

2008 Albert Lasker Award Recipients

The 2008 Albert Lasker Award for Basic Medical Research was awarded to scientists Victor R. Ambros, PhD, David C. Baulcombe, PhD, and Gary B. Ruvkun, PhD, "for discoveries that revealed an unanticipated world of tiny RNAs that regulate gene function in plants and animals." Drs. Ambros and Ruvkun discovered the first example of this molecule type in the worm *Caenorhabditis elegans.* In a study that was initially focused on how the worm develops from a newly hatched larva into an adult, the scientists discovered a gene that codes for microRNAs and can turn down gene expression. Dr. Baulcombe subsequently established that the process of small RNA gene-silencing also occurs in plants. Together, these discoveries opened the door for further study of such RNAs in a broad range of living things. The work also led to identification of biochemical mechanisms that unify the processes by which small RNA regulate gene activity and to the belief that microRNAs may regulate one-third of the genes in the human body. Thus, many research efforts to better understand a variety of diseases as well as therapies now include the study of microRNAs.

Dr. Ambros serves as a Professor at the University of Massachusetts Medical School. He received his PhD from MIT in 1979 and completed his postdoctoral research at the Massachusetts Institute of Technology (MIT), where he also earned his undergraduate and graduate degrees. He joined the faculty at Harvard from 1984 until 1992, at which time he accepted a faculty position at Dartmouth. It was in his lab at Dartmouth that he first discovered a microRNA, known as lin-4. Dr. Ambros has earned numerous honors and awards, including the 2005 Lewis S. Rosentiel Award for Distinguished Work in Basic Medical Research - an award he shared with Dr. Ruvkun and 2006 Nobel Laureates, Andrew Z. Fire, PhD, and Craig C. Mello, PhD. He was awarded the 2006 Genetics Society of America Medal for outstanding contributions in the field of genetics in the past 15 years and was a co-recipient of the 2002 Newcomb Cleveland Prize from the American Association for the Advancement of Science. Dr. Ambros became an elected member of the national Academy of Sciences in 2007.

Dr. Ruvkun is Professor of Genetics at Harvard Medical School. He earned his AB in Biophysics from the University of California at Berkeley and PhD in Biophysics from Harvard University. During his postdoctoral work, Dr. Ruvkun began to work with *C. elegans* with Nobel Prize Winners Bob Horvitz at MIT and Walter Gilbert at Harvard, where he collaborated with Dr. Ambros on the molecular analysis of the heterochronic genes. He has earned numerous awards and honors for his work, including the National Institutes of Health (NIH) Merit Award, the aforementioned Rosenstiel Award, and—along with Dr. Ambros—the 2007 Warren Triennial Prize at Massachusetts General Hospital and the 2008 Gairdner International Award. Along with both Drs. Ambros and Baulcombe, he was also awarded the Franklin Institute 2008 Benjamin Franklin Medal.

Professor of Botany and Royal Society Research Professor in the Department of Plant Sciences at the University of Cambridge in Cambridge, United Kingdom, Dr. Baulcombe earned his BS in botany at Leeds University and his PhD in botany from the University of Edinburgh. Prior to establishing a research group at the Plant Breeding Institute in Cambridge, Dr. Baulcombe completed postdoctoral work at McGill University in Montreal, Canada, and at the University of Georgia in Athens, GA. In 1988 he joined the Sainsbury Laboratory, where he did much of his world-renowned work. His honors and awards include the 2006 Royal Medal from The Royal Society, the University of Southern California Massry Foundation 2005 Massry Prize (shared with Dr. Fire and Dr. Mello), and the Rockefeller University 2003 Wiley Prize in Biomedical Science (shared with Dr. Fire, Dr. Mello and Thomas Tuschl, PhD). Dr. Baulcombe was elected as a Foreign Associate Member of the National Academy of Sciences, USA, in 2005. He is also an elected member of the Academia Europaea (2002), Fellow of the Royal Society (2001) and European Molecular Biology Organization (2001). He is a past president of the International Society of Plant Molecular Biology and recipient of the Royal Netherlands Academy of Arts and Sciences 2004 MW Beijerinck Virology Prize.

NIH Grants $138 Million for Director's Pioneer & New Innovator Awards in 2008

The National Institutes of Health (NIH) announced that 47 scientists will receive funds estimated at up to $138 million over 5 years as part of the 2008 Director's Pioneer and New Innovator Awards. The awards are designed to support high-impact research efforts and to enable scientists to employee innovative

approaches, and awardees are often in the early stages of their careers. While scientists at any career level may receive Pioneer Awards, only those investigators who have not held an NIH regular research (R01) or similar NIH grant are eligible for the New Innovator Awards.

The Pioneer Award program, which provides $2.5 million in direct costs over 5 years, is in its 5th year. Sixty-three awards have been granted, 16 of which were granted this year. The New Innovator Award program provides $1.5 million in direct costs over 5 years. It began in 2007 and supports 61 investigators. 30 awards were made during the inaugural year, and 31 in 2008.

Both programs are key components of the NIH Roadmap for Medical Research, and recipients are selected through special application and evaluation processes. Distinguished outside experts identify the most highly competitive applicants, while the Advisory Committee to the Director, NIH, performs the second level of review and the Director makes final decisions based on the outside evaluations and programmatic considerations.

2008 award recipients were named at the start of the NIH Director's Pioneer Award Symposium.

A listing of recipients of the Pioneer awardees and their biographical sketches is available online at http://nihroadmap.nih.gov/pioneer/Recipients08.aspx. For a list of this year's New Innovator Award recipients and details pertaining to their research plans, visit http://nihroadmap.nih.gov/newinnovator/Recipients08.asp

NIH Announces New Transformative R01 Funding Program

The National Institutes of Health (NIH) announced that more than $250 million over the next five years will be invested to promote bold and creative investigator-initiated research through a new transformative R01 (T-R01) Program. The NIH is developing the T-R01 as an alternate program to the R01, which supports the bulk of mainstream NIH investigator-initiated efforts. The focus will be to attract bold and innovative research proposals that may be inhibited by the structure and review of R01 proposals by initiating novel approaches to peer review and encouraging original and high-risk proposals in biomedical or behavioral sciences. Review criteria will focus on a project's transformative potential. The first cohort of T-R01 awards is planned in 2009. Extending the program again in 2010 will depend upon availability of funds.

The TR01 program is a trans-NIH effort coordinated by the Office of Portfolio Analysis and Strategic Initiatives as part of the NIH Roadmap for Medical Research. The NIH has identified that efforts to achieve T-R01 program goals will include support of original studies that:

- Forge the synthesis of new paradigms for biomedical or behavioral sciences.
- Reflect an exceptional level of creativity in proposing bold and ground-breaking approaches to fundamental problems.
- Promote radical changes in a field of study with a profound impact in other scientific areas.
- Be evaluated by new procedures being piloted by the NIH Center for Scientific Review that are distinct from the traditional NIH peer review process.

Scientists from all disciplines that are relevant to the NIH mission are eligible to submit T-R01 applications. This includes the biological, behavioral, clinical, social, physical, chemical, computational, engineering, and mathematical sciences. The NIH strategic planning process has identified a list of areas of highlighted need, including: Science of Behavior Change; Protein Capture; Functional Variation in Mitochondria; Complex 3-D Tissue Models; Acute to Chronic Pain Transition; and Pharmacogenomics. For additional information about the Transformative R01 Program, visit http://www.nihroadmap.nih.gov/grants/index.asp. Applications for new five-year grants are currently being accepted.

National Neurology Advisory Council Names Four New Members

Four new members were recently appointed to the National Institute of Neurological Disorders and Stroke, a component of the National Institutes of Health (NIH), National Advisory Neurological Disorders and Stroke Council. The Council meets three times annually to review applications for financial support of biomedical research and research training on brain and nervous system disorders. Additionally, members advise the Institute on research program planning and priorities. Composed of 18 members, the Council includes physicians, scientists and members of the public. The new members will serve through July 2012. They are:

Emery N. Brown, MD, PhD

Dr. Brown is a professor of Health Sciences and Technology and a professor of computational neuroscience at Massachusetts Institute of Technology; and the Massachusetts General Hospital Professor of Anesthesia at Harvard Medical School. Dr. Brown earned his MD from Harvard Medical School and a PhD in statistics from Harvard

University. He is an anesthesiologist-statistician whose research focus is the development of signal processing algorithms to characterize how the patterns of electrical discharges from neurons in the brain represent information from the outside world. He has been using a systems neuroscience approach to examine the way in which anesthetic drugs behave in the brain to create the state of general anesthesia. Dr. Brown's awards are numerous and include elected membership in the Association of University Anesthesiologists, and the Institute of Medicine. He received the NIH Director's Pioneer Award in 2007 and is a fellow of the American Association for the Advancement of Science and a fellow of the IEEE.

Robert M. Friedlander, MD

Dr. Friedlander serves as vice chairman of the Department of Neurosurgery at Brigham and Women's Hospital in Boston. He is also an associate professor in surgery (Neurosurgery) at Harvard Medical School, Brigham and Women's Hospital. Dr. Friedlander earned his MD from Harvard Medical School and completed his residency in neurosurgery at Massachusetts General Hospital. An associate neurosurgeon at Children's Hospital and Brigham and Women's Hospital, Dr. Friedlander also serves on the consulting staff at Dana Farber Cancer Institute. His clinical specialties include aneurysms and vascular malformations, brain tumors, carotid disease, cerebrovascular disease, Chiari malformation, microvascular decompression, and radiosurgery. His research focuses on mechanisms of apoptosis, and he and his colleagues are investigating ways to halt or deter the progression of cell death in Huntington's disease, ALS, and stroke. Dr. Friedlander was the first to demonstrate the functional role of apoptotic pathways in a number of neurologic diseases. Along with his colleagues, he has reported success with using pharmacological interventions to reduce apoptosis in mouse models of neurological disease. Dr. Friedlander is widely published in his field and is a member of numerous professional societies.

Katie Hood, MBA

Katie Hood is chief executive officer of The Michael J. Fox Foundation for Parkinson's Research (MJFF). Second only to the US government, MJFF is the single largest Parkinson's disease (PD) research funder in the world, having funded over $126 million in PD research. Ms. Hood has been instrumental in shaping MJFF's strategy of closing critical gaps that slow the translational process. She has also been key to the development of a team of in-house research experts responsible for implementing that strategy. Ms. Hood joined MJFF in 2002 after working as a consultant at Bain & Company. Her prior professional experiences include work as an analyst in the Credit Department of Goldman, Sachs & Co., and as a program coordinator with Duke University's Hart Leadership Program. Ms. Hood earned her undergraduate degree in Public Policy Studies from Duke University and her MBA from Harvard Business School. She serves as a member of the Board of Directors of the Parkinson's Action Network.

Louis Ptáček, MD

Dr. Louis J. Ptáček serves as Director of the Division of Neurogenetics at the University of California, San Francisco School of Medicine, where he holds the John C. Coleman Distinguished Professorship in Neurodegenerative Diseases. He is also an investigator with Howard Hughes Medical Institute. He received his MD from the University of Wisconsin and completed his residency in neurology at the University of Utah. His research examines genetic diseases of muscle, heart and brain, and hereditary variation of human sleep behavior. By demonstrating that disorders such as periodic paralysis and non-dystrophic myotonias are caused by genetic mutations that affect ion channel proteins in neurons, Dr. Ptáček pioneered the field of channelopathies. He and his collaborators continue to investigate the pathophysiology of episodic neurological diseases. His group has also identified genes causing cardiac arrhythmias, epilepsy and migraine headache. More recently, he and collaborators have identified the first Mendelian human circadian rhythm variants and cloned a number of genes causing altered sleep schedules. Such studies are leading to insights about the molecular basis of these human phenotypes and to similarities and differences between human physiology and that of other organisms.

NIEHS Awards $21.5 Million for Study of Environmental Causes of Parkinson's Disease

The National Institute of Environmental Health Sciences (NIEHS), part of the National Institutes of Health (NIH), announced three new grants totaling $21.5 million to study how environmental factors influence the cause, treatment and prevention of Parkinson's disease and related disorders. The 5-year grants are part of the NIEHS' Centers for Neurodegeneration Science announcement issued in 2007. The three grant recipients are:

Gary Miller, PhD, Emory University, Atlanta, GA

Dr. Miller and his team will focus on how environmental and genetic factors interact to alter dopamine neurons. The group will examine links between mitochondrial damage, altered storage of dopamine, and exposure to environmental components such as pesticides to the development of the disease. Additionally, the group will seek to develop new biomarkers in the blood that will facilitate the identification

of people who are at-risk for developing the disease.

Marie-Francoise Chesselet, MD, PhD, University of California, Los Angeles, CA (UCLA)

UCLA researchers have previously demonstrated associations between high levels of exposure to specific environmental pesticides and Parkinson's, and will further this link to determine the mechanisms of action that may be causing this association. The group will use a multidisciplinary approach to identify additional agricultural pesticides that are disrupting similar molecular pathways in order to determine if these pesticides also increase the risk of Parkinson's. Their investigation is expected to further explain the pathological processes involved in sporadic Parkinson's disease and may have implications for precautions in the use of some pesticides.

Stuart Lipton, MD, PhD, Burnham Institute for Medical Research, La Jolla, CA

Burnham Institute investigators will focus on how environmental toxicants may contribute to Parkinson's disease by producing free radical stress mimicking or enhancing the effects of known genetic mutations. Researchers will work to determine how chemical reactions that donate extra electrons lead to damaging modifications of proteins known to be related to Parkinson's disease - such as DJ-1, PINK1 and parkin. The clinical implications of these processes will be examined through biomarker development efforts and a screen to identify new lead compounds that preserve protein function through reduction of free radical stress.

NIH Announces Funding for the Molecular Libraries Probe Production Centers Network

The National Institutes of Health (NIH) recently announced the funding of a network of nine centers across the United States that will implement high tech screening methods to identify small molecules for use as probes to investigate the diverse functions of cells. The network will produce chemical probes in order to explore new therapeutic targets, and will be funded at approximately $70 million annually over the four-year production phase. The National Institute of Mental Health and the National Human Genome Research Institute will co-administer the network on behalf of NIH, and Program funding is scheduled to transition out of the Roadmap in years five and six.

The Molecular Libraries Probe Production Centers Network is the second phase of a program that was started in 2004 as part of the Molecular Libraries and Imaging Initiative under NIH's Roadmap for Medical Research. The network will use assays solicited by NIH from the research community to screen a library of over 300,000 small molecules maintained in the programs Molecular Libraries Small Molecule Repository in San Francisco at Biofocus DPI, a drug discovery research company. Information obtained by the screening is available to the public through PubChem.

The nine institutions funded as part of the network are as follows:

Comprehensive Centers

- The Burnham Center for Chemical Genomics, La Jolla, CA; *John Reed, Principal Investigator*
- Broad Institute Comprehensive Screening Center, Cambridge, MA; *Stuart Schreiber, Principal Investigator*
- National Institutes of Health Chemical Genomics Center, Bethesda, MD; *Christopher Austin, Principal Investigator*
- The Comprehensive Center for Chemical Probe Discovery and Optimization at Scripps, La Jolla, CA; *Hugh Rosen, Principal Investigator*

Specialized Screening Centers

- Johns Hopkins Ion Channel Center, Baltimore, MD; *Min Li, Principal Investigator*
- Southern Research Specialized Biocontainment Screening Center, Birmingham, AL; *Colleen Jonsson, Principal Investigator*
- University of New Mexico Center for Molecular Discovery, Albuquerque, NM; *Larry Sklar, Principal Investigator*

Specialized Chemistry Centers

- University of Kansas Specialized Chemistry Center, Lawrence, KS; *Jeffrey Aube, Principal Investigator*
- The Vanderbilt Specialized Chemistry Center for Accelerated Probe Development, Nashville, TN; *Craig Lindsley, Principal Investigator*

First Eureka Grants for Exceptionally Innovative Research Awarded

The National Institutes of Health (NIH) has awarded $42.2 million to fund 38 exceptionally innovative research projects as part of the Exceptional, Unconventional Research Enabling Knowledge Acceleration (EUREKA) program. The program funds research efforts focused on testing novel—and often

unconventional—hypotheses and projects aimed at resolving major methodological or technical challenges.

Awardees will receive direct costs of approximately $200,000 per year for up to four years, subject to the availability of appropriations. The NIH components funding EUREKA projects include the National Institute of General Medical Sciences, National Institute of Neurological Disorders and Stroke, the National Institute of Mental Health, and the National Institute on Drug Abuse. The awardees are:

National Institute of General Medical Sciences

- John Chaput, Arizona State University, Tempe *"Discovering a Hidden Proteome in the Human Genome"*
- Daniel T. Chiu, University of Washington *"Super-Resolution Imaging with Difference Deconvolution Microscopy"*
- Tanja Dominko, Worcester Polytechnic Institute *"De-Differentiating Adult Human Fibroblasts into Stem-Like Cells Using Defined Conditions"*
- Iswar K. Hariharan, University of California, Berkeley *"Identifying Novel Strategies to Promote Tissue Regeneration"*
- James M. Hogle, Harvard University Medical School *"Correlative Cyro-Miscroscopy: A New Approach for Characterizing the Structure and Function of Intracellular Macromolecular Machines in Situ"*
- Laurence H. Hurley, University of Arizona, Tucson *"Establishing a Molecular System for Drug Targeting of Transcriptional Control"*
- Masayori Inouye, University of Medicine & Dentistry of New Jersey/Robert Wood Johnson Medical School *"The Method forDetermination of Membrane Protein Structures Without Purification"*
- Michelle Krogsgaard, New York University School of Medicine *"Visualizing Ligand-Induced Signal Propagation in the TCR-Signaling Complex"*
- Jeanne B. Lawrence, University of Massachusetts Medical School, Worcester *"Is Chromosome Therapy Possible for Down Syndrome and Other Karyotypic Imbalances?"*
- Joseph A. Loo, University of California, Los Angeles *"Impact of Non-Canonical Decoding on the Proteome"*
- Lee Makowski, University of Chicago *"MADMAX: Precise Measurement of Conformational Changes in Proteins"*
- Scott R. Manalis, Massachusetts Institute of Technology *"High-Throughput Monitoring of Mass, Density, and Fluorescence of Single Cells"*
- William E. Moerner, Stanford University *"Three-Dimensional Super-Resolution Imaging in Living Cells Using Single-Molecule Active Control"*
- John W. Sedat, University of California, San Francisco *"Enabling High-Resolution Imaging Deep in Live Tissue with Adaptive Optics"*
- David F. Smith, Emory University *"Shotgun Glycomics: Linking Glycan Structure and Function"*
- Charles E. Stebbins, Rockefeller University *"Exploiting a Bacterial Nano-Syringe for Protein Therapeutics"*
- Michael H. Stowell, University of Colorado at Boulder *"Self Assembled Lipid Icosohedra for High-Throughput Membrane Protein Structure Determination"*
- Brian D. Strahl, University of North Carolina, Chapel Hill *"A High-Throughput Approach Towards Deciphering the Histone Code"*
- Russell N. Van Gelder, University of Washington Medical Center, Seattle *"Mechanisms of Light-Mediated Protein Degradation"*
- Alexander J. Varshavsky, California Institute of Technology *"Split Proteins as Boolean Circuits and Drugs of a New Kind"*

National Institute of Neurological Diseases and Stroke

- Karen Ashe, University of Minnesota, Twin Cities *"In Search of the Molecular Basis of Memory Loss in Tauopathy"*
- Akira Chiba, University of Miami, Coral Gables *"Mechanical Force and Neural Regeneration"*
- Janis J. Daly, Case Western Reserve University *"Non-Invasive Brain-Signal Training to Induce Motor Control Recovery After Stroke"*
- Beverly Davidson, University of Iowa *"RNA Aptamers for Brain Delivery"*
- David Sretavan, University of California, San Francisco *"Microscale Axon Repair as a Novel Paradigm for Nerve Injuries"*

National Institute of Mental Health

- Paul Brehm, Oregon Health & Science University *"A Genetic Indicator Provides Long Term Mapping of Neuronal and Calcium Activity"*
- David Kleinfeld, University of California, San Diego *"Neuronal Signaling, Network Topology, and Blood Flow Dynamics in Cortex"*
- Todd Lencz, Feinstein Institute for Medical Research *"Identifying Molecular Subtypes of Schizophrenia: A Novel Genomic Approach"*
- Rick C.S. Lin, University of Mississippi Medical Center *"Serotonin, Corpus Callosum, and Autism"*
- Joshua Ahab Maurer, Washington University in St. Louis *"Unraveling Development: New Materials for Understanding Neuronal Wiring"*
- Andras Jagy and Hongkui Zeng, Allen Institute for Brain Science *"Generation and Characterization of Novel and Highly Specific Neuronal Subtype TRA"*

- Bernardo L. Sabatini, Harvard University Medical School *"Regulation of Neuron and Synapse Function by Neuropeptides"*
- Yi Eve Sun, University of California, Los Angeles *"A Novel Approach to Identify Neuronal mRNA Targets for Individual microRNAs"*
- Michael J. Tarr, Brown University *"Using Functional Physiology to Uncover the Fundamental Principles of Visual Cortex"*

National Institute on Drug Abuse

- Jeff W. Bulte, Johns Hopkins University *"Developing MPI for Non-Invasive and Quantitative Imaging of Stem Cells"*
- Benjamin F. Cravatt, Scripps Research Institute *"Toward a Potent and Selective Inhibitor for Every Mammalian Serine Hydrolase"*
- Rodolfo E. Diaz, Arizona State University, Tempe *"Feasibility Demonstration of an Artificial Electrocyte for Neuronal Observation & Stimulation"*
- Emad N. Eskandar, Massachusetts General Hospital *"Striatal Deep Brain Stimulation for Learning Enhancement"*

San Diego Consortium for Regenerative Medicine Receives $30 Million

T. Denny Sanford has given $30 million to the San Diego Consortium for Regenerative Medicine, which has been renamed in his honor as the Sanford Consortium for Regenerative Medicine. The newly named Sanford Consortium for Regenerative Medicine was started in 2005 as a means of linking local research centers in an effort to more rapidly advance stem cell research toward the improvement of medical testing, diagnosis and treatment options. Consortium resources come from the Burnham Institute for Medical Research, the University of San Diego, the Scripps Research Institute and the Salk Institute for Biological Studies.

Mr. Sanford, a South Dakota philanthropist, has made various donations to hospitals and medical schools in South Dakota and Minnesota. The University of South Dakota School of Medicine as well as the pediatric center at the Mayo Clinic in Rochester, MN, are named in his honor. Mr. Sanford was born at the Bethesda Hospital in St. Paul, MN, and previously donated $500,000 to the HealthEast Foundation in St. Paul to build the William Sanford welcome center at Bethesda Hospital in honor of his parents and family members. Last year, Mr. Sanford gave $400 million to the Sioux Valley Hospitals and Health System in Sioux Falls, SD. His generosity put him at Number 14 on the *Chronicle of Philanthropy*'*s* list of top donors for 2005, at number 49 on *BusinessWeek*'*s* "50 Most Generous Philanthropists" in 2006, and he has received numerous awards for his generous contributions.