

## ANNUAL REPORT 2012





# esearch to Prevent Blindness

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#### On the cover: Survival of the cones



Rods and cones convert light into the image-forming signals that allow our brain to create vision. The rods are low-light photoreceptors, allowing us to perceive shapes in black and white, and to navigate in dimly lit conditions.

Cones, although only three to five percent of retinal photoreceptors, are responsible for daylight sight, color vision and visual acuity. They depend on rods for survival and when rods die from retinal degenerative diseases, such as retinitis pigmentosa, cones next to them die, too. RPB investigators are identifying the mechanism underlying this relationship and developing ways of either stimulating natural factors that promote cone survival or blocking the rod signals that initiate cone death. For patients with advanced retinal disease, RPB scientists are developing stem cell therapies to replace cones.

Front and back cover image: Clouds Hill Imaging Ltd / Science Source Images

#### LETTER FROM THE CHAIRMAN



In my two decades (and more) at Research to Prevent Blindness, I have never seen anything like the current acceleration in the pace of vision-saving discoveries. Fueled by the urgency of need and made possible by funding from RPB and others, advances in technology and the incredible persistence of risk-taking researchers have generated headline-grabbing breakthroughs that were dreams only a short time ago:

- improved vision;
- ocular melanoma, will spread to other parts of the body;
- The ability to pinpoint the moment when age-related macular degeneration goes from its dry form to wet, allowing the possibility that a drug can be developed to halt that progression;
- The intriguing finding that patients using statins to reduce risk of cardiovascular disease also had a reduced risk of glaucoma; and
- The identification of a compound that interrupts both mechanisms causing diabetic retinopathy.

But these discoveries do not tell the entire story. Unpublished updates from our currently funded 142 individual researchers and 55 departments of ophthalmology also are extremely exciting—including word that a gene therapy treatment for dry and wet age-related macular degeneration will soon enter early human trials.

Significant challenges remain, however, given the rapidly rising prevalence of eye diseases, largely due to an aging population. The huge numbers of retiring baby boomers entering our country's healthcare and financial safety net systems, while facing diminishing vision, create an urgency to even further accelerate vision research and treatment advances.

Unfortunately, the largest source of research funding and our greatest ally in the battle against blindnessthe National Eye Institute-has had to restrict access to grants as the fallout from the recession continues and the politically-determined limitation on federal "discretionary" spending, i.e., sequestration, forces further cutbacks in research spending.

It's clear that we will have to do a lot more to meet these urgent challenges. To address these issues, RPB is undertaking a re-visioning of its goals and strategies. As we enter into this process, we are joined by several new members of the Board of Trustees, and by Brian F. Hofland, our new President (about whom you can read more at www.rpbusa.org). Brian's experience in identifying and addressing key issues confronting the elderly makes him a natural fit for RPB at this critical time.

I have great confidence that RPB will not miss a beat as we attempt to move nimbly and creatively toward the prevention of blindness.

Diane S. Swift Chairman

• An experimental treatment for blindness, derived from a patient's skin cells, that

• A genetic test that predicts whether or not the most common form of eye cancer,

### LETTER FROM THE PRESIDENT



"The road ahead is always connected to the path already traveled by the point at which we find ourselves now."

-Anonymous

Since joining Research to Prevent Blindness earlier this year, I have been impressed with our "path already traveled." RPB has a rich history and strong track record of success over the past five decades, which is the story of the creation, growth and development of today's vision research community. So far, I have focused on gaining

a deeper understanding of the work that's going on at RPB-supported departments of ophthalmology at U.S. universities, the vision research that's taking place at the National Eye Institute, and the work of other organizations focused on treating diseases of the eye and preserving vision.

One characteristic that sets RPB apart from other vision organizations, yet complements their work, is our unique approach to grantmaking: by offering extremely flexible grants, RPB unfetters scientists to engage in the essential act of discovery.

This isn't a recent idea. In fact, it's an RPB founding principle, one that still guides our work today. This flexibility is also fundamental to maintaining vitality and productivity, and to providing gap support when other resources dwindle or disappear. Researchers tell us that an RPB grant provides freedom to pursue sudden discoveries, and to follow one's gut. The proof is in the pudding: an impressive 1,599 papers published in scientific journals cited RPB support in 2012.

That freedom to innovate, and the potential it clearly unleashes, is pretty powerful stuff. Which, of course, begs the question: Can we leverage our past successes to achieve even more, and be more strategic?

To develop "the road ahead" for RPB, over the next year we will reach out to, and engage, our stakeholders to identify the most pressing needs of vision researchers who may need even greater support, particularly given reduced federal and state resources, and a private sector still recovering from the Great Recession of the last five years. We also want to expand the growing body of information on the true costs of vision loss to individuals, caregivers, and a society that is rapidly aging. Concurrently, we need to know more about the capacity of U.S. vision care services to meet the needs of those with vision loss. Perhaps this last step-in transforming hope into help—will be our most important task.

Our goals will remain the prevention of blindness and the restoration of sight. But our future may well include new strategic alliances with our stakeholders and partners, and new tactical approaches, which we believe will increase our impact and contributions to this incredibly important and much needed work.

Brin J. Hopland

Brian F. Hofland, PhD President

#### ADVANCES IN EYE RESEARCH 2012

Scientists, like artists, are motivated by an inner drive to discover fundamental truths, to solve a problem, and to improve the human condition. To do their best work, they require an environment conducive to exploration. They work with an awareness of those who have come before them. They invent new tools or use existing tools in new ways to achieve their goals. They are inspired by colleagues and influenced by societal trends. From every generation, excellent scientists emerge.

Our tasks at RPB are to support vibrant research environments and to help vision scientists excel. The remarkable vision science discoveries in this annual report represent one measure of our success. But in evaluating the full impact of our endeavors, we also have to take into account the careers we transform and nourish, the thought leaders we help develop, and the future generations of vision researchers they mentor. That narrative follows in the form of three stories, which we have called *Insights*.



Institute, received RPB awards in 1975, '82, '89 and '96. As a member of RPB's Scientific



Doctors Maureen and Jay Neitz met as lab partners in an electron microscopy class in their last semester before graduate school. "We had discovered our passion for science at the time but not eye research," recalls Jay. "We fell in love and got married a little more than a year after our first date."

After graduation, PhDs in hand, they pooled their intellectual resourcescombining Maureen's expertise in molecular genetics with Jay's in neuroscience-to focus on the biological basis of visual perception. "We discovered the joy of being able to collaborate on truly translational research when we took our first faculty positions at the Medical College of Wisconsin," says Jay. "Maureen was offered a position in Ophthalmology and I eventually joined her there, where we were introduced to RPB."

Maureen: "I received an RPB Career Development Award, which really helped me to get launched. I was able to use it to purchase equipment and move into new technology."

Jay: "Working in Ophthalmology opened a new world of research opportunities for us. Suddenly we had ideas of ways our basic science work on vision could be translated into applications that could help people with vision disorders. Maureen's RPB money was instrumental in getting those first, more translational, experiments started, including discovering the genetic basis of common eye disorders, developing diagnostic tests and gene therapies. But having access to unrestricted departmental funds has also been important to us."

The Neitzes, whose goal is to develop a set of gene-based tools that can be useful in treating many human blinding disorders, have fully leveraged the flexibility of RPB's unrestricted grants. Their first step toward their goal came when they demonstrated that color blindness (which affects four percent of the population and eight percent of men) can potentially be cured through gene therapy.

"An RPB award says 'We believe in you. Now, go innovate,' " says Jay. "Once you innovate, then you have data with which to access other funding."

"But that funding, from the government and other sources, tends to be project-specific and restricted," adds Maureen. "There have been times when our most significant finding was unrelated to an NIH grant, and RPB was critical in allowing us to pivot and pursue

#### A PASSION FUELED BY RPB

The Neitzes are internationally recognized for developing a potential gene therapy cure for color blindness and are among the world's thought leaders on genetic and environmental factors that may be influencing a global epidemic of myopia.

Maureen Neitz, PhD, has received four RPB grants, a distinction achieved by only four other researchers. Jay Neitz, PhD, has received an RPB Senior Scientific Investigator Award. They have also received RPB unrestricted departmental support for more than 20 years, including the University of Washington School of Medicine in Seattle, their current home. "There's no way we'd be who we are today without RPB," says Maureen.

some of the really exciting things we are still working on."

"When you're working away in research, you are doing things that no one has ever done before and unexpected things come up," says Jay. "RPB's model makes eminent sense."

For years, the Neitzes have been examining genetic and environmental factors involved in extreme myopia (nearsightedness), which develops during childhood. Contemporary scientific literature holds that there are two main factors that influence risk of myopia: if both parents are myopic, and if a child spends little time outside. The forest as hunter-gatherers. "They Neitzes discovered that excessive close work (like reading, or working on something held close to the face) and exposure to the red light spectrum both activate the same

genes that cause the developing eyeball to elongate, which leads to myopia.

That finding helped explain why there is a higher prevalence of myopia in cultures where there is an emphasis on close work performed under artificial lighting (incandescent light is largely red), such as in Singapore—where 90 percent of high school graduates are myopic—and in many other Asian cultures.

Recently, the Neitzes were told by a colleague about an aboriginal Argentinian tribe that lives in the have no artificial lighting, they spend the entire day outside, they have no electricity and, genetically, they are related to other Asian peoples," says Jay. "They represented a

### ADVANCES IN EYE RESEARCH

perfect opportunity to study myopia in a people who carry the genes but are unaffected by today's artificial environments. To test their eyes, we identified a portable device, but we had no money to buy it with. So we went to our Chair, here in Seattle, and he allocated some RPB unrestricted funds for the purchase.'

Jay spent a month in the Argentinian jungle and found compelling evidence for the role played by environmental factors in myopia. Not a single tribe member was myopic.

Maureen, meanwhile, stayed back in the laboratory to push forward with a large scale DNA analysis that examines the association between lightsensitive protein mutations and myopia. "Jay loves the work in the field," says Maureen. "I love the lab work. We are perfectly matched."

#### ADVANCES IN EYE RESEARCH

#### Building a Foundation for **Future Breakthroughs**

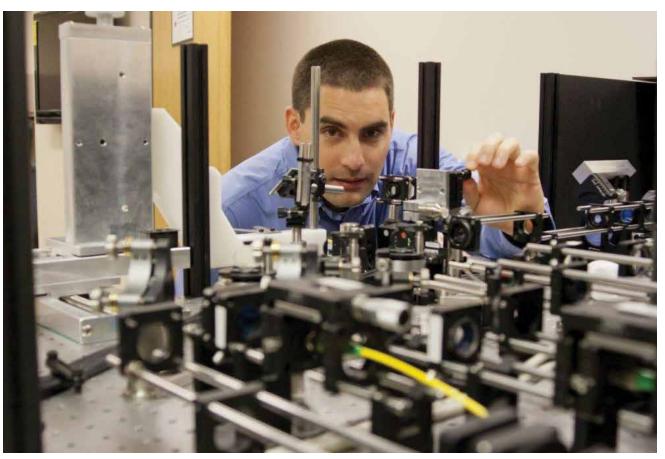
New treatments for eye diseases are built on basic science. Once the molecular pathways, genetic components and biological systems that cause dysfunction are understood, it may become possible to repair, replace or repress the problematic mechanism.

Among the many basic breakthroughs reported in 2012. RPB researchers:

• Confirmed the existence of a previously hypothesized fluid circulation system within the lens that delivers nutrients and removes waste, opening the possibility of enhancing it in order to delay the development of nuclear cataracts;

• Demonstrated a way to isolate stem cells from the corneas of patients, expand the cells in tissue culture, and inject them into patients' scarred corneas where they make opaque scar tissue clear again; and

• Provided evidence that enhancing the eye's immune response mechanism may help prepare the eye in advance for a potential infection, such as endophthalmitis, which is associated with cataract surgery and the injections used to treat age-related macular degeneration (AMD) and diabetic retinopathy.



Using a new imaging tool adapted from astronomy and built in his lab, 2011 RPB Career Development Awardee Alfredo Dubra, PhD, Medical College of Wisconsin, discovered a never-before-seen structure in glaucoma patients. Both the adaptive-optics scanning laser ophthalmoscope and the finding will be important for monitoring the progression of the disease.



Prior to receiving a 2012 RPB Career Development Award (see page 19), Sai H. Chavala, MD, used RPB unrestricted funds at the University of North Carolina at Chapel Hill to uncover the foundation for a promising new blood test to detect the earliest progression of macular degeneration from the dry form to its more serious wet form.

#### Translating Breakthroughs into **New Treatments**

Investigators announced a major advance in the field of vision restoration for patients with retinal degeneration: the creation of a chemical photo-switch that makes normally "blind" cells in the retina sensitive to light. The chemical eventually wears off, making it potentially safer than experimental gene or stem cell therapies, which permanently change the retina.

RPB scientists are working toward human clinical trials of a sustained gene therapy approach for the wet form of AMD. They have already shown that a single injection of a gene-carrying virus (engineered to produce an already existing treatment for AMD) can suppress the growth of leaky blood vessels for six months.

### ADVANCES IN EYE RESEARCH

Scientists studying the structure and function of eye muscles found that many cases of acquired strabismus (arising from tumors, trauma, infections and other defects) are due to degeneration of connective tissues, rather than neurological causes. They also showed that the resulting double vision can be corrected by novel surgeries involving only the dysfunctional portions of individual eye muscles.

In the course of developing biomaterials for cornea regeneration, an RPB Jules Stein Professor has created contact lenses that bind hyaluronic acid, a lubricant that not only helps the lens stay more hydrated but improves healing after surgery. Taking this concept a step further, she is now moving to apply the bonding strategy directly to the cornea's surface to combat dry eye.

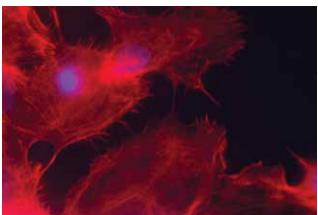
#### ADVANCES IN EYE RESEARCH



Microneedles less than a millimeter in length may soon become the preferred method to deliver therapeutics to diseased areas at the back of the eye. The new technology, developed in part with RPB support, is less invasive, more accurate, and more efficient than current methods.

Two teams of RPB investigators have discovered different ways to stimulate regeneration of nerves following corneal refractive surgeries and injuries. Currently, there are no FDA-approved products for this purpose. Loss of corneal nerves from trauma, infection, or inflammation can lead to corneal ulceration, perforation and blindness; restoring them helps maintain the health of the corneal surface and can reduce dry eye symptoms.

To help patients with chronic corneal disorders, an RPB researcher is developing a topical formulation of rapamycin, which he has shown can inhibit ocular surface fibrosis. The same



Cells near the healing edge of a corneal wound.

researcher has developed a technique for using human cadaver corneas as a scaffold to regrow a patient's own corneal cells for those whose immune systems have rejected multiple corneal transplants.

A nationwide study of more than 300,000 patients found that the risk for glaucoma was reduced by eight percent in patients who took statins continuously for two years, compared with patients who did not take statins. The study also suggested that statin use may be most important before glaucoma is diagnosed, or in the early stages of the disease, pointing toward a potential preemptive use of statins by groups at increased risk, including African-Americans, Hispanics and those with a family history of glaucoma.

#### Improving the Detection of Eye Disease

According to the World Health Organization and the American Academy of Ophthalmology, 80 percent of blindness can be prevented or cured ... if detected. For patients with progressive diseases, such as glaucoma, AMD, some corneal conditions and diabetic retinopathy, the sooner they are diagnosed and treated, the greater the likelihood that treatment can help retain vision.

In many cases, however, an eye disorder is discovered only after symptoms are severe enough to drive a patient to an eye care specialist ... and some vision has already been lost. With glaucoma, for example, it is estimated that there are nearly three million cases in the U.S. alone, but half of those people do not know they have the condition because there are no apparent symptoms. Early detection, then, is the Holy Grail of prevention and the focus of many RPBsupported initiatives.

Several RPB-supported labs reported inventive uses of advanced Optical Coherence Tomography (OCT, a technology that captures high-resolution, *(continued on page 12)* 

# SHAPING A LIFE

"I know I speak for every clinicianscientist who has received one of RPB's Career Development Awards when I say that RPB shaped my life and enhanced my career," says Natalie Afshari, MD, Professor of Ophthalmology, and Chief of Cornea and Refractive Surgery at the University of California San Diego, Shiley Eye Center.

Today, Dr. Afshari is an accomplished research scientist and clinician, widely sought by both patients and colleagues for her skills in cataract surgery, LASIK, complex corneal transplantations and other sophisticated treatments for diseases of the cornea. She has received grant support to study corneal disorders, and she is active in teaching and training the next generation of ophthalmologists.

"Within academic medicine, it is becoming more difficult to protect time for research," says Afshari. "One needs to see patients, train the next generation of physicians and perform research to reduce the burden of disease. An RPB grant allows you to be an intellectual, to be creative. It gives you the valuable time to think, to discover and to develop your talent."



The RPB Career Development Award is primarily intended as bridge funding for early-career scientists emerging from postdoctoral training who want to eventually obtain larger, federal grants.

"At that stage, you are too young to have an NIH grant—and, at the same time, you don't have enough preliminary data to use in an application," says Afshari. "The RPB grant literally allowed me to become a physician-scientist. It helped me grow by providing the time and funding to produce early data for an NIH grant. I was also blessed to have worked with two chairmen who encourage clinician-scientists.

"RPB grants are fabulous, because you can pursue untested ideas. In my case, I conducted multiple projects and published over 30 papers with that award. My primary investigation was on Fuchs endothelial corneal dystrophy [an inherited, progressive degeneration of the front of the eye that can cause vision loss]. One of my additional projects was to develop a potential new antifungal treatment for corneal infections which may cause blindness.

"The antifungals that are FDAapproved are too expensive for people in developing countries. There is a medicine used for amoebic infections of the eye. It's made from—you will not believe this—diluted pool cleaner and is well tolerated. Patients treated with it also show improvement in their fungal infection. So we tested it in the lab, and found that it was safe and effective.

"Around the world, doctors can dilute this pool cleaner to a safe strength, and treat fungal infections of the eye for less than ten cents per application. Surprising and great vision-saving discoveries can come from RPB support."

#### ADVANCES IN EYE RESEARCH

3-D images) to characterize structures within the eyes of patients in normal and diseased states. This allows investigators to create predictive models for-or to monitor-eye disease progression. For instance, one researcher indicated that OCT "will allow improved diagnosis of corneal stem cell loss, more accurate harvesting of stem cells for transplantation, and better monitoring of treatment."

Portability and miniaturization are also prominent in detection breakthroughs reported by RPB investigators. One group has helped develop a contact lens sensor which can provide continuous, intraocular pressure (IOP) measurements for 24 hours, including during undisturbed sleep, when IOP is believed to be highest in glaucoma patients—and the period of time that is hardest to track.



The contact lens sensor was safe and tolerable, and is awaiting U.S. approval.

Another group has developed a contrast sensitivity test to measure a patient's ability to differentiate between light and dark (an early indication of a retinal degenerative disease) that can be performed in even the remotest of locations, with an iPad, in 2-3 minutes. This development may prove to be timely in light of another discovery: that some of the earliest visual problems reported by glaucoma patients, such as difficulty functioning at night or under dark lighting conditions, may be due to increased eye pressure.

A test with an entirely different purpose was created by an RPB-supported researcher last year for patients with ocular melanoma. The test can

accurately identify those diagnosed with this cancer who have the often fatal, metastatic form, in which tumors spread from the eye to the liver. While some clinicians feel that there is no value in telling patients that death is inevitable, the researcher believes that patients should have the knowledge and be able to plan accordingly. The good news is that the test is based on the discovery of a new cancer gene involved in eye melanoma, which opens the door to potential new therapies.

#### Vision Loss and Depression: Can Alleviating One Treat the Other?

A recent study, conducted by the National Center for Health Statistics and the National Institutes of Health, revealed that more than one out of every ten U.S. adults who report vision loss has clinically meaningful symptoms of major depression.

This could prove problematic because other studies have drawn a connection between depression and patients' reduced adherence to treatments for chronic diseases, including diabetes and glaucoma, both of which can lead to permanent loss of sight if untreated. The implication is that depression itself, if untreated, may stand in the way of treating a chronic, progressive eye disorder.

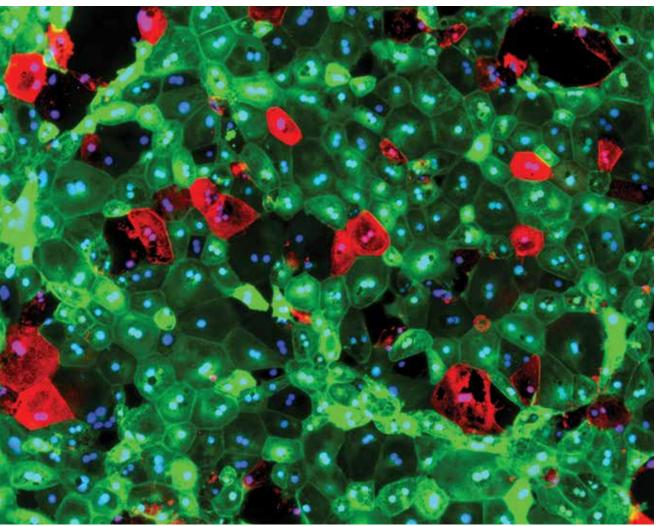
Fortunately, RPB-supported studies of the depression-vision loss connection have uncovered a subtlety that might offer solutions: while diminished sight can, indeed, limit one's ability to perform specific tasks, it is a person's self-reported sense of limitations that are connected to depression rather than actual measures of visual acuity. So, according to investigators, the presence of an eye disorder should alert eye care providers to the possible presence of depression, even if vision testing finds no medical cause for concern.

In fact, RPB researchers studying glaucoma and depression suggest that symptoms of

depression may be alleviated through low vision interventions that help patients improve function in glaucoma-impaired activities.

Both vision loss and depression affect quality of life, but does one cause the other? In an RPB study of veterans receiving eye care services, Dry Eye Syndrome (DES) was associated with depression and post-traumatic stress disorder. While it is known that certain medications used to treat psy-

#### Gene Therapy for Both Dry and Wet AMD Entering Human Clinical Trials



RPB researchers have developed a technique for injecting a gene-carrying virus into the clear gel that fills the eye, where it expresses a protein that blocks formation of damaging levels of Membrane Attack Complex (MAC). Normally, MAC attacks viruses and bacteria. In AMD patients, excessive MAC destroys retinal pigment epithelial cells (shown here in red, with their nuclei stained blue) which nourish the retina.

### ADVANCES IN EYE RESEARCH

chiatric conditions cause DES, the study's authors also found evidence of the possibility that depression and post-traumatic stress disorder may impact the health of the ocular surface. They concluded: "Regardless of the causal link, this suggests that individuals with a known psychiatric diagnosis should be questioned about dry eye symptoms and, if applicable, referred to an eye care physician."

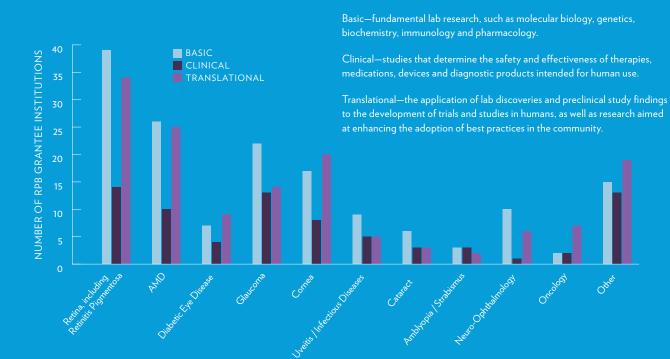
#### **RPB GRANTS PROGRAM**

RPB's grant support is available to those U.S. departments of ophthalmology with a demonstrated commitment to cutting-edge clinical, basic and translational research. The grants, in general, fall into two categories: unrestricted departmental grants (to be used at the discretion of the chairman) and awards to individual researchers.

Unrestricted grants to departments of ophthalmology represent a largely untold element of the RPB story. Roughly half of RPB's annual support is distributed

in the form of these unrestricted grants. Beyond the dollar amount of the Unrestricted Grant, its true value resides in the flexibility it provides: to support junior researchers who are seeking their first government grants; to amplify restricted grant work that is supported by other sources; to promote collaborations with other schools; and to explore surprising findings that arise in the midst of studies. In fact, there may be as many unique uses as there are RPB-supported medical schools.

#### **RPB-SUPPORTED RESEARCH REPORTED DURING 2012**



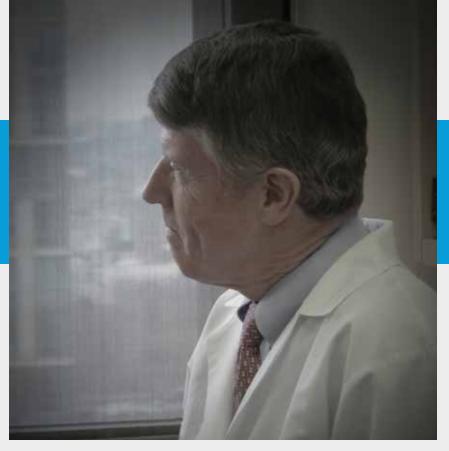
ln 2012, **RPB** provided \$5.71 million in unrestricted departmental support.

# A HOUSE THAT RPB BUILT

"This place is held together by RPB money," says Douglas Jabs, MD, MBA, referring to the uses he makes of RPB's annual \$110,000 Unrestricted Grant to the department of ophthalmology at Mount Sinai School of Medicine, where he has been Chairman since 2007. "We could not survive without it."

The RPB Unrestricted Grant is intended to provide opportunities for creative planning and maximum flexibility in developing and expanding eye research programs. "From a department chair's perspective, the entire RPB portfolio of grants generates success," says Jabs, who received three individual RPB grants prior to his chairmanship. "But that Unrestricted Grant to the department is essential to our ongoing endeavors.

"There are three ways we typically use the RPB Unrestricted Grant." he continues. "Bio-statistical support, equipment purchases, and bridge funds. I am of the opinion that every scientist can benefit from the power of statistical analysis. We are moving into an era where reviewers of grants are much more statistically sophisticated. So one way I use the RPB grant is to make sure that every researcher, for any paper produced by this depart-



ment, has access to help from our Statistical Consulting Service.

"Another reason I feel so strongly about the use of the grant for statistical support is the educational perspective. For example, something like 60 percent of ophthalmic literature incorrectly analyzes followup visual acuity. So it's important to educate residents in how to analyze their data. Let's face it, they are the future generation of researchers and manuscript reviewers.

"A further use of the grant is for strategic equipment purchases that multiple users can take advantage of, which allows us to leverage those purchases. Equipment is expensive. Research is expensive!

"But here's an example of why the departmental Unrestricted Grant is invaluable in today's environment, where funding rates are falling. The Fiscal Cliff created all sorts of

problems with National Eye Institute funding and now sequestration is going to force a further reduction by ten percent.

"We have a cadre of mature researchers in this department, some of them with labs they have developed across ten years or more, and it is critical to be able to support these skilled personnel if they need to recover from a temporary reduction in grant funding.

"If I'm going to provide bridge funding for a scientist for a year, I may need to support his or her lab technician, and that's going to be \$60,000 per year. If I support part of a statistician, that's going to be \$10-15,000 per year. You can see how quickly \$110,000 can be spent.

"I wish it could be more, because it is that important to what we do, but I am extremely grateful to have it."

#### 2012 RPB APPROVED GRANTS TOTAL: \$10,688,881

#### U.S. medical schools receiving new 2012 departmental and/or individual investigator awards

STATE	RPB GRANTEE INSTITUTIONS	TOTAL GRANTS 2012	TOTAL SUPPORT INCLUDING 2012
ALABAMA	University of Alabama at Birmingham School of Medicine	\$110,000	\$ 3,745,000
ARIZONA	University of Arizona College of Medicine	110,000	2,155,000
CALIFORNIĂ	University of California, Davis, School of Medicine	110,000	3,383,900
	David Geffen School of Medicine at UCLA	360,000	8,550,750
	University of California, San Diego, School of Medicine	110,000	3,175,000
	University of California, San Francisco, School of Medicine	460,000*	6,829,256
	Keck School of Medicine of the University of Southern California	390,000*	5,163,500
FLORIDA	University of Florida College of Medicine	110,000	3,595,600
	University of Miami Miller School of Medicine	110,000	4,285,200
GEORGIA	Emory University School of Medicine	110,000	3,597,100
ILLINOIS	Northwestern University Feinberg School of Medicine	200,000	2,670,000
	University of Illinois at Chicago	110,000	3,916,712
IOWA	University of Iowa Carver College of Medicine	210,000	4,242,425
KENTUCKY	University of Kentucky College of Medicine	360,000*	1,630,000
	University of Louisville School of Medicine	185,000	3,734,800
LOUISIANA	Louisiana State University Health Sciences Center in New Orleans	110,000	2,492,100
MARYLAND	The Johns Hopkins University School of Medicine	215,000	8,365,140
MASSACHUSETTS	Harvard Medical School	535,000*	8,107,315
	Tufts University School of Medicine	110,000	3,303,697
MICHIGAN	The Regents of the University of Michigan School of Medicine	460,000*	6,493,050
	Wayne State University School of Medicine	110,000	3,803,000
MINNESOTA	Mayo Medical School	110,000	2,944,600
	University of Minnesota, Academic Health Center, Medical School	110,000	3,038,701
MISSOURI	University of Missouri - Kansas City School of Medicine	220,000	220,000
	Washington University in Saint Louis School of Medicine	112,081	6,784,981
NEBRASKA	University of Nebraska Medical Center	110,000	1,750,000
NEW YORK	Albert Einstein College of Medicine of Yeshiva University Columbia University College of Physicians & Surgeons Weill Cornell Medical College Mount Sinai School of Medicine New York University University of Rochester School of Medicine & Dentistry SUNY at Buffalo School of Medicine & Biomedical Sciences SUNY Downstate Medical Center SUNY Upstate Medical University	260,000 110,000 110,000 250,000 110,000 110,000 30,000 110,000	1,867,500 4,603,167 4,433,700 3,958,200 2,177,250 2,645,250 790,000 250,000 2,520,000
NORTH CAROLINA	Duke University School of Medicine	296,800	6,530,150
	University of North Carolina at Chapel Hill School of Medicine	250,000*	1,520,500
OHIO	Case Western Reserve University School of Medicine	110,000	3,152,500
	Cleveland Clinic Lerner College of Medicine	885,000#	2,625,000
	University of Cincinnati College of Medicine	110,000	1,600,250
OKLAHOMA	University of Oklahoma Health Sciences Center	110,000	4,826,600
DREGON	Oregon Health & Science University School of Medicine	360,000*	4,482,150
PENNSYLVANIA	University of Pennsylvania School of Medicine	160,000	5,528,500
	University of Pittsburgh School of Medicine	210,000	4,188,372
SOUTH CAROLINA	Medical University of South Carolina College of Medicine	110,000	2,287,500
TENNESSEE	University of Tennessee Health Science Center	210,000	2,545,000
	Vanderbilt University School of Medicine	260,000	2,610,500
TEXAS	Baylor College of Medicine	140,000	4,244,060
	University of Texas Health Science Center at Houston	150,000	3,225,000
	University of Texas Southwestern Medical Center at Dallas	110,000	4,006,000
JTAH	University of Utah Health Sciences Center	210,000	5,125,300
WASHINGTON	University of Washington School of Medicine	185,000	3,447,638
WISCONSIN	Medical College of Wisconsin	110,000	4,224,215

\*Includes a four-year \$250,000 Research to Prevent Blindness Career Development Award, payable at the rate of \$62,500 per year.

#Includes a five-year \$625,000 Jules and Doris Stein Research to Prevent Blindness Professorship payable at \$125,000 per year and a \$150,000 Stein Professorship Laboratory renovation grant. A \$30,000 Medical Student Fellowship was declined following approval.

Schools that received RPB support but no new grants in 2012: University of California, Irvine, College of Medicine and West Virginia University.

Within the 55 schools that received unrestricted grant support in 2012, RPB also approved and funded 33 new grants to individual researchers. RPB only awards individual grants to researchers working with or within departments of ophthalmology that have been approved for unrestricted support.

"It's heartening to know that there's an organization that wants to make sure that vision is preserved and that people who could be at risk for blindness have help," says William T. Driebe, Jr., MD, Chair, Department of Ophthalmology, University of Florida College of Medicine. "RPB seeks to support institutions that demonstrate outstanding accomplishments in research. You can't do the research without funding."

#### HOW RPB GRANTS TO INDIVIDUALS WERE APPLIED IN 2012

OTHER (including drug delivery, imaging, epidemiology) S580,000 AMBLYOPIA / STRABISMUS \$125,000 NEURO-OPHTHALMOLOGY \$130,000 GLAUCOMA \$866,800 CORNEA \$477,081 DIABETIC EYE DISEASE \$280,000

#### NEW GRANTS

In 2012, RPB awarded \$4.7 million in grants to individual researchers.



### JULES & DORIS STEIN **RPB PROFESSORSHIP**

RPB's premier award is designed to foster translational research by recruiting outstanding basic scientists to conduct clinically relevant research in a department of ophthalmology. It provides \$625,000 over a five-year period, and offers a matching grant of up to \$150,000 to help renovate and equip lab space to be utilized by the Awardee. After the fourth year of funding, RPB will accept applications to extend support for an additional two years, bringing the total potential support to \$1,025,000.

Brian Perkins, PhD, Cleveland Clinic Lerner College of Medicine–Identifying new gene mutations that cause early onset retinal degeneration; using libraries of FDA-approved drugs to conduct high-throughput drug screens for compounds that reduce or prevent loss of vision in models of macular degeneration.

## JULES & DORIS STEIN RPB **PROFESSORSHIP EXTENSION**

David S. Williams, PhD, David Geffen School of Medicine, University of California, Los Angeles-Delivering large genes in gene therapy for Usher 1B; developing a live-cell imaging procedure to understand the process of photoreceptor protein transport.

### **RPB CAREER DEVELOPMENT AWARDS**

\$250,000 over four years for outstanding young clinical and basic scientists; a valuable recruiting tool for department chairs.

Brenda L. Bohnsack, MD, PhD, The Regents of the University of Michigan School of Medicine-Identifying and understanding the role of genes involved in the inherited childhood diseases Axenfeld-Rieger Syndrome and primary congenital glaucoma.

Yvonne Ou, MD, University of California, San Francisco, School of Medicine–Testing a novel hypothesis that retinal synapse loss occurs early in the glaucoma disease process in both the retina and brain.

Sai H. Chavala, MD, University of North Carolina at Chapel Hill School of Medicine–Streamlining the process of generating new, patient-specific retinal epithelial cells for age-related macular degeneration patients.

Joseph Bowers Ciolino, MD, Harvard Medical School–Advancing contact lens drug delivery.

J. Martin Heur. MD. PhD. Keck School of Medicine of the University of Southern California-Investigating the mechanism of cell cycle arrest in human corneal endothelial cells.

Mark E. Kleinman, MD, University of Kentucky College of Medicine–Understanding vascular and aging diseases of the eye.



### RPB WALT AND LILLY DISNEY AWARDS FOR AMBLYOPIA RESEARCH

Created through a pledge from The Walt and Lilly Disney Foundation, \$100,000 is allocated to one or more scientists for research into improved detection, treatment or cures for amblyopia, the leading cause of childhood vision loss, affecting two to four percent of U.S. children.

Michael Mustari, PhD, University of Washington School of Medicine–Examining the visual response properties of neurons in the extrastriate cortex of strabismus and amblyopia.

Howard S. Ying, MD, PhD, The Johns Hopkins University School of Medicine–Using high-speed, high-resolution video-oculography to detect small eye rotations to improve the diagnosis of amblyopia.

### NEW GRANTS



Mark E. Pennesi, MD, PhD, Oregon Health & Science University School of Medicine–Determining if selective serotonin reuptake inhibitors (SSRIs) and serotonin receptor modulators (SRMs) can prolong visual function in retinal degeneration.

#### NEW GRANTS

### **RPB SENIOR SCIENTIFIC** INVESTIGATOR AWARDS

\$150,000 to extend the productivity of seasoned vision scientists who can play a crucial role in training the next generation of vision scientists.

Nicholas E. Baker, PhD, Albert Einstein College of Medicine of Yeshiva University–Elucidating the contribution of cell competition to eye disease and its significance for cell transplantation and cell replacement procedures.

David J. Calkins, PhD, Vanderbilt University School of Medicine–Using novel inhibitors to rescue function and prevent neuronal tissue loss in glaucoma.

Reza Dana, MD, MPH, MSc, Harvard Medical School–Initiating new translational studies of the cellular and molecular markers of corneal transplant rejection in the setting of "high-risk" inflamed host beds.

Stephen C. Massey, PhD, University of Texas Health Science Center at Houston–Investigating the connections and electrical coupling of horizontal cells in the retina using new methods for multichannel confocal imaging.

### **RPB PHYSICIAN-SCIENTIST** AWARDS

\$100,000 to nationally recognized MDs who bring to the laboratory a practical understanding of patients' needs while their research efforts yield new knowledge in treating patients.



Arlene Drack, MD, University of Iowa Carver College of Medicine-Testing different vectors, expression levels and concentrations using induced pluripotent stem cells to develop treatments for Bardet Biedl Syndrome and other ciliopathies.

Alessandro Iannaccone, MD, MS, University of Tennessee Health Science Center–Identifying the types of autoimmune antibodies present in AMD patients to monitor the progression of the disease.

Thomas M. Lietman, MD, University of California, San Francisco. School of Medicine–Conducting a community-randomized trial in South India to see if corneal ulcer prophylaxis with antibiotic ointment can reduce or prevent corneal ulcers.

Joshua D. Stein, MD, MS, The Regents of the University of Michigan School of Medicine–Identifying the additional barriers to eye care —beyond limited access due to lack of health insurance—which vary by race, education level, and income.

### **RPB SPECIAL SCHOLAR** AWARDS

\$25,000 - \$75,000 given to promising young scientists of exceptional merit, in honor of former RPB trustees and others who have made generous contributions of time, energy and financial resources in support of eye research.

Qiutang Li, PhD, Ernest & Elizabeth Althouse Scholar Award - University of Louisville School of Medicine–Understanding the molecular network that controls corneal epithelial homeostasis and wound healing.

Xiaorong Liu, PhD, William & Mary Greve Scholar Award - Northwestern University Feinberg School of Medicine–Understanding how brain-derived neurotrophic factor prevents retinal ganglion cell degeneration in glaucoma.

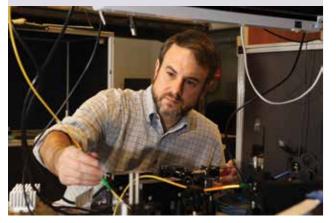
Goldis Malek, PhD, Sybil B. Harrington Scholar Award - Duke University School of Medicine-Identifying important signaling pathways involved in the clearance of lipids and extracellular matrix molecules found in deposits common in AMD.

Debasish Sinha, PhD, Sybil B. Harrington Scholar Award (Macular Degeneration) - The Johns Hopkins University School of Medicine–Elucidating phagocytosis impairment and identifying targets for treatment/prevention of retinal degeneration.

Tatjana C. Jakobs, MD, Dolly Green Scholar Award -Harvard Medical School–Understanding the stages of glial reactivity after injury to the optic nerve.

George D. Stetten, MD, PhD, University of Pittsburgh School of Medicine–Improving the ability of the ophthalmologic surgeon to see and feel structures by combining a new Optical Coherence Tomography guidance system and new Hand-Held Force Magnifier surgical tool platform into a single surgical environment for eye surgery.

Adam Wax, PhD, Duke University School of Medicine–Developing a novel in vivo optical imaging system that will quantify structural biomarkers of neurodegenerative disorders.



### NEW GRANTS

### **RPB INNOVATIVE OPHTHALMIC RESEARCH AWARDS**

\$100,000 to basic scientists (PhD or MD/PhD) actively engaged in out-of-the-box research in collaboration with the school's department of ophthalmology. This award is intended to bring basic science into ophthalmology and/or new collaborations between ophthalmology and other scientific disciplines.

Monica L. Vetter, PhD, University of Utah Health Sciences Center–Defining mechanisms driving retinal ganglion cell degeneration in glaucoma.

### **RPB RESEARCH** SABBATICAL GRANT

\$50,000 to mid-career researchers involved in educational and scientific programs that either enhance their scientific expertise or allow them to pursue a new ophthalmic research career path.

Joshua L. Dunaief, MD, PhD, University of Pennsylvania School of Medicine-Expanding knowledge and research direction of the complement biology field; increasing knowledge of genetics, neuroinflammation and neurodegeneration.

### **RPB INTERNATIONAL RESEARCH SCHOLAR AWARDS**

These travel grants enable foreign researchers to collaborate with U.S. colleagues.

Darryl R. Overby, PhD, traveling from Imperial College London to Duke University School of Medicine–Testing the hypothesis that a biomechanical "feedback loop" regulates intraocular pressure by modulating the resistance of aqueous humor outflow from the eye.

Morio Ueno, MD, PhD, traveling from Kyoto Prefectural University of Medicine to Washington University School of Medicine–Using stem cells to replace the corneal epithelium without the need for immuno-suppression.

### **RPB MEDICAL STUDENT EYE RESEARCH FELLOWSHIPS**

\$30,000 enables a student to take a year off to pursue a laboratory research project within a department of ophthalmology.

Meng Chen, Baylor College of Medicine–Revealing the pathway by which mucus production is regulated in normal eyes, in dry eye diseases, and in other body surfaces.

Jonathan Chou, Northwestern University Feinberg School of Medicine–Understanding blood flow and oxygen levels in the retinal circulation in early diabetic retinopathy.



Qianwen Dong, SUNY Downstate Medical Center–Determining the molecular mechanisms that lead to retinal ganglion cell and axonal loss in glaucoma.

Michele Lee, New York University School of Medicine–Measuring oxygen saturation of retinal vessels and the spectral signature of drusen to gain insight into the pathogenesis of retinal diseases.

Billy Pan, Keck School of Medicine of the University of Southern California–Predicting the progression of axonal loss within Leber's hereditary optic neuropathy nerves.

Terry Singhapricha, Duke University School of Medicine–Determining the impact of the immune system on age-related macular degeneration progression.

The RPB grant approval process is highly competitive. A standing Scientific Advisory Panel (SAP) and rotating Ad Hoc Committees convene each spring and fall to review all grant applications. Ad Hoc Committees are comprised of selected ophthalmology department heads whose recommendations are forwarded to the SAP for further evaluation. The SAP includes distinguished scientists representing a broad range of scientific disciplines and interests. Their recommendations are presented to the RPB Board of Trustees for final approval.

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Dr. Ryan passed away April 29, 2013. We will miss his warmth and wisdom.

#### SHEILA WEST. PhD

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RPB's mission is to preserve and restore vision by supporting research to develop treatments, preventives and cures for all conditions that damage and destroy sight.

