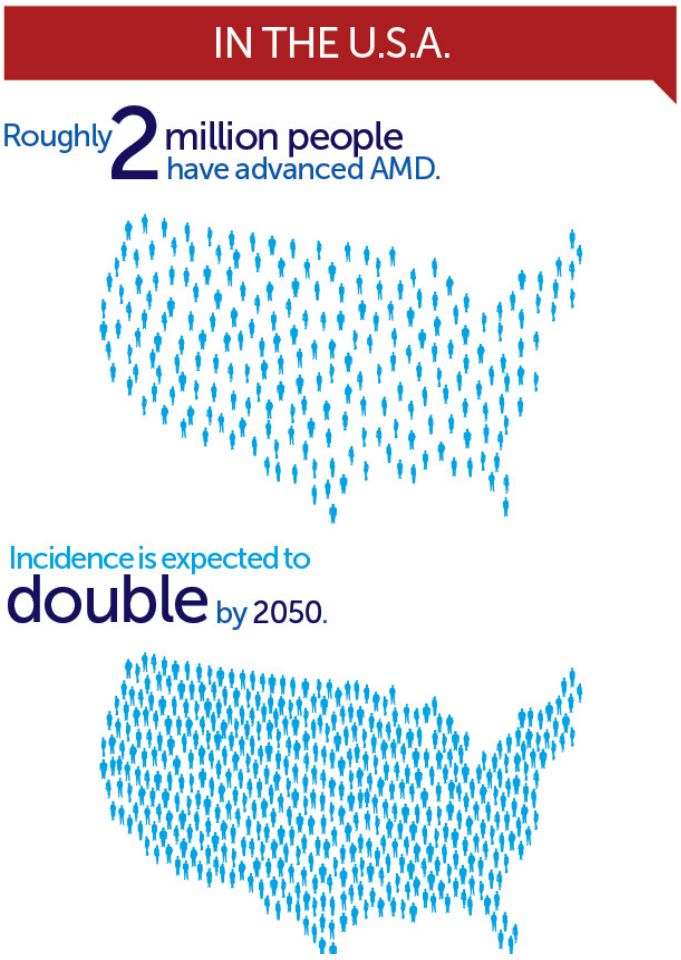
BrightFocus® Foundation
Cure in Mind. Cure in Sight.

Macular Degeneration Research Current Grantees

Advanced forms of age-related macular degeneration (AMD) are a leading cause of vision loss and irreversible blindness in Americans age 60 years and older, as well as throughout the world. As many as 11 million Americans have some form of macular degeneration, including both early and later stages of the wet and dry forms. This number is expected to double by 2050. Since treatments are limited or nonexistent for several AMD stages and types, much more work is needed to develop effective treatments and cures.

With the support of our donors, Macular Degeneration Research (MDR), which began in 1999, has awarded more than $21.5 million to fund research projects on the cause, potential prevention, and treatment of this disease. The standard MDR grant is $160,000 over two years. In the past three years alone, MDR has funded 55 research projects totaling $7.5 million.

MDR is currently supporting 40 research projects, which are described in this yearbook.



Understanding Early AMD

As the eyes age, there's a breakdown in its ability to get rid of cholesterol, which collects in drusen and triggers an immune response. That may be one of the tipping points leading to early AMD, followed by inflammation and eventual cell death. A stiffening of blood vessels, which happens in the eye as it does in the body, may contribute further. AMD is a disease linked to not just one, but many, causes. Foremost are changes in the eye that happen with age—the strongest risk factor. MDR is funding scientific exploration that will expand our understanding of AMD and open new and earlier avenues for treatment.

Maria Valeria Canto-Soler, PhD (7/1/2016 – 6/30/2018)
Wilmer Eye Institute, Johns Hopkins University
A New Model of a Human Retina in a Dish to Study AMD

"Our goal is to develop the first 'human retina in a dish' model to provide a unique biological system to investigate the initial triggers leading to AMD and to develop treatments to stop its progress."
www.brightfocus.org/grant/M2016119

Patrick Daugherty, PhD (7/1/2016 - 6/30/2018)
University of California, Santa Barbara
Characterization of Circulating Antibodies Specific to AMD

"The objective of this study is to characterize the changes in the immune response as individuals develop AMD. The end result of this effort will be to develop diagnostics for early detection."
www.brightfocus.org/grant/M2016219

Sarah Doyle, PhD (7/1/2016 - 6/30/2018)
Trinity College Dublin, Ireland
Investigating How Loss of an "Off Switch" for Inflammation Contributes to AMD

"AMD has elements that indicate that the inflammatory response is uncontrolled and persistent when low-level inflammation is observed. Our research question asks whether this active process of switching off the inflammatory response is lost in people with AMD."
www.brightfocus.org/grant/M2016030

Malia Edwards, PhD (7/1/2016 – 6/30/2018)

Wilmer Eye Institute, Johns Hopkins University

A Study of Why Retinal Support Cells, Called Glia, Exit the Retina in AMD

"The goal of my research is to identify glial cell changes in AMD and determine how these may affect AMD progression and treatment."

www.brightfocus.org/grant/M2016198

Kaustabh Ghosh, PhD (7/1/2016 – 6/30/2018)

University of California, Riverside

Understanding the Role of Increased Vessel Stiffness in Cell Death Associated with AMD

"In this project, we are investigating the hypothesis that aging leads to stiffening of blood vessels in the eye that, in turn, exacerbates the pathogenesis of AMD by causing inflammation-mediated vascular degeneration."

www.brightfocus.org/grant/M2016161

Goldis Malek, PhD (7/1/2015 – 6/30/2017)

Duke University

The Role of an Immune Cell Attractant in a Blinding Disease

"The experiments in this study are designed to address the following question: What is the role of a specific factor called osteopontin that may be responsible for recruiting immune cells to the eye in the development and progression of AMD?"

www.brightfocus.org/grant/M2015421

Ruchira Singh, PhD (7/1/2015 – 6/30/2017)

University of Rochester Medical Center

Understanding the Role of Different Cells in the Eye that Are Affected in AMD

"The overall goal of this project is to understand the role of different cells in the eye that are affected in macular degeneration, and which of them initiate disease processes; culminating in visual dysfunction."

www.brightfocus.org/grant/M2015267

RPE and Drusen Formation

It's widely thought that macular degeneration. and its most common form AMD, begins with activation of immune cells in the retinal pigment epithelium (RPE), a pigmented layer of cells next to the retina. The RPE's role is to protect and nourish the nerve cells by transporting molecules in and out. As one of the starting points of AMD, the RPE is sensitive to damage brought on by age, oxidative stress, and other factors. That may trigger chronic inflammation, which confuses RPE cells and impairs their ability to clear away debris ("autophagy"). Small fatty particles, known as exosomes, are released by RPE cells and may be involved in drusen formation, yellow deposits under the retina that are made up of fatty protein. When spotted on an eye exam, drusen often are the first obvious sign of disease.

Francesco Giorgianni, PhD (7/1/2016 – 6/30/2018)
University of Tennessee Health Science Center
Basic and Clinical Studies to Understand the Role of the CD5L/AIM Protein in AMD

"The main objective of our research project is to reveal the cellular and molecular mechanisms involved in AMD by studying two key pathological events that characterize AMD: the accumulation of drusen deposits and the death of RPE cells."
www.brightfocus.org/grant/M2016068

Nady Golestaneh, PhD (7/1/2014 – 6/29/2017)
Georgetown University
Cellular Self-Eating: An Important Mechanism in AMD

"We propose to study the role of autophagy and its possible dysfunction in AMD"
www.brightfocus.org/grant/M2014039

Robyn Guymer, PhD (7/1/2016 – 6/30/2018)
Centre for Eye Research Australia, University of Melbourne
Too Much Debris as a Cause of AMD

"The goal of our project is to provide a novel explanation for the accumulation of debris that contributes to AMD."
www.brightfocus.org/grant/M2016061

Aparna Lakkaraju, PhD (7/1/2015 – 6/30/2017)
University of Wisconsin-Madison
Insight into the Formation of Harmful Deposits in the Retina

"My group has identified FDA-approved drugs that help clear garbage and prevent inflammation in the RPE. This project will evaluate the potential of these drugs to prevent the release of these harmful exosomes from the RPE."
www.brightfocus.org/grant/M2015350

Ernesto Moreira, MD (7/1/2015 – 6/30/2017)
Medical University of South Carolina
Using Patient-Derived Stem Cells as a New Model to Study Disease Mechanisms in AMD

"In this project we are investigating how toxins derived from cigarette smoke lead to RPE cell damage through the activation of the immune complement system."
www.brightfocus.org/grant/M2015356

New Approaches to AMD Treatment

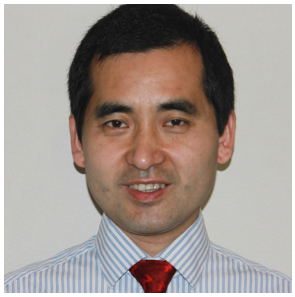
What will be the next "gold standard" treatment for AMD? MDR is funding research into unique ways to protect the retinal pigment epithelium (RPE) and retina at earlier stages, before damage to sight has occurred. These include drugs that enhance immune functioning and improve the eye's ability to clear lipids and other waste that might otherwise lead to inflammation. Researchers are also looking at ways to restore health to the aging eye by improving its metabolism, or efficiency at storing and using energy.

Kip Connor, PhD (7/1/2016 – 6/30/2018)
Schepens Eye Research Institute/Massachusetts Eye and Ear, Harvard
Lipid Regulators of AMD

"Our proposal has clear potential to lead to new therapeutic molecules, targets, and strategies for specifically inhibiting neovascular AMD, a leading cause of blindness in the elderly which, if left untreated, rapidly leads to substantial vision loss."
www.brightfocus.org/grant/M2016183

Ivan Conte, PhD (7/1/2015 – 6/30/2017)
Fondazione Telethon, Italy
MicroRNAs in AMD: Novel Molecules for Future Therapies

"The goal of this project is the study of the possible role of a microRNA in RPE physiology, survival, and homeostasis both as causative agents and as therapeutic agents."
www.brightfocus.org/grant/M2015317



Jianhai Du, PhD (7/1/2016 – 6/30/2018)
West Virginia University
A New Method to Decrease Cell Death by Supplementation with NAD Metabolites

"The goal of this project is to understand how energy metabolism is altered in AMD and test a nutritional approach to boost metabolism to prevent or rescue dry AMD."
www.brightfocus.org/grant/M2016047

John Hulleman, PhD (7/1/2016 – 6/30/2018)
University of Texas Southwestern Medical Center
A Single Genetic Manipulation for Treating ML/Dry AMD

"Malattia Leventinese (ML) and AMD are two eye diseases that disrupt the normal physiology of the retina, the back portion of the eye which is responsible for sensing light. The goal of our project is to slow or prevent damage to a cell layer in the eye called the RPE by genetically reducing expression of a target protein in the retina that has been associated with inflammation and risk of AMD."
www.brightfocus.org/grant/M2016200

Eric Yin Shan Ng, PhD (7/1/2015 – 6/30/2017)
Schepens Eye Research Institute/ Massachusetts Eye and Ear, Harvard
TLR2 As A Novel Therapeutic Target For Wet AMD

"The objective of this proposal is to validate the functional role of toll-like receptors (TLRs), a component of the innate immune system, in choroidal neovascularization (CNV) pathogenesis, and to evaluate TLRs as a novel, effective and safe therapeutic target for the treatment of CNV."
www.brightfocus.org/grant/M2015214

Diana Pauly, PhD (7/1/2015 – 6/30/2017)
University Hospital Regensburg, Germany
Can Proteins Block Inflammation in the Eye and Ameliorate AMD-Pathology?

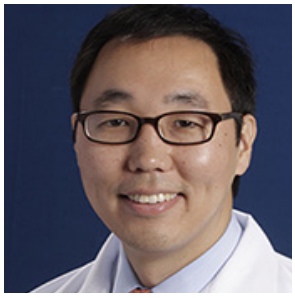
"For this research, we will generate an array of protein tools, designed such that some of them will eventually be developed into novel therapeutics for the inhibition of overactive inflammation in the human eye during AMD pathology."
www.brightfocus.org/grant/M2015186

Drug Discovery & Development

There are currently no drugs to treat advanced forms of dry AMD, also called geographic atrophy (GA). And despite the success of drug injections for wet AMD, not all patients respond. New therapies are needed, and MDR-funded investigators are exploring new drugs and conducting early tests of their potential. These include new agents that work to block blood vessel growth in wet AMD, as well as compounds to treat dry AMD which, if successful, could be among the first drug treatments.

Tim Corson, PhD (7/1/2015 – 6/30/2017)
Indiana University School of Medicine
New Drugs to Treat Abnormal Blood Vessel Growth in Wet AMD

"Starting with a new chemical that we developed that blocks blood vessel growth, we will design and produce related chemicals that are even more potent and selective for blood vessel cells over other cell types."
www.brightfocus.org/grant/M2015301



Benjamin Kim, MD (1/1/2016 – 6/30/2017)
University of Pennsylvania
Therapeutic Evaluation of Alpha Lipoic Acid for GA

"Through a Phase II pilot clinical trial, we are testing alpha lipoic acid as a treatment for GA."
www.brightfocus.org/grant/CM2016971

Alfred Lewin, PhD (7/1/2015 – 6/30/2017)
University of Florida
A Novel Antioxidant Therapy for Retinal Degeneration

"The goal of this project is to develop a novel drug to prevent GA, the advanced form of dry AMD."
www.brightfocus.org/grant/M2015348

Marcelo Nociari, PhD (7/1/2016 – 6/30/2018)
Weill Cornell Medical College, Cornell University
Identification of Novel Treatments for Macular Degeneration by Alleviating Endoplasmic Reticulum Stress

"We found a novel mechanism by which lipid-bisretinoids (LBs) kill RPE cells. In the current project, we propose to fully characterize this new damaging pathway, and test whether by targeting this pathway we can prevent blindness in animal models of LB-driven retinal disease."
www.brightfocus.org/grant/M2016124

Debasish Sinha, PhD (7/1/2016 – 6/30/2018)
Wilmer Eye Institute, The Johns Hopkins University
Novel Therapeutic Targets for the Treatment of Early AMD

"Our proposed studies are aimed at developing novel small molecules that could be tested as a therapy for early AMD."
www.brightfocus.org/grant/M2016056

Hongli Wu, PhD (5/1/2015 – 4/30/2017)
University of North Texas Health Science Center
The Role of Glutaredoxin 2 (Grx2) in AMD

"Understanding the function of antioxidant enzymes in the retina is critical for developing new therapies for AMD."
www.brightfocus.org/grant/M2015180

Insights into Geographic Atrophy

Geographic atrophy (GA) is a type of AMD that's also called "dry" AMD because it lacks the fragile, leaky blood vessels seen in late-stage "wet" AMD. Instead, photoreceptors weaken and die ("atrophy"), resulting in dead zones and an expanding blind spot near the center of the visual field. There is no treatment for late-stage GA and MDR is funding projects aimed at finding better treatments and a cure. New imaging and analysis techniques will help us understand GA lesions and do a better job of tracking progression over time. Additional projects focus on genetics: conducting rapid analysis on the world's largest collection of AMD samples to pinpoint genes that interact in both dry and wet AMD, and experimenting with gene therapy.

Paul Baird, PhD (7/1/2016 – 6/30/2017)
Centre for Eye Research Australia, The University of Melbourne
Identifying Gene Pathways in Late-Stage AMD

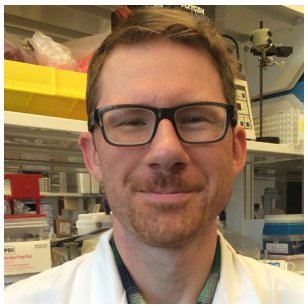
"This project will look at how different regions of our genetic background interact with other genetic regions and lead to AMD."
www.brightfocus.org/grant/M2016178

Vera Bonilha, PhD (7/1/2016 – 6/30/2018)
The Cleveland Clinic Foundation
Atrophic Lesion Borders in AMD: What Can They Tell Us?

"The information gained from this study will aid in understanding the pathophysiological mechanisms causally involved in GA and may offer additional insight in clinical diagnosis and therapeutic decision-making for GA."
www.brightfocus.org/grant/M2016079

Zhihong Hu, PhD (7/1/2016 – 6/30/2018)
Doheny Eye Institute, University of California, Los Angeles
An Automated Method to Detect and Analyze Atrophic Lesions in AMD.

"The deliverable from this research program is a fully automated system for the detection of GA lesions and the quantitative analysis of GA progression."
www.brightfocus.org/grant/M2016088



Mikael Klingeborn, PhD (7/1/2015 – 6/30/2017)
Duke Eye Center, Duke University
The Role of Cell-Derived Lipid Vesicles in Early & Atrophic AMD

"Our research seeks to understand what causes the earliest stages of the dry form of AMD."
www.brightfocus.org/grant/M2015221

Rescue & Regeneration of Sight Damaged by AMD

Eyesight is precious and irreplaceable. Unlike skin and other parts of the human body, the nerve cells of the eyes do not, for the most part, regrow or regenerate after damage has occurred. However, there is hope, and researchers are exploring whether it might be possible to "rescue" cells from early AMD damage using growth factors and other natural hormones that offer protection. In addition, work is moving forward to regenerate and reconnect the eye's retinal cells, and to restore the underlying RPE that provides its nourishment and support. They are recreating parts of the eye using induced pluripotent stem cell (iPSC) technology (stem cells derived from living adult tissue), and cell growth is being studied in other members of the animal kingdon with the hope of gleaning information that will benefit human eyes.

Derek van der Kooy, PhD (7/1/2016 – 6/30/2018)
University of Toronto, Canada
Biomaterial-based Stem Cell Therapies for Blinding Eye Disease

"We are focused on the goal of producing large quantities of cone photoreceptors for transplantation directly into the retina. Cones are the cells responsible for high-resolution/color vision and are lost in AMD."
www.brightfocus.org/grant/M2016173

Petr Baronov, MD, PhD (7/1/2016 – 6/30/2018)
Schepens Eye Research Institute/Massachusetts Eye and Ear, Harvard
A New Approach to Rescuing Photoreceptors from Death through Activation of Endogenous Neuroprotective Mechanisms

"We aim to identify small molecules that can induce endogenous growth factors in the sensory part of the eye, the retina. The results of our studies should lead to the development of novel, accessible and effective molecular therapies for retinal degeneration and other neurodegenerative disorders."
www.brightfocus.org/grant/M2016046

Behzad Gerami-Naini, PhD (7/1/2014 – 6/30/2017)

Tufts University

Using Cells from Teeth to Replace Damaged Cells in AMD

"Our work is focused on developing a novel cell therapy intervention to treat AMD by replacing diseased RPE cells with newly made replacement cells, generated from cells found in the dental pulp of extracted teeth and grown on a special silk surface."

www.brightfocus.org/grant/M2014059

Jeffrey Gross, PhD (7/1/2016 – 6/30/2018)

University of Pittsburgh

Identification of Factors that Can Stimulate Regeneration of the RPE

"The goal of our work is to determine whether the human RPE can be stimulated to regeneration."

www.brightfocus.org/grant/M2016067

Biju Thomas, PhD (7/1/2016 – 6/30/2018)

Roski Eye Institute, University of Southern California

Transplantation of iPS-RPE as a Polarized Monolayer

"We propose to treat such diseases by transplanting a polarized monolayer of retinal pigment epithelium (RPE) sheets derived from human induced pluripotent stem cells (iPS), which are stem cells that have been derived from adult human tissue."

www.brightfocus.org/grant/M2016186

Sara Venters, PhD (7/1/2014 – 6/30/2017)

University of California, San Francisco

Investigating Central Neural Retina Development in a Vertebrate Model

"The central retina is specifically affected in several eye diseases, and discovering what makes it unique can help with treatment and future therapies for re-growing this portion of the eye. Our research goal is to identify the definitive origin and specific developmental mechanisms underlying the establishment of central neural retina cells."

www.brightfocus.org/grant/M2014060

New Hope for Preventing AMD

One day we may be able to detect signs that AMD is developing and prevent it from damaging eyesight. Many different strategies are being explored. One early sign, there may be inflammatory factors in blood that could serve as preclinical biomarkers for AMD; also it may be possible to image inflammation at the cellular level to alleviate the early immune response. Another early sign of AMD is a slowdown in the retina's ability to recover from light exposure, and a simple test of the eye's response to light is being evaluated for predictive value. Knowledge of genetics is advancing to the point that it may be possible to replace or block gene signaling that triggers AMD. As yet another protective mechanism, researchers are looking at ways to increase stress resistance and promote survival and integrity of the retinal pigment epithelium (RPE) as it encounters oxidative stress from aging and other causes.

Marie Burns, PhD (7/1/2015 – 6/30/2017)
University of California, Davis
Window to Health: New Ways to Detect the First Signs of Cell Sickness in the Eye

"One big-picture goal of our research is to identify cells in distress and to heal them before they die and before patients lose their sight."
www.brightfocus.org/grant/M2015379

Noriko Esumi, MD, PhD (7/1/2015 – 6/30/2017)
Johns Hopkins University
Resistance to Oxidative Stress: A New Strategy for AMD

"The goal of this project is to develop new strategies for prevention and treatment of AMD, more specifically to test a molecule that promotes stress resistance and longevity on the survival and integrity of the RPE, a critical cell for AMD."
www.brightfocus.org/grant/M2015220

Stefanie Hauck, PhD (7/1/2015 – 6/30/2017)
Helmholtz Zentrum Muenchen, Germany
Identification of Protein Complexes Binding to Genomic AMD Risk Variants

"As a result of this project, we expect to uncover novel pathways that are associated with AMD genetic risk factors and these may provide the basis for innovative preventive therapies."
www.brightfocus.org/grant/M2015370

Qiuhong Li, PhD (7/1/2015 – 6/30/2017)
University of Florida, Gainesville
Enhancing Endogenous Protective Pathways as a Therapeutic Intervention for AMD

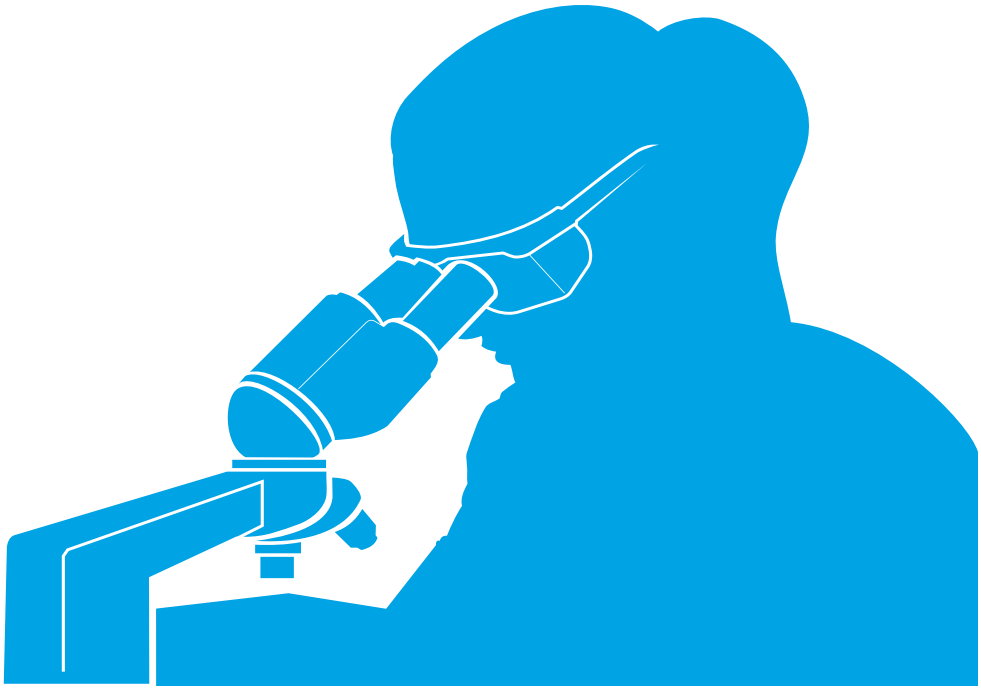
"The overall goals of this research are to study the protective mechanism of a new signaling pathway and its downstream target gene, and to test their therapeutic efficacy in animal models of AMD."
www.brightfocus.org/grant/M2015178

Omid Masihzadeh, PhD (7/1/2014 – 3/1/2017)
University of Colorado Eye Center
A Method to Study AMD Using a New Microscopic Technique

"In this proposal, we investigate the possibility of vivo imaging studies for AMD and ocular diseases in general."
www.brightfocus.org/grant/M2014014

Steven Nusinowitz, PhD (7/1/2015 – 6/30/2017)
Jules Stein Eye Institute, University of California, Los Angeles
Scotopic Critical Flicker Fusion in Preclinical AMD

"The main goal of this research project is to develop and test a novel method of evaluating retinal function in patients who are at risk of developing AMD, but who have not yet developed signs of the disease."
www.brightfocus.org/grant/M2015295

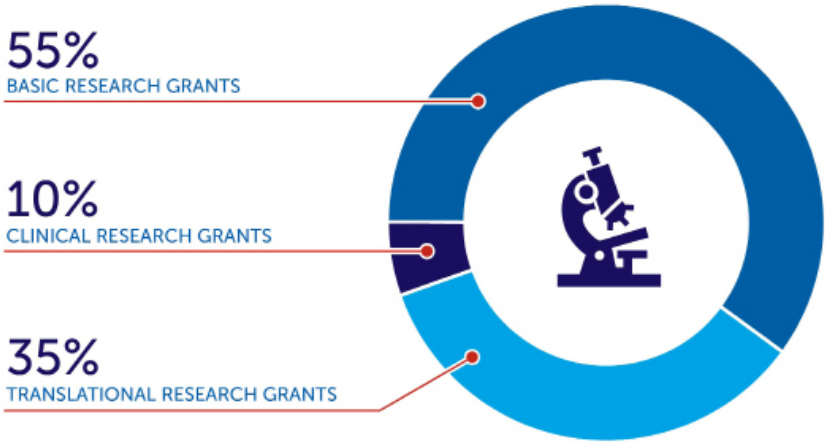


Sowing the Seeds of Scientific Progress

Thanks to our early support, most researchers go on to receive government and industry grants that, on average, are ten times larger than the original BrightFocus award,

a **1,000%** return on investment.

BrightFocus Grants at a Glance



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**Note: These are active Macular Degeneration Research grants as of 7/1/2016.*

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Foundation

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Macular Degeneration Research, a BrightFocus Foundation program