Introduction

Genes are the blueprint for our bodies, governing factors such as growth, development and functioning. Genes are carried on chromosomes, are the basic physical and functional units of heredity. Genes are specific sequences of bases that encode instructions on how to make proteins. Although genes get a lot of attention, it's the proteins that perform most life functions and even make up the majority of cellular structures. When genes are altered so that the encoded proteins are unable to carry out their normal functions, genetic disorders can result **(National Cancer Institute**, **2004**).

Methods of Gene Therapy:

Broad Methods:

There are a variety of different methods to replace or repair the genes targeted in gene therapy.

- 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 **(Gardlik, Roman et al., 2004).**

Hybrid methods:

Due to every method of gene transfer having shortcomings, there have been some hybrid methods developed that combine two or more techniques. Virosomes are one example; they combine liposomes with an inactivated HIV or influenza virus. This has been shown to have more efficient gene transfer in respiratory epithelial cells than either viral or liposomal methods alone. Other methods involve mixing other viral vectors with cationic lipids or hybridising viruses **(Gardlik, Roman et al., 2004).**

Indication of Gene Therapy:

Cancer research has been looking in using gene therapy for potential prevention methods or cures. There is strong belief that when certain genes are missing completely or mutated, they can lead to cancer or keep cancer from developing. Using viral vectors to insert these genes may prevent cancer. Another approach is to manipulate the immune system, promoting the attack on cancer cells **(National Cancer Institute, 2004)**.

Researchers are also looking for a way to prevent the damage that comes from a heart attack as well. They have located a gene in animals that is activated when their oxygen supplies are low. This gene synthesizes a protein and prevents cells from dying. Humans have the gene that synthesizes the protein as well, but they do not have the promoter. The promoter is activated by low oxygen supply. Injecting human cells with this gene could reduce the damage created from a heart attack or stroke, saving individuals from death. Testing of this method on

rats has already been proven successful **(Christopher Haslett et al., 2004).**

Experiments, using both in vivo and ex vivo, are targeting hemophilia and searching for a way to produce the clotting factors the diseased individual lacks. Using the in vivo technique, the liver cells are exposed to vectors. The liver creates most of the needed clotting factor, and by modifying it, the hemophiliacs would no longer lack the clotting factors. With the ex vivo technique, a variety of different cells including skin cells and various blood cells are manipulated. With successful manipulation, the cells are then injected into the human, and continue manufacturing the needed clotting factors.

Lastly, most hereditary diseases are not confined to a single gene. Many disorders are caused by multiple genes which have defects. Alzheimer's, diabetes, and heart disease are a few examples of disease caused by the multiple genes. These are called multigene disorders, or multifactorial disorders. It is hard to "find a cure" for diseases such as these, and gene therapy would not be extremely effective **(National Cancer Institute, 2004)**.