TESTIMONY SUPPORTING INCREASED FISCAL YEAR 2019 FUNDING FOR THE NATIONAL INSTITUTES OF HEALTH (NIH) AND NATIONAL EYE INSTITUTE (NEI)

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EXECUTIVE SUMMARY

NAEVR, on behalf of the vision community, thanks Congress for the $2 billion NIH funding increases in FY2016 and FY2017 and the $3 billion increase in FY2018. Congress is helping NIH to regain lost ground after years of effectively flat budgets that did not keep up with biomedical inflation, thereby reducing purchasing power. With the FY2018 increase, Congress continued to make progress in reversing those losses by providing a substantial increase to all NIH Institutes and Centers (I/Cs), in addition to dedicated funding through the 21st Century Cures Act and other funding devoted to specific programs.

In FY2019, NAEVR recommends at least $39.3 billion for the NIH, including funds provided through the 21st Century Cures Act for targeted initiatives. This funding level would continue the momentum of recent years by enabling meaningful base budget growth above inflation to expand NIH’s capacity to support promising science in all disciplines, and would also ensure that the Innovation Account supplements NIH’s base budget, as intended, through dedicated funding for specific programs.

NAEVR also recommends at least $800 million in FY2019 NEI funding. In 2018, NEI celebrates the 50th anniversary of its creation by Congress as the lead Institute for
our nation’s sight-saving and vision restoring research. Congress must ensure robust NEI funding to address the challenges of *The Decade of Vision 2010-2020*—as recognized by Congress in H. Res. 366 in 2009—including an aging population, disproportionate risk/incidence of eye disease in fast-growing minority populations, and the impact on vision of numerous chronic diseases. Despite recent NIH increases, NEI’s FY2018 enacted funding of $772.3 million is just ten percent greater than the pre-sequester FY2012 funding of $702 million. Averaged over the six fiscal years, the 1.6 percent annual growth rate is less than the average annual biomedical inflation rate of 2.8 percent, thereby eroding purchasing power. We must maintain the momentum of vision research since vision health is vital to overall health and quality of life. Since the United States is a world leader in vision research and in training the next generation of vision scientists, the very health of the global vision research community is at stake.

**NEI LEADS IN GENETIC AND REGENERATIVE MEDICINE RESEARCH**

As recently as March 21, 2018, during the NEI’s 50th Anniversary Congressional Reception, NIH Director Francis Collins, MD, PHD stated the following about the NEI:

“Due to the architecture, accessibility, and the elegance of the eye, vision research has always been a few steps ahead in biomedical research. Understanding the genetic basis of eye diseases has led the way for understanding the genetic basis of many common diseases.”

The NEI has been a leader in genetics/genomics research and regenerative medicine.

- Genetics/Genomics: Vision researchers have found more than 50 gene variants that cause a risk of developing age-related macular degeneration (AMD). For glaucoma, more than 16 genes have been identified. NEI support also made discoveries of dozens of rare eye disease genes possible, including the discovery of RPE65, which causes congenital blindness called Leber congenital
amaurosis (LCA). Just within the past year, NEI’s initial efforts have led to a commercialized, Food and Drug Administration (FDA)-approved gene therapy for this condition. These gene-based discoveries are forming the basis of new therapies that not only treat the disease, but may ultimately prevent it entirely.

- Regenerative Medicine: NEI is at the forefront of regenerative medicine with its Audacious Goals Initiative (AGI), which was launched in 2013 with the goal of restoring vision. Initially asking a broad constituency of scientists within the vision community and beyond 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 image the visual system. Researchers can now look at individual nerve cells in the eyes of patients in an examination room and learn quite directly whether new treatments are successful. Another consortium is identifying biological factors that allow neurons to regenerate in the retina. And the AGI is gathering considerable momentum with current proposals to develop disease models that may result in clinical trials for therapies within the next decade.

This year, NEI scientists on the NIH campus will launch the first-ever clinical trial in the U.S. to test tissues derived from induced pluripotent stem cells. Retina pigment epithelium—tissue in the back of the eye that supports the light-sensing cells in the retina—is being created in a lab starting with patient blood cells. These tissues, when mature, will be implanted in patients with AMD. The hope is that this will be enough to save dying cells and vision.
THE NATION’S INVESTMENT IN THE NEI IS RESULTING IN NEW THERAPIES TO TREAT MAJOR EYE DISEASES

Speaking after Dr. Collins at the March 21 Reception, NEI Director Paul Sieving, MD, PhD observed that:

“As we look back 50 years, we remember times when people had untreatable eye diseases. These included AMD, diabetic retinopathy, and glaucoma. These were blinding conditions, and doctors had little more than hope to offer patients.”

The federal commitment—made in 1968 when President Lyndon Johnson signed legislation creating the NEI—has made possible treatments and therapies for the very diseases that Dr. Sieving cited as previously resulting in blindness or severe vision loss:

- **AMD:** The treatment of the “wet” form of AMD has made great strides resulting from use of Anti-Vascular Endothelial Growth Factor (VEGF) therapies—which emerged from initial NIH-funded research—that stabilize vision loss and may improve lost vision. The NEI has established an AMD Pathobiology Working Group within its National Advisory Eye Council to evaluate knowledge learned from its extensive AMD portfolio and identify what is still uncertain, such as the relationship between genes and biological pathways, therapies for the more-prevalent “dry” form of the disease, and how to diagnose and treat the disease much earlier. The NEI has launched 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:** Over the span of 50 years, NEI has funded a number of randomized controlled trials (RCTs), which have led to major vision health improvements. In the 1960s, about half of patients with diabetic retinopathy were blind within five years of diagnosis. NEI-sponsored clinical trials—starting in the
1970s with the Diabetic Retinopathy Study and most currently with the Diabetic Retinopathy Clinical Research Network—have reduced the incidence of severe vision loss from diabetic retinopathy by 90 percent.

- **Glaucoma:** The FDA has approved two new drug therapies emerging from decades of NEI research into the role of high intraocular pressure (IOP) as a causal risk factor for primary open-angle glaucoma (POAG), the most common form of the disease and a leading cause of vision loss and blindness. Targeting the eye’s trabecular meshwork—which is one of the pathways responsible for regulating fluid flow within the eye—the new generation of therapies reflects an expanding menu of drugs that lower IOP and better meet the needs of patients.

Critical to the diagnosis and monitoring of treatments for these eye diseases is Optical Coherence Tomography (OCT), which is a non-invasive, high-resolution imaging technology that 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 has enabled better personalization of eye care to facilitate more efficient use of effective but costly drug therapies. 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.