

Gene Therapy Information

What is gene therapy?

Gene therapy is a technique for correcting defective genes responsible for disease development. The most common approach for correcting faulty genes is to insert a “normal” gene into the genome to replace an “abnormal” disease-causing gene.

There are two types of gene therapy:

- Somatic: Somatic Gene therapy is provided by introducing a new therapeutic gene (transgene) into the diseased cells of a patient.
- Germ Line: changes occur in the cells responsible for reproduction, hence permanently altering the genes of all future generations of the person receiving the therapy.

Is gene therapy currently practised anywhere in the world?

- It is important to note that although there is much research being done, gene therapy is still in the experimental stage and has not proven very successful in clinical trials.
- The USA Food and Drug Administration (FDA) placed a temporary halt in 2003 on certain gene therapy trials after the death of a child in France undergoing experimental gene therapy treatment.
- In 1999 an 18-year-old named Jesse Gelsinger died of multiple organ failure four days after starting gene therapy for OTCD.
- Several research teams around the world are experimenting with gene therapy on mice, but after the devastating results of the clinical trials mentioned above, many safeguards have been put into place to discourage clinical trials on human.

What are the potential benefits for individuals of gene therapy?

- Cures could be developed for hereditary diseases such as Huntington’s Disease and various cancers.
- By removing the faulty gene from a family line, all future generations could be saved from developing terrible life-threatening diseases.

What are the potential social implications of gene therapy?

- In the case of genetic enhancement, such manipulation could become a luxury available only to the rich and powerful
- Widespread use of this technology could lead to new definitions of “normal” which would have huge implications for persons with disabilities. This could lead to widespread use of the technology to “weed out” disability.
- Gene therapy is currently focused on correcting genetic flaws and curing life-threatening disease, and regulations are in place for conducting these types of studies. But in the future, when the techniques of gene therapy have become simpler and more accessible, society will need to deal with more complex questions, such as the implications of using gene therapy to change behavioural traits.
- Germline gene therapy would forever change the genetic make-up of an individual’s descendants. Thus, the human gene pool would be permanently affected. Although these changes would presumably be for the better, an error in technology or judgment could have far-reaching consequences.

USEFUL WEBSITES:

1. Otago University “For High Schools” Gene Therapy Info Page

<http://osms.otago.ac.nz/main/bursary/genether.html>

This page provides a comprehensive overview of Gene Therapy, including history of the research, ethical considerations and information on the one Gene Therapy trial ever done in New Zealand. There are also links to other useful Gene Therapy sites.

2. Biotechnology Australia: Gene Therapy Page

http://www.biotechnology.gov.au/biotechnologyOnline/human/h_gene_therapy.htm

This portal outlines the uses, challenges, trials and possible futures of Gene Therapy research.

3. Human Genome Project Information: Gene Therapy Page

http://www.ornl.gov/sci/techresources/Human_Genome/medicine/genetherapy.shtml

One of the most comprehensive Gene Therapy websites available, this site addresses questions such as:

- [What is gene therapy?](#)
- [How does gene therapy work?](#)
- [What is the current status of gene therapy research?](#)
- [What factors have kept gene therapy from becoming an effective treatment for genetic disease?](#)
- [What are some recent developments in gene therapy research?](#)
- [What are some of the ethical considerations for using gene therapy?](#)
- [Gene therapy links](#)

4. UK Department of Health: Gene Therapy Advisory Committee (GTAC)

<http://www.doh.gov.uk/genetics/gtac/>

The 1992 report of the Committee on the Ethics of Gene Therapy (the Clothier Committee) recommended that gene therapy (genetic engineering in humans) should be limited to life threatening diseases or disorders. To oversee and implement this, GTAC was established in 1993. GTAC advises on the ethical acceptability of proposals for gene therapy research on humans taking account of the scientific merits and the potential benefits and risks, and provides advice to UK health Ministers on developments in gene therapy research.

5. American Society of Gene Therapy (ASGT)

<http://www.asgt.org/>

This society, established in 1996, represents researchers and scientists dedicated to discovering new gene therapies. It includes almost 3,000 members, and is “committed to promoting and fostering the exchange and dissemination of information and ideas related to gene therapy, encouraging the general field of research involving gene therapy and promoting professional and public education in all areas of gene therapy.”

ASGT has an official journal, *Molecular Therapy*, and holds an annual meeting each year.

6. European Society of Gene Therapy (ESGT)

<http://www.esgt.org/>

The objectives of ESGT are:

- To promote basic and clinical research in gene therapy

- To promote education and the exchange of information and technology related to gene transfer and therapy
- To serve as a professional adviser to the community and to the regulatory bodies in Europe.

7. The Journal of Gene Medicine

<http://www.wiley.co.uk/genetherapy/clinical/>

Claims to be, “the most comprehensive source of information on gene therapy clinical trials available on the internet.” The site includes some interesting charts and statistics and an interactive database where you can search for gene therapy information by country, disease etc.