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Adeno-Associated Virus-Based Gene Therapy for Neurological Disorders

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Message from the Guest Editors

Neurological disorders are among the most difficult pathologies to treat because of the limited access to brain structures, which are protected by physical barriers such as the blood-brain barrier (BBB); the complexity of the CNS; and the multitude of cell types involved in disease progression.

Gene therapy is an alternative to traditional pharmacological approaches that has made important advances in the past few years for treating diseases of the nervous system. Adeno-associated viral vectors (AAVs) have opened the opportunity of widespread targeting of the CNS due to their non-integrative and low immunogenic profile.

While exciting progress has been made, AAV gene therapy is still a young and emerging field that faces multiple challenges and hurdles such as manufacturing, safety concerns at high doses, delivery to deeper brain structures and more.

This Special Issue aims to address the current challenges and potential solutions by showcasing the advances in the research of AAV gene therapy for all kind of neurological disorders. We welcome all papers of original research, critical reviews, clinical trials, etc.







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Message from the Editor-in-Chief

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