Gene therapy in neurology

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Gene therapy is a novel, rapidly developing form of therapy for both genetic and acquired diseases. In this strategy, the nucleic acid in the form of DNA is administered into somatic cells to correct or replace a genetic defect or to modify the genetic properties of cells or the direct use of genes as a therapeutic agent is shortly defined as the use of DNA as a drug. Such technology allows for the possible treatment and cure of genetic diseases such as cancer, vascular diseases, neurodegenerative disorders, myopathies and Parkinson disease.Gene therapy was first performed in 1990 , to treat a four years old girl complaining of a severs disease; severe combined immuno-deficiency disease using ex vivo technique, this disease is caused by a faulty gene that fail to produce a vital enzyme ; adenosine deaminase (ADA). It is one of the successful stories of gene therapy.The goal of gene therapy is to treat and/or cure genetic diseases by the survival of genetically engineered cells.Diseases candidates for human gene therapy must be an incurable, life threatening disease, the normal counterpart of the defective gene has been isolated, the gene can be expressed adequately, Techniques are available and Organ, tissue and cell types affected by the disease must be identified.Two main types of gene therapy; somatic gene therapy and germ line gene therapy, somatic gene therapy aim to insert a healthy gene into specific tissues of an individual, in order to treat disease resulting from a genetic defect, somatic therapy cannot be passed into future generations.Germ line gene therapy is an approach that deliver genes to sperm or ovum (or to the cells that produce them) to prevent the defective genes from being transmitted to subsequent generation.There are two main approaches to this treatment: in vivo gene therapy, in which genes are delivered directly to target cells in the body. And ex vivo gene therapy, in which the target cells are genetically modified outside the body and then re-implanted.Gene therapy has both advantages and disadvantages which allow or prohibit its use.Gene delivery is the second big step in gene therapy which can be performed by two major categories; viral and non-viral vectors.Different types of viruses are used such as retroviruses and adenoviruses; they differ in their mechanisms of action, results, advantages and disadvantages.Gene therapy approaches for neurological diseases used to protect against neurological disease, to slow the progression of disease or repair of damaged tissues.This review will focus on the potential of gene replacement therapy for treatment neurodegenerative diseases, Parkinson’s disease, Multiple sclerosis, Gillian-Barre’ syndrome, Huntington’s disease, Alzheimer’s Disease, Muscular Dystrophy, Brain tumors, Neurofibromatosis, Migraine and The Lysosomal storage disorders are a major subset of genetic

enzyme deficiencies.In conclusion:Throughout the years our technology has greatly increased in many ways, gene therapy is just one of these many advances in treatment of some neurological disorders. Gene therapy has the potential to treat, create, a possibly be destructive to those who receive the therapy. Many more trials and researches still need to be done to insure its safety and effectiveness. The possibilities through gene therapy may be endless. Decisions on how far the possibilities may stretch must be decided on and therefore be made into laws in order to protect ourselves and the future generations. The benefits of using gene therapy should not be over looked, for it has the potential to make ourselves free of disease and thus will make difference in life of ourselves and our loved onesRecommendations1. Gene therapy should proceed with cautious optimism and shouldn’t be considered as the only alternative. Additionally, genes should not be viewed as the sole source of diseases such as cancer in which the environment may also contribute to disease development.2. Genetic manipulation should only be used to treat diseased genes. At no time should this technology be used for other means such as physical appearance or mental capability.3. What are the features of a good gene delivery system? Explaining the difference between an in vivo and an ex vivo approach, and why certain disease is ”fit” over the other.4. More understanding of human genome to know farther genetic diseases.5. Explaining the role of a basic research institute for gene therapy studies.6. Explaining the advantages & disadvantages of the viral and non viral vectors.7. Conduct more inspections to increase oversight of gene therapy.8. Application of gene therapy in more incurable diseases.