Abstract

Gene therapy is a revolutionary approach to treating diseases, targeting the genetic basis of disease. In the future, it could become the predominant therapeutic approach for many diseases. Current approved gene therapies target immunodeficiencies, cancers, hereditary haematological and neurological diseases, thus presenting hope for patients suffering from rare and previously untreatable diseases. At present, gene therapy is primarily intended for the treatment of monogenic diseases, but in the future its use could extend to the field of acquired diseases. In this way, gene therapy may provide new treatment options and improve the quality of life of patients. Advances in this field are due not only to the development of genetic diagnostics, but also to the improvement of techniques for the application of nucleic acid to target sites, which allows genetic defects to be addressed precisely and effectively. The bachelor thesis focuses on the use of gene therapy in the field of hereditary neurological diseases, the prevalence of which is increasing in both the pediatric and adult population. The thesis also summarizes the historical events leading to the discovery of gene therapy, the methodological possibilities of gene therapy and the risks associated with it. The last chapter is devoted to the latest advancement in gene therapy, which is a personalized treatment approach targeting patients with extremely rare mutations.

Key words: gene therapy, genome editing, RNA antisense oligonucleotides, neurogenetic diseases, viral vectors