GENE THERAPY FOR HUMAN DISEASES: CLINICAL ACHIEVEMENTS AND PROBLEMS

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Relevance: Over the past decades, a completely new approach to the treatment of diseases has appeared. This approach is called gene therapy. The fundamental difference between the new and traditional methods of treatment is that it is aimed at eliminating the root cause of the disease, and not its consequences. At the present stage, gene therapy can be defined as the treatment of hereditary and non-hereditary diseases by introducing genes into the cells of patients in order to purposefully change gene defects or give cells new functions.

Objective: The treatment of diseases at the genetic level is a very attractive prospect. But, like any intervention in the human body, it can lead to completely unpredictable results. Over the half-century period of development of this field of medicine, both positive and negative experience in the use of gene therapy has been accumulated. However, it cannot be said unequivocally whether gene-level therapy is a good or an irreparable mistake.

Material and methods: Such methods of transfer include either direct injection of DNA into the recipient cells, or utilising methods to induce membranes permeabilisation, receptor- mediated uptake or endocytosis. Transduction utilises recombinant virus as a vector for gene transfer**.**

Results: Significant results were obtained in the field of neurodegenerative diseases, such as Parkinson's disease, Huntington's chorea, and others. In this case, the principle of gene therapy intervention, which is at the stage of clinical trials, is based on the introduction of cell cultures synthesizing a set of proteins that prevent nerve degeneration into certain subcortical regions of the brain cells. In addition, one cannot fail to note the achievements of gene therapy approaches in the treatment of HIV-infected patients, in cardiology, as well as in a number of other diseases.

The tremendous success of gene therapy is accompanied by significant failures. In 2003, the FDA decided to temporarily discontinue clinical trials using retroviruses on blood stem cells. The reason was the development of leukemia in two out of ten children undergoing severe combined immunodeficiency (SCID) therapy. It was found that the side effect is due to the fact that the virus used to deliver the therapeutic gene activated the oncogen. In April 2004, the FDA, given the successes of this type of gene therapy, nevertheless eased the ban on its implementation, allowing the use of retroviral vectors for the treatment of those patients for whom other methods were ineffective. Gene therapy can give humanity a powerful tool for moving medicine to a whole new level of development. Where the methods of traditional medicine were powerless, gene therapy came to the rescue, making it possible for the terminally ill to recover. Nevertheless, it cannot be denied that, having in its hands such a powerful weapon as the ability to change genes, humanity faces the problem of the competent use of this technology. Already, theoretically, modifications of the genome are quite possible in order to improve some physical, mental and intellectual parameters. Modern science at its new round of development has returned to the idea of improving the human breed, which in itself is dangerous.

Conclusions: Thus, the therapeutic potential of genomic transformations is truly enormous, but we should not forget about the dangers that lie ahead. Along with the development of a methodological experimental base for gene therapy, attention should be paid to solving a number of issues of a social and ethical nature. Despite the nearly half-century history of the development of gene therapy, this industry is still at the initial stage of its formation. Since its inception, it has attracted the attention of people around the world. Having penetrated into an area previously not accessible to mankind, gene therapy has opened fantastic prospects.

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