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Anti-Sense Therapeutic for VCP Diseases and Familial ALS

Tech ID: 24745 / UC Case 2015-144-0

FULL DESCRIPTION

Inclusion body myopathy with Paget disease of the bone and/or frontotemporal dementia (IBMPFD) is an autosomal dominant disorder caused by mutations in the valosin containing protein (VCP) gene. Individuals affected with IBMPFD exhibit scapular winging and die from progressive muscle weakness, and cardiac and respiratory failure typically in their 40's to 60's.

VCP protein is essential for the maturation of ubiquitin-containing autophagosomes, and mutant VCP toxicity is partially mediated through its effect on TDP-43 protein, a major constituent of ubiquitin inclusions that neuropathologically characterize ALS. Histologically, IBMPFD patients show the presence of rimmed vacuoles and inclusion bodies containing the ubiquinated protein TDP-43 in their muscles. Mutations in the VCP gene have also been associated with amyotrophic lateral sclerosis (ALS) in 10-15% of individuals with IBMPFD and 2-3% of isolated familial amotrophic lateral sclerosis (fALS).

Researchers at the University of California Irvine and Murdoch University have developed anti-sense oligonucleotides (AONs) that skip or silence VCP exon 5 to treat VCP disease like IBMPFD and fALS.

SUGGESTED USES

There are currently no treatments for VCP-associated neurodegenerative diseases and patients are dying early from respiratory failure related to muscle weakness. These AONs may become the first therapies to treat VCP diseases like IBMPFD and fALS.

PATENT STATUS

| Country | Туре | Number | Dated | Case |
|--------------------------|---------------|------------|------------|----------|
| United States Of America | Issued Patent | 10,093,932 | 10/09/2018 | 2015-144 |

TESTING

These AONs have been tested in vitro. The researchers have shown through a VCP hR155H mutant cre-lox mouse model that knocking-out the VCP mutation yields beneficial outcomes in VCP diseases like IBMPFD.

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OTHER INFORMATION

KEYWORDS

VCP Disease, IBMPFD, fALS, ALS

CATEGORIZED AS

» Medical

» Disease:
Musculoskeletal
Disorders
» Therapeutics

RELATED CASES

2015-144-0



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