of change in the faulty gene classifies gene therapy as either gene replacement or gene addition.

3.4 Gene Replacement

Gene replacement means the replacement of defective gene with a corrected one.

3.5 Gene Addiction Therapy

Gene addition means restoring the normal function of cell by adding normal or functional copy of gene into genome. This concept is primarily used in various gene therapy related research on cancer. It is important to understand some terms that are commonly associated with gene therapy, because laymen and biologists often use terms such as genetic engineering, molecular therapy or DNA-based therapy. Many genetic engineering tools that can help alleviate genetic diseases are available, Gene therapy is one of them [7].

A. It can be classified on the basis of modification of cells by therapeutic genes into gene therapy of somatic cell and gene therapy of germ cell.

3.6 Gene therapy of Somatic cell

Genetic changes in somatic cells are targeted in this type. It is comparatively safer as somatic cells are non reproductive, so the effect will not be passed on to the off springs.

3.7 Gene therapy of Germ cell

Genetic changes in germ cells are targeted in this type. In this type of gene therapy, genetic changes are heritable because the germ cells (either egg or sperm) are introduced into the therapeutic gene. This type can affect future generations.

B. It can be classified on the basis of delivery technique of vectors into target cells as Invivo or Ex-vivo

3.8 Gene Therapy of Ex-vivo Type

In this method defective cells are taken out from the body and are targeted with gene of therapeutic activity. Once the cells are altered successfully, they are cultivated in ex-vivo conditions and are transported back to the host from where replication of modified gene takes place.

3.9 Gene Therapy of In-vivo Type

In this type of gene therapy a vector is injected into the host which carries the healing gene to the host cells.

C. It can be classified based on the type of changes that are brought in the defective gene into gene replacement and gene addition therapy

3.10 Gene Replacement

In this method, the faulty gene is replaced with a modified one.

3.11 Gene Addiction Therapy

In this method, regular function of the cell is restored by the addition of a functional or standard copy of the gene into the genome.

Above mentioned type is principally used in research related to gene therapy [8].

4. Prerequisites For Gene Therapy

Fundamentals for gene therapy consist of choosing the most suited delivery system for the gene (usually a virus, often called a viral vector), indicating that the transported gene can be expressed in the host cell, also defining that used technology is harmless. Very few human gene therapy clinical trials can pass all of these conditions, generally due to the delivery system not reaching the cell or because the cell does not express the gene. Nanotechnology is being used to develop better-quality gene therapy systems. One hopeful application of this research involves targeting cancer cells by packaging genes into nanoparticles, thereby precisely killing cells causing cancer and protecting healthy cells from damage [9].

5. Vectors For Gene Therapy

A cell-carrying substance called a vector can be used for delivering cells to DNA by various methods. The two main categories are nonviral and viral vectors.

5.1 Viral Vector

During the replication process, the virus introduces into the host cell its genetic material, enticing the cellular apparatus of the host to use it as a blueprint for viral proteins. The retrovirus takes it a step further, in which the genetic material is copied into the host cell genome. The advantage of this is taken by scientists by placing healing DNA in place of the genetic material of the virus. (Some viruses have RNA as genetic material, so gene therapy can also use RNA.) Numerous viruses are being

utilized in human gene therapy, including adenoviruses, herpes simplex virus, retrovirus, and adenoassociated and vaccinia viruses. Like the hereditary substance (DNA or RNA) of a virus, remedial DNA can be used simply as a transitory outline, either naturally degraded or (in theory at least) entering the host's genome, turning into an everlasting part of the DNA of the host in the infected cells [10].

5.2 Non-Viral Vectors

Big-scale manufacture and lower host immunogenicity are advantages of non-viral vectors over viral vectors. However, the nonviral methods first produce minor gene expression and transfection stages and therefore have lesser therapeutic effects. After the onset of subcellular transport control and cell-specific targeting, new technologies can solve these problems. Gene gun, nonoperation, naked DNA injection, magnetotransfection, electroporation, oligonucleotides, lipoplexes, inorganic nanoparticles, and dendrimers are methods of non-viral gene therapy [11].

Newer methods, such as those implemented by companies such as Ligandal, provide opportunities to produce targeting technologies that are cell-specific for various gene therapy methods, including some gene excision tools such as RNA, DNA, and CRISPR. Some other companies, such as Arcturus Therapeutics and Arbutus Biopharma, provide non-targeted and non-viral methods that mainly incluthe de liver nutrthe ition. Recently, startups like GenEdit, Spotlight Therapeutics and Sixfold Bio have commenced to resolve the problem of nonviral gene delivery. Benefit of non viral methods is that it provides opportunities for repeated administration and greater adaptability of gene payloads, which will replace virus-based delivery systems in the future. Companies including

Intellia Therapeutics, Editas Medicine, CRISPR Therapy, Cellectis, Casebia, Precision Biosciences, Sangamo and bluebird bio have invented non-viral gene editing technologies; though, they usually even now use viruses after being guided by nucleases for genome cleavage to carry the gene insertion material. The above mentioned companies focus on gene editing, but even though they face major delivery problems [12].

Moderna Therapeutics, and CureVac and BioNTech pay attention on the transport of mRNA payloads, that are usually non-viral transfer problem. Ionis Pharmaceuticals, Alnylam and Dicerna Pharmaceuticals, emphasize the transport of siRNA (antisense oligonucleotides) to supress gene, which also requires non-viral delivery systems.

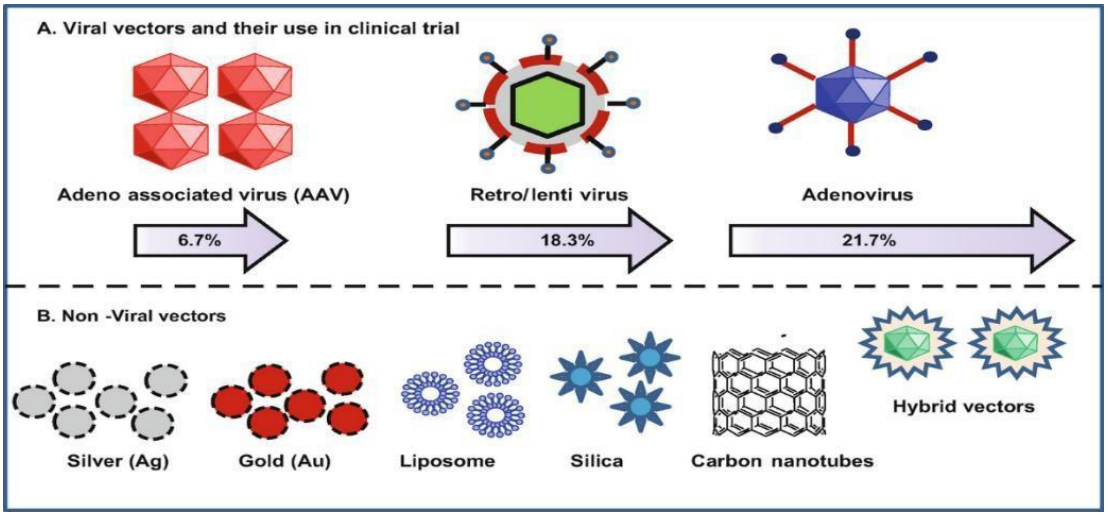


Fig. 3. Types of vectors in gene therapy

6. Approved Gene Therapy Products

Table 1. A list of already approved gene therapy products Product

Product	Indication	Company	Viral Vector	Delivery	Status
Luxturna	Retinal dystrophy	Spark Therapeutics (Roche)	Adeno associated virus	In vivo	Approved in United States
Zolgensma	Spinal muscular atrophy	AveXis (Novartis)	Adeno associated virus	In vivo	Approved in United States
Kymriah	B-cell lymphoma	Novartis	Lentivirus	Ex vivo	Approved in United States
Yescarta	B-cell lymphoma	Kite Pharma (Gilead Sciences)	Lentivirus	Ex vivo	Approved in United States
Zynteglo	Thalassemia, Sickle Cell Disease	Bluebird Bio	Lentivirus	Ex vivo	Approved in Europe
Collategene	Critical Limb ischaemia	AnGes	Plasmid	In vivo	Approved in Japan
Liso-cell	B-cell lymphoma	Bristol-Myers Squibb	Lentivirus	Ex vivo	Biologics Licence Application (FDA)
OTL-200	Metachromatic Leukodystrophy	Orchard Therapeutics	Lentivirus	Ex vivo	Market Authorization Application (EMA)
Valrox	Haemophilia -A	BioMarin	Adeno associated virus	In vivo	Biologics Licence Application (FDA)

7. Applications Of Gene Therapy

7.1 Cancer

Gene therapy-related research and its clinical application have been mostly utilized in the field of malignancy. By the end of 2009, nearly two third of gene therapy-related research was concentrated on cancers (8).

Oncolytic viruses are used to introduce genes into malignant cells, thereby causing death of Gene Therapy in Iraq- Current Status malignant cells. Another approach is to deliver p53 gene (tumor suppressor gene) and thereby induce oncolysis. Gendicine that was first approved

anticancer drug which was based on this gene therapy principle. Suicide gene therapy is another attempt to treat tumor by delivering of gene coding for enzyme that metabolizes prodrugs into locally active chemotherapeutic drug moiety. Research related to gene therapy and its clinical presentation have been generally applied in the concept of malignancy. Cancer amounts to nearly two-third of all gene therapy-related research being conducted. Oncolytic viruses are being used to administer genes into the malignant cells, thus producing death of the involved malignant cell. A different strategy is to administer p53 gene (tumor suppressor gene) inducing oncolysis. The first permitted anticancer drug which was created on this principle of gene therapy was Gendicine . Suicide gene therapy is one more effort to cure cancer by delivering of gene coding for enzyme that metabolizes prodrugs into locally active chemotherapeutic drug moiety [13]

7.2 Single Gene Disorder

There are numerous single gene disorders namely alpha-1-antitrypsin deficiency, cystic fibrosis, muscular dystrophies, lysosomal storage disease, chronic granulomatous disease, Huntington's disease, junctional epidermolysis bullosa, haemophilia , ornithine transcarbamylase deficiency, in which an important role is played by gene therapy for their treatment.

7.3 Immunodeficiency

It has been years since the improvement of gene therapy started but the first remarkable development seen ever since the first trial was in early 90s. During the preliminary set back where two patients had died after being treated for Xlinked severe combined immunodeficiency (XSCID) using retroviral vectors due to leukemia, even though there were clinical trials that showed strong therapeutic benefits of gene therapy in management of both X-SCID and SCID caused by the deficiency of adenosine deaminase (ADA). Secondary immunodeficiency states like Human Immunodeficiency Virus (HIV) in addition to primary immunodeficiency infection has likewise grew as a probable contestant for gene therapy. For specific defence against HIV infection to these cells, Transgenes can be transported into haematopoietic stem cells or into T-cells. They make the milieu unsuitable for HIV1replication or disable the HIV-1 protein [14].

7.4 Eye Diseases

After the preliminary set back seen in SCID the regeneration of trust in gene therapy occurred due to Leber's congenital amaurosis. Eye is a small organ, hence there is possibility of transfecting a great amount of ocular cells. Leber's hereditary optic neuropathy, glaucoma, macular degeneration and red-green colour blindness are clinical ophthalmologic conditions for gene therapy. A phase I study is going on to treat age-related macular degeneration which shows effects of antiangiogenic cytokine Pigment Epithelium-derived Factor (PEDF). By injecting subretinal injections of adeno-associated virus containing a L-opsin gene, significant improvement is seen in creating trichromatic colour vision in adult red-green colour-blind monkeys [15].

7.5 Cardiac Diseases

Cardiac diseases are difficult to treat as they are multigenic in origin. Trials are being conducted in which scientists have developed methods to transport genes for different growth factors like Fibroblast Growth Factors (FGF), Vascular Endothelial Growth Factors (VEGF) to encourage vascular angiogenesis. However their outcomes did not display remarkable enhancement in stress-induced myocardial perfusion but enhanced regional wall motion indicated a favourable anti-ischemic effect inspiring more research in this field.

7.6 Central Nervous System (CNS) Disorders

Gene therapy has revealed hopeful results in neurological disorders to cure Alzheimer's and Parkinsonism disease unlike cardiac diseases. Several trials are being conducted on gene therapy in Parkinsonism which are now either in phase 1 and phase 2 but are screening gene therapy to be potential , harmless and tolerable for in-vivo studies. Numerous methods used are, delivery of the gene in putamen cell bodies for neurturin or transmitting the gene into the subthalamic nucleus for glutamic acid decarboxylase. Similarly in Alzheimer's disease delivery of nerve growth factor into the CNS is being attempted using gene therapy[16].

8. Gene Therapy In Iraq

Monetary assistance given by different government agencies has helped the nation to achieve speedy development in research associated to gene therapy. In the context of gene therapy laboratories, Iraq ranks third among Asian countries. The main goal should be to invent new research institutions for gene therapy, while solidifying currently present

institutions with better experience in molecular genetics to reduce the load of genetic diseases in Iraq. The Advanced Cancer Treatment, Research and Education Center (ACTREC) is a pioneer in gene therapy-related research in our country. The center is using synthetic vectors to conduct research on head and neck cancer. It should be pointed out that Iraqn scientists are working hard day after day to contribute to the development of gene therapy in Iraq [17].

Hareendran et al. suggested that in order to weaken the immune barrier, one should target specific cellular host proteins, which are a key obstacle in the promotion of the clinical application of gene therapy by adeno-associated viruses. Kochat et al and his team studied the use of allogeneic liver transplantation. In vitro, regulatory T cells triggered by allogeneic antigens can induce tolerance to donor antigens. Shetty et al have shown that by designing a genetically modified technique to express human orthologs of the Asrij protein, immature stem cells can be generated as pluripotent cells. Asrij protein is present in mouse embryonic stem cells, it is important for sustaining pluripotency. Misra et al are working on a possible strategy to use selective gene transfection to rule out nontarget gene toxicity and expression. To overcome the barriers formed by various layers of the skin to treat skin diseases Vij et al. have used nucleic acid therapy as an efficient local delivery system. Kumar et al are committed to alternative and effective nucleic acid transport. The effectiveness of a combination of BH3 mimics and a new type of cancer terminator in the treatment of advanced prostate cancer is studied by Sarkar et al [18].

9. Future Of Gene Therapy

Dr. Michael Gottesman (NIH) is working on the growth of a dominant selectable marker which is to be used in gene therapy. An international combined work to produce high quality genetic maps of the human genome and also the wideranging DNA sequence is known as The Human Genome Project. Main intention behind this is to recognize and arrange all of the genes that have an important function in human disease and human health. In future it is expected to be more cost effective. There has been significant progress in the production of maps of the genome. Due to increasing accuracy of map information, isolating the genes responsible for human genetic diseases upto 4000, which are inherited in a Mendelian style by using a positional cloning technique is made possible. The technology involves mapping disease genes by linking disease occurrence in the family with identified DNA markers, and then separating the gene from DNA clones that span the region of interest[19].

The genes secluded in this way include those used for Huntington's disease, Duchenne muscular dystrophy and CF. It has been predicted that hundreds of these disease causing genes would be isolated in the next few years, so that gene therapy can cure or be expected to treat these diseases. In different diseases that incline to be inherited in the family, such as diabetes, cancer or heart disease, the inheritance is even more complicated. The reason for seems to be the affected susceptibility by various environmental factors and genes. In this case, the plotting tools of the Human Genome Project might be used to identify susceptible genes. This information will help predict the possibility of disease development and targeted interventions (including somatic gene therapy) to decrease the severity or likelihood of the disease. Laboratory of Dr. Gottesman's focuses on the problem of missing expression of transferred

genes, which do not have any selective advantage over target cells. One way to solve this problem is to associate the gene of interest with the gene encoding the selectable marker which is dominant.

One of those genes, MDR1, encodes a 1280 amino acid protein that acts as an energy-based pump conferring resistance to many cytotoxic capsules utilized in most cancers treatment. It does this via way of means of binding capsules to the mobileular membrane (or cytoplasm) and expelling them from the mobileular. dr. Gottesman and his colleagues have correctly added MDR1 into bone marrow cells and feature proven that it's miles drug resistant and does now no longer look like poisonous to goal cells. In different experiments, his lab connected the MDR1 gene to genes that reason sickness in people and proved the expression of those genes. Using MDR1 as a choice marker has numerous advantages.

It is probably relevant to somatic cells and hematopoietic stem cells, consisting of muscle, pores and skin or liver cells, and may be utilized in vivo and in vitro. Because the MDR1 gene product is expressed at the cellular surface, monoclonal antibodies may be used to pick out and classify cells that explicit it. Since the pump can apprehend masses of reagents, it's miles feasible to pick a reagent to choose transfected cells with out trendy systemic toxicity. In addition, the advent of factor mutations withinside the MDR1 gene might also additionally lessen pump specificity and enhance the ability to choose agents with useful properties. We are keen to switch this approach from the lab to the clinic [20].

10. Safety And Ethical Issue

From the time since first gene therapy clinical trial was accepted in 1988 and launched in 1989, more than 3,000 patients have received gene

therapy. Most of the early security concerns raised in early trials still exist till date. Some of them can be broadly classified as related to the expression of transfer vehicles or transferred genes. Viruses are used to transfer the expression of genetic material into cells in most of the clinical trials. Inflammation or active infection can be caused by administration of viruses. In a study by University of Pennsylvania, personally experienced the risk of excessive inflammation caused by administration of viruses. It caused death of a participant who was 18 years old.

Second, infections with unrestrained activity can occur by multiple recombination events (since currently the design is unlikely) or contaminate stocks of viruses that do not replicate through helper viruses. There are no known cases of transmission of infected viruses to patients. Obviously, testing the materials used in clinical trials is extremely important and very regular. Third, the administration of retroviruses haphazardly incorporated into the genome can lead to insertional mutations and malignant alteration [21].

As stated above, autologous stem cell transplantation infested with a retrovirus stating the defective gene is used to treat severe combined immunodeficiency, resulting in a reduction in patients' symptoms. However, 2 out of 11 patients aquired T-cell leukemia due to the virus integrated downstream of the oncogene, causing it to be up-regulated. The expression of several healing genes makes patients prone to adverse reactions. As stated above, the use of growth factors to treat neurodegenerative diseases or the usage of proangiogenic molecules to treat CAD can encourage tumor development. Similarly, the appearance of proinflammatory cytokines used to treat malignant tumors can lead to abnormal inflammatory problems. Though with the insertion of any therapeutic agent, side effects are associated the total incapability to

withdraw drugs delivered through gene therapy is predominantly problematic. Lastly, theoretically there is danger of accidentally disrupting germ line cells. This event has been testified in animal models, but has not yet been precisely defined after human insertion. The possibility of genetically modifying germ lines has long been a hot topic in the scientific community. When creating new technologies, bioethics always exist to evaluate the risks of the process and the honest impact it brings. Somatic gene therapy is recognized by most people in the scientific community, especially for serious diseases such as Duchenne muscular dystrophy and Cystic Fibrosis.

Chinese researchers had gone past ethical issues and for the first time announced the use of CRISPRCas9 technology to genetically modify embryonic cells. Then a different Chinese team also conveyed the same procedure of inserting mutations in the CCR5 gene to confer HIV resistance. It showed that 4 of the 26 embryos were successfully modified according to the genetic analysis. The results evidently reveal the requirement for improved technology and remind people that such experiments can be tested in animal models sooner [22].

These fresh publications have reignited the debate about gene editing. On the one hand, the Ethics Committee of Japan announced that the experiment was carried out in the correct way, because the research carried out has been permitted by the home-grown ethics committee and the consent of the egg donor has been obtained. In the UK, the first healthy human embryo deletion project was approved. On the other hand, the US research team remains traditionalist, restating their position that they do not support such experiments and stating that they are awaiting improvements in defining technical and ethical issues [23-28].

Conclusion :

In deduction, gene therapy remains to offer great hope for the treatment of diseases for which there are ineffective forms or no cure present. The simple model of gene delivery has remained the same in vivo and in vitro. However, the techniques for delivering genetic material in these models are highly flexible, and new and revealing technologies continue to emerge. Currently, clinical trials based on gene therapy are ongoing in many important disease procedures and rare inherited diseases, which are generally well defined. With the continued advancement of basic science in the pitch of gene therapy, more efficient clinical trials will be launched one after another, and will at the end efficaciously cure and even treat patients. When this happens, all the clinicians would adapt with these gene therapies to treat their patients. So, we can say that gene therapy has a great future ahead.

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