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Short Communication

Viral Vectors and the Gene Therapy

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Abstract:

The discovery of DNA as the biomolecule of genetic inheritance and disease opened the prospect of therapies in which mutant and damaged genes could be altered for the improvement of the human condition. Genome-editing is a technology that allows specific changes in the genes of interest. This approach is capable of manipulating the genome of living cells or organisms in various ways: insertions or deletions of chosen genes, introduction of point mutations, knockout or correction of specific genes3 Gene therapy provides a unique approach to treat a variety of both inherited and acquired diseases2 by delivering a therapeutic gene material to correct the loss of function caused by mutation or to express the deficient gene product. Despite years of preclinical studies, it was not until the early 1990s that the first gene therapies were studied in humans In fact, the first clinical trial to gain approval for transfer of a foreign gene into humans was conducted at the National Cancer Institute in Bethesda in 1990.6 In spite of numerous setbacks, efficacious gene-based therapies still hold the great promise to revolutionize the clinical management of human diseases. Numerous preclinical and clinical studies of gene therapy strategies for preventing or treating a wide range of neurodegenerative diseases have been carried out in recent decades,8 however, safety concerns remain one of the biggest barriers to successful clinical application. Potential gene-based therapeutic strategies to treat neurodegenerative disorders should therefore be carefully scrutinized for clinical development, including evaluation of available safety profiles and pharmacological effects, and identification of individuals who can benefit.8 Safe and efficacious gene delivery requires a suitable vector and viruses are designed by nature for in vivo gene delivery. The role of viral vectors in gene delivery is primary due to their function in the delivery of genetic material into host cells. The suitability of a viral vector for a given application depends on multiple factors, including target cells or tissues, tropism, use for ex vivo versus in vivo gene transfer, packaging capacity, potential for genome integration

Biography:

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