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In the Works: A Replacement CFH Protein to Treat and Maybe Cure AMD

Writing provided by Richman Associates, LLC
(www.richmanassociates.com)

Discoveries in genetics are happening faster than ever. It is already old news about the gene found in 2005 that causes about half of all cases of age-related macular degeneration (AMD). Based on the discovery, scientists are now making advances in “translational research” to find new AMD treatments.

AMD is the most common cause of irreversible visual impairment in the developed world. Forms of the disease are dry AMD, geographic atrophy, and choroidal neovascularization (also known as wet AMD). All three types can impair vision—especially central vision—and seriously affect quality of life. Central vision is used for straight-ahead tasks like reading, watching television, and seeing a face across a table.

An estimated 15 million people in the U.S. have AMD. Worldwide the number is easily twice that.

The 2005 AMD gene is called complement factor H (CFH). It instructs cells, mainly liver cells, to produce a protein of the same name. The protein is involved in the body’s immune system. More and more, researchers are finding diseases associated with variants (abnormalities, mutations, polymorphisms) in the CFH gene. The normal protein protects against AMD, but exactly how it does so is not yet fully known.

Dr. Hageman

The goal of translational research is to convert basic scientific discoveries into products for treating and preventing disease. In AMD, work is ongoing to find ways to replace the defective CFH protein with normal CFH protein.

Macular Degeneration Foundation

P.O. Box 531313
Henderson, NV 89053

Website:
www.eyesight.org

Telephone:
1-888-633-3937

Groundbreaking progress to commercially produce the normal CFH protein was described at a September 16, 2008, briefing on Capitol Hill in Washington, DC, by vision researcher Gregory Hageman, Ph.D., of University of Iowa. Dr. Hageman was a member of one of the first teams to discover the role of CFH in AMD.1

He described the effort by the University of Iowa, National Institutes of Health, and Optherion, Inc, an early-stage biotechnology company, to develop an AMD treatment that could be similar to insulin therapy used for treating diabetes. Insulin is a protein made by cells of the pancreas. It controls the amount of sugar in the blood. People whose pancreatic cells make too little insulin can take the protein by injection. Similarly, people whose CFH gene is malfunctioning can, theoretically, take infusions of normal CFH.

Do we have proof that this would work, or what scientists call “proof of principle”? The answer is yes. Dr. Hageman reported on serendipitous findings in patients with liver disease who had received a healthy new liver transplant. In the patients with AMD, as their new livers started producing the normal CFH protein, their AMD improved. In some cases, it was even cured! This does not mean, however, that lost vision was entirely restored. Vision cells that die because of AMD cannot be revived. This is why early treatment is so important.

Dr. Hageman believes that Phase I clinical trials to test the safety of the synthesized CFH protein could begin as early as summer of 2009. He describes progress as “spectacular.”

The genetics of AMD is a hot area with several new discoveries, and more expected. The Macular Degeneration Foundation will keep readers updated as new developments occur.

References

1. Hageman GS, Anderson DH, Johnson LV, et al. A common haplotype in the complement regulatory gene factor H (HF1/CFH) predisposes individuals to age-related macular degeneration. Proc Natl Acad Sci USA 2005;102:7227-7232.]

2. Khan JC, Thurlby DA, Shahid H, et al. Smoking and age related macular degeneration: the number of pack years of cigarette smoking is a major determinant of risk for both geographic atrophy and choroidal neovascularisation. Br J Ophthalmol. 2006;90(1):75-80.

Clinical Trial for Macular Degeneration Seeks a New Way of Seeing

Plasticity, the brain’s ability to reorganize itself to compensate for vision loss, may help those with MD see better. A new study between Emory Eye Center and the Georgia Institute of Technology (Psychology) is designed to train patients who have retinal damage to focus on using those good retinal cells so they may experience increased visual acuity.

Susan Primo, OD, MPH, of Emory Eye Center, reports that the Phase 2 portion of the clinical trial “Age-Related Macular Degeneration and Cortical Reorganization” will help bridge the knowledge gap between cortical plasticity and visual function. This will create rehabilitation therapies and technologies that will expedite efficient use of fixation strategies ultimately fostering cortical reorganization. Contacts: Joy Bell, Tel: (404) 778-3711 source: Emory University

Pfizer buys into biotech working on eye disease

http://www.theday.com

Jun 24, 2008 (The Day - McClatchy-Tribune Information Services)

Pfizer Inc. announced that it is funding a new San Diego biotech company, EyeCyte, Inc that will test novel adult stem-cell treatments for use in fighting eye disease.

“Martin Friedlander has developed a method, using the bone and blood-marrow stem cells of animals, to preserve eyesight after detection of early-stage blood vessel damage. ‘These cells know where to go, and they target the site of the injury,’ said Friedlander in a statement.”

Paul A. Sieving, director of the National eye Institue said, “Something interesting and potentially very important is happening here — we may be seeing discoveries of investigation-initiated research, funded by NIH, pushed to the clinic more rapidly by the investigators through creative collaborations among nonprofits, biotech and big pharmaceuticals”.

The stem-cell treatments should be ready for human trials within three years, said Mohammad A. El-Kalay, president and chief executive of EyeCyte, according to a report on Forbes.com. The 3 million dollar financing from Pfizer will fund the company into 2010 and will be primarily used to drive product development of the company’s initial clinical target, diabetic retinopathy.

Can the Aging Marker in the Human Retina be Reversed?

Dr. Stuart Richer OD, PhD, Chief, Optometry Section at the Veterans Medical Center in North Chicago, speaking at the 111th annual American Academy of Optometry meeting in Seattle, says this may be the first time an intervention has been shown to reverse aging changes in the retina.

A nutraceutical matrix may effectively remove cellular debris from the human eye that accumulates with advancing age. The accumulation of cellular debris in the retina is believed to be the first sign of AMD.

A study done with an 80-year old patient, showed that after 5 months on the dietary supplement regimen, Longevinex, five measurable parameters of vision improved to varying but significant degrees including night (contrast) vision, visual acuity, color, and side vision. Upon testing, it was also found the patient’s mental capacity had improved. The patient said, “My night vision and thinking have gotten much better.”

Longevinex is designed to remove excess minerals by a process called chelation. These minerals, calcium, iron and copper can build up in retinal tissues over time. Lipofuscin, or cellular garbage that pollutes cells as they age, can generate free radicals, gene mutations, and even cause premature cellular death.

This case presentation will be published in Optometry-Journal of the American Optometric Association. Dr. Richer has no financial interest in the product. Resveratrol Partners LLC, makers of patent-pending Longevinex(R), provided the nutraceuticals for this patient. To learn more about Longevinex(R) go to www.longevinex.com.

OPKO Health Announces Completion of Enrollment for Its Phase III Clinical Trial of Bevasiranib for Treatment of AMD

http://www.businesswire.com

OPKO Health, Inc. (AMEX:OPK) announced today that it has completed enrollment in the Company's Phase III clinical trial of bevasiranib for the treatment of wet age-related macular degeneration (wet AMD). The multi-national study has enrolled more than 330 patients and is designed to assess the efficacy and safety of bevasiranib administered every 8 or 12 weeks in preventing vision loss due to wet AMD. (The current injections with lucentis or avastin is 4-6 weeks).

Bevasiranib is a first-in-class small interfering RNA (siRNA) drug designed to silence the genes that produce vascular endothelial growth factor (VEGF). VEGF is believed to be largely responsible for the vision loss from wet AMD and bevasiranib is the first drug based on the Nobel Prize-winning RNA interference (RNAi) concept to be in Phase III clinical trials. "This first-ever Phase III trial of an agent based on RNAi technology is a milestone in the field of RNAi," said Phillip Frost, M.D., Chairman and CEO of OPKO Health. "With the completion of enrollment, we are one step closer to our goal of submitting a New Drug Application to regulatory agencies worldwide."

Wet macular degeneration is a leading cause of irreversible vision loss in the developed world. Until recently, treatments for wet AMD were of limited efficacy. In the search for more effective treatments, researchers targeted VEGF, shown to be a key cause of the excess growth and leakiness of ocular blood vessels that result in loss of vision in these patients. Current VEGF antagonists, such as Lucentis®, slow this vision loss, but require injections into the eye every four weeks, a particular issue for elderly patients who often have limited mobility. For more information about the COBALT bevasiranib clinical study, please visit
www.opko.com/clinicaltrials.

Nicole Valio Walk-a-Thon Fundraiser
RAISES OVER $9,000 FOR MACULAR DEGENERATION FOUNDATION

Nicole Valio initiated this walk-a-thon fundraiser in honor of her grandfather, August Valio. Nicole writes the following: "After watching my grandpa, August Valio, suffer with macular degeneration for the last ten years, I decided that it was about time I do something. By organizing a walk-a-thon and collecting donations, I was able to raise $9,080 for the Macular Degeneration Foundation. I am extremely appreciative of the generosity and enthusiasm of my family, friends, and all the donors. It is a group of people like this that offer my grandpa and me hope for future generations."

CONTACTING MDF
To speak to a support representative directly, you may call 1-888-633-3937. If you reach our voice mail, please speak slowly and distinctly.

MAKING CONTRIBUTIONS:
Please make checks payable to Macular Degeneration Foundation, Inc., P.O. Box 531313, Henderson, Nevada 89053, or you may use your credit card on our web site http://www.eyesight.org. Your contributions make our services available as a support system for macular degeneration patients in the following ways:

1. We provide toll-free lines for personal contact assistance.
2. We mail brochures and other printed materials upon request.
3. We support an award-winning web site that provides the latest up-to-date information.
4. We fund research proposal grants to provide therapies for both the wet and dry form of AMD. Contributions marked "research" are used 100% for research.

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MDF was founded in 1992 by Edmund J. Aleksandrovich Ph.D (a victim of macular degeneration). It provides MD patients and their families with the information necessary to understand the disease, the latest news concerning ways to cope with the disease, and supports the efforts of researchers to find a cure.