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Regenerative Medicine: A Game Changer in Alzheimer's Disease

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Abstract

Regenerative medicine is able to restore, repair, and regenerate diseased cells and tissues. This is a highly promising method of treating neurodegenerative disorders, such as Alzheimer's disease. Alzheimer's is a progressive disease that negatively impacts memory, cognition, and quality of life for millions of people around the world. The literature review that follows examines new regenerative strategies that show potential to reverse, slow, or stop the progression of Alzheimer's, including gene therapy, stem cell therapy, neurotrophic factors, and tissue engineering. However, these strategies all come with limitations. For example, treatments have a hard time crossing through the blood-brain barrier. In addition, ethical issues can also arise. Although there is still no cure, the following evidence indicates that regenerative medicine may pave the way towards long-term, targeted therapies.

Keywords: Alzheimer's disease, regenerative medicine, gene therapy, stem cells, neurodegeneration

1. Introduction

Alzheimer's disease (AD) is the most prevalent type of neurodegenerative disease, manifesting as progressive loss of brain regions related to memory, language, and higher-order cognitive functions (Selkoe, 1999). The disease begins with subtle memory impairment and slowly develops into total loss of communicative function as well as the loss of ability to carry out activities of daily living. Age remains the strongest predictor of AD risk. The risk of developing AD increases significantly after age 60, although early-onset cases can appear as early as the mid-40s; AD is also a top ten leading cause of death in the United States and was the seventh leading cause of death among U.S. adults in 2022 (CDC, 2024). A cure is yet to be discovered, but existing research suggests promising regenerative treatments, such as stem cell therapy, gene therapy, and delivery of neurotrophic factors. They have the potential to inhibit the advancement of AD and promote the regeneration of damaged neural tissue. In the past, treatments for AD were focused more on managing or reducing symptoms, rather than trying to find an actual cure for the disease itself. The purpose of this literature review is to examine and analyze strategies that could potentially lead to such a cure. While existing literature does examine individual strategies, it often does not compare multiple approaches by identifying the advantages and limitations. This literature review uses existing evidence, appraises the therapeutic potential of these strategies, and outlines the major challenges and the pathways for future development within the discipline.

2. Materials and Methods

This narrative literature review is a compilation of peer-reviewed articles, government health agency databases, and recent studies from institutions committed to Alzheimer's research. The sources were selected with regard to their relevance to regenerative medicine and its therapeutic uses in AD, such as stem cell therapy, gene therapy, neurotrophic factors, and tissue engineering. Specifically, the sources were gathered from reputable medical

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publications, peer-reviewed journals, and health organizations. Most of the sources in this paper were published within the past seven years; however, earlier research was included when necessary to explain the biological basis of Alzheimer's disease.

3. Results

The exact causes of AD are still unknown, but current research suggests that aberrant protein behavior has a primary role in neuronal dysfunction and degeneration (Mayo Clinic, 2024a). Two signature proteins—amyloid-beta and tau—are the most extensively studied in AD research. These proteins form amyloid plaques and tau neurofibrillary tangles, which, in turn, cause the progressive cognitive decline associated with AD (Figure 1). Amyloid plaques develop when fragments of the larger amyloid-beta precursor protein are cleaved. Some of these fragments are beta-amyloid peptides, which aggregate together to form extracellular amyloid deposits (Selkoe, 1999). These plaques interfere with intercellular communication and initiate inflammatory processes that result in further neuronal damage (Mayo Clinic, 2024a). The tau proteins from which tau neurofibrillary tangles develop normally stabilize microtubules and promote intracellular transport; however, in AD, they become hyperphosphorylated, meaning excessive phosphate groups attach to the protein, which, in turn, causes their regular function to be impaired. The tangles that develop as a result of this hyperphosphorylation disrupt neuronal structure and function, with eventual cell death (Mayo Clinic, 2024a). Together, amyloid plaques and tau neurofibrillary tangles create a neurotoxic environment that underlies the cognitive decline process that characterizes AD. Because AD damages neurons, researchers study regenerative therapies to minimize the effects of the damage and offer a potential treatment.

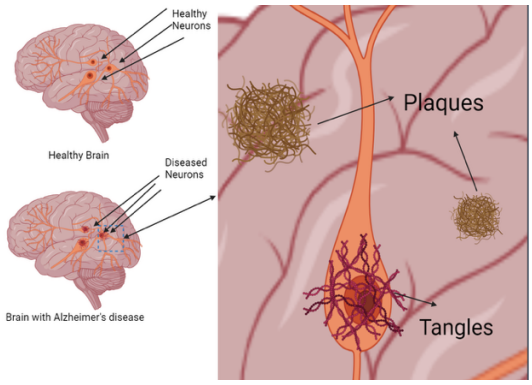

Figure 1. Healthy Brain vs. AD Brain (Mayo Clinic, 2024a).

4. Discussion

Alzheimer's disease remains incurable despite the presence of several drugs. Some of these are cholinesterase inhibitors, which increase acetylcholine—a neurotransmitter that is deficient in AD—and memantine, which regulates glutamate signaling to facilitate neural transmission (Mayo Clinic, 2024b). The medications can improve symptoms and slow the progression of the disease to some extent, but they cannot reverse or prevent neurodegeneration. Regenerative medicine, aimed at replacing or repairing damaged cells, tissues, or organs, is another promising approach. Since AD is characterized by widespread neuronal loss, regenerative strategies are a biologically reasonable therapeutic approach. Three major regenerative approaches currently being considered for AD are discussed in this section: stem cell therapy, gene therapy, and delivery of neurotrophic factors.

4.1 Stem Cell Therapy

Stem cells can differentiate into many cell types, including neurons. Stem cell therapy refers to the use of stem cells to treat conditions by restoring dying neurons or reconstructing brain tissue (Liu et al., 2020). Alzheimer's leads to complete mental disability through neuronal death and degeneration. Techniques have been established for the differentiation of stem cells into a neuron lineage, so that there is hope for the transplantation of neuron-like cells into the brain. Various types of stem cells are being explored for the treatment of AD, with various risks and advantages. Researchers at Stanford Medicine have reported that stem cell therapy in mice relieved brain abnormalities typical of Alzheimer's disease (Stanford Medicine, 2023). In this study, hematopoietic stem cell transplants, which specifically transplant stem cells that produce blood cells, replaced dysfunctional microglia, the brain's immune cells, in mice with healthy cells, restoring normal TREM2 activity and reducing amyloid plaque deposits in the brain (Stanford

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Medicine, 2023). These results suggest that stem cell therapy may offer a path to reduce the effects of AD. In fact, it has also shown strong results in clinical studies (Liu et al., 2020). However, researchers called this practice too dangerous for humans, because it would require an excessive amount of chemotherapy or radiation treatment in the current procedure.

4.2 Gene Therapy

Stem cell therapy is not the only therapeutic strategy that can be used for the treatment of Alzheimer's disease. Another option is gene therapy. Genes are parts of cells that contain DNA. Gene therapy is the process of transplanting genes into cells that have either missing or defective genes. Gene therapy can also manipulate genetic factors that cause Alzheimer's disease; for example, it can target beta-amyloid plaques and replace neurons (Ruwa, 2023). This is important because plaques are one of the two major proteins that cause Alzheimer's disease, and one of the major problems with Alzheimer's disease is damaged neurons. Gene therapy can aid these processes by targeting the genetic and molecular causes of AD; however, it is still experimental, and its safety has yet to be proven (Levy, 2023). When neurons are damaged, they typically die without intervention; however, gene therapy can replace faulty DNA, allowing damaged neurons to survive.

4.3 Neurotrophic Factors

There are other methods to treat Alzheimer's disease besides cell-or gene-based therapies. Using neurotrophic factors can be very beneficial. Neurotrophic factors are biological molecules that support a neuron's growth and survival; in addition, they even decrease the rate of neurodegeneration (Gao et al., 2022). Delivering neurotrophic factors directly to the brain is a viable strategy for treating Alzheimer's disease. Neurotrophic factors also preserve synaptic function; this is especially important because memory and learning, both of which are negatively impacted by AD, heavily rely on healthy synaptic function (Kim et al., 2025). AD causes neurons to die, but with neurotrophic factors promoting neuron survival, the neurons have a much better chance of survival. However, this method does not come without its limitations. AD is a complex disease, and because neurotrophic factors target only one biological pathway, the process may not fully halt the disease; additionally, it is very difficult for neurotrophic factors to cross the blood-brain barrier since it blocks out most large molecules (Gao et al., 2022). Because of these limitations, it is best to use neurotrophic factors alongside other therapies rather than using them alone in order to maximize their effects.

4.4 Tissue Engineering

There are other methods that are not as common for AD treatment, such as tissue engineering. Tissue engineering is the process of creating biological tissue. Tissue engineering often uses synthetic polymeric scaffolds, 3D engineering frameworks, that are designed to mimic the extracellular structure of the brain (Kumar et al., 2025). Because these scaffolds mimic the physical environment of the brain, they promote both cell survival and regeneration (Zhang et al., 2023). Since Alzheimer's disease is a neurodegenerative disorder, tissue engineering may help replace parts of the brain that have been lost by generating new tissue. However, limitations still exist. The use of this process is not fully unrestricted because it can cause instability in patients; in addition, the polymeric scaffolds may leave harmful monomers or harmful chemicals behind in the brain (Kumar et al., 2025). This method is still in an early stage, but it offers a new way to potentially treat AD (Zhang et al., 2023).

5. Hurdles to Overcome

Regenerative medicine shows immense potential in offering a treatment for AD, yet there are still hurdles that must be overcome. Because the brain has a barrier called the blood-brain barrier, drugs have a difficult time reaching the brain when used to treat the disease (Liu et al., 2020). Sometimes, when transplanting foreign substances into the brain, the patient can have cell rejection or an abnormal immune response. These risks raise ethical concerns,

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particularly regarding patient safety and long-term outcomes. Cost is also a major limitation (Yiannopoulou & Papageorgiou, 2020). Patient safety is a primary concern in clinical trials because immune rejection can cause even more brain damage. Additionally, the long-term effects of transplanted cells are still unknown. If scientists do not know the potential risks, then the method cannot be used to treat real people. These challenges currently hinder scientists from confidently using regenerative medicine for human treatment.

6. Our Potential Future

While regenerative medicine for Alzheimer's disease shows immense potential, several challenges must be addressed before these therapies can become widely available. In the future, every patient might receive personalized medicine through an approach called precision medicine, which tailors treatment strategies based on an individual patient's biological characteristics (Yiannopoulou & Papageorgiou, 2020). Precision medicine uses biomarkers, defined as measurable biological indicators of disease presence, progression, or response to treatment, and neuroimaging (Yiannopoulou & Papageorgiou, 2020). Neuroimaging allows doctors to assess the brain's health and the extent of disease progression and damage. Biomarkers allow doctors to evaluate how well a patient responds to a particular treatment. Precision medicine may reduce the risk of cell rejection and make the treatment of Alzheimer's disease much safer.

7. Conclusion

Many approaches exist to treat Alzheimer's disease. Each approach has its own advantages and disadvantages, but based on the current research, regenerative medicine is the most promising approach towards a possible cure. Regenerative medicine can potentially restore the damaged part of an AD-affected brain. In the future, the major concerns will be treatment-associated risks and the development of a permanent cure. Ongoing research continues to explore better methods for treating Alzheimer's disease, and one day, a complete cure may be found. Regenerative medicine has already given new hope to patients who were once considered untreatable. It also offers broader potential for treating other diseases characterized by tissue degeneration. Since the field continues to advance, sustained dedication by clinicians and researchers can lead to developments that save and improve many lives.

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