International Age-Related Macular Degeneration (AMD) Awareness Week
Congressional Briefing:
New Stem Cell-Based Therapies for AMD and Blast-Related Eye Injuries

DECADE OF VISION 2010-2020
an initiative of the Alliance For Eye And Vision Research

NEED TO KNOW:

*On September 18, in recognition of both **Healthy Aging Month** and **International Age-related Macular Degeneration (AMD) Awareness Week 2019**, AEVR's **Decade of Vision 2010-2020 Initiative** and co-sponsors (see box) held a Congressional Briefing entitled **New Stem Cell-Based Therapies for AMD and Blast-Related Eye Injuries** featuring tenured NIH intramural researcher Kapil Bharti, PhD. This event began AEVR's **Fifth Annual Emerging Vision Scientists Day on Capitol Hill**, summarized in stories on the next two pages.*

AEVR wishes to thank its co-sponsors:

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

AEVR also thanks Regeneron for support for event management.

AMD, which is one of the leading causes of blindness and low vision in the United States and the developed world, destroys central vision through proliferation of new blood vessels in the "wet" form of the disease or gradual breakdown of cells in the retina, the light-sensitive tissue at the back of the eye, in the "dry" form of the disease, also called geographic atrophy. Vision loss from AMD makes it increasingly difficult to read, drive, and perform other everyday tasks.

Featured speaker Kapil Bharti, PhD (NEI)

AEVR President Dr. Paul Lee provided a welcome to the Briefing that featured Dr. Bharti, who serves as a Senior Investigator in the Ocular and Stem Cell Translational Research Section at the NEI. He has also received funding from the Department of Defense's (DOD) Psychological Health/ Traumatic Brain Injury (PH-TBI) Program for new stem cell approaches to treat blast-related eye injuries, complementing his NIH Common Fund and NIH intramural-funded AMD and blast injury research.

Dr. Bharti explained that tremendous strides in the treatment of patients with wet AMD have resulted from Food and Drug Administration (FDA)-approved anti-Vascular Endothelial Growth Factor (VEGF) therapies—which emerged from initial NIH funded research—that stabilize vision loss and may improve lost vision. Although research to develop new therapies to treat geographic atrophy or the dry form of AMD has lagged behind that for wet AMD, he described the Phase I first-in-human clinical trial that will test the safety of using an induced pluripotent stem cell (iPSC)-based therapy to treat AMD. The study, in which he serves as Lead Investigator, had recently been submitted to the FDA for review.

The therapy involves taking a patient's blood cells and, in a lab, converting them into iPS cells, which can become any type of cell in the body. The iPS cells are programmed to become Retinal Pigment Epithelium (RPE), the type of cell that dies early in the geographic atrophy stage of AMD. RPE cells nurture photoreceptors, the light-sensing cells in the retina. In geographic atrophy, once RPE cells die, photoreceptors eventually also die, resulting in blindness.

"Although it could be another 10 years until we have an approved therapy, we are one step closer to providing hope for patients with vision loss."—Dr. Bharti

The therapy is an attempt to shore up the health of remaining photoreceptors by replacing dying RPE with patient's own iPSC-derived RPE.

Through dramatic multi-dimensional graphics that documented prior work in animal studies, Dr. Bharti demonstrated how, before they are transplanted, the iPSC-derived RPE are grown in tiny sheets one cell thick, replicating the natural structure within the eye. This monolayer of iPSC-derived RPE is grown on a biodegradable scaffold designed to promote the integration of the cells under the retina. A specifically designed surgical tool was built for the task of inserting the patch of cells under the retina. Proof of concept in the initial animal studies focused on the structural and functional assessment of the transplanted tissue and the RPE injury. For example, imaging studies confirmed that the lab-made cells had integrated within the animal eye, while electrical responses recorded from photoreceptors "rescued" by RPE patches were closer to normal, whereas photoreceptors treated with a control empty scaffold had died.

In describing the types of stem cell-derived tissues that may be needed at different stages of retinal disease or retinal injury to various structures in the eye—including the choroid, which is the vascular layer that provides nutrients to the RPE—Dr. Bharti spoke about his complementary DOD-funded research to develop an RPE patch, an RPE/choroid patch, or retina/RPE/choroid patch, depending on the extent of blunt or blast injury that can lead to complex tears and tissue loss in the retina.

He concluded by stating, "Although the timeline for development of an approved stem cell therapy for dry AMD is lengthy, we have successfully completed the preclinical work in animals and are now ready to embark on the Phase I safety trial in humans."

Dr. Bharti speaks with Cong. Scott DesJarlais, MD (R-TN), a physician who attended the briefing

Dr. Bharti with Shefa Gordon, PhD, NEI's Director of the Office of Program Planning and Analysis, left, and AEVR Board President Dr. Paul Lee, right

Left to right: Representatives from event co-sponsor AMDF, which provided an eye-healthy luncheon, included Neena Haider, PhD (Mass Eye & Ear/Harvard), Paul Gariepy, Sydney Ruth Torrey, Dr. Bharti, Chip Goehring, and Matthew Levine. Ms. Torrey, who has dry AMD and serves as a Patient Liaison for AMDF, speaks daily with patients whose quality of life has been affected by the disease.