Geographic atrophy is an advanced form of dry age-related macular degeneration (AMD). It damages the center of the retina at the back of the eye and can lead to blind spots. Currently untreatable, it’s responsible for about one in five cases of legal blindness in the U.S.

But that may be changing, thanks to Macular Degeneration Research-funded scientist Benjamin Kim, MD, from the Scheie Eye Institute at the University of Pennsylvania.

A major school of thought among researchers is that oxidative stress contributes to retinal damage, and one of those stressors may be iron overload. However, an oral supplement called alpha-lipoic acid is able to remove excess iron from the body. Since it already has a good safety profile, Dr. Kim is conducting a pilot Phase 2 clinical trial to test it as a treatment for people with geographic atrophy.

The grant Dr. Kim received from Macular Degeneration Research was pivotal to getting his study up and running. It “helped snowball this… from an idea that was talked about to now a fully enrolled clinical trial,” he said. His study is expected to finish in the spring of 2019.

As a clinician and researcher, Dr. Kim sees both the important advances that have been made in treating AMD (continued on page 2)
PRESIDENT’S CORNER

An oral treatment for advanced dry AMD... a therapy that could combat both the dry and wet forms of the disease... “good cholesterol” that might help protect the eye against AMD...

Not that long ago, treatments like these were scarcely imaginable. But today, scientists funded by Macular Degeneration Research are testing these and many other innovative approaches to stop this terrible disease. You can learn more about these promising studies in this issue of Macular Degeneration Research News.

The progress we’re making is only possible because of the generosity of you and other caring supporters. You enable us to blaze new trails in the fight against AMD, while also providing valuable information to the public to help protect sight.

Every day, we get another step closer to finding a cure for AMD. Thank you for supporting this vital work!

Stacy Pagos Haller
President

CLINICAL TRIAL UNDER WAY FOR TREATMENT OF DRY AMD

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and how much more needs to be accomplished. “When you’re seeing patients, you’re helping one patient at a time, which is incredibly personal and important for you and that patient,” he said. “But if you make a major scientific discovery... you can help many people at one time.”

He added, it’s “only through research that we’re going to push the envelope and improve patient care. We are trying to find a treatment that can slow down this disease and give patients more years of better vision.”

GENE THERAPY FOR EYE DISEASES

A recent gene therapy breakthrough could open the door for new treatments for retinal diseases, including AMD.

The U.S. Food and Drug Administration has approved the first gene to be injected into people to treat a disease. In a clinical trial, researchers at the Scheie Eye Institute at the University of Pennsylvania were able to use a gene called RPE65 to improve the vision of children with an inherited form of blindness.

Scientists packaged RPE65 into a safe, genetically modified adeno-associated virus (AAV)—which does not cause disease—and injected it under the retina. It significantly restored vision in blind children, enabling them to complete tasks such as walking through a maze without bumping into soft objects and catching a ball.

This important advance also paves the way for gene therapies to treat other retinal diseases. Clinical trials are

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Please share this newsletter with others who may be interested!
already under way to test this approach in treating AMD. Some genes involved in vision loss are too large to be carried into the retina by the AAV virus, so other approaches must be developed. For example, a clinical trial is currently evaluating another type of virus, called a lentivirus, which can carry larger genes. For some complex diseases, such as AMD, it is not clear which genes will protect vision.

Wet AMD

For wet AMD, a gene therapy called “Retinostat” has proven safe in a Phase 1 clinical trial. After being injected under the retina, it continues to make proteins for at least one year that inhibit abnormal blood vessel growth in the eye.

Another company is testing injections of a helpful gene into the retina, called sFLT, which creates a vascular endothelial growth factor (VEGF) blocker. In a Phase 1 trial, the therapy was safe and appeared to nearly eliminate the need for additional treatments over a one-year period. This is an advantage over current treatments such as Lucentis® and Avastin®, which also target VEGF but are delivered through eye injections, sometimes as often as once every four weeks.

A second company is testing the same gene but delivering it into the eye instead of the retina, which may be safer and more convenient.

Dry AMD

Gene therapy for dry AMD is not as far along as that for wet AMD. There is currently one clinical trial going on for dry AMD.

While gene therapy holds promise, one of the potential downsides is that in most cases, it is not possible to turn off the therapy once it’s delivered into the eye. The cells that receive the therapeutic genes will continue to express them for years. Therefore, clinical trials are very important to determine the long-term safety of this approach.
5 CLINICAL TRIAL MYTH BUSTERS

Clinical trials play a critical role in scientific research seeking a cure for AMD. There are often common misperceptions about these clinical trials. Learn more about the myths versus the reality:

Myth #1: I can’t participate in clinical trials because of my age or my health.
Reality: Each trial has its own criteria for who can take part, and even if you don’t qualify for one trial you’re interested in, a different trial may be a match for you.

Myth #2: Taking part in a clinical trial is too time-consuming.
Reality: Some clinical trials do require regular visits or overnight stays, while others may have visits only every few months. Some trials are even conducted virtually. Ask about the time commitment to see if it is right for you.

Myth #3: Clinical trials are too risky.
Reality: It is important to weigh the benefits against the risks. Possible risks include drug side effects, and that the drug doesn’t work. Benefits include the possibility that the treatment will help research and improve the standard of care.

Myth #4: Once I join a clinical trial, I can’t quit if I want to.
Reality: All trial participants are free to leave a trial at any time, for any reason.

Myth #5: Clinical trials are only a last resort.
Reality: Clinical trials can offer an option for those who have exhausted other treatments, or you can choose to participate if you’re unhappy with your current options. Many people also choose to participate to help medical research on their condition.

To learn more about participating in an AMD clinical trial near you, please visit our clinical trial finder, powered by Antidote, at brightfocus.org/MacularTrials.

This series of articles promoting awareness of clinical trials is supported in part by an educational sponsorship from Biogen. BrightFocus is solely responsible for the content of this article.
“Good cholesterol” might help protect against AMD

High-density lipoprotein (HDL), the so-called “good cholesterol,” has been shown to have antioxidant and anti-inflammatory properties that reduce people’s risk for cardiovascular disease. Since the latter shares many common risk factors with AMD, could good cholesterol help protect against AMD as well?

Chi Luu, PhD, of the Center for Eye Research Australia, believes the answer could be “yes.” In this study, he will collect blood samples from subjects with and without AMD and assess them for HDL’s antioxidant and anti-inflammatory properties. He will also investigate if AMD can be treated with a reconstituted form of good cholesterol. If so, this could pave the way for the development of new therapies to manage the earliest changes in AMD to prevent vision loss.

A novel method to treat both wet and dry AMD

Currently there is no treatment for dry AMD, where waste deposits called drusen build up under the retina. And although wet AMD can be treated with regular eye injections of anti-vascular endothelial growth factor to inhibit abnormal blood vessel growth, clinical trials have shown that long-term visual outcomes are less than optimal.

In this study, Yingbin Fu, PhD, of Baylor College of Medicine, is developing a highly innovative and effective strategy to target the causes of both wet and dry AMD simultaneously. He will use apolipoprotein A-1 binding protein to inhibit the growth of fragile, leaky blood vessels in wet AMD and also reduce drusen formation in dry AMD—something which no other single treatment can currently do. This research could potentially guide the development of a new generation of therapy for AMD.

These are just 2 of the 18 new grants Macular Degeneration Research recently awarded to help fight AMD. You can read about all the studies we funded in 2018 at brightfocus.org/2018grantsawards.
When someone close to us passes away, celebrating the person’s life and reflecting on favorite memories can help us cope.

One way to help the person’s legacy live on is to establish a memorial gift in his or her honor. Before you make a memorial gift, consider a few key factors:

**Purpose:** Memorial gifts honor the lives of loved ones and friends. You can make a gift to our organization to help fund research that could one day bring health to others and further our efforts to discover a cure.

**Timing:** You may give your gift to us today to help support our most immediate needs, or you can include a gift in your will or living trust, stating that a specific asset, certain dollar amount, or percentage of your estate will pass to us after your lifetime in honor of your loved one.

**Type:** Cash is a popular and easy way to make a gift, but you can also use securities or other assets. Like all charitable donations, a memorial gift can offer tax benefits.

**Amount:** No matter the size of your gift, you can be certain your support will make a lasting impact.

If the mission of Macular Degeneration Research was important to a loved one and you want to know more, please contact Lauren Fields, Manager, Planned Giving, at 855-345-6637 or Lfields@brightfocus.org. We would be happy to discuss ways you can create a gift that properly honors his or her memory.

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