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Foundation Fighting Blindness Announces $2.4 Million in Funding for Eight New Sight-Saving Research Projects

Studies Involve Gene Therapy, Cell Transplantation Methods to Target Retinal Diseases Including AMD

COLUMBIA, MD (August 21, 2012) – The Foundation Fighting Blindness announces an investment of $2.4 million in eight promising research projects aimed at providing treatments and identifying causes of retinal degenerative diseases including age-related macular degeneration (AMD), retinitis pigmentosa (RP), Leber congenital amaurosis (LCA), and Usher syndrome, among other conditions. The spectrum of funded work spans from innovating gene therapies, to advancing cell transplantation, to understanding AMD risk, to developing new therapies for an inherited condition that causes blindness at birth. Each of the eight investigative teams will receive $300,000 for its three-year research efforts.

"These eight grants are going to a truly outstanding cadre of scientists," says Dr. Stephen Rose, chief research officer, Foundation Fighting Blindness. "The funding will help investigate several approaches to saving sight and provides strong momentum for moving treatments forward, out of the lab and into the clinic."

The projects receiving funding were selected through a rigorous review process conducted by the Foundation's Scientific Advisory Board, a group comprised of the field's leading retinal researchers. A total of 35 proposals were reviewed during the grant allocation process.

Eight New Foundation Fighting Blindness-Funded Projects

- **Oklahoma University Health Sciences Center in Oklahoma City, Muna Naash, Ph.D.**
  Dr. Naash's project focuses on the development of a nanoparticle-based gene therapy for Usher syndrome type 2A (USH2A). While human-engineered viruses, including adeno-associated viruses (AAVs), have worked well in delivering therapeutic genes to retinal cells in clinical trials, they aren't able to deliver large genes, such as USH2A. Nanoparticles appear to have no capacity limitations, and Dr. Naash's USH2A gene therapy development effort could facilitate the advancement of delivery systems for other large genes that cause retinitis pigmentosa and related conditions.

- **University of Florida in Gainesville, Shannon Boye, Ph.D.**
  An expert in AAV development, Dr. Boye is working on a gene therapy delivery system that can be used to deliver therapeutic genes through the vitreous to the front of the retina, instead of underneath it. Her new approach may be safer for the retina and enable gene therapies to more effectively reach cones, the cells that provide central vision.

- **Tufts University Medical Center in Boston, Johanna Seddon, M.D., ScM**
  Dr. Seddon's project aims to define new variants in genes that put people at risk for the dry form of AMD. By identifying disease-causing genes, she can provide the research community with clear targets for treatments and cures.

- **Johns Hopkins University School of Medicine in Baltimore, Donald Zack, M.D., Ph.D.**
  Dr. Zack's goal is to pinpoint biomarkers — proteins or molecules — in the blood that can help identify who is at risk for AMD. Through his research, a simple blood test might someday be developed to determine if a person is likely to be affected by this vision-robbing condition.

(MORE)

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- **Columbia University in New York City, Ted Smith, M.D., Ph.D.**
  Dr. Smith will be analyzing the genetic and physical characteristics of reticular macular degeneration, a distinct form of AMD that puts people at high risk for vision loss. His research may help doctors identify these high risk individuals as well as potential treatments.

- **University of Medicine and Dentistry, New Jersey in Newark, Marco Zarbin, M.D., Ph.D.**
  One of the big challenges in transplanting therapeutic cells to the retina is getting them to survive, integrate and become functional, especially when the patient's retina is compromised by disease or age. Dr. Zarbin will be developing and analyzing a mixture of biological molecules that helps transplanted retinal cells integrate and survive in the eyes of people affected by AMD.

- **Radboud University Nijmegen Medical Centre in Nijmegen, The Netherlands, Rob Collin, Ph.D.**
  With the eventual goal of launching a clinical trial, Dr. Collin is developing an innovative gene correction treatment for people with the most common type of Leber congenital amaurosis (LCA), caused by defects in the gene CEP290. LCA is a severe form of RP that causes blindness or severe vision loss at birth.

- **University of Massachusetts Medical School in Worcester, Hemant Khanna, Ph.D.**
  Also focusing on LCA caused by defects in the gene CEP290, Dr. Khanna will be investigating causes of the disease, and potential treatments for preventing or reversing vision loss.

About Foundation Fighting Blindness

The Foundation Fighting Blindness is a national nonprofit driving research that will lead to preventions, treatments and cures for retinitis pigmentosa, macular degeneration, Usher syndrome and the entire spectrum of retinal degenerative diseases that affect more than 10 million Americans. Since 1971, the Foundation has raised more than $450 million as the leading non-governmental funder of retinal research. Breakthrough Foundation-funded studies using gene therapy have restored significant vision in children and young adults who were previously blind, paving the way for using this method to treat a variety of retinal degenerative diseases, and proving a cure is in sight. With a network of nearly 50 chapters, the Foundation also provides support and resources to affected individuals and their families in communities across the country.

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