Essay about Gene Therapy

Gene therapy refers to using DNA as medication for the treatment of diseases by the delivery of therapeutic DNA into a patient's bodily cells (Sheridan, 2011). Commonly, it is done through DNA to encode functions and therapeutic genes to replace transformed genes. Other forms of this technology involve direct correction of mutated genes, or the use of DNA in encoding a therapeutic protein medication instead of natural human genes in the provision of treatment. In the technology, DNA used in encoding therapeutic proteins is packaged with 'vectors' that are used in obtaining DNA in cells found in the body. Once inserted, the DNA is expressed by the cell mechanism, which results in a therapeutic protein that offers treatment to patients.

In 1972 studies on gene therapy commenced in various medical institutions, and in 1990, FDA approved the first gene therapy procedure in the US during the ADA-SCID treatment of Ashanti DaSilva. By January of this year, approximately 2,000 medical attempts had been made or fully approved using various techniques for gene therapy. Despite the fact that previous failures led to controversies and pessimism over gene therapy, various successes from 2006 have led to new hope in the technology. Such include major successes in treatment of patients with Leber's congenital amaurosis disease of the retina, X-linked SCID, ADA-SCID, acute lymphocytic leukemia (ALL), chronic lymphocytic leukemia (CLL), adrenoleukodystrophy, Parkinson's disease, hemophilia, and myeloma. Such clinical successes have resulted in a new interest in the technology, with various publications calling for additional research. Between 2013 and mid-2014, United States pharmaceutical companies and other related firms invested more than $600 million in this field. The European Commission approved Glybera for clinical use in Europe and the US for the first time in 2012 (Sheridan, 2011).

There are two major types of gene therapy used in humans:

Somatic gene therapy

In this type, the therapeutic genes are inserted into non-sex cells of a patient. Any alterations and effects are restricted to the individual body and are not inherited by the offsprings or other generations. This type of gene therapy represents the mainstream line of existent medical research, where therapeutic DNA genes are used in the treatment of diseases of an individual patient (Strachnan & Read, 2004).

Various somatic cell gene transfer procedures are currently conducted in clinical experiments with different successes. Above 600 clinical experiments using somatic cell therapy are being conducted in the United States. Most of these experiments are focused on the treatment of acute genetic disorders, including hemophilia, cystic fibrosis, thalassemia, and various immunodeficiency. Such disorders are proper areas for somatic cell gene therapy because they result from single gene problems. While this type is effective for the treatment, there is no complete correction of genetic disorder or replacement of genes (Baum et al., 2003).

Germline gene therapy

In this type, sperm or egg cells are transformed by introducing functional genes integrated into the genomes. These cells combine to form a zygote that divides to produce all other cells existent in an organism. Therefore, genetically modifying a germ cell causes other cells in an organism to have a modified gene. This allows the therapy to be passed on to future generations and offsprings. Despite the imminent effectiveness in counteracting genetic disorder and other hereditary diseases, various countries, including Netherlands, Canada, Australia, Switzerland, Israel, and Germany presently outlaw the application of this technology to humans for ethical and technical concerns. These concerns include insufficient knowhow on possible effects to the future generations and higher risks than the use of non-interactive vectors. The United States has no federal provisions that particularly address human somatic or germ-line gene modification that are beyond the common FDA regulations for general therapies (Strachnan & Read, 2004).

Quantifying the success of treatment is one major challenge of gene therapy. Research is troubled with ethical and technological challenges. With clinical drug experiments, the reason of human gene therapy experiments is determination of the safety, effectiveness, administration, and the functionality of gene therapy. Diseases are picked for experiments basing on the severity of the disorder, treatment feasibility, and prediction of success of treatment basing on animal models. Despite reasonability, it is evident thatmany people lack objectivity in sending their relatives for research in finding treatment for a serious condition (Strachnan & Read, 2004).

The method of determining disorders or traits warranting gene therapy is also inconclusive. Unfortunately, the difference between gene therapies to improve desirable traits and disease genes is unclear. It is irrational to argue that diseases causing suffering, disability, and death directly qualify for gene therapy. Nevertheless, there is a thin line between what is regarded as a 'disease' and a 'trait' in an otherwise healthy person, for instance, dwarfism disorder, achondroplasia, and normal shortness. Despite the fact that gene therapy for correcting potential socially unacceptable traits, or the improvement of desirable traits, may enhance life quality for a person, ethical concerns fear that gene therapy for trait improvement has negative on social norms, and thus promote discrimination towards people thought to harbor 'undesirable' traits. As science continues to unearth many genes, it may became impossible to define particular genetic traits considered to be diseases against the ones that should be categorized as psychological, mental, or physical traits.

To the current times, acceptable gene therapy clinical experiments involve somatic cell therapy that uses genes causing diseases. Nonetheless, various ethical circles are concerned that the feasibility of germ-line therapy improves, and more genes with different traits are known. There could be risks regarding which genes to be used in future therapies and trials. Particularly, it is suspected that the universal acceptance of germ-line therapy can lead to acceptance of genetic enhancement. Public concerns about the issue around germ-line therapy can therapies for genetic enhancement must continue as advancement in science goes on. This will enable full appreciation of the effectiveness of newer therapies and provision of ethical guidelines for these advancements (Strachnan & Read, 2004).

From a personal perspective, it is evident that gene therapy should be adopted despite the ethical claims, but with proper feasibility tests and gradual adoption. It is evident that death is the major issue why many ethicists consider gene therapy inappropriate. Only three patients have died during gene therapy trials. These unfortunate events have put the field under severe assessment. Jesse Gelsinger's death in 1999 was a major setback for gene therapy. Other deaths were reported in 2003 and 2007, but investigations determined that gene therapy was not the cause of the deaths. Therefore, it is important to look at gene therapy for a cost-benefit analysis, and it will be evident that the benefits of this technology far much outweigh the ethical concerns. In fact, most upheld scientific discoveries and vaccines have led to more deaths and initially appeared to go against humanity and medical ethics. Therefore, with continuing discoveries, gene therapy should be invested on and encouraged.

References

Baum C, Düllmann J, Li Z, Fehse B, Meyer J, Williams DA, von Kalle C (Jan 2003). "Side effects of retroviral gene transfer into hematopoietic stem cells". Blood 101 (6): 2099–114

Sheridan C (2011). "Gene therapy finds its niche". Nature Biotechnology 29 (2): 121–8.

Strachnan, T. and Read, A. P. (2004) Human Molecular Genetics, 3rd Edition, Garland Publishing