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PROSPECTS AND LIMITATIONS OF GENE THERAPY

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The emergence of fundamentally new technologies that allow active manipulation of genes and their fragments, and which provide targeted delivery of new blocks of genetic information in a given area of the genome, has become an important event in biology and medicine. Even now, at the current level of knowledge about the human genome, it is theoretically possible to modify it in order to improve some physical and mental parameters. Genetic engineering is also possible at the level of germ cells.

Prevention of hereditary diseases can be as complete and effective as possible if the zygote is embedded in a gene that replaces the mutant gene. Eliminating the cause of hereditary disease (namely, this is the most profound approach to prevention) means a serious modification of genetic information in the zygote. These may be the introduction of a normal allele into the genome, the reverse mutation of a pathological allele, the «inclusion» of a normal gene in the work, if it is blocked, the «exclusion» of the mutant gene. The difficulties of these problems are obvious, but intensive experimental developments in the field of genetic engineering indicate the fundamental possibility of their solution. The question of genetic engineering prevention of hereditary diseases is no longer a utopia, but a prospect, although not close.

Genetic engineering prevention of hereditary diseases at the zygote level is still poorly developed, although the choice of methods for gene synthesis and ways to «deliver» them to cells is already quite wide (Kuhn et al., 2016). The solution of transgenesis in humans today rests not only on genetic engineering difficulties, but also on ethical issues. After all, there is a talk about «compositions» of new genomes, which are not created by evolution, but by man. Human genetics is still far from fully understanding all the features of the genome. It is unclear how the genome will work after the introduction of additional genetic information, how it will behave after meiosis, reduction in the number of chromosomes, in combination with a new embryonic cell, and so on.

The current level of knowledge does not allow the correction of genetic defects at the level of germ cells and cells of early pre-implantation human embryos due to the real danger of clogging the gene pool with unwanted artificial gene constructs or mutations with unpredictable consequences for future humanity. However, in the scientific literature there are more and more calls for the resumption of the debate on the feasibility of gene correction of human germ cells (Rubeis & Steger, 2018).

Thus, the genetic revolution, which culminated in gene therapy, not only offers real ways to treat severe hereditary and non-hereditary diseases, but also in its rapid development poses new challenges to society, the solution of which is urgently needed in the near future.

References:

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