

Methods For Modulating Expression Of C9orf72 Antisense Transcript

Tech ID: 24544 / UC Case 2015-007-0

BACKGROUND

Amyotrophic lateral sclerosis (ALS) is a fatal neurodegenerative disease characterized clinically by progressive paralysis leading to death from respiratory failure, typically within two to three years of symptom onset. ALS is the third most common neurodegenerative disease in the Western world, and there are currently no effective therapies. Approximately 10% of cases are familial in nature, whereas the bulk of the cases occur randomly throughout the population. Recently a mutation in the C9ORF72 gene has been linked to ALS, frontotemporal degeneration (FTD) and ALS-FTD. It is postulated that the ALS-FTD causing mutation is a large hexanucleotide (GGGGCC) repeat expansion in the first intron of the C9ORF72 gene. There are currently no effective therapies to treat such neurodegenerative diseases. Therefore, it is an object to provide methods for the treatment of such neurodegenerative diseases.

TECHNOLOGY DESCRIPTION

UC San Diego researchers have developed methods to inhibit expression of C9ORF72 antisense transcript.

APPLICATIONS

Such methods are useful to treat, prevent, or ameliorate neurodegenerative diseases, including amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), corticalbasal degeneration syndrome (CBD), atypical Parkinsonian syndrome, and olivopontocerellar degeneration (OPCD).

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	9,963,699	05/08/2018	2015-007
United States Of America	Published Application	20180142240	05/24/2018	2015-007

Additional Patents Pending

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OTHER INFORMATION

KEYWORDS

Amyotrophic lateral sclerosis, ALS,
frontotemporal degeneration, FTD,
antisense, neurodegenerative disease

CATEGORIZED AS

- Medical
 - Disease: Central Nervous System
 - Therapeutics

RELATED CASES

2015-007-0