7. INDICATIONS AND LIMITS OF GENE THERAPY

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Introduction. Gene therapies represent advanced medical tools that aim to fix or improve genes by correcting mutations or making modifications at a specific site. While there are several successful protocols, it remains a complex field, with many techniques requiring enhancements. Applicability of techniques are limited due to their innovative nature, risks and ethical concerns.

Aim of study. Analysis and synthesis of information on Gene Therapies, highlighting indications and limitations in modern medicine.

Methods and materials. The study was based on a systematic review of publications over the last decade on selected topics using Google Academic, PubMed and Scopus electronic databases, combining keywords related to gene therapy, indications, limits of gene therapy. A total of 343 articles were retrieved, 42 of which met the inclusion criteria.

Results. Genetic modification involves the transfer of DNA, but can also include oligonucleotides or RNA-based therapies. A relatively new technique is CRISPR-Cas9, a system adapted from the natural defense mechanisms of bacteria and archaea and enable highly precise molecular modifications. The approach of gene therapy is broad and has the potential to treat diseases caused by recessive genetic disorders, acquired conditions, and certain viral infections such as AIDS. Recombinant DNA technology, a commonly used technique, introduces the gene of interest into a vector, but risks include the presence of viral genetic material and the potential for oncogenic transformation. Limits, particularly in cancer treatment, arise from recurrences and neutralizing antibodies, impacting therapy effectiveness. The prevalence of neutralizing antibodies against certain subtypes of the virus reaches up to 70% in the general population, reducing the therapy's effectiveness and limiting the categories of patients who can participate in studies. Similarly, high costs limit access to treatments, for example, gene therapy for hemophilia B, Hemgenix, has become the most expensive therapy in the world, priced at 3.5 million dollars. Not least, ethical dilemmas raise numerous questions to be resolved, such as impact of germline genetic modifications and the possibility of creating "designer babies," as well as genetic diversity, informed consent and equitable access for all.

Conclusion. Gene therapies represent an alternative to conventional treatments. The design of new experimental vectors and the specificity of delivery systems can lead to the expansion of techniques in clinical applications. Similarly, it is necessary to understand the scientific and technological challenges, ethical concerns and public opinion to manage the challenges that could hinder the further development of the field.