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## Modified Form of Vitamin A Could Help Treat AMD

*Discoveries supported by Macular Degeneration Research could prevent blindness*



Scientists funded by Macular Degeneration Research have shown that a modified form of vitamin A may help prevent vision loss due to age-related macular degeneration (AMD) and Stargardt's disease, the most common cause of juvenile macular degeneration.

Researchers at Columbia University, led by AHAF-grantee Ilyas Washington, Ph.D., were able to reduce the formation of clumpy deposits of vitamin A in the eye, known as "vitamin A dimers," which are associated with these degenerative eye diseases.

During the sequence of events that enables vision, vitamin A undergoes chemical changes and sometimes reacts with other vitamin A molecules to form dimers. In dry AMD, these dimers are believed to accumulate over decades. In Stargardt's disease, however, it can happen much faster,

leading to vision loss as early as age eight.

Rather than changing the way the eye processes vitamin A, Dr. Washington's team focused on changing the structure of vitamin A itself. In studies of mice that mimic the effects of macular degeneration, they synthesized a modified vitamin A drug. When they fed this new drug to mice with Stargardt's disease, they were able to reduce the amount of vitamin A dimers without any observed side effects and improve vision and overall eye health.

Most importantly, they also observed that the modified vitamin A behaved exactly as normal vitamin A does in all other aspects, making it an attractive potential therapy for preventing blindness in humans. This work is detailed in a series of articles recently published in the *Journal of Biological Chemistry*.

A variety of resources are available for people who suffer from macular degeneration. For a list of agencies that offer counseling, training, and other special services, please call Macular Degeneration Research at **800-437-2423** or visit our website at [www.ahaf.org/macular/resources](http://www.ahaf.org/macular/resources).

## Chairman's Corner

A bright future is in sight



When eyesight is lost to AMD, it currently can't be restored. But thanks to the groundbreaking work of dedicated scientists, that could one day change.

In this edition of **Macular Degeneration Research News**, you'll discover how vision activity was restored in mice who have a retinal degenerative disease using stem cells derived from skin. You'll also learn how an out-of-control immune system response can cause this disease in the first place. And you'll find out how another set of scientists has discovered an innovative form of vitamin A that could treat it.

We haven't won the battle against AMD yet. But the progress that's being made is simply breathtaking. And when you look at the promising new research projects that have just been launched with the help of Macular Degeneration Research grants—including one aimed at slowing the progression of AMD through a minimally invasive cellular treatment—there is every reason for hope.

With the determined efforts of Macular Degeneration Research-funded scientists and the committed support of friends like you, I know that one day AMD will be a thing of the past.

Brian K. Regan, Ph.D.

## Stem Cells Regenerate Sections of Damaged Retinas and Increase Visual Function

*Scientists use stem cells derived from skin in animals with retinal degenerative disease*

The dream of restoring lost vision is now one step closer to reality.

In a major breakthrough, scientists from Schepens Eye Research Institute in Massachusetts have for the first time been able to regenerate large areas of damaged retinas and improve visual function using stem cells derived from skin. The results of their study, published in the online scientific journal *PLoS ONE*, could pave the way for future treatments and cures for retinal diseases such as AMD that affect millions of people worldwide.

In AMD and other retinal degenerative diseases, photoreceptors and retinal pigment epithelial cells in the retina can die, damaging the eye's ability to capture light and transmit this information to the brain. Researchers used skin cells from the tails of red mice to develop immature photoreceptor cells called precursor cells and transplant them into the eyes of a mouse model of retinal degenerative disease.

After four to six weeks, tests showed that the electrical activity in the newly reconstructed retinal tissue was about half of what would be expected in a normal retina.

These results are very promising. "Stem cell regeneration of this precious tissue is our best hope for treating and someday curing these disorders," said Dr. Michael J. Young, the study's principle investigator and head of the Institute's regenerative medicine center.

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## SPOTLIGHT: AHAF Announces Latest Grants for Innovative AMD Research

**Could a minimally invasive cellular therapy prevent progress of AMD?** That's the question that Michael E. Boulton, Ph.D., of the University of Florida, hopes to answer, with the help of a \$100,000 grant from Macular Degeneration Research, a program of the American Health Assistance Foundation (AHAF). Dr. Boulton and his colleagues are investigating whether injecting specialized retinal-like cells in the blood of animals engineered to show features of AMD will allow the cells to migrate to the retina and re-establish normal vision. If this treatment is effective, it could avert the need for invasive eye surgery and offer the potential for prevention, since it could be given much earlier in the disease's progress.

This is just one of 12 new grants awarded by AHAF to understand and stop AMD. Learn more about the cutting-edge research being funded by AHAF at [www.ahaf.org/maculargrants](http://www.ahaf.org/maculargrants).

# Research Roundup

## *Gene therapy creates light-detecting retina cells with potential to restore vision*

Researchers have developed a potential treatment for blindness using a gene that delivers a light-sensitive protein to retinal cells, restoring visual function in mice.

The study, reported in the journal *Molecular Therapy*, targeted different retinal cells called bipolar cells, which are normally not light detectors. After the light-sensitive protein was delivered to them using a virus, they became light-sensitive and took over the light-capturing function of the photoreceptors damaged by a retinal degenerative disease. Multiple studies have been performed to ensure this technology does not harm the eye.

“It’s a very targeted approach that maintains the natural processing of the retina,” said lead researcher Alan Horsager, Ph.D., neuroscientist at the University of Southern California. “There’s a lot more to understand, but initial indications suggest we have developed something that can have enormous benefit to people.”

## *Scientists discover how rogue cells “eat our eyes”*

Researchers have shown how light-damaged retinas can lead to an out-of-control immune system response that causes macular degeneration.

In rat studies, scientists at the Vision Centre in Australia found that when the retina is damaged by bright light, it increases production of a molecule called Cc12. “When the cells in our eyes are damaged, the immune response kicks in and sends macrophages—white blood cells that ‘eat’ invading or dead cells—to clean up the mess,” the researchers said. “If the immune system is not properly regulated, the invading cells linger and call in even more macrophages.” This creates a vicious cycle that destroys retinal cells.

Identifying Cc12 as a key culprit in macular degeneration will help scientists target potential treatments. The team is currently exploring whether shining a soft infrared light on light-damaged cells can limit Cc12 growth. If it works, it could be a cheap, non-intrusive way to slow the progression of macular degeneration.

## Questions & Answers

### *Is AMD hereditary?*

AMD typically affects individuals older than the age of 50. Scientific evidence shows that genes may play a role in the development of nearly three out of four cases of this devastating eye disease. While there is definitely a strong genetic component to this disease, it is highly likely that its development is due to a combination of multiple factors, including gene mutations or variations, and environmental factors, such as sunlight exposure, diet, and smoking.

### *Is there a surgery for dry macular degeneration that can prevent the progression to the wet form of the disease?*

No surgery exists to prevent the conversion of dry AMD to the wet subtype. However, smoking cessation and nutritional supplementation may slow the conversion. Ask your retina specialist if you are a candidate for AMD nutritional supplements. It is important to note that these supplements are only beneficial for a subgroup of patients with dry AMD.

### *Can a botched cataract operation cause macular degeneration?*

There is no evidence that strongly supports a relationship between cataract surgery and increased rates of AMD. A complicated cataract surgery can cause visual decline for a variety of reasons; however, an increased rate of AMD is not thought to be one of them.

# Become a Year-Round Force in AMD Research



## *Monthly giving makes your dollars go further to fight AMD*

Many of our donors find that the easiest and most efficient way to give to Macular Degeneration Research is to make monthly contributions of \$10, \$20, \$100, or more.

Automatic payments are particularly effective because they save on the cost of stamps and envelopes. This makes us more efficient and allows us to allocate more of every dollar to the fight against AMD.

Becoming a monthly donor is easy to do, and you can change or cancel your monthly gift at any time. For more information on this unique way of giving, please contact Cristel Siaobungco at 800-437-2423.

**Thank you for advancing the work of Macular Degeneration Research!**



Visit our website at [www.ahaf.org/connect](http://www.ahaf.org/connect) to learn more about what's new in the world of research, as well as important information about risk factors for macular degeneration. You can also follow us on Twitter (@macular) or become a fan on Facebook!

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