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THERAPEUTIC POTENTIAL OF CRISPR- CAS9 TECHNOLOGY IN THE TREATMENT OF GENETIC DISEASES: IMPLICATIONS AND ACTIONS OF NURSES IN CLINICAL PRACTICE

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Abstract: **INTRODUCTION:** CRISPR/Cas9 technology has made a name for itself in the field of science, especially in the context of gene editing therapies. The aim of this study was to analyze the contribution of nursing in supporting care in this area, considering the role of nurses in implementing these therapies. **MATERIALS AND METHODS:** This study was based on data collected from scientific articles available on Google Scholar, COFEN and various specialized scientific journals, which provided an in-depth analysis of the subject. **RESULTS AND DISCUSSION:** Analysis of the data extracted from the scientific literature reveals that nursing plays a crucial role in various stages of the therapeutic process. Its duties include the prevention of pathologies, early diagnosis, administration of therapies, collection of tests, promotion of health education, continuous monitoring of patients and guidance for families. The articles reviewed show that, although CRISPR/Cas9 is a recent technology, nursing is directly involved in providing care, guidance and emotional support to patients during the therapeutic process. **FINAL CONSIDERATION:** Nurses play an essential role in patient care, not only in conventional practices, but also in emerging areas such as gene editing therapy. In the context of CRISPR/Cas9, nursing actively contributes by providing guidance, monitoring patients, mediating the necessary tests, promoting comfort in the face of adverse effects and accompanying the patient during all phases of treatment until its conclusion.

Keywords: Nursing, CRISPR/Cas9, Nursing Care, Nurse.

INTRODUCTION

The CRISPR/Cas9 technique is an advanced genetic editing tool based on an adaptive bacterial defense system. The name derives from the combination of the CRISPR system (Clustered Regularly Interspaced Short Palindromic Repeats) with the Cas9 endonuclease protein (CRISPR-associated protein 9). In this mechanism, a molecule of guide RNA (gRNA) directs the Cas9 enzyme to specific target DNA sequences, promoting cutting at predetermined sites. This action is compared

to a pair of “molecular scissors”, which allow the deletion, insertion or replacement of specific stretches of genetic material. Once the DNA double strand has been cleaved, natural cell repair mechanisms such as homologous recombination or non-homologous end joining (NHEJ) are activated, allowing the gene of interest to be modified. In this way, the technique allows genetic mutations to be corrected with high precision and is promising for the treatment of various hereditary and acquired diseases (LI et al., 2023). According to the aforementioned authors, the CRISPR/Cas9 technique is a promising tool for treating various genetic diseases, including cancer, muscular dystrophy and sickle cell anemia. Its application is based on the deletion or correction of genes that have suffered pathogenic mutations, making it possible to restore normal gene function. In the case of cancer, for example, the technique can be used to inactivate mutated oncogenes or restore tumor suppressor genes, while in monogenic diseases such as muscular dystrophy and sickle cell anemia, it acts directly to modify the defective gene, contributing to the development of personalized and potentially curative therapies.

Genetic diseases represent a significant challenge for public health worldwide, being responsible for high morbidity rates and a negative impact on the quality of life of affected individuals. For example, the World Health Organization (WORLD HEALTH ORGANIZATION, 2006) already recognizes hemoglobinopathies as a relevant public health problem, due to their high global prevalence and the burden they place on health systems, especially in low- and middle-income countries. This scenario is corroborated by recent studies that point to the seriousness of genetic disorders of hemoglobin as a persistent and significant problem in the pediatric and clinical context (RODIGARI; BRUGNERA; COLOMBATTI, 2022). In addition, rare ge-

netic diseases, which affect millions of people worldwide, are associated with high treatment costs and challenges in clinical communication, which further increases their complexity for healthcare systems (ALLEN et al., 2021).

The constant evolution of biomolecular technologies has enabled promising advances in the therapeutic field, especially with the advent of the CRISPR-Cas9 technique, a gene editing tool that allows DNA to be modified in a precise, efficient and relatively affordable way (JAVAID et al., 2022). This innovative approach has aroused the interest of the scientific community due to its potential in the treatment of various genetic diseases, such as muscular dystrophies (FATEHI et al., 2023), cystic fibrosis (GRAHAM; HART, 2021), sickle cell anemia (TARIQ et al., 2024) and other hereditary-based pathologies.

In the context of clinical practice, the incorporation of new therapeutic technologies requires qualified and up-to-date multi-professional action, with nurses being one of the key players in comprehensive patient care. Understanding the technical, ethical and legal aspects related to the application of CRISPR-Cas9 is essential for nursing professionals to be able to act safely and ethically in providing care, health education and welcoming patients undergoing this type of therapy.

This highlights the relevant role of nursing in the application of emerging technologies, such as therapies based on gene editing. Nurses, as essential professionals on the front line of patient care, must constantly seek to update and improve their knowledge of these new therapeutic approaches. Specific qualifications are essential for health professionals who wish to work in this field, considering that although it is still an area that has been little explored in clinical practice, there is already promising evidence of its effectiveness. Furthermore, it is essential that nurses understand the multiple aspects involved in their work, such as

knowledge about the diagnostic tests indicated, treatment protocols, possible adverse reactions and appropriate guidance for patients throughout the therapeutic process (Vasques; Lacerda, 2022).

In this way, nurses play a crucial role in mediating between scientific advances and the humanization of care, making a significant contribution to informed decision-making and dealing with the psychosocial challenges arising from gene therapies. This requires not only technical-scientific mastery, but also ethical, communicative and empathetic skills, which are essential for welcoming patients in the face of innovative and often complex therapies. According to the Code of Ethics for Nursing Professionals, it is the nurse's duty to ensure the patient's right to clear and objective information, respecting their autonomy and promoting care based on respect for human dignity (COFEN, 2017). This guideline is especially relevant in the context of gene therapies, which involve clinical decisions and possible ethical and emotional implications for patients and their families.

This review aims to explore the therapeutic potential of CRISPR-Cas9 technology in the treatment of genetic diseases, as well as to discuss the implications and possibilities for nurses in the current clinical scenario. The critical analysis of these aspects will contribute to expanding knowledge about the interface between biotechnological innovation and care practice, promoting the qualification of care and ethical reflection on the direction of genetic therapy.

CRISPR-CAS9 TECHNOLOGY AND ITS THERAPEUTIC APPLICATION

The mechanism of action of CRISPR-Cas9 gene editing therapy occurs through the association between the CRISPR system and Cas proteins, forming a complex called CRISPR-

-Cas9, which acts like “molecular scissors”. This activity is mediated by a guide RNA (gRNA), which directs the Cas9 enzyme to a specific point in the DNA sequence. The gRNA hybridizes to the complementary nitrogenous bases, allowing the Cas9 enzyme to recognize the target site and cleave the DNA molecule, promoting a precise break in the double helix (BERNARDES et al., 2021). After DNA cleavage, the cell activates its own natural repair mechanisms, which can result in changes to the genetic sequence, such as deletions, insertions or, in some cases, the correction of pre-existing mutations. This process makes it possible to modify or delete specific regions of the genetic code, thus allowing science to precisely manipulate the cell’s genetic material (BERNARDES et al., 2021).

With regard to advances in the clinical use of gene editing, there have been significant milestones in the treatment of genetic pathologies. In this context, the use of CRISPR-Cas9 technology aims to correct diseases at the cellular level, restoring gene function and providing more effective, precise and positive impacts. This process includes both the modification of stem cells and the direct editing of genes in the affected tissues or organs (LOPES DE ALMEIDA, 2024).

Among the advances observed is the cure of hereditary conditions such as Hypertrophic Cardiomyopathy, a genetic disease caused by mutations in genes that code for heart muscle proteins, resulting in thickening and stiffening of the ventricular walls (MARTINS et al., 2025). In this sense, gene therapy makes it possible to edit the altered genes, promoting remission of the disease. Other advances include the correction of muscular dystrophies (JULLIANNE et al., 2023), the treatment of heart conditions (AREND; PEREIRA; MARKOSKI, 2017) and advances in gene therapy applied to neurological diseases (CAVASSANI NETO et al., 2024). These

approaches make up the set of pathologies positively impacted by gene editing therapies, such as CRISPR-Cas9, and demonstrate significant advances in their respective treatments (DUTRA; ANDRADE, 2024).

In the context of genetic diseases targeted by the CRISPR-Cas9 technique, the treatment of Sickle Cell Anemia, a genetically-based hereditary disease, stands out. CRISPR-Cas9 therapy works by modifying the patient’s own stem cells, reintroducing them with the aim of promoting the production of functional hemoglobins, resulting in a significant improvement in quality of life (MACHADO; KRUL, 2022). Another example is the application of gene editing in cancer treatment. In this approach, the technique locates and deletes the mutation in the DNA, allowing the previously altered cell itself to start a repair process and resume homeostasis. Due to its high precision, cancer therapy based on CRISPR-Cas9 provides more effective and personalized interventions (BARROSO et al., 2024)

Cystic Fibrosis is also a potential target for the application of this technology. This pathology results from mutations in the CFTR gene, responsible for producing the CFTR protein, which acts as a chloride ion channel in cell membranes. When mutated, the gene causes changes in the viscosity of mucus and sweat, affecting various systems in the body (BUENO; ROCHA, 2022). The use of CRISPR-Cas9 makes it possible to identify and correct these mutations, restoring the functionality of the gene and the CFTR protein (BUENO; ROCHA, 2022).

Similarly, gene editing has been investigated in the treatment of lung neoplasms, with the technique acting to correct specific mutations in the DNA of lung cells, promoting the restoration of their functional integrity (ALMEIDA; CLEIDE; SOUZA, 2021).

BENEFITS AND ETHICAL CHALLENGES OF GENE THERAPY

With regard to its therapeutic and preventive potential in the treatment of hereditary diseases, CRISPR-Cas9 technology is highly effective in correcting mutated genes in various pathologies of genetic origin (SERPELONI et al., 2022), as well as in intervening in infectious diseases by interrupting the expression of viral genes or modifying the immune response (BURILLE et al., 2024).

In general, the technique makes it possible to edit DNA, correcting defective genes and altering gene expression, with a view to treating and preventing hereditary diseases. Considering that, in hereditary processes, genes are transmitted from parents to children, the correction of these genes through CRISPR-Cas9 prevents the transmission of pathogenic mutations to future generations (SOUZA et al., 2023). Furthermore, the technology's high precision and efficiency reinforce its therapeutic potential.

With regard to the limitations and risks associated with gene editing, we highlight the possibility of editing errors, such as the occurrence of cuts outside the target site, which can result in unwanted mutations. Therefore, continuous improvement of the technique is fundamental to increasing clinical safety, minimizing risks and improving the system's accuracy (GOMES; APARECIDA DA CUNHA; CRUZ, 2021).

The bioethical issues and social implications arising from the clinical application of CRISPR-Cas9 raise concerns related to the unpredictable effects on future generations, especially due to the possibility of manipulating germ cells (VILELA; REGIS PEIXOTO; FRANCO TAKETANI, 2021). In short, the use of technology must be aligned with the principles of beneficence, aimed exclusively at promoting human well-being (VILELA; REGIS PEIXOTO; FRANCO TAKETANI, 2021).

NURSES AND GENETIC COUNSELING

Another relevant aspect to be considered in the field of genetics is the role of nurses as mediators of genetic information. In this context, nursing acts as a link between scientific knowledge and the patient. The nursing professional acts as a health educator, transmitting information and guidance in a clear and assertive manner about individual needs, analyzing the patient's genetic history and promoting the appropriate mediation of information (GOMES; VALE, 2022).

Nurses provide comprehensive care to individuals, considering their care needs at all stages of care. With regard to genetics, nurses carry out a broad analysis of family hereditary issues, use tools such as genetic maps and communicate information in an understandable way, guiding the patient, clarifying doubts and providing emotional support (NASCI-MENTO; SILVA, 2023).

Through therapeutic communication, nurses address and assess the genetic issues involved, propose solutions and help patients make informed decisions. In this way, nursing plays an essential role in providing guidance on hereditary genetic aspects, therapeutic methods and prophylactic strategies, accompanying the patient throughout the care process (MURAD, 2023).

During the genetic counseling process, the nursing professional must have adequate technical and scientific training, demonstrating in-depth knowledge of genetics and related care practices, as well as ensuring strict compliance with ethical principles. Health care demands comprehensive attention to the needs of patients and their families, respecting confidentiality, professionalism and promoting ethical, qualified and resolute care (GOMES; VALE, 2022).

Table 1 highlights the main duties of nurses in the context of genetic information me-

diation, an essential field for clinical practice in genetics. The nurse's role in this scenario goes beyond physical care, including education, counseling and psychological support, which are fundamental aspects for ensuring comprehensive care for the patient and their family.

It is therefore essential for nursing professionals to continually update themselves on advances in the field of genetics, which is constantly evolving, so that they can offer care based on the latest scientific evidence and best practices. In this context, nurses play an essential role as facilitators in the communication of genetic information, contributing to patient understanding and promoting informed decision-making, always with respect for autonomy and individual values. Integrating this information is crucial not only for nurses' academic and professional training, but also for promoting ethical and efficient practice in the field of genetics.

THE ROLE OF NURSES IN CLINICAL PRACTICE WITH CRISPR-CAS9

Nurses are professionals who take an active part in various stages of patient care. In this context, nurses are involved from screening the patient through to gathering information, family history and laboratory and genetic tests. In addition, the professional provides emotional support, welcomes and promotes humanization, providing guidance and helping to understand the treatment, as well as adherence to preventive measures, in addition to post-exam follow-up (NUNES; CANABARRO, 2021).

Another of the nurse's competencies is monitoring side effects and promoting well-being, since nursing accompanies patients at various times, with the aim of ensuring their health and well-being (NUNES; CANABARRO, 2021).

Studies show that nurses act as a link between scientific knowledge and the patient, promoting accessible and humanized communication, which is essential for individuals to understand the risks, benefits and limitations of gene therapies (Kawasaki et al., 2021). In addition, nursing work in the genetic context requires the incorporation of knowledge related to genomics, counseling and health education, strengthening the patient's ability to make informed decisions (Flória-Santos et al., 2013).

The role of nursing in advanced therapies, such as CAR-T cell therapy, one of the main advances in the field of gene therapy, is fundamental to ensuring the safety and well-being of patients. Although genetic manipulation is not a direct responsibility of nursing professionals, their contribution is essential at various stages of treatment (Steinbach et al., 2023).

In therapies such as chemotherapy, radiotherapy and immunotherapies, nurses play crucial roles, including: the assessment and clinical monitoring of the patient, with a focus on the early identification of adverse effects and complications; patient and family education, promoting appropriate guidance regarding treatment, symptom management and home care; as well as the coordination of care, collaborating with the multiprofessional team to ensure an integrated and patient-centered approach (Dailah et al., 2024).

In the specific context of CAR-T cell therapy, nurses are responsible for several essential stages of the therapeutic process (Steinbach et al., 2023). These include preparing the patient, assessing eligibility and monitoring the collection of T-cells. During the infusion of the cell product, the nurse monitors it closely, looking out for possible serious complications such as cytokine release syndrome. After the procedure, ongoing support is essential, both in terms of education and emotional support

Role	Description	Reference
Family history collection and analysis	The nurse carries out detailed collection of the patient's family history, identifying hereditary patterns and genetic predispositions. This analysis is essential for the early detection of possible genetic conditions and for planning appropriate interventions.	(HÉBERT et al., 2022)
Health education and therapeutic communication	Acts as an educator, providing clear and accessible information about genetics, genetic testing and the implications of hereditary conditions. Uses therapeutic communication to emotionally support the patient and their family, promoting understanding and adherence to the care plan.	(CALZONE et al., 2024)
Genetic counseling and decision-making support	Actively participates in the genetic counseling process, assisting patients in understanding genetic risks, testing options and possible outcomes. Offers support in making informed decisions, respecting the autonomy and individual values of each patient.	(HERNANDES; COIMBRA; SILVA, 2025)
Emotional and psychological support	Provides continuous emotional support to patients and their families, especially in the face of challenging genetic diagnoses. Facilitates adaptation to genetic conditions, promoting psychological well-being and resilience.	(MANCL et al., 2024)
Ethical behavior and confidentiality of genetic information	Guarantees the confidentiality of patients' genetic information, respecting ethical and legal principles. Acts with sensitivity and respect for patients' decisions, ensuring that the information is used appropriately and safely.	(HERNANDES; COIMBRA; SILVA, 2025)

Table 1: The Nurse’s Role in Mediating Genetic Information

Nurse Action	Description	Reference
Clinical assessment and monitoring	Continuous observation of vital signs and symptoms, early identification of adverse effects and complications, especially in CAR-T therapy.	(Steinbach et al., 2023)
Patient and family education	Guidance on treatment, side effects, home care and self-care.	(Dailah et al., 2024)
Emotional support and welcome	Psychological support for the patient and family, facilitating adherence to treatment.	(Lyu et al., 2024)
Preparing the patient for advanced therapy	Assessment of eligibility and monitoring of cell collection in the case of CAR-T therapy.	(Baer, 2021)
Administration and monitoring during infusions	Supervision of the infusion of drugs and cell products, such as CAR-T cells, with attention to reactions such as cytokine release syndrome.	(Baer, 2021)
Coordination of multi-professional care	Collaboration with doctors, pharmacists and other health professionals to ensure focused and integrated care.	(Beaupierre, RN, BSN, OCN et al., 2019)
Participation in the management of the nursing routine	Organizing the hospital routine and acting on patient safety care protocols.	(Beaupierre, RN, BSN, OCN et al., 2019)
Training and professional updating	Participation in training and continuing education programs to keep up with advances in cancer and cell therapies.	(Kenyon et al., 2024)

Table 2: Descriptions of nurses’ actions in CAR-T therapy.

for the patient and their family throughout the recovery process. Table 2 shows the nurse's main actions in therapies such as CAR-T, chemotherapy, radiotherapy and immunotherapies.

Although practices related to biotechnology, such as genetic manipulation, are not the direct responsibility of nurses, their role in the field of care remains indispensable. Nursing remains a fundamental pillar in promoting safety, humanization and effectiveness in the care provided to patients in complex and innovative therapies. These responsibilities highlight the importance of the nursing professional in the implementation and success of cell therapies. Continuous training and the development of specific skills are therefore essential in order to meet the growing demands generated by these innovative therapeutic approaches.

Nursing plays an essential role in multifactorial patient care, especially in the context of cancer treatment. According to Silva, Marinho and Imbiriba (Silva; Marinho; Imbiriba, 2021), in the management of breast cancer, the nurse accompanies the patient from the beginning of the diagnosis, during all the therapeutic stages and in the post-treatment follow-up. In this scenario, the nurse's role includes carrying out nursing consultations, monitoring and mitigating the adverse effects of treatment, drawing up therapeutic plans with a focus on preventing complications, as well as mediating referrals and communicating with multidisciplinary teams. Other duties involve collecting laboratory tests and administering chemotherapy drugs. Although the study mentioned focuses mainly on chemotherapy, it should be noted that in immunotherapy, nurses' duties do not differ substantially, and remain centered on comprehensive care, emotional support and promoting patient safety throughout the therapeutic process.

TRAINING AND CHALLENGES FOR NURSING IN CLINICAL GENETICS

In addition, it is of the utmost importance that nursing professionals working in this area seek continuing training in genetics in order to achieve specialization and, consequently, be able to provide high-quality, timely and assertive care in the clinical field of genetics (DUTRA; ANDRADE, 2024).

Integrating biotechnology into care practice is essential because, in order to provide qualified care, professionals must have scientific knowledge of the biotechnological processes that make up the therapy applied to the patient (BERNARDES et al., 2021).

In addition, the limitations associated with care must be considered, whether they are structural, such as the precision of genetic editing, the possibility of errors and the need to improve the technique with a view to greater safety, efficacy and precision, or related to the needs of the multidisciplinary team, such as the qualification and specialization of professionals and the continuous improvement of scientific knowledge and care practices (VILELA; REGIS PEIXOTO; FRANCO TAKE-TANI, 2021).

CONCLUSION

CRISPR-Cas9 technology represents a significant advance in the field of gene editing therapies, with great therapeutic potential for the treatment of various genetic diseases. By analyzing data from relevant scientific sources, it was possible to verify that nurses play an essential role in the process of assisting patients undergoing genetic therapies, such as CRISPR-Cas9. Nurses work on a number of fronts, such as the prevention of pathologies, early diagnosis, test collection, health education, patient follow-up and family support. In addition, their presence is crucial to ensure that patients receive the information they

need to make informed decisions, promoting the humanization of care and comfort in the face of the adverse effects of therapy. The nurse's role is fundamental to optimizing therapeutic results and, at the same time, ensuring the promotion of ethical, welcoming and quality care, meeting the physical, emotional and psychological needs of patients during the therapeutic process. In this way, nurses not only take an active part in the application of gene therapies, but also assume a crucial role in education, emotional support and guidance, essential elements for the success of gene

editing therapies and the well-being of the patient. The study concludes that, with the evolution of therapeutic technologies, nurses continue to play an indispensable role, ensuring excellence in care and the application of these innovations in clinical practice.

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