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Commentary

Potential of Nanomedicine and Biotherapeutics in Neurodegenerative Disease Therapy (NDT)

Yuki Ishida*

Department of Engineering Science, Meiji University, Tokyo, Japan

DESCRIPTION

Neurodegenerative diseases, such as Alzheimer's disease, Parkinson's disease, Huntington's disease, and Amyotrophic Lateral Sclerosis (ALS), present significant challenges to modern medicine. These disorders are characterized by progressive neuronal degeneration, often accompanied by cognitive decline, motor dysfunction, and eventual loss of independent function. Nanomedicine, defined as the application of nanotechnology in medicine, focuses on the use of nanoscale materials (typically 1 nm to 100 nm) to improve drug delivery, diagnostic tools, and therapeutic strategies. In the context of neurodegenerative diseases, nanoparticles have attracted considerable attention due to their ability to cross the Blood-Brain Barrier (BBB), a major barrier to drug delivery to the brain. The BBB, a selective permeability barrier between the blood and the brain, protects against harmful substances, but also prevents most therapeutic agents from reaching neural tissue. Nanoparticles, due to their small size and surface modification, can be engineered to bypass the BBB through receptor-mediated transport, endocytosis, or transient barrier disruption. This paves the way for delivering a wide range of biotherapeutic agents, including small molecules, proteins, nucleic acids, and even gene editing tools such as CRISPR-Cas9 (Clustered Regularly Interspaced Short Palindromic Repeats-associated protein 9), directly into the brain.

Biotherapies, including biologics such as monoclonal antibodies, gene therapies, and protein-based therapies, offer promising solutions for neurodegenerative diseases by targeting the underlying molecular mechanisms. For example, in Alzheimer's disease (AD), the accumulation of beta-amyloid plaques and tau tangles is a hallmark of the disease. Recent clinical trials have explored the use of monoclonal antibodies such as aducanumab and lecanemab to target and clear amyloid plaques, although clinical results have been mixed. The potential to improve the delivery of these biologics to the brain using nanocarriers may improve their delivery effectiveness and minimize side effects. In Parkinson's Disease (PD), the loss of dopamine neurons in the substantia nigra is essential for disease progression. Traditional treatments, such as levodopa, provide symptomatic relief but do not prevent neuronal degeneration. However, advances in gene therapies, such as the delivery of neurotrophic factors like Glial Cell Line-Derived Neurotrophic Factor (GDNF) by nanoparticles, aim to provide long-term neuroprotection by promoting the survival and regeneration of dopaminergic neurons.

The integration of nanomedicine with biotherapies holds great promise for improving the treatment of neurodegenerative diseases. Nanoparticles can be designed to encapsulate and deliver therapeutic proteins, nucleic acids, and small molecules with high specificity and controlled release. For example, Lipid Nanoparticles (LNPs), which are already used to deliver mRNA vaccines, can be adapted to deliver small interfering RNAs (siRNAs) or Antisense Oligonucleotides (ASOs) that specifically silence disease-causing genes, such as those encoding mutant Huntington's disease or ALS proteins. In addition, nanocarriers can be functionalized with targeting ligands, such as antibodies or peptides, to selectively bind to specific receptors or disease biomarkers in neurons, thereby improving the accuracy of drug delivery. This targeting capability reduces systemic toxicity and increases the therapeutic index of biotherapies. Recent studies have shown that nanoparticles Polymeric nanoparticles, gold, and silica-based nanocarriers can be used to target neuroinflammation, an important component of neurodegenerative diseases.

Despite the promise, many challenges remain in translating nanomedicine and biotherapy into clinical therapies for neurodegenerative diseases. One of the main obstacles is the toxicity and biocompatibility of nanomaterials. Although nanoparticles can provide better distribution, their long-term accumulation in the body, especially in organs such as the liver and spleen, can cause side effects. Therefore, careful design and testing are necessary to ensure the safety of these nanomaterials. Additionally, scalability and regulatory hurdles present significant challenges in bringing these therapies to the clinic. Regulators require comprehensive safety and efficacy data prior to approval, which can slow the journey from bench to bedside.

Correspondence to: Yuki Ishida, Department of Engineering Science, Meiji University, Tokyo, Japan, E-mail: yuki.ishida@meiji-u.ac.jp

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In addition, personalized medicine will play an important role in future developments. The use of nanomedicine may allow for the personalization of therapies based on individual genetic profiles, disease stages, and specific biomarkers, providing a more targeted approach to the treatment of neurodegenerative diseases. Nanomedicine and biotherapeutics represent an exciting frontier in the fight against neurodegenerative diseases. By using nanotechnology to improve the delivery of biotherapeutic agents, it is possible to overcome some of the major obstacles to the treatment of these devastating diseases. Although significant challenges remain, the convergence of these two fields has great potential to improve the lives of millions of people affected by Alzheimer's disease, Parkinson's disease and other neurodegenerative diseases, paving the way for more effective and personalized therapies in the near future.