



## Ocular Disease

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### Overview

Ocular diseases and anomalies affect the human eye and visual system. They cover disorders that range from infections to conditions that produce blindness and vision loss. Several systemic diseases can also affect the cornea, eyelids, retina and optic nerve head as age increases. However, the leading causes of blindness in the United States are age-related eye diseases, including age-related macular degeneration (AMD), cataracts, diabetic retinopathy and glaucoma.

- AMD afflicts more than 30 million people worldwide and is the leading cause of blindness in people over 60 in the U.S.<sup>1</sup> An estimated 1.8 million Americans above the age of 40 are affected by AMD, with the total number expected to rise to 2.95 million by 2020.<sup>2</sup>
- Stargardt's disease is the most common form of inherited juvenile macular degeneration. It is usually diagnosed in people below the age of 20 if there are signs of reduced central vision.
- Glaucoma, an eye disease that causes damage to the optic nerve, affects around 50 million people worldwide and between three and four million people in the U.S.<sup>2</sup>
- Cataracts are cloudy areas in the lens caused by a change in the chemical makeup within the lens. They cause blurred and double vision, as well as poor night vision.

- Diabetic retinopathy refers to the damage to the blood vessels of the retina caused by diabetes. The damage results in weakened and broken blood vessels that either leak fluid into the retina or cause it to swell.

Though surgery and laser treatments can be used for some ocular disorders, others remain difficult to treat. As the aging population increases in size, it is becoming more imperative to explore different treatment options for age-related eye diseases.

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### Ocular Diseases and Regenerative Medicine

Many of the field's key opinion leaders consider ophthalmology to be an area where regenerative medicine will have a tremendous impact over the coming years. Promising results from animal models have been published in leading academic journals, and several companies are moving into mid-stage clinical trials for a variety of ocular disorders—especially age-related macular degeneration.

**Advanced Cell Technology (ACT)** is targeting degenerative retinal disease. They are focusing on commercializing human embryonic stem cell-derived retinal pigment epithelial cells (RPE). A Phase 1/2 clinical trial for AMD and Stargardt's disease is underway with several major eye institutes, including the Wills Eye Institute in

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Philadelphia to test the safety and tolerability of the hESC-derived RPEs in patients with dry AMD.

StemCells, Inc. is developing a human neural stem cell product, HuCNS-SC, for AMD. Preclinical results revealed that their product preserved the visual acuity in rats, protecting the retina from progressive degeneration. Phase 1/2 trials began in 2012. The trial will evaluate the safety and preliminary efficacy of HuCNS-SC cells as a treatment for dry AMD.

University College London's (UCL) *The London Project to Cure Blindness* is also focused on using embryonic-derived RPE cells to target AMD. The scientists at UCL have established a formal collaboration with Pfizer for clinical development of a small patch of cells to cover the damaged macula, protecting the eye from age-related blindness. They have completed their preclinical safety experiments and are hoping to start clinical trials in 2013.

The RIKEN Center for Developmental Biology in Kobe, Japan, is also working toward solutions for AMD using induced pluripotent stem cells. RIKEN scientists are currently taking mature skin cells from patients, reprogramming them into stem cells, and then coaxing them into a certain type of retinal cell to be transplanted into patients' eyes. The team, led by Masayo Takahashi, M.D., Ph.D., plans to submit an application for a clinical study to the Japanese health ministry next month, and could be recruiting patients as early as September.

## Ocular Disease: Economic Impact



**\$51.4 Billion**

Estimated U.S. annual cost of care for those who are visually impaired or blind.<sup>3</sup>



**\$570 Million**

Annual portion of U.S. direct medical costs due to age-related macular degeneration.<sup>3</sup>

<sup>1</sup> Advanced Cell Technology, "Retinal Pigment Epithelial Cell Program," 2013, available at [www.advancedcell.com/our-technology/act-stem-cell-related-research-pipeline/retinal-pigment-epithelial-cell-program/](http://www.advancedcell.com/our-technology/act-stem-cell-related-research-pipeline/retinal-pigment-epithelial-cell-program/)

<sup>2</sup> Vision Health Initiative (VHI), "Common Eye Disorders," July 31, 2009, Centers for Disease Control and Prevention website, [www.cdc.gov/visionhealth/basic\\_information/eye\\_disorders.htm](http://www.cdc.gov/visionhealth/basic_information/eye_disorders.htm)

<sup>3</sup> *The Economic Impact of Vision Problems: The Toll of Major Adult Eye Disorders, Visual Impairment and Blindness on the U.S. Economy* available at [www.preventblindness.net/site/DocServer/Impact\\_of\\_Vision\\_Problems.pdf?docID=1321](http://www.preventblindness.net/site/DocServer/Impact_of_Vision_Problems.pdf?docID=1321) (published by Prevent Blindness America, 2007)