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УДК 811.111

Шпаков О. В.
Саламатіна О.О.

GENE THERAPY FOR THE TREATMENT OF INCURABLE DISEASES – SUCCESSES AND CHALLENGES IN THE TREATMENT OF HEREDITARY DISEASES (ГЕННА ТЕРАПІЯ ЛІКУВАННЯ НЕВИЛІКОВНИХ ХВОРОБ – УСПІХИ ТА ВИКЛИКИ В ЛІКУВАННІ СПАДКОВИХ ЗАХВОРЮВАНЬ)

У публікації проведено огляд сучасних досягнень у сфері генної терапії як методу лікування спадкових захворювань. Розглянуто успіхи у використанні генної терапії для корекції серповидно-клітинної анемії, спінальної м'язової атрофії та спадкової сліпоти. Проаналізовано основні виклики, пов'язані з високою вартістю лікування, безпекою та етичними аспектами редагування геному, перспективи подальшого розвитку генної терапії та її впливу на майбутнє медицини.

Ключові слова: генна терапія, спадкові захворювання, генетична модифікація, серповидноклітинна анемія, спінальна м'язова атрофія (сма), спадкова сліпота, зольгенсма, редагування геному, етичні проблеми, медична біотехнологія, доставка генетичного матеріалу.

The publication provides an overview of current achievements in the field of gene therapy as a method of treating hereditary diseases. Successes in the use of gene therapy to correct sickle cell anaemia, spinal muscular atrophy and hereditary blindness are discussed. The main challenges related to the high cost of treatment, safety and ethical aspects of genome editing, prospects for further development of gene therapy and its impact on the future of medicine are analysed.

Keywords: gene therapy, hereditary diseases, genetic modification, sickle cell anaemia, spinal muscular atrophy (SMA), hereditary blindness, zolgensma, genome editing, ethical issues, medical biotechnology, genetic material delivery.

Gene therapy is one of the most promising areas of modern medicine, allowing the treatment of hereditary diseases by modifying the patient's genetic material. This method opens up opportunities for treating pathologies such as sickle cell anaemia, spinal muscular atrophy (SMA), hereditary blindness and other diseases that were previously considered incurable. However, along with its successes, gene therapy faces a number of challenges that require further research and resolution [5].

One of the most significant achievements in the field of gene therapy was the treatment of sickle cell anaemia, which allows correcting mutations in the β-globin gene, which significantly improves the condition of patients [5]. In addition, in 2017, a drug was approved for the treatment of Leber's amaurosis, a severe inherited retinal disease. Thanks to the therapy, patients who were previously blind were able to partially restore their vision [1].

Another breakthrough was the use of Zolgensma to treat spinal muscular atrophy. This disease, which leads to loss of motor function of children, was previously considered fatal. Gene therapy can stabilise patients' condition and, in some cases, significantly improve their mobility and quality of life [4].

Despite significant progress, gene therapy faces a number of challenges. One of the main limitations is the high cost of treatment. For example, some drugs cost several million dollars, making them unaffordable for most patients.

Another problem is the issue of safety and ethics. Interfering with human genetic material raises concerns about possible long-term consequences. This is especially true of embryo genome editing, which can have unpredictable effects on future generations [2]. In addition, scientists face technical difficulties related to ensuring stable expression of the introduced genes and efficient delivery of genetic material to target cells [3].

Gene therapy has the potential to become the main treatment for hereditary and many acquired diseases. Further research, the development of more efficient methods of delivering genetic material, and a reduction in the cost of therapy could make it more affordable and safer. The introduction of these technologies into medical practice will open up new opportunities for patients who previously had no chance of living a full life.

In summary, gene therapy is one of the most promising areas of modern medicine, demonstrating significant progress in the treatment of incurable diseases. At the same time, it requires further development to overcome challenges related to high costs, safety and ethical issues. Despite these difficulties, the development of gene therapy has the potential to significantly change the way in which hereditary diseases are treated and improve the quality of life for millions of patients worldwide.

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