International Journal of Science and Research Archive
eISSN: 2582-8185
Cross Ref DOI: 10.30574/ijsra
Journal homepage: https://ijsra.net/

(REVIEW ARTICLE)

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# Use of stem cells in the recovery of neurodegenerative diseases: Advances and clinical barriers

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International Journal of Science and Research Archive, 2025, 14(02), 1336-1339

Publication history: Received on 13 January 2025; revised on 20 February 2025; accepted on 23 February 2025

Article DOI: https://doi.org/10.30574/ijsra.2025.14.2.0532

## Abstract

Neural stem cells, a type of multipotent cell, can generate neurons and glial cells, contributing to nervous tissue repair. Additionally, induced pluripotent stem cells (iPSCs) can be reprogrammed from the patient's own cells, reducing the risk of rejection and enabling personalized therapies. Studies investigate the injection of these cells into the brain to replace lost neurons, modulate inflammation, and promote the release of neurotrophic factors that protect remaining neurons. Thus, the objective of this study is to evaluate the potential of stem cells in neural regeneration and neuroprotection in neurodegenerative diseases, exploring their therapeutic applicability and the challenges involved. This study was conducted through a literature review, analyzing scientific articles published in the last five years. The search was carried out in major scientific databases, including PubMed, Scopus, Web of Science, ScienceDirect, and SciELO, ensuring a comprehensive and up-to-date selection on the application of stem cells in neurodegenerative diseases. Findings suggest that, in addition to gene therapy, the use of stem cells can be an effective tool in treating various neurodegenerative diseases, with the potential to restore neuronal function and improve patients' quality of life. The combination of gene and cell therapies represents a promising frontier in regenerative medicine, especially for diseases such as Alzheimer's, Huntington's, ALS, and others, with the promise of significant advancements in treating these debilitating conditions. However, the prospects for using stem cells in the treatment of neurodegenerative diseases continue to grow, offering renewed hope for millions of patients. If technical obstacles are overcome, stem cells could become a crucial tool in regenerative medicine, providing new therapeutic options and improving the quality of life for patients affected by these devastating diseases.

**Keywords:** Stem Cells; Cell Therapy; Neurodegeneration; Regenerative Medicine

## 1. Introduction

Stem cells have the ability to self-renew and differentiate into various cell types, being classified as totipotent, pluripotent, multipotent, oligopotent, and unipotent. While totipotent cells can give rise to all embryonic and extraembryonic tissues, pluripotent cells generate cells from the three germ layers. Multipotent, oligopotent, and unipotent cells, in turn, exhibit a progressively more restricted specialization (CARVALHO & RODRIGUES, 2022).

In regenerative medicine, stem cells have been explored for the treatment of various diseases, including neurodegenerative conditions such as Alzheimer's, Parkinson's, and amyotrophic lateral sclerosis (ALS). Since these diseases are associated with the progressive degeneration of neurons, which lack natural regeneration capacity, cell therapies emerge as a promising approach (CARVALHO & RODRIGUES, 2023).

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According to Neves (2024), despite advancements, challenges such as controlling cell differentiation and the risk of tumor formation still need to be overcome. However, with research progress, stem cell-based therapies have the potential to revolutionize the treatment of neurodegenerative diseases, offering hope to patients with no effective therapeutic options. Thus, the objective of this study is to evaluate the potential of stem cells in neural regeneration and neuroprotection in neurodegenerative diseases, exploring their therapeutic applicability and the challenges involved.

## 2. Methodology

This study was conducted through a literature review, analyzing scientific articles published in the last five years. The search was carried out in major scientific databases, including PubMed, Scopus, Web of Science, ScienceDirect, and SciELO, ensuring a comprehensive and up-to-date selection on the application of stem cells in neurodegenerative diseases.

The search strategy utilized controlled and uncontrolled descriptors, combined with Boolean operators (AND, OR), in both Portuguese and English to expand the scope of the studies found. The main terms used were: "Células-tronco," "Neurodegeneração," "Doenças neurodegenerativas," "Terapia celular," "Regeneração neural," and "Medicina regenerativa" in Portuguese, and "Stem cells," "Neurodegeneration," "neurodegenerative diseases," "Cell therapy," "Neural regeneration," and "Regenerative medicine" in English.

Articles published between 2019 and 2024, in English or Portuguese, that address the use of stem cells in the treatment of neurodegenerative diseases, both in experimental and clinical models, were included. Studies exclusively focusing on basic mechanisms without therapeutic applications, opinion articles, reviews without a clear methodology, and duplicate publications were excluded.

The selected articles were critically analyzed regarding methodology, key findings, and clinical implications. The synthesis of the findings allowed for the identification of trends, challenges, and perspectives on the use of stem cells in neural regeneration and the treatment of neurodegenerative diseases.

## 3. Literature Review

Recent studies on gene therapy for neurodegenerative diseases have shown promising results, with different therapeutic approaches being tested to treat conditions such as Canavan disease, Krabbe disease, Alzheimer's disease, Huntington's disease, amyotrophic lateral sclerosis (ALS), and others. Among the most promising approaches, the use of stem cells has gained prominence due to their ability to regenerate tissues and restore impaired neurological functions (BELÉM & FELIPIN, 2021; CARVALHO & RODRIGUES, 2023).

In Canavan disease, a clinical study using gene therapy with adeno-associated virus vectors (AAV-ASPA) demonstrated a significant reversal of N-acetyl-aspartate (NAA) accumulation in affected brain regions, leading to improved brain function in patients. Although some complications associated with the vector delivery procedure were observed, the use of stem cells in this context may enhance therapeutic effects and accelerate neuronal regeneration, promoting more effective brain function recovery (BELÉM & FELIPIN, 2021; CARVALHO & RODRIGUES, 2023).

For Krabbe disease, a therapeutic approach in newborn mice involving injections of viral particles into various brain regions showed an increase in GALC enzyme activity, resulting in weight gain and significant improvements in overall health. In this scenario, stem cells could be used to regenerate damaged nerve cells, amplifying beneficial effects and prolonging patient survival (BELÉM & FELIPIN, 2021; VARELA et al., 2024).

In the treatment of Alzheimer's disease, experiments with gene therapy using nerve growth factors (NGF) have shown favorable results in activating neuronal responses, such as axonal growth, and have proven to be a safe approach with long-lasting therapeutic effects. Additionally, stem cell application could aid in neuronal regeneration in affected areas, promoting the restoration of cognitive functions (BELÉM & FELIPIN, 2021; SILVA et al., 2024).

Other studies have demonstrated that gene therapy with specific miRNAs effectively reduces the translation of the huntingtin (HTT) gene, responsible for Huntington's disease. The use of stem cells in this context may help replace or regenerate damaged cells, allowing for a more efficient therapeutic approach to the disease (BITTAR et al., 2024).

Furthermore, gene therapy using adeno-associated viral vectors and genetic manipulation with CRISPR/Cas9 have shown potential to halt the production of proteins associated with neurodegenerative diseases, such as amyloid protein in Alzheimer's disease and SOD1 protein in ALS. The integration of stem cells into these treatments could further improve neuronal regeneration and therapeutic efficacy, increasing the survival and quality of life of patients affected by these pathologies (SILVA et al., 2024).

These findings suggest that, in addition to gene therapy, the use of stem cells may be an effective tool in treating various neurodegenerative diseases, with the potential to restore neuronal function and improve patients' quality of life. The combination of gene and cell therapies represents a promising frontier in regenerative medicine, especially for diseases such as Alzheimer's, Huntington's, ALS, and others, with the potential for significant advancements in the treatment of these debilitating conditions (BELÉM & FELIPIN, 2021; CARVALHO & RODRIGUES, 2023; SILVA et al., 2024).

## 4. Conclusion

Stem cells offer a promising approach for the treatment of neurodegenerative diseases due to their unique ability to regenerate and repair damaged tissues. With the potential to replace compromised nerve cells, stem cells can play a crucial role in diseases such as Alzheimer's, Parkinson's, amyotrophic lateral sclerosis (ALS), and other conditions affecting the central nervous system. The use of stem cells, particularly induced pluripotent stem cells (iPSCs) and adult stem cells, enables neuronal regeneration, restoration of cognitive and motor functions, and may even slow or reverse the progression of these diseases.

Despite promising experimental advances in animal models, stem cell treatments in humans still face significant challenges, such as the effective integration of transplanted cells, prevention of immune rejection, and the risk of tumor formation. The long-term safety and efficacy of these treatments need to be further investigated through rigorous clinical trials.

Nevertheless, the prospects for stem cell use in treating neurodegenerative diseases continue to expand, offering renewed hope for millions of patients. If technical obstacles are overcome, stem cells could become a crucial tool in regenerative medicine, providing new therapeutic options and improving the quality of life for patients affected by these devastating diseases.

## Compliance with ethical standards

*Disclosure of conflict of interest*

No conflict of interest to be disclosed.

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