Introduction

Gene therapy is the direct use of genes as therapeutic agents. Such technology allows for the possible treatment and cure of genetic diseases such as vascular diseases, neurodegenerative disorders, myopathies and Parkinson disease **(Kay et al., 2001).**

Gene therapy is based around the concept of replacing a faulty gene, which causes a disease, with a normal gene. This normal gene can then carry out the function of the faulty gene, thus eliminating the cause of the disease. Gene therapy is relatively new in the field of medicine. The goal of gene therapy is to treat and/or cure genetic diseases by the survival of genetically engineered cells **(Russell and Stephen, 2004).**

Gene therapy can be divided into two main areas:

1-Somatic gene therapy: It relates to the correction of genes in most of cell types within the body, such as muscle cells and brain cells. This is the area where most of the work on gene therapy is being done.

2-Germ line gene therapy: It concentrates on the correction of genes in the ova or sperm.

There are two main approaches to this treatment:

1-In vivo gene therapy, which genes are delivered directly to target cells in the body.

2-Ex vivo gene therapy, in which the target cells are genetically modified outside the body and then reimplanted **(Lyerly and DiMaio, 1999).**

Stem cell transplantation, it is specialized ex vivo gene therapy perhaps one of the most remarkable advances for the field of CNS gene therapy has been identified. Isolation of neural stem cells (NSCs) from embryonic or foetal neural tissue then transplantation into developing or adult mouse brain, human embryonic NSCs can migrate over distance, integrate into the surrounding microenvironment and demonstrate multipotency (i.e. an ability to differentiate into regionally appropriate neuronal cells) **(Scheffler et al., 1999).**

Vectors are used to transfer the therapeutic genes into human somatic cells. They protect the new genes and transport them safely to the target area. In some cases, the virus is used as a vector mainly because of its replication ability. To use a virus, it must be first deactivated; this is done by taking out the bad

genes found in the virus and then replacing them with the good therapeutic genes. This way when the virus replicates, it is only replicating the good genes, therefore producing gene defect the needed therapy for the patient **(Mc Conkey et al., 2000).**

Gene therapy was first performed in 1990, to treat a four years old girl with severe combined immuno-deficiency disease (SCID) using ex vivo technique, this disease is caused by a faulty gene that fail to produce a vital enzyme adenosine deaminase (ADA). This is one of successful stories of the therapy, but process wasn't a cure but only work for a few months and should be repeated every few months **(Thompson, 1995).**

Unfortunately, unlike many types of cells, neurons in the central nervous system of adults are typically unable to divide. That fact of life creates the neurological illness or injury, but scientific advances aims to replace lost cells in damaged tissue by transplanting neurons or by delivering growth factors that can stimulate surviving neurons that can awaken the cells dormant ability to regenerate. This review will focus on the potential of gene replacement therapy for correction many neurological problems e.g. neurodegenerative, metabolic enzymatic and muscular disorders **(Neuwelt et al., 1995& Rosenberg, 1994).**

The lysosomal storage disorders are a major subset of genetic enzyme deficiencies. Treatment of genetic enzyme deficiencies with somatic cell gene replacement bypasses many of the problems inherent in transplantation and enzyme replacement techniques and instead focuses on the source of the problem a mutation resulting in a defective gene. For gene replacement therapy, a normal gene is introduced into the patient in the appropriate tissue (CNS), in the appropriate cells (neurons and/or glia), thereby correcting the consequences of the defective gene **(Fleischman, 1995)**

The goal with CNS gene therapy would be to intervene as early as possible in course of the disease in order to not only halt progression neurodegeneration, but also actually cure the disease. In addition to inborn errors of metabolism, gene therapy has the potential for restoration of neuronal function. In late-onset localized neurodegenerative disorders such as Parkinson disease or Huntington's disease and even brain tumors gene therapy may be a mechanism for the treatment of these disorders. Reversal of the transformed phenotype of individual tumor cells that can be accomplished by replacement or augmentation of a tumor suppressor gene such as P53 **(Huang, et al., 1998).**

Aim of the Work

- Give a short idea about the strategies and different types of gene therapy.
- Demonstration of gene therapy in neurological disorders.