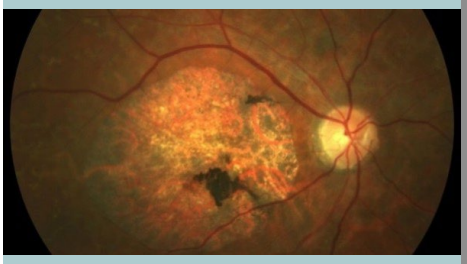
PENN CENTER FOR INNOVATION
Empowering Ideas

Gene Therapy for Macular Degeneration



Brief Description

Gene therapy targeting disposal of toxic intracellular lipid debris and lipofuscin in wet age related macular degeneration.

Inventor

Kathleen Boesze-Battaglia

STATE OF DEVELOPMENT

- *In vitro* and *In vivo* proof of concept

INTELLECTUAL PROPERTY

Provisional pending

DESIRED PARTNERSHIPS

- License
- Co-development

Problem

Macular degeneration is a genetic eye disorder that affects the retina resulting in progressive vision loss. Macular degeneration has two main forms, Stargardt's macular degeneration (STGD) which affects juveniles, and age related macular degeneration (AMD) which affects older individuals. While a fairly small proportion of the population has STGD, with an estimated prevalence of 1 in 8,000 to 10,000, AMD affects a significantly larger segment at 2 million people in 2010 in the U.S. alone. There are currently few treatment options for macular degeneration disorders and therefore a serious need for innovative solutions.

Solution

Work in the lab of Dr. Kathleen Boesze-Battaglia has resulted in a novel therapeutic approach which targets the underlying cause of macular degeneration vision loss, the accumulation of toxic lipid debris including lipofuscin. Lipofuscin is a fatty yellow pigment which builds up in cells underlying the macula, eventually causing cell death and vision loss. Utilizing a unique gene therapy method, Dr. Boesze-Battaglia has achieved a significant reduction in the amount of lipofuscin accumulation in a well-established *in vivo* model system. Taken together, this technology could be used to potentially delay or prevent the onset of vision loss in patients suffering from macular degeneration disorders.

Advantages

- Potential treatment for wet AMD – accounts for 80% of cases.
- Target is gene defect independent
- Limits lipid debris accumulation
- May delay serious vison loss if used early

LEARN MORE

Melissa Kelly
kellymel@upenn.edu
215-898-9877
Docket #15-7361