

## Plenary Lecture

*Human Ocular Gene Therapy: Considerations from X-linked Retinoschisis*



**Paul Sieving, M.D., Ph.D.**  
Director, National Eye Institute  
National Institutes of Health  
Bethesda, Md.

Paul Sieving, M.D., Ph.D., is director of the National Eye Institute, National Institutes of Health. After graduate studies in nuclear physics at Yale University, he attended Yale Law School, and then received his medical degree and doctoral degree in bioengineering from the University of Illinois. He performed his ophthalmology residency under Morton F. Goldberg, M.D., at the University of Illinois Eye and Ear Infirmary, and was a post-doctoral fellow in retinal physiology with Roy Steinberg at University of California, San Francisco

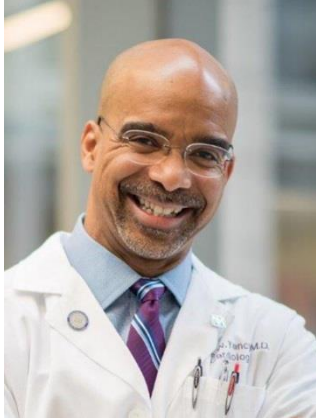
Dr. Sieving joined the faculty of the University of Michigan and held the Paul R. Lichter Chair in Ophthalmic Genetics. He founded the Center for Hereditary Retinal and Macular Degenerations at U-M and established the first Clinical Laboratory Improvement Amendments-certified laboratory in the United States for Ophthalmic Molecular Diagnostics for hereditary retinal dystrophies. He holds elected membership in the National Academy of Medicine USA and the German National Academy of Sciences.

He originated the “NEI Audacious Goals Initiative,” a 15-year effort in human regenerative medicine to replace photoreceptors and retinal ganglion cells lost to disease. He continues clinical and research engagement as a tenured senior investigator in the NIH Intramural Research Program.

He is known internationally for studies of human retinal neurodegenerative diseases, termed retinitis pigmentosa and has published 260 peer-reviewed papers. Dr. Sieving has worked extensively on X-linked retinoschisis (XLRS). He created a transgenic XLRS mouse model (IOVS 2004) and demonstrated that XLRS is a synaptic disease with direct involvement of the rod-to-bipolar synapse. He used gene therapy to deliver a normal RS1 gene into eyes of XLRS mice, reversing the synaptic pathology and closing the retinal schisis cavities. These pre-clinical studies culminated in his successful U.S. Food and Drug Administration submission for an Investigational New Drug Application to initiate a human RS1 gene therapy trial for human XLRS subjects (2015) now underway at the NEI.

## Plenary Lecture

### *Heart Failure in African Americans; a Puzzle Resolved*



**Clyde Yancy, M.D., M.Sc.**

Vice Dean of Diversity and Inclusion  
Magerstadt Professor of Medicine  
Professor of Medical Social Sciences  
Chief of Cardiology  
Northwestern University Feinberg School of Medicine  
Chicago, Ill.

Clyde Yancy, M.D., M.Sc. is chief of Cardiology at Northwestern University, Feinberg School of Medicine, and associate director of the Bluhm Cardiovascular Institute at Northwestern Memorial Hospital. He holds the Magerstadt Endowed Professor of Medicine Chair and also holds an appointment as Professor of Medical Social Sciences. He concurrently serves as vice dean of Diversity and Inclusion, Northwestern University, Feinberg School of Medicine.

The Louisiana native earned a bachelor's degree (honors) from Southern University, a medical degree (Alpha Omega Alpha) from Tulane University School of Medicine and an master's of science degree from the University of Texas - Dallas School of Business and Management (Beta Gamma Sigma). He completed an internship and residency in Internal Medicine at Parkland Memorial Hospital in Dallas. He completed his fellowship in cardiology at the University of Texas Southwestern Medical Center at Dallas. He has held professional appointments at the University of Texas Southwestern Medical Center (professor of Medicine, medical director- Heart Failure and Heart Transplantation and Carl Westcott Chair in Cardiovascular Research) and Baylor University Medical Center (chief of Cardiothoracic Transplantation and director of the Baylor Heart and Vascular Institute).

He is a former president of the American Heart Association and has held several volunteer leadership positions with the American College of Cardiology. He has also served in various positions with the National Institutes of Health, the National Heart, Lung, and Blood Institute, the Patient-Centered Outcomes Research Institute and the U.S. Food and Drug Administration. He has more than 450 peer-reviewed publications, has been named one of the most highly cited investigators and is a deputy editor for JAMA Cardiology. In 2016, he was elected to the National Academy of Medicine.

## Plenary Lecture

### *Tuning Depression Circuits Using Deep Brain Stimulation*



**Helen Mayberg, M.D.**

Director of the Center for Advanced Circuit Therapeutics  
Icahn School of Medicine at Mount Sinai  
New York, N.Y.

Helen Mayberg, M.D. is professor of Neurology, Neurosurgery, Psychiatry and Neuroscience, and the Mount Sinai Professor in Neurotherapeutics at the Icahn School of Medicine. She was recruited to New York after 14 years at Emory University in Atlanta, where she was professor of Psychiatry, Neurology and Radiology, and the Dorothy Fuqua Chair in Psychiatry Neuroimaging and Therapeutics.

Her research has characterized neural systems mediating major depression and its recovery, defined imaging-based illness subtypes to optimize treatment selection and introduced the first use of deep-brain stimulation for treatment-resistant patients.

Dr. Mayberg received a bachelor's degree in Psychobiology from the University of California, Los Angeles, and a medical degree from the University of Southern California. She completed her Neurology residency at the Neurological Institute of New York, and fellowship training in nuclear medicine at Johns Hopkins. She is a member of the National Academy of Medicine, the National Academy of Arts and Sciences, and the National Academy of Inventors, and has written more than 200 publications. She participates in a wide variety of advisory and scientific activities across multiple fields in neuroscience.

Dr. Mayberg is renowned for her study of brain circuits in depression and for her pioneering deep-brain stimulation research, which has been heralded as one of the first hypothesis-driven treatment strategies for a major mental illness. She is the founding director of Mount Sinai Health System's Center for Advanced Circuit Therapeutics, which advances precision surgical treatments for neuropsychiatric disorders through the rapid conversion of neuroscience and neuroengineering innovations that correct brain circuit abnormalities to restore mood as well as motor and cognitive functioning.

# Plenary Lecture

## *Lymphocytes as a Drug for the Treatment of Cancer*



**Steven Rosenberg, M.D., Ph.D.**

Chief of the Surgery Branch

National Cancer Institute of the National Institutes of Health  
Bethesda, Md.

Steven Rosenberg, M.D., Ph.D., is chief of the Surgery Branch at the National Cancer Institute and professor of Surgery at the Uniformed Services University of Health Sciences and at the George Washington University School of Medicine and Health Sciences.

He received a bachelor's degree and medical degree at The Johns Hopkins University, a doctoral degree in Biophysics at Harvard University. After completing his residency in surgery in 1974 at the Peter Bent Brigham Hospital in Boston, Dr. Rosenberg became chief of Surgery at the National Cancer Institute.

Dr. Rosenberg pioneered the development of immunotherapy that resulted in the first effective immunotherapies for selected patients with advanced cancer. His studies of cell transfer immunotherapy resulted in durable complete remissions in patients with metastatic melanoma. He pioneered the development of gene therapy and was the first to successfully insert foreign genes into humans. His studies of the adoptive transfer of genetically-modified lymphocytes resulted in the regression of metastatic cancer in patients with melanoma, sarcomas and lymphomas.

He received the Meritorious Service Medal from the U.S. Public Health Service in 1981 and 1986, the Friedrich Sasse Prize from the University of West Berlin in 1986, the Nils Alwell Prize from Stockholm in 1987, the Distinguished Alumnus Award from The Johns Hopkins University in 1987, the Griffuel Prize for Research from the French Association for Research on Cancer in 1988 and the Milken Family Foundation Cancer Award in 1988. He received the Armand Hammer Cancer Prize "for pioneering work in cancer research" in 1985 and 1988. In 1991, he received the Karnofsky Prize, the highest honor given by the American Society of Clinical Oncology. He received the Flance-Karl Award, the highest honor given by the American Surgical Association in 2002 and in 2003 received the annual prize for scientific excellence in medicine from the American-Italian Cancer Foundation. He also has received the Richard V. Smalley, M.D., Memorial Award, the highest honor given by the International Society for Biological Therapy of Cancer; the Karl Landsteiner Prize from the American Association of Blood Banks, the Keio Medical Science Prize, the Massry Prize and the Medal of Honor from the American Cancer Society.

A member of the American Society of Clinical Oncology, he is also a member of the National Academy of Medicine, the Society of University Surgeons, the American Surgical Association, the American Association for Cancer Research and the American Association of Immunologists. Dr. Rosenberg has written more than 1,100 articles on cancer research and eight books.

## Plenary Lecture

*Why Don't We Get More Cancer?: The Importance of Extracellular Matrix and Organ Architecture*



**Mina Bissell, Ph.D.**

Distinguished Scientist, Biological Systems and Engineering  
Lawrence Berkeley National Laboratory  
University of California, Berkeley  
Berkeley, Calif.

Mina Bissell, Ph.D. is a Distinguished Scientist, the highest rank bestowed at Lawrence Berkeley National Laboratory, and serves as senior advisor to the Laboratory Director on Biology. She is a faculty member of four graduate groups at University of California, Berkeley: Comparative Biochemistry, Endocrinology, Molecular Toxicology and Bioengineering. The breast cancer research pioneer's body of work has provided much impetus for the current recognition of the significant role that extracellular matrix signaling and microenvironment play in gene expression regulation in both normal and malignant cells. Her laboratory developed novel 3D assays and techniques that demonstrate her signature phrase: after conception, "phenotype is dominant over genotype."

Dr. Bissell earned her doctoral degree in microbiology and molecular genetics from Harvard Medical School, won an American Cancer Society fellowship for her postdoctoral studies, and soon after joined LBNL. She was founding director of the Cell and Molecular Biology Division and associate laboratory director for all Life Sciences at Berkeley Lab, where she recruited outstanding scientists and developed a strong program in cell and molecular biology and breast cancer. She has more than 400 publications. Her honors include the U.S. Department of Energy's E.O. Lawrence Award, the American Association for Cancer Research's G.H.A. Clowes Memorial Award, the Pezcoller Foundation-AACR International Award, the Susan G. Komen Foundation's Brinker Award, the Breast Cancer Research Foundation Jill Rose Award, Berkeley Lab's inaugural Lifetime Achievement Prize, the American Cancer Society's Medal of Honor, the MD Anderson Cancer Center's highest honor – the Ernst W. Bertner Award, the Honorary Medal from the Signaling Societies in Germany, the American Society for Cell Biology's highest honor – the E.B. Wilson Medal, and the 2017 AACR Award for Lifetime Achievement in Cancer Research.

The University of Porto, Portugal, established the Mina J. Bissell Award, given every three years to a person who has dramatically changed a field. She is the recipient of honorary doctorates from both Pierre & Marie Curie University in Paris, France, and University of Copenhagen in Denmark. Dr. Bissell is an elected fellow of most U.S. honorary scientific academies, including the National Academy of Sciences, the National Academy of Medicine and the American Philosophical Society. She sits on many national and international scientific boards and continues to engage in full-time research.

# Plenary Lecture

## *Big Data and Health*



**Michael Snyder, Ph.D.**

Chair of the Department of Genetics  
Stanford W. Ascherman Professor  
Director, Center for Genomics and Personalized Medicine  
Stanford University  
Stanford, Calif.

Michael Snyder, Ph.D. is the Stanford Ascherman Professor and Chair of Genetics and the director of the Center of Genomics and Personalized Medicine at Stanford University. He received his doctoral training at the California Institute of Technology and carried out postdoctoral training at Stanford University. He is a leader in the field of functional genomics and proteomics, and is one of the major participants of the Encyclopedia Of DNA Elements, or ENCODE project.

His study was the first to perform a large-scale functional genomics project in any organism, and he has developed many technologies in genomics and proteomics. These including the development of proteome chips, high-resolution tiling arrays for the human genome, methods for global mapping of transcription factor binding sites (ChIP-chip now replaced by ChIP-seq), paired end sequencing for mapping of structural variation in eukaryotes, de novo genome sequencing of genomes using high-throughput technologies and RNA-Seq. These technologies have been used for characterizing genomes, proteomes and regulatory networks.

Seminal findings from the Snyder laboratory include the discovery that much more of the human genome is transcribed and contains regulatory information than was previously appreciated, and a high diversity of transcription factor binding occurs both between and within species.

He has combined state-of-the-art “omics” technologies to perform the first longitudinal detailed integrative personal omics profile (iPOP) of person and used this to assess disease risk and monitor disease states for personalized medicine. He is a cofounder of several biotechnology companies, including Protometrix (now part of Life Technologies), Affomix (now part of Illumina), Excelix, Personalis and Q Bio. He serves on the board of a number of companies.