The Association for Research in Vision and Ophthalmology
1801 Rockville Pike, Suite 400 ■ Rockville, Maryland 20852-5622
arvo@arvo.org ■ +1.240.221.2900 (Tel) ■ +1.240.221.0370 (Fax)
www.arvo.org

Iris M. Rush, CAE
Executive Director
Association for Research in Vision and Ophthalmology
Irush@ARVO.org or 240-221-2906

TESTIMONY SUPPORTING INCREASED FISCAL YEAR 2020 FUNDING FOR THE NATIONAL INSTITUTES OF HEALTH (NIH) AND NATIONAL EYE INSTITUTE (NEI)

LABOR, HEALTH AND HUMAN SERVICES, EDUCATION AND RELATED AGENCIES SUBCOMMITTEE OF THE SENATE COMMITTEE ON APPROPRIATIONS

June 1, 2019

EXECUTIVE SUMMARY

The Association for Research in Vision and Ophthalmology (ARVO), on behalf of its nearly 12,000 members—half in the US—thanks Congress, particularly the House and Senate Appropriations Subcommittees on Labor, Health and Human Services, and Education (LHHS), for strong bipartisan support for the National Institutes of Health (NIH) funding increases from Fiscal Year (FY) 2016 through FY2019. ARVO appreciates the $9 billion increase that has helped the agency regain ground lost after years of effectively flat budgets. For FY2020, ARVO urges Congress to appropriate at least $41.6 billion to the NIH, a $2.5 billion or 6.4 percent increase over the FY2019 funding level. This increase would allow for meaningful growth above inflation in the base budget to support promising science across all Institutes and Centers (I/Cs). Funding at this level would also ensure that dollars from the Innovation Account, established through the *21st Century Cures Act,* would supplement NIH's base budget, as intended, through dedicated funding for specific programs.

For FY2020, ARVO also encourages Congress to appropriate at least $850 million for the National Eye Institute (NEI), which is a $53 million or 6.4 percent increase over enacted FY2019. ARVO is concerned that the NIH and NEI budgets have not kept pace with biomedical inflation. For example, despite the total FY2016–2019 funding increases of $120 million, NEI's FY2019 enacted budget of $797 million is just 14 percent greater than the pre-sequester FY2012 budget of $702 million. When this amount is averaged over the past seven fiscal years, the 2 percent annual growth rate is less than the average annual biomedical inflation rate of 2.8 percent, thereby eroding the purchasing power and impact of these funds.

The NEI, which celebrated its 50th anniversary in 2018, is the world leader in sight-saving and vision-restoring research. We encourage Congress to ensure robust NEI funding that can help address the challenges of *The Decade of Vision 2010–2020*—as recognized by Congress in S. Res. 209 in 2009—which includes an aging population, disproportionate risk/incidence of eye disease in fast-growing minority populations, and the visual impact of numerous chronic diseases and their therapies.

Vision loss is costly to Americans. Investing in vision research to improve eye health can help to reduce healthcare costs and increase quality of life. The NEI's breakthrough research is a cost-effective investment that leads to treatments and therapies that delay, save and prevent

healthcare expenditures. The NEI has been a leader in genetics/genomics research and regenerative medicine. For example:

Genetics/Genomics:

- NEI's Glaucoma Genetics Collaboration Heritable Overall Operational Database (NEIGHBORHOOD) Consortium has identified 133 genetic traits that predict with 75 percent accuracy a person's risk for developing glaucoma related to elevated intraocular pressure (IOP). Among the 133 traits, 68 had not been previously linked to IOP or the relate cellular processes, such as lipid metabolism and mitochondrial function, that contribute to IOP. By understanding these cellular processes that can increase IOP and cause optic nerve damage, clinicians may be able to make an earlier diagnosis and researchers may be able to develop therapies to potentially halt disease progression.

- NEI-funded research has also led to the discovery of genetic defects underlying dozens of rare eye disease, including the discovery of RPE65. When mutated, this gene causes an inherited form of blindness called Leber congenital amaurosis (LCA). In 2017, based on NEI's initial efforts, the Food and Drug Administration (FDA) approved a gene therapy that cures this condition. These gene-based discoveries are forming the basis for new therapies that treat or prevent disease deemed untreatable until now.

Regenerative Medicine:

- In 2013, NEI launched its *Audacious Goals Initiative (AGI)* focused on regenerative medicine with the goal of restoring vision in blind patients that had lost portions of their retina. After initially asking a broad constituency of scientists within the vision research community to consider what could be done if researchers employed this new era of biology, the AGI currently funds major research consortia that are developing innovative ways to visualize the retina system. Researchers can now look at individual nerve cells in the eyes of patients in an examination room and directly observe whether new treatments are successful. Another consortium is identifying biological factors that allow damaged nerve cells of the retina to regenerate. The AGI is gathering considerable momentum with current proposals to develop disease models that may accelerate clinical trials for therapies within the next decade.

- NEI plans a first-in-human clinical trial that would test a stem cell-based therapy using a patient's own cells, so called induced pluripotent stem cells (iPSC), to treat the "dry" form of Age-related Macular Degeneration (AMD), the leading cause of vision loss among people age 65 and older. In this trial, a patient's own blood-making cells, called CD34+ cells, are converted to iPSCs and then programmed to become retinal pigment epithelial (RPE) cells. The RPE cells nurture the photoreceptors cells, which are necessary for vision and which die in AMD. The therapy replaces RPE cells, which have died due to AMD, with new, fully functioning, iPSC-derived RPE, thereby rescuing remaining photoreceptor cells and ultimately vision.

THE NATION'S INVESTMENT IN THE NEI RESULTS IN NEW THERAPIES TO TREAT MAJOR EYE DISEASES

Since President Lyndon Johnson signed legislation creating the NEI in 1968, the federal commitment has resulted in treatments and therapies for devastating diseases that cause vision loss. For example:

- AMD: The treatment of the "wet" form of AMD has made great strides resulting from use of Anti-Vascular Endothelial Growth Factor (VEGF) therapies. Last year, the NEI launched the AMD Ryan Initiative Study (ARIS), a prospective international study of patients that uses the latest advances in retinal imaging to identify biomarkers of the disease and targets for early therapeutic interventions.

- Diabetic Retinopathy: In the 1960s, about half of patients with diabetic retinopathy were blind within five years of diagnosis. NEI-sponsored clinical trials—starting in the early 1970s with the Diabetic Retinopathy Study and most recently with the Diabetic Retinopathy Clinical Research Network—have reduced the incidence of severe vision loss from diabetic retinopathy by 90 percent.

- Glaucoma: Eye pressure (IOP) appears to be the primary determining factor in the development of glaucoma for many patients. NEI research into primary open-angle glaucoma (POAG), the most common form of the disease, has resulted in two FDA-approved drug therapies in addition to those that have already emerged from NEI research. Targeting the eye's trabecular meshwork, which is a part of the eye responsible for reducing eye pressure by regulating fluid outflow from within the eye, the new generation of therapies reflects an expanding menu of drugs, potentially in combination with therapy, that lower IOP and better meet the needs of patients who do not respond to existing drugs or for whom older therapies have lost efficacy.

- Optical Coherence Tomography (OCT): Critical to the diagnosis and monitoring of treatments for the aforementioned eye diseases is an imaging technology called Optical Coherence Tomography (OCT). This non-invasive, high-resolution imaging technology displays a three-dimensional cross-sectional view of the layers of the retina. Developed over 25 years with $423 million in NIH and National Science Foundation (NSF) funding, OCT enables more personalized eye care and more efficient use of healthcare dollars. A December 2017 *American Journal of Ophthalmology* article reported that OCT saved Medicare $9 billion and patients $2.2 billion in co-pays by reducing unnecessary injections. As the technology continues to be applied to new medical conditions, such as Alzheimer's disease and Parkinson's disease, it supports a private commercial market of $1 billion and more than 16,000 high-paying jobs. These numbers suggest that in just this single area of research, the US government saw a 2,100% return on investment. https://www.ajo.com/article/S0002-9394(17)30419-1/fulltext

The next breakthroughs in technologies or treatments to save sight may be around the corner. Increased funding for the NIH and NEI in FY2020 may expedite the discovery of these technologies and treatments that will impact the lives of those facing vision loss and their families.

NEI's FY2019 enacted budget of $797 million is just 0.55 percent of the $145 billion annual cost (inclusive of direct and indirect costs) of vision impairment and eye disease, which was projected in a 2014 Prevent Blindness study to grow to $317 billion—or $717 billion in inflation-

adjusted dollars—by year 2050. Of the $717 billion annual cost of vison impairment by year 2050, 41 percent will be borne by the federal government as the Baby-Boom generation ages into the Medicare program. A 2013 Prevent Blindness study reported that direct medical costs associated with vision disorders are the fifth highest—only less than heart disease, cancers, emotional disorders, and pulmonary conditions. The U.S. is spending only $2.40 per person, per year for vision research, while the cost of treating low vision and blindness is at least $6,680 per person, per year. https://www.preventblindness.org/cost-vision-problems

In a May 2016 *JAMA Ophthalmology* article, NEI-funded researchers reported that the number of people with legal blindness will increase by 21 percent each decade to 2 million by 2050, while best-corrected visual impairment will grow by 25 percent each decade, doubling to 6.95 million people—with the greatest burden affecting those 80 years or older. http://jamanetwork.com/journals/jamaophthalmology/article-abstract/2523780?resultClick=1

In an August 2016 *JAMA Ophthalmology* article, the Alliance for Eye and Vision Research (AEVR, ARVO's educational foundation) reported that most Americans across all racial and ethnic lines describe losing vision as having the greatest impact on their day-to-day life. Other studies have reported that patients with diabetes who are experiencing vision loss or going blind would be willing to trade years of remaining life to regain perfect vision. http://jamanetwork.com/journals/jamaophthalmology/article-abstract/2540516?resultClick=1

NEI funding is also crucial to young scientists' research. Flat funding and cuts to NIH funding will impact their ability to move their careers and research forward. Recognizing the potential of young scientists, ARVO has focused efforts in recent years to support the next generation of scientists through travel grants to our annual meeting and awards to support research. Additionally, ARVO has created a Science Communication Training Fellowship to teach young scientists how to communicate about their science research to various audiences. It is crucial that these young scientists continue receiving support for their research through NEI grants and funding if they are to prevail as the next generation of leaders in vision research.

Please remember that investing in vision health is an investment in overall health. NEI's breakthrough research leads to treatments and therapies that may delay, save, and prevent healthcare expenditures. It can also increase productivity and improve the quality of life, as vision loss is associated with increased depression and accelerated mortality.

To support this important work, on behalf of the eye and vision research community, ARVO asks Congress to maintain the momentum of research that is vital to vision health, as well as overall health and quality of life and requests FY2020 NIH funding of at least $41.6 billion and NEI funding of $850 million.

About ARVO

ARVO's mission is to advance research worldwide into understanding the visual system and into preventing, treating and curing its disorders. Our members include nearly 12,000 researchers–with about half in the US–representing more 80 countries. This is done through meetings, education, partnerships, fellowships and programs that drive collaboration, innovation and the advancement of eye and vision science with a goal of saving sight. Learn more at www.ARVO.org