AEVR EDUCATION

International Age-Related Macular Degeneration (AMD) Awareness Week Congressional Briefing: *Retinal Regeneration and Stem Cell Therapy*

Featured speaker Mandeep Singh, MD, PhD (Wilmer Eye Institute/Johns Hopkins University School of Medicine)

On September 13, in recognition of both *Healthy Aging Month and International Age-related Macular Degeneration (AMD) Awareness Week* 2017, AEVR's *Decade of Vision 2010-2020 Initiative* and co-sponsors (see box right) held a Congressional Briefing entitled *Advances in the Diagnosis and Treatment of AMD and Retinal Diseases* that focused primarily on retinal regeneration and stem cell therapy for the "dry" form of AMD.

Clinician-scientist Mandeep Singh, MD, PhD, an Assistant Professor of Ophthalmology at the Wilmer Eye Institute at Johns Hopkins University School of Medicine, spoke about his clinical practice and research activities as they relate to AMD and other retinal diseases. AEVR invited Dr. Singh—who participated in its September 2016 *Emerging Vision Scientists Day* on *Capitol Hill*—to be the featured speaker, where he addressed a packed room that included the 18 early-stage investigators

The transplantation of photoreceptor cells is a promising concept for "dry" AMD treatment in the future.

participating in the 2017 *EVS* Day who observed how their colleague described research to a public policy-oriented audience.

Dr. Singh discussed research activities—funded primarily by the NEI—to diagnose and treat AMD, both the "wet" or neovascular form of the disease—where new blood vessels disrupt the retina and which accounts for about 10 percent of AMD cases—as well as for "dry" or atrophic AMD, where the photoreceptors (the light-sensitive cells in the retina) and retinal pigment epithelial cells gradually die away and which accounts for 90 percent of AMD cases.

He described the dramatic improvements in wet AMD treatment from "anti-VEGF" therapy. These therapies, developed in part through NIH-funded research, include Food and Drug Administration (FDA)-approved drugs that are injected into the eye, inhibiting abnormal blood vessel growth due to Vascular Endothelial Growth Factor (VEGF) and stabilizing vision loss—and, in a proportion of cases, improving lost vision. The diagnosis of wet AMD and the efficacy of drug treatments is determined in large part through the use of non-invasive imaging technology such as Optical Coherence Tomography (OCT) that can show microscopic changes in the eye caused by AMD. Since OCT was also developed through NIH-funded research, he emphasized that sustained funding has resulted in both the imaging technology to diagnose and monitor wet AMD disease progression, as well as to monitor the effect of the drug therapies to treat it.

Dr. Singh acknowledged that, although dry AMD is more prevalent, no current treatments exist to restore vision in this condition. However, thanks to the NEI-funded *Age-Related Eye Disease Study (AREDS)* trials, an anti-oxidant regimen is available and can significantly decrease the rate of progression of dry AMD. He reported that there are also several promising therapies on the horizon for dry AMD, including retinal stem cell therapy clinical trials which are now proceeding after many years of preclinical development in animal studies. He showed how several groups have pioneered the concept of stem cell-derived retinal pigment epithelial cell transplantation in human clinical trials. He highlighted the transplantation of photoreceptor cells as a promising concept for dry AMD treatment in the future, where photoreceptors can be grown in the laboratory—essentially a "mini-retina in a dish"—and then injected into the eye to "patch" the degenerated retina in dry AMD. The goal for these transplanted cells is to make synapses—or connections—with host inner retinal neurons and regenerate the visual circuit. His most recent research has shown that these photoreceptor cells fuse with each other.

He concluded by noting that stem cells hold great promise as a tool for regenerative medicine and that further progress will lead to treatments that can regenerate photoreceptors in dry AMD.

After Dr. Singh's presentation, patient advocate Paul Gariepy offered his perspective on the impact of AMD on activities of daily living and how he is channeling his efforts through the American Macular Degeneration Foundation (see box below) to support effective treatments.

Dawn Prall George of Briefing co-sponsor organization Macula Vision Research Foundation with Dr. Singh

From left: Mark Lenker (Shire), AEVR Executive Director James Jorkasky, and Scott Haber (American Academy of Ophthalmology)

AMD is the leading cause of blindness and low vision in the United States—especially in the age 60-plus population—and is increasingly prevalent due to the aging population, with greatest growth in the age 90-plus segment. NEI estimates that 200,000 Americans each year go on to develop advanced AMD. Since AMD affects central vision–specifically the macula, which is the central part of the light-sensitive retina in the back of the eye–it impacts an individual's ability to read and drive, significantly affecting quality of life. One-third of AMD patients experience clinical depression. Numerous risk factors are associated with AMD. Non-modifiable factors include aging, genetics, gender, and race, while modifiable factors include smoking, diet, obesity, high blood pressure, high cholesterol, cardiovascular disease, and light exposure.

AEVR wishes to thank its co-sponsors for this event:

Research to Prevent Blindness
Alliance for Aging Research
American Macular Degeneration Foundation
Association for Research in Vision and Ophthalmology
European Vision Institute
Lighthouse Guild
Macula Vision Research Foundation
Macular Degeneration Partnership

AEVR also thanks Regeneron for a grant to support event management.

Representatives from new NAEVR/AEVR member organization American Macular Degeneration Foundation (AMDF) participated in the Congressional Briefing in between visits with the Connecticut, Massachusetts, New York, and Vermont delegations.

Cong. Joe Kennedy III (D-MA), center, hosted AMDF representatives, including from left: Matt Levine, Paul Gariepy, Neena Haider, PhD (Mass Eye & Ear/Harvard), Jennifer Williams, Mark Torrey, and Chip Goehring