



Summer 2008

President's Message

NAEVR Advocacy Results in Real and Proposed Dollars for NIH, Serves as Model for Global Activities to Support Vision Research Funding

As you read this, Congress has returned for three weeks of work prior to the elections recess. Congress may not return after the November election, meaning that Fiscal Year (FY) 2009 appropriations will not be finalized until the 111th Congress convenes in early 2009. Prior to the election recess, Congress must pass a Continuing Resolution (CR) that funds the government into FY2009, which begins October 1. Whether the CR is stand-alone legislation or included in "must-pass" legislation, such as the FY2009 Defense appropriations bill, is currently unclear.

Our Congressional champions for medical research have been very active in securing real, additional dollars for the National Institutes of Health (NIH) in FY2008 beyond the initial appropriation—\$150 million in supplemental appropriations, with \$3.5 million for the National Eye Institute (NEI).

Senate Labor, Health and Human Services, and Education (LHHS) Appropriations Subcommittee Chair Tom Harkin (D-IA) and Ranking Member Arlen Specter (R-PA) have also introduced a bill for \$5.2 billion in additional FY2008 NIH funding, primarily to restore the 13 percent loss in purchasing power due to flat funding and biomedical inflation in the past five funding cycles. Both the House and Senate have proposed \$1 billion increases for the NIH in respective FY2009 appropriations bills, which also include

\$20 million-plus increases for the NEI.

I was fortunate to have met with Senator Harkin during his late May visit to Los Angeles to thank him for his efforts. I also hosted other champions, such as Cong. Pete Sessions (R-TX) and Cong. Gene Green (D-TX), at the June NAEVR/AEVR 15th Anniversary Capitol Hill reception. In addition to Members of Congress recognizing the Alliances' efforts, NIH Director Elias Zerhouni, M.D. also sent a letter of congratulations, which I read to attendees, and NEI Director Paul Sieving, M.D., Ph.D. spoke. As recently as mid-July in Senate testimony, Dr. Zerhouni cited the need for funding to build upon discoveries about the genetic basis of disease—such as the NEI-funded research identifying gene variants associated with age-related macular degeneration (AMD)—to develop appropriate prevention, diagnostic, and treatment strategies.

I was pleased to present a case study about NAEVR and AEVR at the recent World Ophthalmology Congress in Hong Kong, highlighting the effectiveness of the Alliances' message about the value of vision research with Congress, regulators, and coalition partners. In that regard, I am proud that NAEVR Executive Director James Jorkasky authored for the Association for Research in Vision and Ophthalmology (ARVO) an *International Advocacy Handbook: Tools to Influence Vision Research Funding*, which was released at the 2008 ARVO Annual Meeting in an interactive workshop that

Contributor Report



Left to right: Roberta Williams, M.D. (Chair, Department of Pediatrics, Keck School of Medicine at the University of Southern California) joined Senator Tom Harkin (D-IA) in discussions with Dr. Ryan about healthcare and medical research issues



Dr. Ryan speaks about the Alliances' effectiveness at the World Ophthalmology Congress in Hong Kong as Hugh Taylor, M.D. (University of Melbourne/Australia) looks on. Jim hosted. This resource, which is posted on the ARVO Web site, provides insights into influencing potential public and private sector funding source decision-makers nationally, regionally, and locally to support vision research.

As always, I thank you for your commitment to and support for the Alliances.

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National Alliance For
 Eye And Vision Research

Alliance For Eye
 And Vision Research

Members of Congress, NIH and NEI Directors Recognize AEVR/NAEVR

On June 9, AEVR and NAEVR celebrated their 15th anniversary with a reception in the foyer of the Rayburn House office building. At the event, NAEVR also released its updated joint fact sheet with Research!America entitled *Vision and Blindness: Investment in Research Saves Lives and Money*.

Cong. Pete Sessions (R-TX) provided an inspirational welcome, lauding the Alliances' 55 member organizations for their efforts to prevent blindness and save and restore vision. Earlier in the evening, Congressional Vision Caucus co-Chair Cong. Gene Green (D-TX) mingled with attendees, which included Congressional staff, administration representatives, coalition partners, and contributor organizations.

"I am pleased to recognize 15 years since the founding of AEVR and NAEVR and I applaud your unwavering efforts to preserve the nation's commitment to eye and vision research." NIH Director Dr. Elias Zerhouni

In opening comments, Dr. Stephen Ryan acknowledged a letter of congratulations from NIH Director Dr. Elias Zerhouni which read, in part, "I am pleased to recognize 15 years since the founding of AEVR and NAEVR and I applaud your unwavering efforts to preserve the nation's commitment to eye and vision research." NIH was represented at the event by Alan Krensky, M.D., Director of NIH's Office of Portfolio Analysis and Strategic Initiatives (OPASI), which manages the NIH Common Fund for trans-Institute research, and NEI Director Dr. Paul Sieving, who thanked the Alliances for their message about the value of the research emerging from the NEI. Dr. Ryan stated that, "During the past 15 years, we have seen dramatic breakthroughs in vision research emerging from the NEI, from understanding the genetic basis of eye disease to developing treatments that save and restore vision," adding that, as a

practicing ophthalmologist, "I can assure you that these therapies are having a fundamental impact on the productivity, independence, and quality of life of all Americans."

Research!America President Mary Woolley highlighted details from the fact sheet, stating that polling data consistently demonstrate that Americans want more spent on medical research, even if it meant an increase in their taxes. In introducing the last speaker, Hyman Shapiro, who is the featured patient on the fact sheet, James Jorkasky emphasized that the focus of the Alliances' work is ultimately patients with vision impairment and eye disease. Mr. Shapiro related his experience with AMD, noting his involvement in the Age-Related Eye Disease Study (AREDS), an NEI-

funded study which demonstrated that high levels of dietary anti-oxidants and zinc can reduce the risk of progression to advanced AMD by a factor of 25 percent. He is hopeful that dietary supplements, coupled with the new AMD treatments also emerging from NEI-funded research, can maintain his remaining vision.

The Alliances were founded in 1993 by the American Academy of Ophthalmology (AAO), the Association for Research in Vision and Ophthalmology (ARVO), and the Association of University Professors of Ophthalmology (AUPO).



Cong. Pete Sessions (R-TX) provides a welcome



Cong. Gene Green (D-TX, center) with NAEVR Advocacy Manager David Epstein (left) and AEVR/NAEVR Executive Director James Jorkasky (right)



Left to right: Craig Geist, M.D. (Ophthalmology Department Chair, George Washington University) and NEI Director Paul Sieving, M.D., Ph.D.



Left to right: Research!America President Mary Woolley and patient Hyman Shapiro in front of his featured profile on the joint RIA/NAEVR fact sheet *Vision and Blindness*



Left to right: ARVO Executive Director Joanne Angle and NIH's Alan Krensky, M.D. (Director, Office of Portfolio Analysis and Strategic Initiatives, OPASI)



Left to right: EyeSight Foundation of Alabama Executive Director Torrey Smitherman, Armand DeKeyser (MITA Group), and Dr. Ryan



Left to right: Blinded Veterans Association's Tom Zampieri, Ph.D., and Executive Director Tom Miller with NAEVR's David Epstein

Legislative Scorecard ISSUES

Congress Acts to Supplement FY2008 NIH Appropriations, Increase FY2009 Funding

	FY 2008* Final	FY 2009 Budget	FY 2009 House	FY 2009 Senate
NIH	\$29.38B	\$29.2B +0%	\$30.4B +3.9%	\$30.2B +3.5%
NEI	\$670.7M	\$667.8M +0.1%	\$690.7M +3.5%	\$687.3M +3.0%

* FY2008 figures include original appropriation sums of \$29.2B NIH / \$667.1M NEI plus Supplemental Appropriations of \$150M NIH / \$3.5M NEI. FY2009 Budget, House, and Senate percent increases based on initial FY2008 appropriations.



Senator Arlen Specter (R-PA) introduces S. 3272, an emergency appropriations bill to increase NIH funding by \$5.2 billion in FY2008



On June 24, Alliance for Aging Research Executive Director Dan Perry (left) was joined by Senator Tom Harkin (D-IA) in presenting Senate LHHS Appropriations Subcommittee staffer Erik Fatemi with the Florence S. Mahoney Making a Difference Award in recognition of public service to the medical research community

ENACTED: \$150 Million in FY2008 Supplemental NIH Appropriations

On June 30, President Bush signed into law a Supplemental Appropriations bill, passed overwhelmingly by the House (416-12) and the Senate (92-6), which included a science package of \$150 million in additional FY2008 NIH funding, as well as \$62.5 million for the National Science Foundation (NSF). The NEI's share of these supplemental funds is \$3.5 million, for an FY2008 total of \$670.7 million, or a 0.5 percent increase over FY2007. This will go primarily to fund R01 grants, about eight additional, as well as some Center grant and bridge award funding. Bipartisan Senate leaders, specifically Senate Labor, Health and Human Services, and Education (LHHS) Appropriations Subcommittee Chair Tom Harkin (D-IA) and Ranking Member Arlen Specter (R-PA), had directed these additional NIH resources to fund 246 research grants beyond that possible in initial FY2008 appropriations

PROPOSED: \$5.2 Billion in FY2008 Supplemental NIH Appropriations

On July 16, Senator Specter (on behalf of Senator Harkin) made a statement on the Senate floor introducing Senate Bill 3272, which would make emergency supplemental appropriations of \$5.2 billion for the NIH in FY2008 – \$4 billion would be provided to the Office of the NIH Director for prorating to Institutes and Centers, and \$1.2 billion

would go to the National Cancer Institute (NCI) to enhance cancer research. Senator Specter, who just completed a chemotherapy regime for Hodgkin's disease, noted that these emergency appropriations were necessary to restore NIH's purchasing power, which has been eroded by 13 percent due to flat funding and biomedical inflation over the past five funding cycles. Whether the supplemental is acted upon in the remaining days of the 110th Congress is currently unclear. However, it is a dramatic statement about the extent of loss of NIH's purchasing power that provides a basis for future actions to increase funding.

PROPOSED: FY2009 NEI Increases of \$23.6 Million (House), \$20.2 Million (Senate)

Per the funding chart, both the House and Senate LHHS Appropriations Subcommittees reported out FY2009 spending bills that would increase NIH funding by \$1 billion (inclusive of Global AIDS funding and other transfers) and \$20 million-plus increases for the NEI. Both bills match the biomedical inflation rate of 3.5 percent for increased funding overall for the NIH, which had been proposed to be flat-funded in the President's proposed FY2009 budget. The House bill, which is slightly more generous, has yet to be reported out of full Committee due to partisan disputes. Both bills are unlikely to be acted on until the 111th Congress convenes in early 2009.

NAEVR Builds Global Community of Support at 2008 ARVO Annual Meeting

From April 27-May 1, ARVO's United States members sent email letters to their Members of Congress from the NAEVR Contact Congress booth at the ARVO Annual Meeting. The timing was pivotal, as the House and Senate were finalizing a Budget Resolution and the respective LHHS Appropriations Subcommittees were soliciting input on FY2009 spending bills. NAEVR Advocacy Manager David

Epstein also spoke with researchers interested in submitting a grant application to the Department of Defense's (DOD) Peer Reviewed Medical Research Program (PRMRP), a \$50 million pool of funds for basic and translational research awards for which vision is eligible.

This year, the booth served as a focal point for building a global community of sup-

port for eye and vision research funding. NAEVR Executive Director James Jorkasky, who authored ARVO's *International Advocacy Handbook: Tools to Influence Vision Research Funding*, premiered this new resource document at an afternoon workshop, then met with interested ARVO members at the booth. ARVO has posted the *Handbook* and its illustrative appendices at www.arvo.org/advocacyhandbook.

ARVO Annual Meeting



Then-Incoming ARVO Board President Todd Margolis, M.D., Ph.D. (University of California at San Francisco) contacts Congressional offices he visited during ARVO's Advocacy Day in January 2008



NAEVR Advocacy Manager David Epstein assists Elaine Chuang, M.D. (University of Washington Eye Center) in sending an email letter



Left to right: Dr. Ryan listens as Peter Wiedemann, M.D., Ph.D. (University of Leipzig/Germany) reviews the just-released *Handbook* with James Jorkasky



In an interactive workshop, James Jorkasky discusses with attendees how to use the *Handbook*

In Senate Hearing, Dr. Zerhouni Describes How He Would Spend \$1 Billion Increase

On July 16, after an opening statement by Chairman Harkin, NIH Director Elias Zerhouni, M.D., testified before the LHHS Appropriations Subcommittee on NIH's accomplishments and challenges. He was joined by four Institute Directors: Francis Collins, M.D., Ph.D. (National Human Genome Research Institute); Anthony Fauci, M.D. (National Institute of Allergy and Infectious Diseases); Elizabeth Nabel, M.D. (National Heart, Lung and Blood Institute); and John Niederhuber, M.D. (National Cancer Institute). Dr. Zerhouni elaborated on previous testimony about NIH advances that are transforming the research and healthcare practice paradigm to one that is predictive, personalized, preemptive, and participatory. He noted that the almost-daily announcement of research findings about genes associated with specific diseases (including gene variants associated with AMD, which has emerged from the Human Genome Project), represents a "revolution of knowledge" that NIH must take advantage of by being flexible and adaptive. For example, he stated that NIH must follow up on these gene discoveries by: confirming the findings in large population-based studies; better understanding the complexity of the associated disease process and how the biology works to identify potential targets; and translating the discoveries into prevention, diagnosis, and treatment.

Regarding NIH's budget, Dr. Zerhouni stated that "medical research is a long-term process that does not operate on a twelve month cycle" and that predictable funding is necessary to sustain the research enterprise. He added that a reasonable success rate is also necessary to maintain the scientific infrastructure (for



NIH Director Elias Zerhouni, M.D.

the first time, the FY2009 NIH success rate is expected to fall under 18 percent), especially to encourage young and first-time investigators. Regarding the latter, when asked what NIH would do with a \$1 billion dollar increase, Dr. Zerhouni stated that the first priority should be funding young/first-time investigators by putting a "lock box" on a specific amount of appropriations. In so stating, he noted that "NIH should be funding at least 3,000 new scientists a year, and it is currently less than that" (FY2008 funding for new/first-time investigators is \$60 million, and proposed FY2009 funding is \$108 million). Other key NIH priorities with increased funding include: encouraging risk-taking/innovation in research; having adequate resources to conduct clinical trials; and encouraging collaboration across disciplines.

Although NEI Director Dr. Paul Sieving did not testify, NEI provided a written statement. In that regard, Chairman Harkin noted that it is the Subcommittee's intent to resume hearings with all Institute and Center Directors in the FY2010 appropriations process, as was done in the FY2008 process.

Full details about these events appear in the [NAEVR Web site Advocacy Center's section on NIH/NEI Appropriations and Defense-related Vision Research](#)

Capitol Hill Briefing Highlights Promising Human Gene Therapy Trial



Alliance For Eye
And Vision Research

AEVR

Educating about the Value of Vision Research



**Foundation
Fighting Blindness™**

Drive research to save and restore sight

On June 24, two top retinal researchers educated Congressional staff about their landmark clinical trial in which they used gene therapy to restore some vision in three young adults who were virtually blind from a severe form of retinitis pigmentosa known as Leber congenital amaurosis (LCA). Seventy people attended the briefing, which was hosted by AEVR and the Foundation Fighting Blindness (FFB), a nonprofit organization that funds research to cure retinal degenerative diseases and a NAEVR member organization.

Jean Bennett, M.D., Ph.D., the study's Scientific Director, and Al Maguire, M.D., the study's Principal Investigator, both of whom are at the University of Pennsylvania, provided details of their clinical study, which is taking place at the Children's Hospital of Philadelphia (CHOP). The researchers noted that critical funding from the NEI and FFB made their advancement possible.

NEI Director Dr. Paul Sieving also presented remarks, in which he called the researchers' work a "stunning outcome" and a great testament to the value of research funding. He added, "This effort is the tip of the iceberg. This validates the process of putting genes in the body for the purpose of restoring vision, liver function, heart function, and treating many other conditions." Initial results of the study were published in the *New England Journal of Medicine* on April 27, 2008. News of the advancement was carried by dozens of major media outlets around the world.

Seven years ago, Dr. Bennett came to Capitol Hill to share the results from a preclinical study of the same gene therapy, which was at the time successfully giving vision to dogs born blind from LCA. During her June 24 Hill visit, she said, "We predicted seven years ago at a simi-

lar venue that this approach could cure blindness in humans. We are thrilled to be here today to tell you that this prediction appears to be coming true." Dr. Maguire, a vitreoretinal surgeon, provided technical details of the study, including how the corrective gene is delivered to the retina using a therapeutic man-made virus or adeno-associated virus (AAV)—an approach that is also being used in studies of diseases such as muscular dystrophy.

Though the primary goal of the Phase I study at CHOP is to ensure safety of the treatment, Dr. Maguire noted that the patients' vision was also tested objectively and subjectively. After treatment, they were able to read several lines on an eye chart. They also had improved peripheral vision and better eyesight in dimly lit settings. Dr. Maguire said that he knew the treatment was working well when the patients asked to receive the therapy in their untreated eyes. The investigators will be treating LCA patients as young as eight years old. They believe the most dramatic results will be seen in young children.

In concluding the briefing, Dr. Sieving put historical perspective on the breakthrough. "The roots of this trial go back many years. A gene that causes this disease, RPE65, was found 15 years ago," he said. Dr. Sieving also noted that the discovery that vitamin A is essential for vision—a Nobel-Prize-winning finding made by George Wald, M.D.(hon), Ph.D., in the 1930s—was also a crucial stepping stone to the recent gene therapy advancement. "This is a very exciting time for all of medicine," he added. "Because this is a time when charitable and taxpayer dollars are being converted into treatments for people."

The research teams working on these projects also received funding from private sources Research to Prevent Blindness and Fight for Sight.



University of Pennsylvania researchers Jean Bennett, M.D., Ph.D., and Al Maguire, M.D., respond to attendee questions



NEI Director Paul Sieving, M.D., Ph.D., who is known internationally for his research into the genetic basis of retinal neurodegenerative diseases, provided an historical context to the current human gene therapy trials



Dr. Bennett and Dr. Maguire with Edward Gollob, a member of the Boards of Directors of both NAEVR/ AEVR and FFB



FFB Chief Research Officer Stephen Rose, Ph.D. (second left) is joined by representatives of the Blinded Veterans Association, including Tom Zampieri, Ph.D., Steve Matthews, and Claudia Perry



Dr. Bennett and Dr. Maguire met with Elizabeth King (right), Senior Legislative Assistant to Cong. Chaka Fattah (D-PA), in whose district the University of Pennsylvania is located. Cong. Fattah is a member of the House Committee on Appropriations



Left to right: Yewlin Chee and Lauren Stein, two students conducting research at University of Pennsylvania, speak with Norm Lanphear of the American Academy of Ophthalmology

NAEVR Joins the Vision Community in Requesting Vision Objectives in Healthy People 2020

At a May 28 Stakeholders Meeting held in Bethesda, Maryland, to solicit input on the objectives within Healthy People 2020, NAEVR's James Jorkasky presented a statement of support for the inclusion of vision, generally, and senior vision, specifically. Sponsored by the U.S. Department of Health and Human Services (DHHS), Healthy People identifies the most preventable threats to health and establishes national goals to reduce those threats. For the first time, Healthy People 2010 included vision objectives, and several vision community representatives attended the National-Capital area meeting to urge vision's continued inclusion in Healthy People 2020. In addition to public comment at the meeting, DHHS is also seeking written comments.

NAEVR's public comments focused on senior vision health, as 2020 is in the middle of the tidal wave of 78 million Baby Boomers who will be turning 65 between the years 2010 and 2028. NAEVR was joined in providing public comments by representatives of the American Optometric Association and the American Foundation for the Blind, with the American Academy of Ophthalmology and the Vision Council of America also in attendance and providing support. The vision community was a significant presence at this meeting, which drew participation from patient and advocacy groups representing a wide spectrum of diseases.



NAEVR's James Jorkasky comments on the importance of senior vision health



Norm Lanphear (American Academy of Ophthalmology) and representatives from the American Optometric Association, including Kelly Hipp, Timothy Wingert, O.D., (University of Missouri St. Louis College of Optometry), and Jeffrey Weaver, O.D., who provided public comment



NAEVR has posted on its Web site home page a link to *Your Candidates, Your Health*, a Web site developed by Research!America to enable voters to read more about candidates' positions on various health-related issues, including NIH funding.

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