Labor Health and Human Services, Education, and Related Agencies
Witness Disclosure Form

Clause 2(g) of rule XI of the Rules of the House of Representatives requires non-governmental witnesses to disclose to the Committee the following information. A non-governmental witness is any witness appearing on behalf of himself/herself or on behalf of an organization other than a federal agency, or a state, local or tribal government.

Your Name, Business Address, and Telephone Number:

Hendrik P.N. Scholl, MD, MA
The Dr. Frieda Derdeyn Bambas Professor of Ophthalmology
The Johns Hopkins University
[redacted]

1. Are you appearing on behalf of yourself or a non-governmental organization? Please list organization(s) you are representing.

National Alliance for Eye and Vision Research (NAEVR)

2. Have you or any organization you are representing received any Federal grants or contracts (including any subgrants or subcontracts) since October 1, 2008?

Yes No

3. If your response to question #2 is "Yes", please list the amount and source (by agency and program) of each grant or contract, and indicate whether the recipient of such grant or contract was you or the organization(s) you are representing.

N/A

Signature: [redacted] Date: March 23, 2012

NAEVR
National Alliance For
Eye And Vision Research
Serving as Friends of the National Eye Institute

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Hearing on Fiscal Year (FY) 2013 Budget Priorities for Labor, Health and Human Services, and Related Agencies Appropriations
before the
Subcommittee on Labor, HHS, and Education and Related Agencies
March 29, 2012
9 am

Testimony by Hendrik P.N. Scholl, M.D., M.A.
The Dr. Frieda Derdeyn Bambas Professor of Ophthalmology
Wilmer Eye Institute/Johns Hopkins University School of Medicine
Baltimore, Maryland
On behalf of the National Alliance for Eye and Vision Research

The National Alliance for Eye and Vision Research (NAEVR) requests Fiscal Year (FY) 2013 National Institutes of Health (NIH) funding at $32 billion and National Eye Institute (NEI) funding at $730 million. This funding recommendation represents the minimum investment necessary to avoid further loss of promising research and at the same time allows the NIH's budget to keep pace with biomedical inflation.

National Alliance for Eye and Vision Research (NAEVR)
Testimony in Support of Increased Funding for the National Institutes of Health (NIH) and National Eye Institute (NEI)
Labor, Health and Human Services, Education and Related Agencies Appropriations Subcommittee of the
U.S. House of Representatives Committee on Appropriations
March 29, 2012

Good morning, and thank you for the opportunity to appear today in support of appropriations for the National Institutes of Health (NIH) and the National Eye Institute (NEI). I am Hendrik Scholl, M.D. and I serve as The Dr. Frieda Bambas Professor of Ophthalmology at the Wilmer Eye Institute of the Johns Hopkins School of Medicine in Baltimore, Maryland.

I am representing the National Alliance for Eye and Vision Research (NAEVR), an Alliance of 55 member organizations representing professional societies in ophthalmology and optometry, patient and consumer groups, and industry. NAEVR serves as the "Friends of the National Eye Institute" and advocates for adequate funding for NEI's mission of saving and restoring vision.

I am here today to urge your support for a Fiscal Year (FY) 2013 NIH funding increase to a level of at least $32 billion, as well as an increase in NEI funding to a level of $730 million. This funding recommendation represents the minimum investment necessary to avoid further loss of promising research and at the same time allows the NIH's budget to keep pace with biomedical inflation.

I received my medical degree in Germany and did a fellowship in London, so I bring an international perspective to the need for adequately funding medical research, especially into blindness and vision impairment. The NIH has long held a unique role in the world as the driver of biomedical research, so it must be adequately funded. The President's FY2013 proposal to level-fund the NIH and cut NEI funding will jeopardize the ability of researchers to build upon breakthroughs resulting from past investment.

For example, the proposed FY2013 budget would cut the NEI's funding by $8.9 million to a level of $693 million, which is just slightly above the FY2009 level. That also results in a net $14 million loss in NEI funding since its highest funding level in FY2010, which translates into about 40 research grants−any one of which could hold the promise of curing a blinding eye disease. The proposed FY2013 NEI funding level of $693 million also represents just a little over one percent of the $68 billion that blindness and vision impairment costs the United States each year.

In 2009, both the House and Senate spoke volumes in passing resolutions that designated 2010-2020 as *The Decade of Vision,* in which the majority of the 78 million "Baby Boomers" will turn 65 years of age and face the greatest risk of aging eye disease. A cut, level funding, or even an inflationary increase is not sufficient for the NEI to meet the extraordinary vision challenges presented by this "Silver Tsunami."

I am a clinician-scientist who focuses on diseases of the retina, which is the light-sensitive back of the eye necessary for vision. My specialty is retinal degenerations, especially age-related macular degeneration, or AMD, which is the leading cause of vision loss in individuals over 60 years old, and has become the leading cause of vision loss overall in the industrialized world due to the aging of the population. Each year, 200,000 Americans develop advanced AMD, resulting in a loss of central vision and an inability to read, drive, and conduct activities of daily living.

Fifteen years ago, there was not a lot new in AMD research, but now it is one of the hottest areas. In June 2010, NIH Director Dr. Francis Collins testified before the House Energy and Commerce Committee, stating that:

> "Twenty years ago, we could do little to prevent or treat AMD. Today, because of new treatments and procedures based in part on NIH research, 1.3 million Americans at risk for severe vision loss over the next five years can receive potentially sight-saving therapies."

The NEI has been a leader in determining the genetic basis of eye disease. In fact, one-quarter of all genes discovered to-date have been associated with both common and rare eye diseases. The NEI has especially been a leader in the genetic basis of AMD. Let me provide two examples:

- NEI's AMD Gene Consortium, which consolidates 15 international Genome Wide Association Studies (GWAS) representing more than 16,900 patients, has identified 19 genomic loci associated with AMD, including 7 new loci. The Consortium could identify 5 distinct biological pathways of disease mechanism. By understanding the genetic basis of the disease and the underlying disease mechanism, NEI can develop appropriate diagnostic and therapeutic applications.

- NEI's successful human gene therapy clinical trial for the neurodegenerative disease Leber Congenital Amaurosis (LCA), as well as subsequent refinement of gene therapy techniques, has enabled it to launch similar clinical trials for AMD and other retinal degenerations.

More than 38 million Americans age 40 and older experience blindness, low vision, or an age-related eye disease such as AMD, glaucoma, diabetic retinopathy, or cataracts. This number is expected to grow to more than 50 million Americans by the year 2020.

In public opinion polls over the past 40 years, Americans have consistently identified fear of vision loss as second only to fear of cancer. Patients with moderate to severe vision loss would trade years of remaining life for perfect vision. For example, patients who are legally blind due to diabetes would be willing to trade up to 36 percent of their remaining life to regain perfect vision.

In summary, NAEVR requests NEI funding at $730 million, reflecting biomedical inflation plus modest growth commensurate with that of NIH overall, since our nation's investment in vision health is an investment in overall health. NEI's breakthrough research is a cost-effective investment, since it is leading to treatments and therapies that can ultimately delay, save, and prevent health expenditures, especially those associated with the Medicare and Medicaid programs. It can also increase productivity, help individuals to maintain their independence, and generally improve the quality of life, especially since vision loss is associated with increased depression and accelerated mortality.

ABOUT NAEVR

The National Alliance for Eye and Vision Research (NAEVR), which serves as the "Friends of the NEI," is a 501(c)4 non-profit advocacy coalition comprised of 55 professional (ophthalmology and optometry), patient and consumer, and industry organizations involved in eye and vision research. Visit NAEVR's Web site at www.eyeresearch.org.

BIOGRAPHICAL SKETCH

NAME	POSITION TITLE
Hendrik P.N. Scholl, MD, MA	Dr. Frieda Derdeyn Bambas Professor of Ophthalmology
eRA COMMONS USER NAME (credential, e.g., agency login) hscholl1	

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	MM/YY	FIELD OF STUDY
University of Tübingen Medical School	M.D.	1989	Medicine
University of Tübingen	M.A.	1989	Philosophy
Brown University Medical School, Providence, RI		1994	Medicine
University Eye Hospital Tübingen	Residency	1997	Ophthalmology
Moorfields Eye Hospital, Institute of Ophthalmology, UCL, London	Fellowship	2001	Retina
mibeg Institute, Cologne	Health Manager	2009	Health Management

B. Positions

12/1/2011 – present: ***The Dr. Frieda Derdeyn Bambas Professor of Ophthalmology***, Wilmer Eye Institute, Johns Hopkins University

3/1/2004 – present: ***Professor of Ophthalmology***, Wilmer Eye Institute, Johns Hopkins University
Head of the Visual Neurophysiology Service

10/1/2004 – 2/14/2010: *Privatdozent* (***Associate Professor***), Medical Faculty, University of Bonn
Consultant Ophthalmologist, ***Vitreoretinal Surgeon*** and ***Research Coordinator***, Dept. of Ophthalmology, University of Bonn
Head of the Clinical Electrophysiology Unit
Head of the Low-Vision-Center
Coordinator and Supervising Ophthalmologist of the Specialized *Clinic Inherited Retinal and Macular Diseases* (7/31/2006 - 2/14/2010)
Head of the Clinical Trial Center „European Vision Institute – Clinical Trials – Sites of Excellence“ (3/1/2008 – 2/14/2010)

Selected Recent Honors:

2002: Elfriede-Aulhorn Award of the German Neuro-Ophthalmological Society
2003: Macula Degeneration Award 2003 of Pro Retina Germany
2004: Heisenberg Fellowship, German Research Foundation (DFG)
2008: KROENER Research Award of the German Ophthalmological Society
2008: European Vision Award 2008
2010: Enhanced Career Development Award, National Neurovision Research Institute (NNRI) / Foundation Fighting Blindness (FFB)
2010: Macular Degeneration Research Award of the American Health Assistance Foundation (AHAF)

C. Selected peer-reviewed publications *from 93* as of January 4, 2012:

Rivera A, White K, Stohr H, Steiner K, Hemmrich N, Grimm T, Jurklies B, Lorenz B, **Scholl HPN**, Apfelstedt-Sylla E, Weber BHF (2000) A comprehensive survey of sequence variation in the ABCA4 (ABCR) gene in Stargardt disease and age-related macular degeneration. Am J Hum Genet 67: 800-813.

Scholl HPN, Kremers J, Vonthein R, White K, Weber BHF (2001) L- and M-cone driven electroretinograms in Stargardt's macular dystrophy-Fundus flavimaculatus. Invest Ophthalmol Vis Sci 42: 1380-1389.

Scholl HPN, Schuster AM, Vonthein R, Zrenner E (2002) Mapping of retinal function in Best macular dystrophy using multifocal electroretinography. Vision Research 42: 1053-1061.

Scholl HPN, Besch D, Vonthein R, Weber BHF, Apfelstedt-Sylla E (2002) Alterations of slow and fast rod ERG signals in patients with molecularly confirmed Stargardt disease type 1. Invest Ophthalmol Vis Sci 43: 1248-1256.

Scholl HPN, Kremers J (2003) Alterations of L- and M-cone driven ERGs in cone and cone-rod dystrophies. Vision Research 43: 2333-2344.

Bellmann C, Neveu MM, **Scholl HPN**, Hogg CR, Rath PP, Jenkins S, Bird AC, Holder GE (2004) Localized retinal electrophysiological and fundus autofluorescence imaging abnormalities in maternal inherited diabetes and deafness. Invest Ophthalmol Vis Sci 45: 2355-2360.

Scholl HPN, Peto T, Dandekar S, Bunce C, Xing W, Jenkins S, Bird AC (2003) Inter- and intra-observer variability in grading lesions of age-related maculopathy and macular degeneration. Graefes Arch Clin Exp Ophthalmol 241: 39-47.

Scholl HPN, Bellmann C, Dandekar SS, Bird AC, Fitzke FW (2004) Photopic and scotopic fine matrix mapping of retinal areas of increased fundus autofluorescence in patients with age-related maculopathy. Invest Ophthalmol Vis Sci 45: 574-583.

Dandekar SS, Jenkins SA, Peto T, **Scholl HPN**, Sehmi KS, Fitzke FW, Bird AC, Webster AR (2005) Autofluorescence imaging of choroidal neovascularization due to age-related macular degeneration. Arch Ophthalmol 123: 1507-1513.

Schmitz-Valckenberg S, Bindewald-Wittich A, Dolar-Szczasny J, Dreyhaupt J, Wolf S, **Scholl HPN**, Holz FG for the Fundus Autofluorescence in Age-Related Macular Degeneration Study Group (2006) Correlation between the Area of Increased Autofluorescence Surrounding Geographic Atrophy and Disease Progression in Patients with AMD. Invest Ophthalmol Vis Sci 47: 2648-2654.

Charbel Issa P, Helb HM, Rohrschneider K, Holz FG, **Scholl HPN** (2007) Microperimetric assessment of patients with type 2 idiopathic macular telangiectasia. Invest Ophthalmol Vis Sci 48: 3788-3795.

Holz FG, Bindewald-Wittich A, Fleckenstein M, Dreyhaupt J, **Scholl HPN**, Schmitz-Valckenberg S, FAM-Study Group (2007) Progression of Geographic Atrophy and Impact of Fundus Autofluorescence Patterns in Age-related Macular Degeneration. Am J Ophthalmol 143: 463-472.

Charbel Issa P, Helb HM, Holz FG, **Scholl HPN** (2007) Correlation of macular function with retinal thickness in nonproliferative type 2 idiopathic macular telangiectasia. Am J Ophthalmol. 145: 169-175.

Charbel Issa P, Holz FG, **Scholl HPN** (2007) Findings in fluorescein angiography and optical coherence tomography after intravitreal bevacizumab in type 2 idiopathic macular telangiectasia. Ophthalmology 114: 1736-1742.

Scholl HPN, Fleckenstein M, Charbel Issa P, Keilhauer C, Holz FG, Weber BHF (2007) An update on the genetics of age-related macular degeneration. Mol Vis 13: 196-205.

Scholl HPN, Charbel Issa P, Walier M, Janzer S, Pollok-Kopp B, Börncke F, Fritsche LG, Chong NV, Fimmers R, Wienker T, Holz FG, Weber BHF, Oppermann M (2008) Systemic complement activation in age-related macular degeneration. PLoS ONE 3(7): e2593.

Fleckenstein M, Charbel Issa P, Helb HM, Schmitz-Valckenberg S, Finger RP, **Scholl HPN**, Löffler KU, Holz FG (2008) High resolution spectral domain-OCT imaging in geographic atrophy associated with age-related macular degeneration. Invest Ophthalmol Vis Sci 49: 4137-4144.

Scholl HPN, Fleckenstein M, Fritsche LG, Schmitz-Valckenberg S, Göbel A, Adrion C, Herold C, Keilhauer CN, Mackensen F, Mößner A, Pauleikhoff A, Weinberger AWA, Mansmann U, Holz FG, Becker T, Weber BHF (2009) CFH, C3 and ARMS2 are significant risk loci for susceptibility but not for disease progression of geographic atrophy due to AMD. PLoS ONE 4: e7418.

Schmitz-Valckenberg S, Fleckenstein M, Helb HM, Charbel Issa P, **Scholl HPN**, Holz FG (2009) In-vivo imaging of foveal sparing in geographic atrophy secondary to age-related macular degeneration. Invest Ophthalmol Vis Sci. 50: 3915-21.

Finger RP, Charbel Issa P, Ladewig MS, Götting C, Szliska C, **Scholl HPN**, Holz FG (2009) Pseudoxanthoma elasticum. Genetics, clinical manifestation and therapeutic approaches. Surv Ophthalmol. 54: 272-285.

Schmitz-Valckenberg S, Fleckenstein M, **Scholl HPN**, Holz FG (2009) Fundus autofluorescence and progression of age-related macular degeneration. Surv Ophthalmol. 54: 96-117.

Charbel Issa P, Finger RP, Holz FG, **Scholl HPN** (2009) Multimodal imaging including spectral domain OCT and confocal near infrared reflectance for characterisation of outer retinal pathology in pseudoxanthoma elasticum. Invest Ophthalmol Vis Sci. 50: 5913-8.

Fleckenstein M, Adrion C, Schmitz-Valckenberg S, Göbel AP, Bindewald-Wittich A, **Scholl HPN**, Mansmann U, Holz FG for the FAM Study group (2009) Concordance of disease progression in bilateral geographic atrophy due to AMD. Invest Ophthalmol Vis Sci 51: 637-42.

Helb HM, Charbel Issa P, Fleckenstein M, Schmitz-Valckenberg S, **Scholl HPN**, Holz FG (2010) Confocal SLO angiography and fundus autofluorescence imaging combined with high-speed, high-resolution, spectral domain optical coherence tomography. Acta Ophthalmol Scand. 88: 842-9.

Hecker LA, Edwards AO, Ryu E, Tosakulwong N, Baratz KH, Brown WL, Charbel Issa P, **Scholl HPN**, Pollok-Kopp B, Schmid-Kubista KE, Bailey KR, Oppermann M (2010) Genetic control of the alternative pathway of complement in humans and age-related macular degeneration. Hum Mol Genet. 19: 209-15.

Charbel Issa P, Troeger E, Finger R, Holz FG, Wilke R, **Scholl HPN** (2010) Structure-function correlation of the human central retina. PLoS One. 22;5(9): e12864.

Finger RP, Charbel Issa P, Kellner U, Schmitz-Valckenberg S, Fleckenstein M, **Scholl HPN**, Holz FG. (2010) Spectral domain optical coherence tomography in adult-onset vitelliform macular dystrophy with cuticular drusen. Retina 30: 1455-64.

Troeger E, Sliesoraityte I, Charbel Issa P, **Scholl HPN**, Zrenner E, Wilke R (2010) An integrated software solution for multi-modal mapping of morphological and functional ocular data. Conf Proc IEEE Eng Med Biol Soc. 2010: 6280-6283.

Finger RP, Fimmers R, Holz FG, **Scholl HPN** (2011) Prevalence and causes of registered blindness in the largest federal state of Germany. Brit J Ophthalmol. 95: 1061-7.

Finger RP, Fimmers R, Holz FG, **Scholl HPN** (2011) Incidence of blindness and severe visual impairment in Germany - projections for 2030. Invest Ophthalmol Vis Sci. 52: 4381-9.

Weismann D, Hartvigsen K, Lauer N, Bennett KL, **Scholl HPN**, Charbel Issa P, Cano M, Tsimikas S, Skerka C, Superti-Furga G, Handa JT, Zipfel PF, Witztum JL, Binder CJ (2011) Complement Factor H binds malondialdehyde-epitopes and protects from oxidative stress. NATURE 478: 76-81.

C. Selected Research Support

FUNDS FROM EUROPEAN COMMISSION (EU), FEDERAL MINISTRY FOR EDUCATION AND RESEARCH (BMBF) or GERMAN RESEARCH FOUNDATION (DFG):

European Commission: Integrated Project EVI-GENORET "Functional Genomics of the Retina in Health and Disease" (EU FP6 LSHG-CT-2005-512036), 4/1/2005 - 3/31/2009; Role: **Co-Investigator/Subproject Leader** "Phenotyping, tissue and DNA collection in patients with age-related macular disease"

European Commission: Concerted Action Project PHOTAGE „Photoreceptor Dynamics in Age-Related Macular Degeneration. Consequences for early diagnosis" (EU FP5 QLRT-2001-02494), 1/1/2004 - 12/31/2006; Role: **PI**.

European Commission: Collaborative Project "EuroVisionNet" (EU FP7 HEALTH-2007-200641), 4/1/2008 - 3/31/2011; Role: **Co-Investigator/Subproject Leader** "The Ageing Eye".

Federal Ministry for Education and Research, Germany (BMBF): Molecular Diagnostics of AMD (MODIAMD; BMBF MBT_011), 7/1/2009 - 6/30/2011; Role: **Co-Investigator**.

FUNDS FROM INDUSTRY/FOUNDATIONS:

National Neurovision Research Institute (NNRI) / Foundation Fighting Blindness (FFB)
Project Period: 03/01/2010 – 03/01/2013 *Enhanced Career Development Award*. Purpose: Structure-Function Correlation in Inherited Retinal Degenerations: Development of Surrogate Endpoints for Clinical Trials. Role: **PI**.

American Health Assistance Foundation (AHAF)
Project Period: 04/01/2010 – 04/01/2012 *Macular Degeneration Research Award*. Purpose: Structure-Function Correlation in Macular Degeneration. Role: **PI**.

Lowy Medical Research Institute, Sydney
Project Period: 01/01/07 – 12/31/07 *Investigator-Initiated Trial (IIT).* Purpose: Microperimetric assessment of patients with Macular Telangiectasia Type 2. Role: **PI**.

Lowy Medical Research Institute, Sydney
Project Period: 01/01/07 – 12/31/07 *Investigator-Initiated Trial (IIT).* Purpose: Fundus autofluorescence imaging and macular pigment distribution in Macular Telangiectasia Type 2. Role: **PI**.

NOVARTIS, Basel
Project Period: 01/01/08 – 12/31/08 *Investigator-Initiated Trial (IIT).* Purpose: Ranibizumab to Treat Type 2 Idiopathic Macular Telangiectasia (RAMA-Trial; ClinicalTrials.gov number, NCT00504400; Paul-Ehrlich-Institute, CRFB002ADE04; EudraCT number 2006-006233-40). Role: **Co-PI**.

NOVARTIS, Basel
Project Period: 01/01/08 – 12/31/08 *Investigator-Initiated Trial (IIT).* Purpose: Ranibizumab to Treat Choroidal Neovascularizations in Patients With Pseudoxanthoma Elasticum (ClinicalTrials.gov number, NCT00510965; Paul-Ehrlich-Institute, CRFB002ADE03; EudraCT number 2006-006233-49). Role: **Co-PI**.