

# 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](mailto: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*<sup>-/-</sup> 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

**UCSF**

**Innovation Ventures**

600 16th St, Genentech Hall, S-272,  
San Francisco, CA 94158

### CONTACT

Tel:

[innovation@ucsf.edu](mailto:innovation@ucsf.edu)

<https://innovation.ucsf.edu>

Fax:

### CONNECT

 Follow  Connect

© 2022 - 2024, The Regents of the University  
of California

[Terms of use](#) [Privacy Notice](#)