FACT SHEET

NATIONAL INSTITUTES OF HEALTH

Age-Related Macular Degeneration

Yesterday

- Age-Related Macular Degeneration (AMD) was an untreatable disease that caused central vision loss. It was a major cause of blindness and the leading cause of new cases of blindness in people over age 65.
- AMD made it difficult, if not impossible, to read, recognize faces, drive a car, or perform even simple tasks that require hand-eye coordination.
- AMD severely restricted mobility, forcing many otherwise healthy seniors to prematurely lose their independence and ultimately to be cared for in costly assisted living facilities.
- From a research perspective, AMD was a poorly understood disease and the quest for treatments seemed daunting.

Today

- With Baby Boomers poised to reach retirement age, AMD is increasingly being viewed as an oncoming epidemic that could affect millions of Americans.
- Thanks to considerable research investment by the National Institutes of Health (NIH), the underlying causes of AMD are becoming understood and several treatments have emerged.
- In 1991 the Macular Photocoagulation Study (http://www.nei.nih.gov/neitrials/static/study60.asp), a large scale clinical trial funded by the NIH, established the value of laser treatment for advanced AMD to stabilize the condition.
- In 2000, the Food and Drug Administration (FDA) approved the use of a photosensitive agent called Visudyne as another laser-based treatment to prevent progression of advanced AMD.
- NIH researchers conducted a large scale clinical study called the Age-Related Eye Disease Study (AREDS - http://www.nei.nih.gov/amd/) and found that a daily regimen of antioxidant vitamins and minerals delayed the onset of advanced AMD by 25 percent. Advanced AMD is the most sight-threatening stage of the disease.
- Based on published data, an estimated 8 million older-age Americans are at high risk to develop advanced AMD. Of these 8 million, 1.3 million would develop advanced AMD within 5 years. However, now with the AREDS treatment, 300,000 of these patients could avoid the severe vision loss associated with advanced AMD over a 5-year period.
- Abnormal blood vessel growth, also known as angiogenesis, is a hallmark of advanced AMD. These faulty blood vessels leak serum and blood, damaging the macula and causing catastrophic central vision loss. NIH-sponsored laboratory research has established that a protein known as vascular endothelial growth factor (VEGF) spurs the development of abnormal blood vessels.
- New treatments that block the action of VEGF have been developed and two of these drugs, Macugen and Lucentis, have been approved by the FDA for treatment of advanced AMD.
- Avastin, a drug that is chemically very similar to Lucentis but much less expensive, is being used off-label for patients who cannot afford the higher priced Lucentis. Although Avastin is thought to be effective in treating AMD, there is no clinical trial data to support its use. To address this issue, the NEI launched the Comparison of Age-Related Macular Degeneration Treatments Trial (http://www.nei.nih.gov/catt/) to assess the safety and effectiveness of Avastin and Lucentis.
- NIH researchers also discovered a second protein, pigment epithelial-derived factor (PEDF), which blocks VEGF to prevent blood vessel formation. Early stage clinical trials evaluating a gene therapy approach with PEDF have shown promise.
- NIH-supported researchers recently identified subtle alterations in two genes, complement factor B and H, which account for 75 percent of the risk of developing AMD. These factors function as part of the immune system and normally respond to pathogens to fight infection. Alterations in these genes may result in an inappropriate response of the immune system, causing chronic inflammation within the macula and surrounding tissues. Chronic inflammation is thought