

# **Lentiviral Vector Based Gene Therapy in the Fight Against Neurodegenerative Diseases**

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## **Abstract**

The efficient development of therapeutic strategies for disorders of the nervous system still remains a major medical challenge. Gene therapy of these diseases is particularly challenging due to the postmitotic nature of neuronal cells and the restricted accessibility of the nervous system. Viral vectors based on lentiviruses are particularly attractive vehicles, routinely used now in developing gene-based therapies to treat human disease. Due to their unique properties, which allow them to transduce most nervous system cell types, maintaining strong, and long-term transgene expression, these vectors represent a versatile and powerful tool for many research and gene therapy applications. In this talk lentiviral vectors and their applications in gene therapy for neurodegenerative diseases will be reviewed, particularly focusing on recent progress in clinical trials for the inherited diseases of childhood X-Adrenoleucodystrophy and Metachromatic Leucodystrophy and the disease of aging Parkinson's Disease.