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Hunting Down Treatment Therapies for Huntington's Disease

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Abstract: Hunting Down Treatment Therapies for Huntington's Disease

Sage Berry

Huntington's disease (HD) is a uniformly fatal, neurodegenerative disease originating from a mutation in one allele of the HTT gene. While global HTT reduction has shown some improvement in treating the progression of HD, the wild type HTT allele has necessary functions for survival and cannot be ablated. Antisense oligonucleotides (ASOs) are a way to target pre-mRNA and prevent the mRNA's translation into a protein. In this study, we examined the efficacy of allele selective ASOs in comparison to pan ASOs in ameliorating HD pathology and symptomology.