**Blocks Transcription of Expanded Microsatellite Repeats to Treat Muscle Degenerating Diseases**

This therapeutic precisely targets microsatellite repeats in the DNA sequence and blocks the transcription of a genetic mutation, called hexanucleotide repeat expansion, providing therapy for a variety of neurological and muscular degenerative diseases. The presence of hexanucleotide repeat expansions in the genetic code causes the development of diseases such as frontotemporal dementia (FTD) and amyotrophic lateral sclerosis (ALS). ALS affects approximately 1 in 25,000 individuals in the U.S., while FTD is the third most common cause of dementia. Many microsatellite repeat diseases like these have no available therapeutics, which is largely due to challenges in targeting individual DNA segments in order to understand the roles they play in these diseases.

Researchers at the University of Florida have developed a therapeutic that blocks the transcription of microsatellite repeats to avert the development of expanded microsatellite repeat diseases. This treatment does not require an inhibitory domain and greatly reduces repeat gene expression.

**Application**

Therapeutic that inhibits the transcription of hexanucleotide repeat expansions by using repeat-targeting guide ribonucleic acids (RNAs)

**Advantages**

- Reduces the expression of microsatellite repeats, preventing development of expansion diseases and improving expansion disease therapy to increase survival
- Treats diseases such as myotonic dystrophy type 1, myotonic dystrophy type 2, C9ALS/FTD, spinocerebellar ataxia, and Fuch’s endothelial corneal dystrophy, advancing the efficacy of CRISPR

**Technology**

The deactivated Cas9 protein inhibits the transcription of expanded microsatellite repeats by using segments of ribonucleic acid (guide RNAs). The guide RNA forms a complex with the protein and directs it to the transcription initiation site. From there, the RNA Polymerase II can no longer bind to the site and produce gene sequences that become expanded microsatellite repeats. Thus, this treatment is an effective solution to treat a number of microsatellite repeat diseases.

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