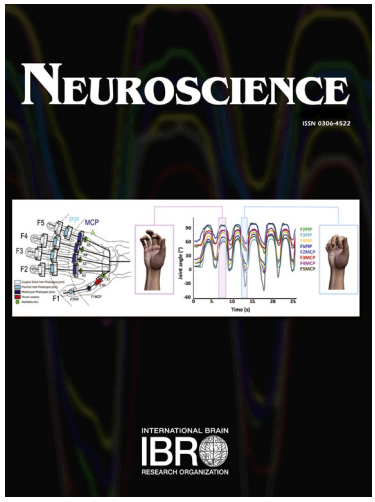

Review

Experimental and clinical strategies for treating spinocerebellar ataxia type 3

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Experimental and clinical strategies for treating spinocerebellar ataxia type 3

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Abstract

Spinocerebellar ataxia type 3 (SCA3), or Machado-Joseph disease (MJD), is an autosomal dominant neurodegenerative disorder caused by the expansion of a polyglutamine (polyQ) tract in the ataxin-3 protein. To date, there is no effective therapy available to prevent progression of this disease. However, clinical strategies for alleviating various symptoms are imperative to promote a better quality of life for SCA3/MJD patients. Furthermore, experimental therapeutic strategies, including gene silencing or mutant protein clearance, mutant polyQ protein modification, stabilizing the native protein conformation, rescue of cellular dysfunction and neuromodulation to slow the progression of SCA3/MJD, have been developed. In this study, based on the current knowledge, I detail the clinical and experimental therapeutic strategies for treating SCA3/MJD, paying particular attention to drug discovery.

Highlights

- Novel insights into potential symptomatic therapeutics and disease-modifying compounds for SCA3 at the experimental level.
- Review of the most promising new experimental candidate approaches to reduce mutant ataxin-3 toxicity.
- Comprehensive update on ongoing clinical trials of pharmaceutical agents for treating SCA3.