19. GENE THERAPY IN SOME GENETIC DISEASES

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Introduction. Gene therapy it's a complex of methods that treats at least 10% of the approximately 9,000 genetic diseases recorded. Genetic diseases involve all dysfunctions that include deficiencies, absence or surplus of certain genetic information. Gene therapy is a complex of treatments that comes to complete the deficiency or lack of genes indispensable for the proper living of patients with an active disease. Gene therapy is a relatively young field and thus an expensive one in production, therefor today some of the drugs (for ex.: Nusinersen, Zolgensma, etc.) can reach from 325 thousand $ to 725 thousand $ a year, any target disease of gene therapy that has a huge degree of specificity and personalization. Approved treatments with a considerable degree of success are: Duchene amyotrophy (DMD), spinal muscular dystrophy (SMA), central nervous system cancer - glioblastoma; and also some therapies that need to be approved for treatment: various forms of hemophilia, Wiskott-Aldrich syndrome, diabetic retinopathy, corneal neovascularization, cancer, etc.

Aim of study. Gene therapy techniques include the direct introduction of substituents into the intercellular space in the body through viral vectors - in-vivo such as: adeno-associated, adenoviral, retroviral, lentiviral vectors, etc. as well as non-viral vectors, for example: CRISPR-Cas9, introduction of plasmids, techniques such as: exon skipping, antisense oligonucleotides, also ex-vivo techniques such as introducing of modified cells, like CAR T. Some obstacles have already been overcome, for example the immune response has been partially resolved, enough to achieve the treatment, by combining it with immunomodulatory treatment with corticosteroids. Individualized forms, such as the degree in a spinal amyodistrophy type I-IV manifestation, are already coordinated with the amount of gene 2 copy.

Methods and materials. The paper was based on a review of the literature, using textbooks and articles published in electronic sources recognized by the international medical community as: PubMed, NEJM, NCBI, GeneCards. Also, two clinical cases of two children suffering from Duchenne progressive muscular dystrophy and belt-shaped muscular dystrophy that could benefit from gene therapy in the Republic of Moldova were described.

Results. During the study it was observed that gene therapy has widely started to be used in various diseases: cardiovascular, neuromuscular, oncology, ophthalmology, etc. Disappointing that in both clinical cases, costs of treatment are too high for the patients, this remains to be the main obstacle.

Conclusion. Gene therapy has a promising future in solving incurable diseases, by proposing a new way of treatment through the involvement in genetic pathophysiology and creation of cells. This encourages medical scientists to include new diseases in research and develop new ways, more accessible to production.