Gene Therapy

Gene therapy means insertion, removal or modification of the defective gene to treat inherited genetic diseases. Modifying genes may lead to the formation of such a drug or a functional enzyme, etc. In the early days of the treatment, it was used especially in immunodeficient states. The first patient was a four-year old girl with a rare immune deficiency (**SCID** - **S**evere **C**ombined **I**mmuno**d**eficiency). Her treatment was based on the genetic adaptation of her own dysfunctional white blood cells by adding genes to ensure their proper function. The result was only partial improvement, but it was a success. It happened on the 14th September 1990.

Use of Gene Therapy

The most common principle of gene therapy is the replacement of a functional gene for the mutated gene. The greatest use of the method is on **monogenic diseases** – cystic fibrosis, hemophilia, muscular dystrophy, etc. With its use is planned in the treatment of cancer.

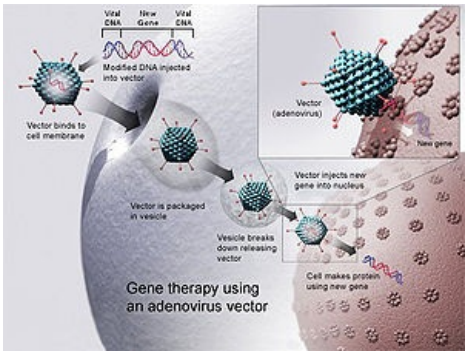
Conditions of Use

- *Knowledge of the exact sequence of the gene examined.*
- *Knowledge of the causes and pathological processes of disease (insufficient amount of product, creating a pathologically mutated gene product acting, etc.).*
- *Selection of suitable vectors - carriers (retroviruses, adenoviruses).*
- *The consent of the patient.*

Methods of Gene Therapy

Ex Vivo Methods

Cells from the affected areas are surgically removed. Then we insert the healthy form of gene to the extracted cells . Following their separation and cultivation in a suitable environment. After their multiplication they are injected back into the original tissue. Very often drawn by the cultivation of tissue sample is *bone marrow*. Bone marrow is a producer of the blood cells which distribute genetically engineered cells throughout the body. The advantage of easier distribution is often oppressed by disadvantages of this technique – surgery is very painful for the patient and must take place in two stages (taking bone marrow and subsequent reimplantation), because the culture itself requires many hours to grow.



Gene Therapy

In Vivo Methods

This method does not require surgery or anesthesia. The modified genes are administered directly into the cells of the body using **viruses** as carriers of information. They are used in two main groups of viruses:

1. Simple retroviruses

Their *advantage* is complete suppression of viral DNA. Only genetically modified DNA is transmitted by them. Their results are long-term and patients are not attacked by these viruses. The *disadvantage* is that it works only on the newly formed daughter cells. There is no effect on the existing defective cells.

2. Adenoviruses

Adenoviruses work faster than retroviruses, but the duration of the effect is shorter – within weeks. The patients immune system has greater tendency to interfere with these viruses, and generate a response. Therefore patients suffer from symptoms of colds and rhinitis. But there are also known cases of serious interactions (see below).

To achieve the same result less adenovirus solution (in milliliters) is needed than retroviruses (in liters).

Gene Therapy Today and Tomorrow

Currently, however, the gene therapy is still used rather rarely. Why? This is due to persistent concerns about its *safety*. It is always a manipulation of human DNA, which may also lead to ethical problems.

In the past, several cases have occurred when there was a serious disability of patients gene therapy. *Several deaths* occured. It was probably (although the cause has long been studying), a massive immune reaction of the organism to the repeated application of adenoviruses.

Gene therapy is now used more **for the serious diseases** that can not be treated otherwise and they are fatal. The considerable disadvantages of this method are its financial and technical demands. Finally, there are also concerns about the impact of therapy on tumor growth. Carried by viral vectors can interfere with other areas such as DNA and alter tumor suppressor genes or protooncogenes.

Links

Related articles

- Vectors
- Protooncogenes

External links

- Genetika.wz.cz (http://genetika.wz.cz/genova_terapie.htm)
- Krize genové terapie (http://www.gate2biotech.cz/krize-genove-terapie/)

Bibliography

- ALBERTS, BRUCE, ET AL,. *Základy buněčné biologie.* 2.vydání edition. 2005. ISBN 80-902906-2-0.