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RNA interference and amyotrophic lateral sclerosis

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Abstract

Amyotrophic lateral sclerosis (ALS) is a debilitating neuro-degenerative disorder characterized by progressive loss of motor neurons. The etiology and molecular pathogenesis of cell death in most sub-types of the disease are largely unknown. The best documented cause of moto-neuron degeneration is the mutation in the superoxide dismutase-1 (SOD 1) gene, which occurs in 10% of the familial forms of ALS. Discovery of RNA interference (RNAi), which plays an important role in the regulation of gene expression, has proven to be a powerful tool to study the pathogenesis and to develop innovative treatment options for hereditary diseases, including familial variants of ALS. This review summarizes current research advances in RNAi in relation to ALS. © 2011 Bentham Science Publishers.

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Keywords

Amyotrophic lateral sclerosis (ALS), Gene expression, Messenger RNA (mRNA), Micro RNA (miRNA), RNA interference (RNAi), Short (or small) hair-pin RNAs (shRNA), Short (or small) interfering RNA (siRNA), Superoxide dismutase (SOD)