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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

CONTACT

Michael X. Papac Michael.Papac@ucsf.edu tel: .



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

ADDRESS	CONTACT	
Innovation Ventures	innovation@ucsf.edu	
600 16th St, Genentech Hall, S-272,	https://innovation.ucsf.edu	© 2022 - 2024, The Regents of the University
San Francisco,CA 94158	Fax:	of California
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