

Therapeutic For Dry Age-Related Macular Degeneration and Stargardt Disease

Tech ID: 32906 / UC Case 2022-156-0

TECHNOLOGY DESCRIPTION

The invention is a therapeutic intended to prevent the onset and progression of age-related macular degeneration (MD) as well as Stargardt disease-related vision loss.

UCSF/University of Wisconsin researchers have identified that abnormal activation of acid sphingomyelinase (ASM) in the retinal pigment epithelium (RPE) serves as a pathological trigger in dry age-related MD and Stargardt's disease. Activation of ASM results in increased ceramide, which makes the RPE (the tissue that is responsible for nourishment and support of light-sensing photoreceptors) susceptible to damage. The RPE is the primary site of damage for both Stargardt inherited and age-related MD.

ADVANTAGES

- ▶ Potential preventative treatment for age-related MD and Stargardt disease
- ▶ ~ 35 million people worldwide suffer from age-related MD
- ▶ Stargardt disease affects ~1 in 8,000 children
- ▶ Treatment with identified therapeutic can prevent ceramide accumulation and preserve the RPE
- ▶ In vivo efficacy in mouse models of MD is observed with a 2,000-fold lower dose than that used for functional ASM inhibitors and 15-fold lower dose of therapeutic than what is currently used to treat osteoporosis.
- ▶ Currently, there are no approved therapies for dry age-related MD (which is characterized by focal loss of the RPE) or Stargardt disease

APPLICATION

- ▶ Therapy to prevent
- ▶ age-related MD onset or progression
- ▶ vision loss due to Stargardt disease
- ▶ Potential therapy for any condition associated with increased ceramide.
- ▶ Excess ceramide has been implicated in many diseases, including Alzheimer's and Parkinson's disease.

STAGE OF DEVELOPMENT

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OTHER INFORMATION

CATEGORIZED AS

- ▶ **Medical**
- ▶ Disease:
 - Ophthalmology and Optometry
- ▶ Therapeutics

RELATED CASES

2022-156-0

Target validation and proof of concept translational studies.

Treatment with an ultra-low dose of thereapeutic has been shown to prevent ceramide accumulation while preserving RPE health and function in cell-based models of AMD and in vivo in the *Abca4*^{-/-} mouse model of Stargardt inherited MD

LOOKING FOR PARTNERS

To further develop the technology

DATA AVAILABILITY

Under CDA

PATENT STATUS

Patent Pending

OTHER INFORMATION

Published application: [WO 2023/220136](#)

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