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ГЕННАЯ ТЕРАПИЯ С ПРИМЕНЕНИЕМ АДЕНОАССОЦИИРОВАННЫХ ВИРУСОВ: РИСКИ И ПРЕИМУЩЕСТВА

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В настоящее время в лечении многих наследственных заболеваний внедряются технологии с применением генной терапии. В данной статье рассмотрена генная терапия с использованием аденоассоциированных вирусов, преимущества данного метода и его возможные осложнения.

Ключевые слова: генная терапия, аденоассоциированный вектор, наследственные заболевания, преимущества, недостатки.

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GENE THERAPY WITH THE USE OF ADENO ASSOCIATED VIRUSES: RISKS AND BENEFITS

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Currently, technologies using gene therapy are being introduced in the treatment of many hereditary diseases. This article discusses gene therapy using adeno-associated viruses, the advantages of the method and its possible occurring complications.

Key words: gene therapy, adeno-associated vector, hereditary diseases, advantage, flaws.

Currently, the treatment of rare genetic diseases using modern gene technologies is becoming highly relevant.

The main aim of the article is to consider the use of adeno associated viruses in gene therapy.

Material and methods: original and review articles in domestic and foreign scientific databases were found and analyzed.

Results and discussions: gene therapy is a set of methods for manipulating genes and introducing them into other organisms to change gene defects in order to give cells new properties and eliminate severe mutations. The most known popular technology of genetic engineering is the use of viruses as vectors for the delivery of modified genes to mutant cells, for example, the lentiviral vector, adenoassociated viruses (AAV), retroviruses, etc.

The AAV vector has gained an advantage in the treatment of hereditary diseases, because it is the safest and most predictable virus in the body of an ill person [5]. The recombinant adeno-associated vector is able to provide expression of transgens and transduce dividing and non-dividing cells [3,6]. This ability is a promising method in the treatment of such diseases like cancerous tumors, monogenic, cardiovascular and neurodegenerative diseases [4].

Scientists have conducted two clinical studies of gene therapy for gastric cancer and squamous cell carcinoma of the head and neck using AAV [1]. The virus vector stably expressed the therapeutic genes of the disease during treatment. Nowadays it is possible to achieve the results of

gene therapy in the treatment of spinal muscular atrophy, hemophilia types A and B, hereditary retinal dystrophy [1]. Despite all the advantages of adeno-associated vectors, the method has some disadvantages. One of the main disadvantages of AAV gene therapy is the limitation of the size of the gene which can be transmitted through viruses. The small amount of packaging of genetic information in the AAV genome limits the possibility of transferring large genes or several genes simultaneously [2].

Rarely, the human immune system reacts to the introduction of a therapeutic vector into the body as to the invasion of any other infectious agent virus, which leads to the development of inflammatory processes and serious consequences [1].

By using a viral vector, gene therapy can cause excessive protein production, which can create various consequences, depending on the nature of the synthesized protein.

Besides, the high cost of developing and manufacturing AAV vectors is also one of the disadvantages of the treatment method. Consequently, due to the high cost of the procedure, gene therapy is unavailable to most patients.

Conclusion: despite discussed risks, the method still remains the most perspective and effective in the field of medicine.

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