

ABSTRACT

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Nanoparticle mediated gene therapy: a trailblazer armament to fight CNS disorders.

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Central nervous system (CNS) disorders account for boundless socioeconomic burdens with devastating effects among the population, especially the elderly. The major symptoms of these disorders are neurodegeneration, neuroinflammation, and cognitive dysfunction caused by inherited genetic mutations or by genetic and epigenetic changes due to injury, environmental factors, and disease-related events. Currently available clinical treatment for CNS diseases, i.e., Alzheimer's disease, Parkinson's disease, stroke, and brain tumor have significant side effects and are largely unable to halt the clinical progression. So, gene therapy displays a new paradigm in the treatment of these disorders with some modalities, varying from suppression of endogenous genes to expression of exogenous genes. Both viral and non-viral vectors are commonly used for gene therapy. Viral vectors are quite effective but associated with immunogenicity and carcinogenicity like severe side effects, and poor target cell specificity. Thus, non-viral vectors, mainly nanotherapeutics like nanoparticles (NPs), opt-out to be a realistic approach in gene therapy in achieving higher efficacy. NPs demonstrate a new avenue in pharmacotherapy for the delivery of drugs or genes to their selective cells or tissue thus providing concentrated and constant drug delivery to targeted tissues, minimizing systemic toxicity and side effects. The current review will emphasize the role of NPs in mediating gene therapy for CNS disorders treatment. Moreover, the challenges and perspectives of NPs in gene therapy will be summarized.

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