This fact sheet describes gene therapy as it is used to treat medical conditions and the benefits and challenges it raises.

In summary

- **Gene therapy is the transfer of a therapeutic or working copy of a gene into specific cells of an individual**
- **The technique may be used to replace a faulty gene, or to introduce a new gene whose function is to cure or to favourably modify the symptoms of a condition**
- **Gene therapy is still an experimental technique and much research remains to be done before this approach to the treatment of conditions will realise its full potential.**

WHAT IS GENE THERAPY?

Gene therapy involves the transfer of a therapeutic or working gene copy into specific cells of an individual. It may be used to:

- Replace a faulty gene
- Introduce a new gene whose function is to cure or to favourably modify the clinical course of a condition
- Inactivate or "knock out" a faulty gene that is not functioning properly.

The potential of gene therapy is very broad, with research involving a number of diseases such as severe combined immune-deficiencies (SCID), haemophilia, Parkinson's disease, many forms of cancer and HIV.

HOW IS GENE THERAPY CARRIED OUT?

There are many challenges to successful gene therapy.

Firstly, the condition in question must be well understood and the underlying causative gene identified. A working copy of the gene involved must be available and the specific cells in the body requiring treatment must be identified, accessible and a means of efficiently delivering working copies of the gene to these cells must be available.

Of all these challenges, the one that is most difficult is the problem of **gene delivery** i.e. how to get the new or replacement genes into the desired tissues.

Some of the **vectors** for delivering the working copy of the gene to the target cells include using:

a) Harmless viruses

One of the most promising methods currently being developed is the use of harmless viruses that can be used to carry genes into cells.

Scientists now have the knowledge and skills to remove the virus' own genes and to replace them with working human genes. These altered viruses can then be used to deliver genes into cells with great efficiency. When viruses are used in this way they are known as **vectors**.

Some of these vectors are capable of not only carrying the gene into the cell but also of inserting the gene into the genetic material of the cell.

Once in the right location within the cell of an affected person, the transplanted gene is switched on. The transplanted gene can then issue the instructions necessary for the cell to make the protein that was previously missing or altered.

b) Stem cells

Another technique with potential is the use of stem cells in delivering gene therapy. Stem cells are cells that have not yet differentiated into a specific tissue or organ cell.

In this technique, stem cells are manipulated in the laboratory in order to make them accept new genes that can then change their behaviour.

For example, a gene might be inserted into a stem cell that could make it better able to survive chemotherapy. This would be of assistance to those patients who could benefit from further chemotherapy following stem cell transplantation.

ETHICAL CONSIDERATIONS

While the body has many billions of cells, only a very small proportion of these cells are involved in reproduction, the process by which our genes are handed on to future generations. In males these cells are located in the testes and in females, in the ovaries. These special reproductive cells are called **germ cells**.

All other cells in the body, irrespective of whether they are brain, lung, skin or bone cells, are known as **somatic cells**.

In gene therapy, only somatic cells are targeted for treatment. Therefore any changes to the genes of an individual by gene therapy will only impact on the cells of their body and cannot be passed on to their children.

An example of somatic gene therapy for an inherited condition

Imagine, for example, a little boy with haemophilia, a condition that is caused by a faulty gene that makes his liver unable to make blood clotting factor 8.

- Gene therapy would involve putting a working copy of the gene which codes for factor 8 into his liver cells so that his liver could then produce adequate levels of factor 8
- While the little boy himself would be cured, the altered genes in his germ cells would remain unchanged and he could still pass the faulty gene on to his children.

CONCERNS WITH GENE THERAPY OF THE EGG OR SPERM CELLS

The possible genetic manipulation of the egg or sperm cells (germ cells) remains the subject of intense ethical and philosophical discussion.

The strong consensus view at present is that the risks of germline manipulation far exceed any potential benefit and should not be attempted.