GENE THERAPY INNOVATIONS IN HEMOPHILIA A

Guilherme Rodrigues Gomes Suzana [1]
Anna Karolyne de Andrade Morais [2]
Augusto José de Oliveira Pereira [3]
Lylian Machado Palmiere Ribeiro [4]
Ruy Abdalla Soares [5]
Jivago Carneiro Jaime [6]

Hemophilia A, a rare and chronic disease characterized by deficiency of coagulation factor VIII, has been the subject of major recent therapeutic advances. Gene therapy has emerged as an innovative approach, offering the prospect of correcting the underlying deficiency and improving patients' quality of life. Therefore, this study aims to analyze the current status of gene therapy in the treatment of hemophilia A, evaluating its efficacy, safety and potential to provide a sustainable cure for patients. An integrative literature review was carried out by searching for articles in the PubMed, LILACS and IBECS databases. Articles published between 2019 and 2023 were selected, which analyzed advances in gene therapy in patients with hemophilia A, as well such as, the clinical outcomes reported, and the techniques used. It was observed that gene therapy in hemophilia A has shown promising advances. Studies have demonstrated that factor VIII gene transfer, mediated by safe viral vectors, can result in the sustainable production of functional factor VIII. Thus, patients treated with gene therapy showed a significant reduction in bleeding rates and an improvement in quality of life. Although challenges persist, such as the duration of gene expression and the immunogenicity of the vectors used, the results are generally encouraging. Therefore, gene therapy emerges as a revolutionary approach in the treatment of hemophilia A. Current studies indicate that transfer of the factor VIII gene through viral vectors, such as Lentivirus, may provide a potential cure for the disease, reducing dependence of infusions of clotting factors and improving patients' quality of life. As research continues to advance, gene therapy offers the promise of transforming the hemophilia A treatment landscape, bringing hope for a healthier, limitless life for patients. However, additional studies are needed to confirm the long-term efficacy and safety of such therapies.

Keywords: Gene therapy; Hemophilia A; Factor VIII;

[1] Discente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGÉILICA, E-mail: guirgs2017@gmail.com
[2] Discente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGÉILICA, E-mail: annakmandrade@gmail.com
[3] Discente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGÉILICA, E-mail: augustoj.op1@gmail.com
[4] Discente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGÉILICA, E-mail: lylianpalmiere2003@hotmail.com
[5] Discente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGÉILICA, E-mail: ruysoares.med@gmail.com
[6] Docente do Curso de Medicina, Universidade Evangélica de Goiás - UniEVANGELICA, E-mail: jivagojaime@gmail.com