On September 16, AEVR sponsored a Congressional briefing in recognition of International AMD Awareness Week 2008. Researcher Gregory Hageman, Ph.D. (University of Iowa, UI), described dramatic advances in age-related macular degeneration (AMD) research that have occurred since 2005, when his and three other research teams first identified a gene variant strongly associated with increased risk for developing AMD. This research, funded by the NEI and the National Human Genome Research Institute (NHGRI) within NIH, was initially described by Dr. Hageman at a September 2005 AEVR briefing. In this update, he announced new findings that could change the way AMD is treated—and potentially cured.

The identified gene Complement Factor H (CFH) and its protein product are normally engaged in the control of a portion of the body’s immune system. Variants in the gene result in poor regulation of this system and can lead to the development of AMD. In the United States, approximately 15 million people have AMD and, worldwide, it affects vision and the concomitant productivity, independence, and quality of life of more than 30 million people.

In addition to directing the Cell Biology and Functional Genomics Laboratory at UI, Dr. Hageman is also the Scientific Founder and Chief Scientific Officer of a newly formed company Optherion, Inc., which is working on developing AMD treatments based on the latest gene discoveries. With funding from the NEI and UI, and additional support from Optherion, he and colleagues around the world are moving toward commercializing a therapy for replacing CFH in patients with AMD and possibly in genetically-susceptible individuals.

His work over the past 25 years has focused on understanding the cellular pathways leading to AMD, which could lead to discoveries of potential therapies, including the manufacture and use of the protective version of the CFH protein in an augmentation strategy similar to that of treating diabetes with insulin. This therapy is under development and expected to enter Phase I clinical safety trials in summer 2009.

He also announced preliminary, unpublished findings in patients with liver transplants that support the direction and scope of his research. Normal levels of healthy CFH protein protect against AMD. Since most of the CFH is made in the liver, liver transplant recipients offer an opportunity to study what occurs when an individual receives a different form of CFH following transplantation. Researchers hope that these studies will reinforce the concept of providing AMD patients with doses of the protective protein or, in the future, with gene therapy approaches that would allow the liver to produce the protein on its own. Dr. Hageman also noted newly published observations of possible relationships between CFH and obesity, coronary artery disease, myocardial infarction, and stroke, among other conditions—the findings of which facilitate new opportunities for trans-NIH research.

For more information about AMD and other aging eye diseases, refer to The Silver Book: Vision Loss, published by the Alliance for Aging Research in partnership with NAERV at:

www.silverbook.org/visionloss