Xournals

Academic Journal of Biotechnological Research

ISSN UA | Volume 01 | Issue 01 | May-2018

Human Gene Therapy: An Overview

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Available online at: www.xournals.com

Received 25th November 2017 | Revised 19th February 2018 | Accepted 18th March 2018

Abstract:

The concept of transferring genes to tissues for clinical applications has been discussed for nearly half a century and advancement in biotechnology have brought gene therapy to the forefront of medical research. The transfer of genetic material to cure diseases or to improve the clinical status of a patient is known as Gene therapy. One of the basic concepts of gene therapy is to transform viruses into genetic shuttles, which will deliver the gene of interest into the target cells. Based on the nature of the viral genome, these gene therapy vectors can be divided into RNA and DNA viral vectors. Gene transfer protocols have been approved for human use in inherited diseases, cancers and acquired disorders. Gene therapy has not offered any permanent cure to any human patients, a breakthrough may come anytime. This paper discusses overview of gene therapy and the areas of significant development and progress in this field with the purpose of identifying strong trends in both research and practice activities.

Keywords: Gene Therapy, Clinical, Diseases, Biotechnology

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Introduction

"We used to think that our fate was in our stars, but now we know, in large measures, our fate is in our genes" -James Watson

Genes the functional unit of heredity, are specific sequences bases that encode instructions to make proteins. If there is any type of alternation in gene, encoded proteins are unable to carry out their normal functions, resulting in genetic disorders **(Mishra, 2013).** Gene therapy is basically defines as nucleic-acid based treatment, or transfer of DNA/RNA to somatic target cells in the intention to treat serious illness'. It is basically to correct defective genes responsible for genetic disorder.

The basic concepts of gene therapy is to transform viruses into genetic shuttles, which would deliver the gene of interest into the target cells. Based on the nature of the viral genome, the vector of gene therapy could be divided into RNA and/or DNA viral vectors. From retroviruses like murine leukemia virus the majority of RNA virus based vectors have been derived. The most commonly used DNA virus vectors are based on adenoviruses and adeno-associated viruses (AAVs) **Akhtar et al. (2011**).

History of Gene Therapy

As humans have understood that characteristics traits of parents could be transmitted to their offspring. The scientific study of genetics began in 1850s, when Austrian monk Gregor Mendel, in a series of experiments with green peas, described the pattern of inheritance. In 1950s, American biochemist James Watson and British biophysicist Francis Crick developed their revolutionary model of double stranded DNA helix which is the key breakout and the another discovery came in the early 1970s, when researchers discovered a series of enzymes that made it possible to snip apart genes at predetermined site along a molecule of DNA and glue them back together in a reproducible manner. Those genetic advances set the stage for the emergence of genetic engineering, which has produced new drugs and antibodies and enabled scientists to contemplate gene therapy.

At the National Institutes of Health's Clinical Center, Bethesda, Maryland. A girl was treated on 14th September, 1990, by Dr. W. French Anderson and his colleagues at the health center, carried out the proceedings. Extraction of white blood cells was done from the body and the genes that produce ADA was implanted and then the cells were again transferred back to the girl's body. In the immune system of the girl considerable improvement was noticed. Meanwhile, the trials of gene therapy continued on various diseases. The patients with skin cancer, melanoma were treated by means of gene therapy.

Process of Gene Therapy

Gene therapy is a very complex techniques and further development is needed in this therapy. The main challenge is development of gene therapy for specific condition. The condition in question must be well understood, the undying faulty gene must be identified and a working copy of the gene involved must be available. Specific cells in the body requiring treatment must be identified and are accessible. A means of efficiently delivering working copies of the gene to the cells must be available. Moreover diseases and their strict genetic link need to be understood thoroughly.

Types of gene therapy

There are 2 types of gene therapy.

1. Germ line gene therapy: Introduction of functional genes is done and the germ cells (sperm or egg) are modified which are integrated into their genome. Therefore due to therapy changes would be heritable and would be passed on to later generation. In counteracting genetic disease and hereditary disorders, this approach should be highly effective. But at present many jurisdictions, a variety of technical difficulties and ethical reasons make it unlikely that germ line therapy would be tried in human beings in near future.
2. Somatic gene therapy: in this therapy therapeutic genes are transferred into the somatic cells of a patient. Any modifications and effects will be restricted to the individual patient only and will not be inherited by the patient's offspring or any later generation.

Review of Literature

Ioannou, (2001) one of the most potent tools of medicine for the treatment of genetic and somatic gene diseases is gene therapy. The problems associated with the design, delivery and fate of therapeutic constructs into patient cells have been grossly underestimated and oversimplified, leading to unrealistic and overoptimistic expectations. The completion of the sequencing phase of the Human Genome Project is expected not only to catalyze the development of effective therapies for many diseases in the first half of this century but also to provide a solid basis for the biological liberation of mankind by the end of the twenty-first century**.**

Touchefeu, *et al.* (2010) the most commonly reported side effects related to gene therapy fever, headache, fatigue, nausea and vomiting. In trials using replication-competent agents, viruses were found in non-injected tumors and regression of both injected and distant disease occurred. This effect could be related to targeting of disseminated tumors by the vector and to local and systemic immune responses. Gene therapy will need to be positioned in the context of existing cancer therapy strategies. In all the therapeutics settings (neo-adjuvant, adjuvant and advanced disease), good safety records could make gene therapy a good candidate. Combining gene therapy with chemotherapy or radiation therapy may be very interesting, as gene therapy can act as a chemo-sensitizer or radio-sensitizer, and chemotherapy and radiation therapy can improve gene transfer efficiency and gene expression.

Akhtar, *et al.* (2011) many exciting innovations are emerging in the area of gene therapy. The therapy and bio products might yet have unknown risks, but they also have the potential for tremendous patient benefit. Many trails of gene therapy have been completed and many are in progress. For the treatment of genetic and acquired diseases viral vectors are used.

Handy, Krudy and Boulis, (2011) for the treatment and management of cancer-related pain Gene therapy is an exciting prospect. Advances in viral vector design and increased understanding of the signaling systems that underlie pain generation, transmission, and maintenance have helped to establish gene-based approaches as a potentially effective means to modulate nociception in a number of diseases. Under tumorigenic conditions the application of gene therapy tools using cancer pain laboratory models provides substantial insight into the molecular and biological features of virus-derived vectors. The development of viral vector-based therapy techniques is timely and necessary required for the high occurrence of pain among the cancer patient population.

Patil, *et al.* (2012) Different types of genetic disorder are cured by gene therapy case of cystic fibrosis, Diabetes, AIDS, Hepatitis melanoma, Alizhmer, Parkinson's diseases etc. One trial is done in case of Parkinson's disease Neurologix a biotech company announced that they have successfully completed its landmark Phase I trial of gene therapy for Park-inson's Disease.

Misra, (2013) in future and will change our lives forever. Genes may eventually be used as medicine and given as simple intravenous injection of gene transfer vehicle that will seek our target cells for stable, site-specific chromosomal integration and subsequent gene expression. The field of medicine gene therapy is rising scientist believed that in future gene therapy will be the last cure of every genetic disease. On the Simplification of procedure of gene therapy will decide how widely this therapy be used. This therapy will play important part in future and will change our lives forever. Genes may eventually be used as medicine and given as simple intravenous injection of gene transfer vehicle that will seek our target cells for stable, site-specific chromosomal integration and subsequent gene expression.

Hasan and Saini, (2014) the permanent solution for genetic diseases is gene therapy. Depending on how the therapy is applied Gene therapy is both beneficial and harmful. Gene therapy is to cure someone who is born with a genetic disorder or who develops deadly diseases like AIDS, cancer etc. to encourage the applications of gene therapy the government, the public groups and the scientific society should cooperate and walk hand in hand.

Wirth and Herttuala, (2014) to gain a therapy effect Gene therapy aims to deliver genetic material into target cells or tissue and to express it. Mostly gene therapy are single type applications and cost effective. It can be administered locally, thereby delivering, locally, a high therapeutic dose without risking systemic adverse effects so this is the main advantage of the gene therapy over conventional therapy.

Ruiz and Santos, (2015) for breast cancer gene therapy is increasing and the main participants in this field are USA and Canada in North America, China, Japan and South Korea in Asia, and England, Germany, and Italy in Europe. A total of 2,043 items are published and 947 patents from 1994 to 2013 including "gene therapy for breast cancer" were retrieved. In publications and patents, adenovirus- and retrovirus-based gene therapies and small interfering RNA (siRNA) interference therapies were the main topics.

Conclusion

Gene therapy is one of the most emerging therapy for the treatment of genetic and somatic gene diseases. It is contradictory that it gives permanent solution of genetic disorder or not, But it is beneficial as well as harmful depending upon that how it be applied. Gene therapy is able to cure someone who is born with a genetic disorder or who develops deadly diseases like AIDS, cancer etc. But it is not as simple as it appears since gene therapy has several inbuilt complexities. Many numbers of gene therapy trials have been completed and still more development in this field must be needed.

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