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## Anti-Sense Therapeutic for VCP Diseases and Familial ALS

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### OTHER INFORMATION

#### KEYWORDS

VCP Disease, IBMPFD, fALS, ALS

#### CATEGORIZED AS

- » **Medical**
  - » Disease: Musculoskeletal Disorders
  - » Therapeutics

#### RELATED CASES

2015-144-0

## BRIEF DESCRIPTION

A groundbreaking approach to treating neurodegenerative diseases associated with VCP mutations through exon skipping technology.

## FULL DESCRIPTION

This technology employs anti-sense oligonucleotides (AONs) to specifically target and silence or skip exon 5 of the VCP gene, offering a potential treatment for VCP-associated diseases such as inclusion body myopathy with Paget disease of the bone and/or frontotemporal dementia (IBMPFD) and familial amyotrophic lateral sclerosis (fALS). Developed by researchers at the University of California Irvine and Murdoch University, these AONs are designed to restore the reading frame within the VCP gene, thereby potentially mitigating the disease's progression.

## SUGGESTED USES

- » Therapeutics for rare genetic disorders, specifically targeting VCP-associated neurodegenerative diseases.
- » Gene therapy applications for a range of conditions beyond IBMPFD and fALS, wherever exon skipping can be beneficial.
- » Research tools in molecular biology and genetics for studying the effects of exon skipping and the role of specific gene mutations in disease.

## ADVANTAGES

- » First potential therapy for VCP-associated neurodegenerative diseases.
- » Targeted exon skipping approach to correct the genetic mutation at the mRNA level.
- » Can lead to the production of a largely functional protein despite the genetic mutation.
- » Offers hope for treating diseases currently considered untreatable, extending patient lifespan and quality of life.

## PATENT STATUS

Country	Type	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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