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BIOTECHNOLOGY IN MODERN MEDICINE: ADVANCES IN GENE, CELL, AND PRECISION THERAPIES AND THEIR IMPACTS ON GLOBAL HEALTH

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ABSTRACT

Biotechnology has assumed a strategic role in transforming modern medicine, especially through the development of gene therapies, cell therapies, and precision medicine strategies. This integrative review aimed to analyze the main advances of biotechnology applied to contemporary medicine, with an emphasis on gene, cellular, and precision therapies, as well as their clinical, scientific, and global impacts. To this end, 33 studies published between 2017 and 2024 were selected from scientific databases and international journals of recognized relevance, including clinical trials, translational studies, follow-up studies, scientific reviews, and methodological research. The studies were organized into three thematic areas: gene therapies and genetic editing; cell therapies and advanced immunotherapy; and precision medicine, personalized vaccines, and global impacts.The results showed that gene therapies, including platforms based on CRISPR-Cas9, viral and lentiviral vectors, have demonstrated potential to modify the natural history of genetic, hematologic, neuromuscular, ophthalmologic, and metabolic diseases. Cell therapies, especially CAR-T cells, have established themselves as important strategies in precision oncology, with relevant clinical responses in refractory or relapsed hematologic malignancies. Precision medicine, in turn, has expanded opportunities for therapeutic personalization through RNA vaccines, neoantigens, molecular biomarkers, and genomic tools. It is concluded that biotechnology represents one of the main transformative forces in 21st-century medicine, although its consolidation depends on long-term safety, economic sustainability, ethical regulation, specialized infrastructure, and equity in global access.

Keywords:biotechnology; gene therapy; cell therapy; precision medicine.

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INTRODUCTION

Biotechnology has become one of the main drivers of transformation in modern medicine, enabling the diagnosis, treatment, and monitoring of diseases to be carried out on the basis of increasingly specific molecular, genetic, cellular, and immunological foundations. Unlike the conventional therapeutic model, which has historically been centered on standardized interventions for large population groups, new biotechnological platforms have made possible approaches tailored to the biological particularities of each patient, ushering in a more precise, personalized, and potentially transformative phase in the natural history of complex, rare diseases with high clinical impact. In this setting, gene therapies, cell therapies, and precision medicine strategies have come to occupy a central position in contemporary scientific development, with significant repercussions for clinical practice, translational research, and the organization of health systems.

The advancement of gene therapies represents one of the most significant expressions of this shift paradigmatic. The possibility of correcting, replacing, modulating, or silencing genes involved in the pathophysiology of inherited and acquired diseases has substantially expanded therapeutic prospects in conditions that were previously considered intractable or managed predominantly with palliative approaches. Studies involving CRISPRCas9 gene editing in hemoglobinopathies, such as sickle cell disease and **β**-thalassemia, have shown that the ex vivo modification of hematopoietic stem cells can induce sustained production of fetal hemoglobin, reduce severe clinical manifestations, and, in some cases, decrease or eliminate depe-ndence on blood transfusions[1-4]. These findings demonstrate that direct intervention on the disease's genetic mechanisms can replace chronic control approaches with potentially transformative strategies.

In addition to ex vivo applications, in vivo gene editing is expanding the frontiers of molecular medicine. The demonstration of a significant reduction in serum transthyretin in patients with amyloidosis inherited after systemic administration of a CRISPR-Cas9 platform represented a historic milestone, by indicating that genetic editing can be performed directly in the human body for therapeutic purposes[5]. Simila-

rly, the use of base editing technologies in CAR-T cells for recurrent T-cell acute lymphoblastic leukemia reinforces the potential of new genetic engineering tools to enhance the safety, specificity, and clinical applicability of advanced cell therapies[6]. These advances indicate that biotechnology not only expands the existing therapeutic repertoire, but also redefines the very limits of medical intervention.

In rare genetic diseases, gene therapies have been demonstrating a significant impact on clinical outcomes previously associated with high morbidity and mortality. Gene replacement therapy for spinal muscular atrophy type 1 showed improved survival and motor function in infants, substantially changing

the prognosis of a severe neuromuscular disease[7,8]. Similarly, ocular gene therapy with voretigene neparvovec for retinal dystrophy associated with mutations in the RPE65 gene has demonstrated improved visual function and therapeutic durability, consolidating the feasibility of genetic interventions in inherited retinal diseases[9, 10]. Similar advances have been observed in hemophilias A and B, in which vector-based therapies are reducing bleeding episodes and the need for continuous prophylaxis[11-13]. These results reinforce the role of biotechnology in the transition from repetitive and compensatory treatments to strategies for sustained molecular correction or compensation.

Lentiviral and hematopoietic platforms have also contributed to the development of gene therapies applied to hematologic and neurometabolic diseases. LentiGlobin therapy demonstrated biological and clinical efficacy in patients with sickle cell disease, while lentiviral gene therapy using hematopoietic stem cells showed promising results in early-onset metachromatic leukodystrophy, especially when administered in the early stages of the disease[14, 15]. These findings reinforce the importance of integrating early diagnosis, genetic screening, laboratory infrastructure, and timely access to advanced therapies. In this sense, gene therapy is no longer only an experimental promise and has begun to form a therapeutic area with progressively greater clinical maturity, although it is still surrounded by challenges related to long-

term safety, cost, scalability, and equity [16].

Alongside gene therapies, cell therapies have produced profound changes in modern oncology, especially in the treatment of refractory or relapsed hematologic malignancies. The engineering of T lymphocytes with chimeric antigen receptors, known as CAR-T cells, enabled the redirection of the immune system against specific tumor targets. Clinical studies using tisagenlecleucel in B-cell acute lymphoblastic leukemia, axicabtagene ciloleucel in large B-cell lymphoma, and KTE-X19 in mantle cell lymphoma demonstrated relevant clinical responses in patients with aggressive disease and limited treatment options[17-22]. These results established cellular immunotherapy as one of the major advances in precision oncology.

In multiple myeloma, CAR-T therapies targeting the B-cell maturation antigen, such as bb2121, idecabtagene vicleucel, and ciltacabtagene autoleucel, expanded treatment prospects for patients

multirefractory, with deep responses in highly complex clinical scenarios[23-25]. However, despite the observed benefits, cell therapies still face important limitations, including immune-mediated toxicities, cytokine release syndrome, neurotoxicity, relapses due to antigen escape, difficulty of access, high cost, and the need for specialized centers. For this reason, strategies based on genetic editing have been investigated to optimize the persistence, safety, antitumor potency, and accessibility of CAR-T cells, pointing to a new generation of more sophisticated cell therapies that may be applicable to solid tumors and other immune-mediated conditions[26-28].

Another central axis of contemporary biotechnology is precision medicine, which integrates genomic, molecular, clinical, immunological, and computational data to guide individualized treatment decisions. In oncology, the identification of actionable mutations, tumor neoantigens, and specific immunological profiles has enabled

the development of personalized vaccines based on RNA and neoantigens. Pioneering studies have shown that personalized vaccines can mobilize specific immune responses against individual tumor mutations in patients with melanoma, while

more recent approaches in pancreatic cancer and high-risk resected melanoma indicate the potential for integrating individualized vaccines with immunotherapy using checkpoint inhibitors[29-32]. These advances demonstrate that biotechnology is enabling a form of medicine capable of producing tailored interventions based on the biological characteristics of the tumor and the patient.

Meanwhile, the impacts of biotechnology on modern medicine are not limited to therapeutic efficacy. The expansion of gene, cell, and precision therapies brings relevant ethical, economic, regulatory, and social implications for global health. The high cost of these technologies, the need for highly specialized infrastructure, the concentration of clinical trials in high-income countries, and the underrepresentation of genetically diverse populations in genomic databases may amplify inequalities that already exist. The use of polygenic risk scores and other genomic tools in global populations requires careful validation, sample diversity, and strategies that prevent the reproduction of scientific and care-related biases[33]. Thus, precision medicine will only achieve an effective global impact if it is associated with equity, responsible regulation, and the democratization of access to innovations.

In this context, it becomes necessary to critically analyze the advances of biotechnology in modern medicine, considering both its transformative potential and the challenges for its safe, sustainable, and equitable implementation. Gene, cell, and precision therapies represent a new phase of translational medicine, in which the molecular understanding of diseases is progressively converted into highly complex clinical interventions. Therefore, this integrative review aims to analyze recent advances in biotechnology applied to modern medicine, with an emphasis on gene, cell, and precision therapies, discussing their main clinical, scientific, and global impacts on contemporary health.

METHODOLOGY

This study is characterized as an integrative literature review, of a qualitative, descriptive, and exploratory nature, developed with the objective of analyzing advances in biotechnology in modern medicine, with an emphasis on gene therapies, cell therapies, and precision medicine strategies, as well as their clinical, scientific, and global impacts. The integrative review was chosen because it allows for the gathering, critical evaluation, and synthesis of evidence from different methodological designs, including clinical trials, translational studies, follow-up studies, scientific reviews, and applied research, enabling a broad and systematized understanding of the investigated topic.

The guiding question of the review was defined as follows: what are the main advances in biotechnology applied to modern medicine, especially in the field of gene, cell, and precision therapies, and what impacts have these technologies produced in clinical practice and global health? Based on this question, the bibliographic search was directed toward studies that addressed advanced biotechnological technologies with medical application, including gene editing by CRISPRCas9, gene therapies based on viral and lentiviral vectors, CAR-T-type cell therapies, personalized vaccines based on RNA and neoantigens, pharmacogenomics, population genomics and precision medicine strategies.

The search for studies was carried out in databases scientific recognized internationally, with priority for PubMed/MEDLINE, in addition to high-impact journals indexed in biomedical and multidisciplinary databases, such as *The New England Journal ofMedicine*,*The Lancet*, *Nature*,*Science*,*Cell*,*Nature Reviews Genetics*,*Frontiers in Immunology*,*Molecular Cancer*,*Biomarker Research*,*Journal of Clinical Oncology* and *Ophthalmology*. English-language descriptors and free terms related to the topic were used, combined using Boolean operators, including: *"*biotechnology*"*, *"*modern medicine*"*,*"*gene therapy*"*, *"*CRISPR-Cas9*"*, *"*genome editing*"*, *"*cell therapy*"*, *"*CART cell therapy*"*, *"*precision medicine*"*,*"*personalized medicine*"*, *"*RNA vaccine*"*,*"*neoantigen vaccine", "hemoglobinopathies", "sickle cell disease", "beta-thalassemia", *"*hemophilia*"*, *"*spinal muscular atrophy*"*, *"*multiple myeloma*"*, *"*lymphoma*"*,*"*global health*"* and *"*polygenic risk scores*"* . The selection of terms aimed to encomp-

ass both therapeutic advances directly applied to clinical practice and the challenges associated with implementing these technologies in different health contexts.

Studies published predominantly between 2017 and 2024 were included, a period considered relevant because it concentrates decisive advances in the clinical consolidation of gene, cell, and precision therapies. The time delimitation is also justified by the rapid development of gene-editing platforms, the approval and expansion of the use of CAR-T therapies, the progress of gene therapies for rare and hematologic diseases, and the growth of personalized vaccines based on RNA and neoantigens. Despite the main time frame, studies with high scientific relevance, clinical impact, and bibliographic traceability were prioritized, especially those published in journals of recognized editorial quality and wide international circulation.

The inclusion criteria adopted were: original scientific articles, clinical trials, translational studies, follow-up studies, relevant scientific reviews, and methodological studies that addressed directly gene therapies, cell therapies, genetic editing, cellular immunotherapy, precision medicine, or global impacts of biotechnology on health; publications available in English; studies with clear identification of the evaluated technology, population or clinical condition, key findings, and contribution to modern medicine; and articles published in indexed and traceable journals. Editorials without relevant analytical data, opinion commentaries, studies without a direct link to the scope of the review, duplicate publications, articles with insufficient information for methodological characterization, and work that addressed biotechnology in a generic way without a defined clinical or translational application were excluded.

After the initial search, the studies were assessed for thematic relevance, scientific robustness, clinical applicability, timeliness, type of technology addressed, and contribution to understanding the impacts of biotechnology on modern medicine. The final selection consisted of 33 studies, organized into three major thematic axes: gene therapies and genetic editing; cell therapies and advanced immunotherapy; and precision medicine, personalized vaccines,

population genomics and global impacts. This organization enabled an integrated analysis of the evidence, considering both the clinical results observed and the scientific, ethical, economic, and social implications associated with the development of these technologies.

Data extraction was carried out from a study characterization matrix, including the following information: author and year of publication, country or study context, study design type, assessed technology, population or clinical condition investigated, main findings, and contribution to modern medicine. This approach made it possible to compare different biotechnological applications, identify innovation trends, recognize existing gaps, and understand how these technologies are modifying the diagnosis, treatment, and prognosis of complex diseases. The extracted data were analyzed in a descriptive and interpretive manner, with a narrative synthesis of the main findings, according to the integrative nature of the review.

Because this is an integrative review based exclusively on previously published studies available in scientific databases, there was no need for submission to the Research Ethics Committee, in accordance with the applicable guidelines for bibliographic review studies. Even so, the principles of scientific integrity, traceability of sources, fidelity to the evidence analyzed, and appropriate author attribution were respected through citations in the Vancouver style. The methodology adopted aimed to ensure rigor, transparency, and coherence in the selection and analysis of the studies, allowing the presented results to consistently reflect the current state of biotechnology applied to modern medicine and its impacts on global health.

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RESULTS

The final sample of this integrative review consisted of 33 studies published between 2017 and 2024, distributed across three main thematic axes: gene therapies and genetic editing; cell therapies and advanced immunotherapy; and precision medicine, personalized vaccines, and global impacts. Organizing by axes made it possible to identify the main areas of progress in biotechnology applied to modern medicine, showing advances ranging from interventions directly aimed at correcting or modulating genetic targets to highly personalized cellular and immunological strategies.

Of the total number of studies analyzed, 16 addressed gene therapies, genetic editing, or related platforms for modifying molecular aspects of hereditary diseases, hematologic, neuromuscular, ophthalmologic, and metabolic[1-16]. The other 12 studies focused on cell therapies, especially CAR-T cells applied to hematological malignancies, as well as emerging strategies to optimize these therapies through genetic editing[17-28]. Finally, five studies investigated precision medicine–associated strategies, including personalized RNA-based vaccines, neoantigen-guided immunotherapy, and global challenges related to the use of genomic scores in diverse populations[29-33].

Table 1 - Synthesis of the Studies Included in the Integrative Review According to Theme Axis and Scientific Contribution

thematic axis and scientific contribution

Thematic axis	Included studies	Technologies/approaches evaluated	Predominant clinical conditions	Main scientific contributions
Gene therapies and gene editing	16 Studies [1-16]	CRISPR-Cas9 ex vivo and in vivo, base editing, AAV vectors, lentiviral vectors, gene replacement, and hematopoietic gene therapy	Sickle cell disease, β-thalassemia, transthyretin amyloidosis, spinal muscular atrophy, inherited retinal dystrophy, hemophilia A and B, metachromatic leukodystrophy	They demonstrated potential to modify the natural history of genetic and hematologic diseases, reduce dependence on chronic therapies, restore biological functions, and expand the clinical application of gene editing and replacement

				genetics.
Cell therapies and advanced immunotherapy	12 studies[17-28]	CAR-T anti-CD19, CAR-T anti-iBCMA, KTE-X19, tisagenlecleucel, axicabtagene ciloleucel, idecabtagene vicleucel, ciltacabtagene autoleucel, CRISPR-optimized CAR-T Acute B-cell	lymphoblastic leukemia, diffuse large B-cell lymphoma, mantle cell lymphoma, multiple myeloma, and refractory hematologic malignancies Demonstrated significant clinical responses in relapsed or refractory oncohematologic diseases, establishing cellular immunotherapy as one of the main frontiers of precision oncology.
Precision medicine, personalized vaccines, and global impact	5 studies [29-33]	Personalized RNA vaccines, neoantigen vaccines, individualized immunotherapy, polygenic risk scores, and population genomics	Melanoma, pancreatic cancer, high-risk solid tumors, and populations genetically diverse	They demonstrated the feasibility of personalized interventions based on the patient's molecular profile and highlighted the importance of genetic diversity, equity, and population validation for global health.

The studies grouped in the first axis showed that gene therapies are reaching an increasingly mature level of clinical development, especially in monogenic and hematologic diseases. Works involving CRISPR-Cas9 in hemoglobinopathies indicated that genetic editing of hematopoietic stem cells can promote sustained increases in fetal hemoglobin, reduce vaso-occlusive crises, and achieve transfusion independence in some patients with sickle cell disease and β-thalassemia[1-4]. In addition, in vivo gene editing for transthyretin amyloidosis demonstrated that CRISPR-based platforms can be administered systemically to directly modulate genes involved in human diseases[5].

Still, in the axis of gene therapies, studies on spinal muscular atrophy, hereditary retinal dystrophy, hemophilias, and metachromatic leukodystrophy reinforced the clinical impact of gene replacement or transfer platforms in rare, highly severe diseases[7-15]. The evidence analyzed indicated that these therapies may reduce reliance on continuous treatments, improve functional outcomes, and significantly alter the prognosis of conditions previously associated with important therapeutic limitations. However, the findings also pointed to challe-

nges related to the durability of the response, long-term safety, cost high, infrastructure

specialized and need for early diagnosis.

In the second axis, studies on cell therapies showed that CAR-T cells significantly modified the treatment of refractory or relapsed hematological malignancies. Clinical trials with tisagenlecleucel, axicabtagene ciloleucel, and KTE-Xl9 demonstrated relevant clinical responses in acute lymphoblastic leukemia of B cells, diffuse large B-cell lymphoma, and mantle cell lymphoma[17-22]. In multiple myeloma, antigen-targeted therapies to BCMA, such as bb2l2l, idecabtagene vicleucel, and ciltacabtagene autoleucel, demonstrated deep responses in patients with multi-refractory disease[23-25].

These findings consolidate cell therapy as one of the main expressions of personalized medicine applied to oncology.

Despite favorable clinical results, the studies also highlighted relevant limitations of cellular therapies, including immune-mediated toxicity, cytokine release syndrome, neurotoxicity, tumor escape, relapses, high cost, and difficulty in scaling up access. In this context, recent reviews emphasized the role of CRISPR/Cas gene editing in optimizing CAR-T cells, with the potential to increase cellular persistence, reduce immune exhaustion, improve safety, and enable the development of more affordable and universal cellular products[26-28].

The third axis brought together studies focused on precision medicine, especially in the field of personalized vaccines and population genomics. RNA vaccines and neoantigens demonstrated the ability to induce specific immune responses against individual tumor mutations in melanoma and pancreatic cancer, pointing to a new stage of personalized immunotherapy[29-32]. These studies show that integrating genomic sequencing, bioinformatics, immunology, and RNA platforms can enable therapeutic interventions tailored to the patient's and tumor's molecular profile.

Finally, the analysis of the studies also demonstrated that biotechnological advances cannot be evaluated by isolated clinical efficacy alone. The global imp-

lementation of precision medicine depends on genetic diversity in databases, validation of predictive models across different populations, technological infrastructure, appropriate regulation, and policies for equitable access[33]. Thus, the results of this Review indicate that biotechnology in modern medicine presents a high transformative potential, but its consolidation requires integration between scientific innovation, economic sustainability, biomedical ethics, and justice in global health.

DISCUSSION

The findings of this integrative review show that biotechnology occupies a strategic position in redefining modern medicine, especially by enabling interventions capable of directly acting on the genetic, cellular, molecular, and immunological foundations of diseases. Analysis of the 33 included studies demonstrated that gene therapies, cell therapies, and precision medicine strategies represent not only isolated therapeutic innovations, but a broader movement to transform health care. This movement progressively shifts clinical practice from a predominantly standardized model to a biomarker-driven medicine oriented by genomic profiles, cellular engineering, targeted immunomodulation, and therapeutic individualization.

In the field of gene therapies and genetic editing, the analyzed studies demonstrate consistent progress in hereditary and hematological diseases of high complexity. Evidence involving CRISPR-Cas9 in sickle cell disease and β-thalassemia indicates that editing hematopoietic stem cells can promote relevant clinical benefits, such as increased fetal hemoglobin, reduced vaso-occlusive events, and transfusion independence in certain patient groups[1-4]. These results are particularly important because they signal a shift in approach: rather than merely controlling complications or continuously replacing deficient components, gene therapy seeks to modify core biological mechanisms of the disease. Thus, biotechnology begins to offer prospects of treatment potentially long-lasting for conditions historically associated with high clinical

burden, dependence on ongoing care, and a significant impact on quality of life.

In vivo gene editing, exemplified by research in transthyretin amyloidosis, represents another important milestone, as it demonstrates the possibility of performing genetic intervention directly in the human body[5]. This advance broadens the therapeutic horizon for diseases in which ex vivo modification of cells would not be sufficient or operationally feasible. However, this strategy also requires rigorous attention to safety, molecular specificity, the risk of off-target effects, the limited reversibility of interventions, and long-term monitoring. The consolidation of in vivo gene editing will therefore depend on the combination of clinical efficacy, technological precision, and robust post-treatment surveillance.

Gene therapies based on viral and lentiviral vectors have also shown clinical relevance in rare, neuromuscular diseases, ophthalmologic, hematologic and neurometabolic. Studies involving spinal muscular atrophy, hereditary retinal dystrophy, hemophilias, and metachromatic leukodystrophy indicate that gene transfer or gene replacement can modify functional outcomes, reduce dependence on recurrent therapies, and alter the natural history of severe diseases [7-15]. These findings reinforce the importance of early diagnosis, genetic screening, and structuring care networks capable of identifying eligible patients before irreversible damage occurs. In progressive diseases, especially neurologic and neuromuscular ones, the timing of intervention may be decisive for therapeutic success.

Despite advances, gene therapy still faces important challenges. Among them are the extremely high cost, manufacturing complexity, the need for specialized centers, limited long-term safety data, the possibility of an immune response against vectors, variability in the duration of the therapeutic effect, and the difficulty of broad adoption in public or private healthcare systems. In addition, the existence of potentially transformative therapies does not, by itself, guarantee equitable access. Low- and middle-income countries, as well as socially vulnerable populations in developed countries, may remain outside these innovations if more inclus-

construídos modelos de financiamento, regulação e transferência tecnológica mais inclusivos.

In the field of cell therapies, the results show that CAR-T cells have established themselves as one of the most important innovations in contemporary oncology. Studies with Tisagenlecleucel, axicabtagene ciloleucel, KTE-Xl9, and anti-BCMA therapies have demonstrated relevant clinical responses in relapsed or refractory leukemias, lymphomas, and multiple myeloma. [17-25]These results are especially significant because they involved populations frequently associated with poor prognosis and limited therapeutic options. The ability to collect cells from the patient themselves, genetically modify them, expand them in the laboratory, and reinfuse them with targeted antitumor function expresses an advanced integration between immunology, genetic engineering, cell biology, and personalized medicine.

The consolidation of CAR-T therapies also highlights a change in concept. of medication. Differently of drugs conventional, these therapies are Living, dynamic, and biologically active products capable of proliferating, persisting, and interacting with the microenvironment. tumoral. That This characteristic explains part of its effectiveness, but it is also related to significant adverse events, such as cytokine release syndrome and neurotoxicity. Therefore, the safe implementation of these therapies requires highly trained teams, rigorous monitoring protocols, adequate hospital infrastructure, and the ability to rapidly manage immunological complications.

Another relevant point is that cell therapies still face significant logistical and economic barriers. Individualized production, the time between collection and infusion, the need for a sophisticated production chain, and the high cost hinder their global dissemination. In this sense, studies on the optimization of CAR-T cells through CRISPR/Cas and other gene editing tools point to promising ways to overcome current limitations. [26-28]Strategies such as increasing cell persistence, reducing immune exhaustion, improving tumor specificity, developing allogeneic therapies, and producing universal cells can broaden the clinical

applicability of these technologies. However, these innovations will also require new standards.

On the third axis, precision medicine has demonstrated strong potential to individualize therapeutic interventions, especially in oncology. Personalized RNA vaccines and neoantigen vaccines indicate that molecular sequencing of the tumor can guide the production of immunotherapies designed to stimulate specific responses against individual mutations [29-32]. This approach represents an important evolution in personalized oncology, as it turns genomic information into individualized therapeutic products. The integration of next-generation sequencing, bioinformatics, tumor immunology, and RNA platforms creates a treatment model in which each patient can receive an intervention based on the specific biology of their disease.

The development of personalized vaccines also demonstrates the growing convergence between biotechnology and data science. Identifying relevant neoantigens requires complex computational analysis, prediction of binding to the major histocompatibility complex, assessment of immunogenicity, and selection of targets with a higher likelihood of response. Thus, precision medicine depends not only on laboratory technologies, but also on digital infrastructure, robust algorithms, qualified databases, and ongoing clinical validation. This integration is expected to expand in the coming years, especially with advances in artificial intelligence applied to biomarker discovery, therapeutic selection, and the design of personalized interventions.

However, precision medicine presents significant challenges related to global equity. Most genomic databases and genetic association studies still have a predominance of populations of European ancestry, which limits the generalizability of predictive models to genetically diverse populations[33]. This limitation is particularly relevant in the use of polygenic risk scores, pharmacogenomics, and genomic stratification of complex diseases. If these tools are applied without adequate population validation, there is a risk of widening diagnostic and therapeutic inequalities, resulting in a form of

precision medicine that is precise only for certain population groups.

In this way, the biotechnological advances analyzed in this review need to be understood from an expanded perspective that includes not only effectiveness and innovation, but also distributive justice, economic sustainability, ethical regulation, and social responsibility. Global health faces the challenge of incorporating high-cost technologies without deepening asymmetries between countries, institutions, and populations. To do so, it will be necessary to strengthen inclusive research policies, increase diversity in clinical trials, encourage regional technological production, promote international cooperation, and develop financing models that reconcile innovation and access.

Another relevant aspect concerns professional training. The incorporation of gene, cellular, and precision therapies requires professionals who are trained to interpret genomic tests, understand molecular mechanisms, manage specific adverse events, work in dialogue with multidisciplinary teams, and guide patients regarding the risks, benefits, and limitations of these therapies. Thus, biotechnology does not only transform the treatments available, but also redefines the clinical, laboratory, regulatory, and educational competencies needed for health care.

The integrated analysis of the studies makes it possible to state that biotechnology is promoting a structural shift in modern medicine. Therapies that previously seemed restricted to the experimental field are already showing measurable clinical impact in severe, rare, and refractory diseases. However, the consolidation of these technologies will depend on long-term evidence, cost reduction, expanded infrastructure, regulatory standardization, biological safety, and equity of access. Innovation, therefore, must be accompanied by scientific responsibility and social commitment.

As a limitation of this review, the heterogeneity of the included studies stands out, since clinical trials, translational studies, follow-up studies, and scientific reviews were analyzed. This diversity is consistent with the integrative nature of the review, but it limits direct quantitative comparisons between technologies, populations, and outcomes. In addition, many of the therapies assessed still have relatively recent follow-up data,

which calls for caution when interpreting durability, long-term safety, and cost-effectiveness. Even so, selecting high-impact studies with clinical relevance made it possible to build a robust synthesis of the current state of biotechnology applied to modern medicine.

In summary, the analyzed studies indicate that gene, cell, and precision therapies are central pillars of a new phase of medicine, characterized by personalization, molecular intervention, and the integration between advanced biology and clinical practice. Their impacts are significant, but their global implementation requires strategic planning, ongoing scientific validation, solid regulatory frameworks, and policies that ensure equitable access. Biotechnology, therefore, represents one of the greatest promises of contemporary medicine, provided that its advancement is guided not only by innovation, but also by safety, sustainability, and health justice.

CONCLUSION

This Integrative Review Showed That Biotechnology Occupies a Central Position in the Transformation of Modern Medicine, Especially Through the Development of Gene Therapies, Cell Therapies, and Precision Medicine Strategies. The Analyzed Studies Demonstrated That These Technologies Have Been Significantly Modifying Diagnosis, Treatment, And

the prognosis of genetic, hematological diseases, neuromuscular, ophthalmological, oncological, and complex conditions of high clinical relevance. By enabling targeted interventions into the molecular, genetic, cellular, and immunological mechanisms of disease, biotechnology expands therapeutic possibilities and strengthens the shift from a predominantly standardized medicine

toward a more individualized, predictive, and personalized approach.

Gene therapies and gene-editing tools, such as CRISPR-Cas9, have shown significant potential to modify the natural history of diseases previously associated with chronic, palliative, or limited-efficacy treatments. Evidence in hemoglobinopathies, transthyretin amyloidosis, spinal muscular atrophy, retinal dystrophies, hemophilias, and leukodystrophies indicate that genetic interventions can restore biological functions, reduce severe clinical manifestations, and provide lasting therapeutic benefits. Th-

ese findings reinforce the role of gene therapies as one of the most relevant frontiers of contemporary translational medicine.

Cell therapies, especially CAR-T cells, have consolidated as a high-impact innovation in precision oncology, with relevant clinical responses in refractory or relapsed hematologic malignancies. The engineering of immune cells has made it possible to turn the immune system itself into a personalized therapeutic tool, expanding treatment prospects for patients with limited alternatives. However, challenges related to toxicity, cost, production logistics, specialized infrastructure, and equitable access still limit its widespread adoption in health systems.

Precision medicine, in turn, has shown great potential by integrating genomic, molecular, immunological, and computational data to guide individualized interventions. Personalized RNA vaccines and neoantigens, biomarker-guided immunotherapies, and genomic tools for risk stratification represent promising advances toward therapeutic personalization. However, its global implementation requires greater population diversity in studies, validation of models across different genetic groups, and policies that prevent the widening of health inequalities.

It is concluded that biotechnology represents one of the main driving forces in 2lst-century medicine, with the potential to redefine therapeutic paradigms and expand the capacity to intervene in complex diseases. However, its consolidation will depend not only on clinical efficacy and technological innovation, but also on long-term safety, economic sustainability, ethical regulation, specialized professional training, and the democratization of access. Thus, the future of biotechnological medicine should be guided by a balance between scientific progress, social responsibility, and equity in global health.

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