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Viral Vectors Paired with Genome-Editing Tools: Evolving Technologies for the Treatment of Neurodegenerative Diseases and Disorders

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Deadline for manuscript submissions:
closed (31 May 2022)

Message from the Guest Editors

Diseases and disorders of the central nervous system (CNS) have historically been among the most difficult to combat using conventional pharmacological strategies. In this chapter, we describe the various tools available for genome editing and summarize in vitro and in vivo preclinical studies of CNS genome editing. We discuss the future progress of these technologies, as well as the current limitations and alternative approaches to overcoming some of their bottlenecks. We will aim here to devote significant attention to viral vectors derived from human immunodeficiency virus type 1 (lentiviral vectors; LVs) and adeno-associated virus (AAVs). The high interest in these viral delivery systems vectors is due to: (i) robust delivery and long-lasting expression; (ii) efficient transduction into postmitotic cells, including the brain; (iii) low immunogenicity and toxicity; and (iv) compatibility with advanced manufacturing techniques. Finally, this chapter will summarize the current therapeutic application and novel approaches of advance genome-editing technology for the prevention and treatment of neurodegenerative diseases (NDDs).

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