INTRODUCTION

The recognition of the role of genetic factors in the causation of human disease has made clinical genetics one of the most rapidly developing fields in medicine *(Baired et al., 1988)*.

The use of genetics and genetic manipulation by humans for the therapy of human disease is a new and rapidly evolving field of both basic science and clinical medicine. The science of gene therapy is derived from significant research advances in the fields of genetics, molecular biology, clinical medicine and human genomics. Thus gene therapy can be defined as the use of genetic manipulation for treatment of disease *(Anderson, 1992)*.

Gene therapy is a technique for correcting defective genes responsible for disease development. Researchers may use one of several approaches for correcting faulty genes.

- A normal gene may be inserted into a nonspecific location within the genome to replace a nonfunctional gene. This approach is most common.
- An abnormal gene could be swapped for a normal gene through homologous recombination.
- The abnormal gene could be repaired through selective reverse mutation, which returns the gene to its normal function.

- The regulation (the degree to which a gene is turned on or off) of a particular gene could be altered. ***(Mulligan, 1993).***

Ideally there are four basic prerequisites that should be met for any genetic therapy targeted to an ocular disease. First, a gene delivery technique must be available that is efficient and nontoxic. Second, the genetic basis of the disease, or its biochemical basis, should be well characterized so that an appropriate matched therapeutic approach can be selected. Third, expression of the therapeutic gene needs to be properly controlled. Finally, having an experimental animal model of the disease available for preclinical testing of the therapy is clearly key in demonstrating proof of principle ***(Hauswirth, 1998).***

In most gene therapy studies, a "normal" gene is inserted into the genome to replace an "abnormal" disease-causing gene. A carrier molecule called a vector must be used to deliver the therapeutic gene to the patient's target cells. Currently, the most common vector is a virus that has been genetically altered to carry normal human DNA. Viruses have evolved a way of encapsulating and delivering their genes to human cells in a pathogenic manner. Scientists have tried to take advantage of this capability and manipulate the virus genome to remove disease-causing genes and insert therapeutic genes.

Target cells are infected with the viral vector. The vector then unloads its genetic material containing the therapeutic human gene into the target cell. The generation of a functional protein production

from the therapeutic gene restores the target cell to a normal state ***(Francisco, 1997)***.

Besides virus-mediated gene-delivery systems, there are several non-viral options for gene delivery. The simplest method is the direct introduction of therapeutic DNA into target cells. This approach is limited in its application because it can be used only with certain tissues and requires large amounts of DNA ***(Dickson, 1995)***.

The first approved gene therapy clinical protocol began in September 1990, using retroviral vectors to introduce copies of the adenosine deaminase (ADA) gene into T cells from a patient with ADA deficiency ***(Morgan and Anderson, 1995)***.

Restriction mapping and DNA sequencing has already led to a better understanding of normal gene structure and function, as well as the molecular pathology of certain single gene disorders ***(Lawn et al., 1980)***.

In this work the defective genes or the abnormally manifested genes have been discussed also the therapeutic gene, its vector and the route of entrance to the target cells have been mentioned.

The ocular diseases have been discussed in this work include; inherited retinal dystrophies, retinoblastoma, choroidal neovascularization, glaucoma and ocular scarring and also the role

of gene therapy in its management and the results have been mentioned.

In conclusion, the vision community stands on the brink of a revolution in therapies for blinding and acuity-altering conditions. It is an exciting time, because ocular gene therapy seems to be among the earliest successful applications of this new approach to disease and pathology.