

CURRICULUM VITAE

HELEN M. BLAU

Donald E. and Delia B. Baxter Professor

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Personal Data: Birthplace: London, England
Dual Citizenship: USA & Great Britain
Languages: French and German

Education:

1969	B.A.	University of York, York, England
1970	M.A.	Harvard University, Cambridge, MA
1975	Ph.D.	Harvard University, Cambridge, MA

Professional Experience:

1969 - 1975: Predoctoral Fellow, Department of Biology, Harvard University, Cambridge, MA (advisor: F.C. Kafatos)

1975 - 1978: Postdoctoral Fellow, Division of Medical Genetics, Departments of Biochemistry and Biophysics, University of California, San Francisco, CA (advisor: C.J. Epstein)

1978 - 1986: Assistant Professor, Department of Pharmacology, Stanford University School of Medicine, Stanford, CA

1986 - 1991: Associate Professor, Department of Pharmacology, Stanford University School of Medicine, Stanford, CA

1991 - 1999: Professor, Department of Molecular Pharmacology, Stanford University School of Medicine, Stanford, CA

1997 - 2002: Chair, Department of Molecular Pharmacology, Stanford University School of Medicine, Stanford, CA

1997 - present: Director, Gene Therapy Technology, Stanford University School of Medicine, Stanford, CA

1999 - present: Donald E. and Delia B. Baxter Professor, Stanford University School of Medicine, Stanford, CA

2002 - present: Director, Baxter Laboratory in Genetic Pharmacology, Department of Microbiology and Immunology, Stanford University School of Medicine, Stanford, CA

Major Honors and Awards:

Basil O'Connor Faculty Fellow (1978-1981)
Mellon Foundation Faculty Fellow (1979-1980)
William M. Hume Faculty Scholar (1981-1984)
Research Career Development Award, National Institutes of Health (1984-1989)
SmithKline and Beecham Junior Faculty Scholar Award (1989-1991)
Fellow, American Association for the Advancement of Science (AAAS) (1991)
U.S. National Academy of Sciences Delegation to The People's Republic of China (1991)
Resident Scholar, Bellagio Study Center of Rockefeller Foundation, Lake Como, Italy (1992)
Senior Career Recognition Award of WICB, American Society of Cell Biology (1992)
Excellence in Teaching Recognition, Committee on Courses and Curriculum, Stanford University School of Medicine (1992-1993); (1993-1994); (1995-1996); (1997-1998)
54th President, American Society for Developmental Biology (1994-1995)
MERIT Award, National Institutes of Health (1995-2005)
Nobel Forum Lecture, Karolinska Inst., Sweden (1995)
Yvette Mayent-Rothschild Visiting Professor, Institut Curie and Pasteur, Paris (5 months, 1995)
Institute of Medicine of the National Academy of Sciences (elected 1995)
National Advisory Council, NIH Institute on Aging (1996-2000)
American Academy of Arts and Sciences (elected 1996)
Initiator and Liaison, Stanford/Aventis Inc. \$5 Million Innovative Grant Agreement (1997-2002)
NATO Collaborative Research Programme, France (1997-98)
Board of Directors, American Society for Gene Therapy (1999-2002)
FASEB Excellence in Science Award (1999)
Donald E. and Delia B. Baxter Endowed Professorship (1999)
NIH Director's (Harold Varmus) Lectureship (2000)
President, International Society of Differentiation (2002-2004)
Initiator and P.I. of Aventis/Gencell-Stanford Collaborative Agreement on Angiogenesis for \$12 million (2001-2005)
Fellow, American Heart Association, Council on Basic Cardiovascular Sciences (2001)
McKnight Endowment Fund for Neuroscience Award (2001)
Council Member, American Society for Cell Biology (ASCB)(2002-2004)
Honorary Doctorate, University of Nijmegen, Holland (2003)
Rolf-Sammet-Fonds Visiting Professorship, University of Frankfurt (2003)
400th Annual Pontifical Academy, the Vatican in Rome, plenary talk on stem cells, Modern Biotechnologies Symposium, Audience with the Pope (2003)
Institute of Medicine (IOM), The Membership Nominating Committee (Chair of Section 2), and Council Nominating Committee Working Group on Reassessing the IOM sections and elections (2003)
Council Member, Institute of Medicine (IOM) (2004-2006)
Board Member, Harvard Board of Overseer's (2004-2010)

Professional Societies:

American Association for the Advancement of Science
American Heart Association
American Society for Biochemistry and Molecular Biology
American Society for Cell Biology
American Society for Developmental Biology
American Society of Gene Therapy
American Society of Human Genetics
Hastings Institute of Society, Ethics, and the Life Sciences
International Society of Differentiation
Society for Neuroscience

Editorial Boards:

The Journal of Cell Biology (1987-1994)
Neuromuscular Disorders (1990-1994)
Molecular and Cellular Biology (1991-1994)
Developmental Biology, consulting editor (1995-1998)
Journal of Molecular Medicine (1994-2001)
Advisory Board, Biochimica et Biophysica Acta Reviews on Cancer (1995-2002)
Molecular Therapy (2000-2004)
Somatic Cell and Molecular Genetics (1988-present)
Experimental Cell Research (1987-2000)
Molecular and Cellular Differentiation (1992-present)
Genes to Cells (Senior Editor, 1994-present)
Molecular Medicine Today/Trends in Molecular Medicine (1994-present)
Molecular Interventions (2000-present)

Organizer, National and International Meetings:

Co-organizer with M. Karin, FASEB Meeting, Regulation of Tissue-Specific Gene Expression (1990)
Co-organizer with A. Kelly, Keystone Symposium, Gene Expression in Neuromuscular Development (1991)
Co-organizer with N. Rosenthal, 54th Annual Meeting of the American Society for Developmental Biology (1995)
Co-organizer with J. Wilson, Keystone Symposium, Molecular and Cellular Biology of Gene Therapy (1997)
Organizer, Gene Therapy: Prospects for the Next Decade – First Gene Therapy Symposium at Stanford (1999)
International Scientific Advisory Committee, 8th Meeting of the European Society of Gene Therapy (ESGT), Stockholm, Sweden (2000)
Co-organizer with W. Mobley and A. Garber, Aging: Biology, Disease and Economics, First Interdisciplinary Aging Symposium at Stanford (2001)
President/Co-organizer, 13th International Society of Differentiation Meeting, Hawaii (2004)

Experience In Training (Students, Postdoctoral Fellows and Medical Residents):

Faculty Affiliate and Preceptor, NIH Cancer Biology Predoctoral/Postdoctoral Training Program, Stanford School of Medicine (1978-present)
Admissions Coordinator, NIH Pharmacology Predoctoral Training Program, Stanford School of Medicine (1981-1988; 1994-1996)
Program Committee, NIH Neurosciences Predoctoral Training Program at Stanford School of Medicine (1987-1991; 1993-1996); Faculty Preceptor (1998-present)
Associate Program Director, NIH Developmental & Neonatal Biology Predoctoral and Postdoctoral Training Program at Stanford School of Medicine (1988-present)
Co-director, CAM with J. Boothroyd (First Stanford Predoctoral Multidepartmental Combined Admission Mode) (1990-1993)
Faculty Affiliate and Preceptor, NIH Biotechnology Predoctoral Training Program, Stanford School of Medicine (1993-present)
Special Advisor, Cell Sciences Program, Stanford School of Medicine (1994-present)
Program Committee, Flexible Graduate Admissions Program at Stanford (1995-1996)
Head, Task Force on Training, National Institute on Aging, Washington, D.C. (1998-1999)

P.I. and Director, NIH Pharmacology Predoctoral Training Program, Stanford School of Medicine (1998-2003)
P.I. and Director, NIH Molecular Pharmacology of Diseases of Aging Predoctoral Training Program, Stanford School of Medicine (1998-2004)
Member, Women's Reproductive Health Research (WRHR) Career Development Program at Stanford School of Medicine (2000-present)
Member, Microbiology and Immunology Department Graduate Steering Committee (2002-2003)

Selected Administrative Experience (University and Public Service):

Genetic Counseling, Department of Medical Genetics, U.C., San Francisco (1975-1978)
Reproductive Outcome Research for Medfly Health Advisory Committee, State of California (1981-1982)
Faculty Senator for Dept. of Pharmacology, Academic Senate of Stanford School of Medicine (1982-85)
Faculty Affiliate, Institute for Research on Women and Gender, Stanford University (1982-present)
Dean Search Committee, Stanford School of Medicine (1984)
Nominating Committee, American Society for Cell Biology (1985-1986)
Faculty Senator-At-Large, Stanford School of Medicine (1986-1989)
NIH Molecular Cytology Study Section (Ad Hoc) (1987, 1988)
Steering Committee of the Faculty Senate, "Committee of Five," Stanford School of Medicine (1986-1989)
Chair, Dean's Advisory Committee on Junior Faculty Awards, Stanford School of Medicine (1987-1989)
Advisory Board, Program in Molecular and Genetic Medicine at Stanford (1988-1995)
Chair, Katharine Dexter McCormick Distinguished Lectureship Committee, Stanford (1989-1998)
NICHD/NIH Five-Year Planning Committee, Genetics & Teratology Branch Washington, D.C. (1989)
Program Committee, American Society for Cell Biology (1990, 2004)
Executive Committee, Basic Science Council, American Heart Association (1991-1992)
NIH (RAC) Oversight Committee of Gene Therapy (created by Harold Varmus) (1995)
Chair, Nominating Committee, American Society for Biochemistry and Molecular Biology (1996)
Co-Chair, Dean's Task Force on Gene Therapy, Stanford Medical School (1995-1996)
National Advisory Council, National Institute on Aging, NIH, Washington, D.C. (1996-2000)
Board of Directors, American Society of Gene Therapy (1998-2001)
Director, Gene Therapy Technology Stanford School of Medicine (1998-present)
Co-Organizer (with Mark Kay), Frontiers in Cell and Gene Therapy Seminar Series, Stanford (1999-present)
Co-Organizer, French Rhône-Poulenc Rorer/Stanford Workshop on Gene Therapy (1999)
American Society for Cell Biology (ASCB), Joint Steering Committee on Public Policy (1999-present)
Member, Congressional Liaison Committee for Public Policy for ASCB (2000-present)
Chair, American Society of Gene Therapy, Scientific Committee for 3rd Annual Meeting on Neuromuscular Disorders (2000); Committee Member (2003)
Fellow, American Heart Association, Council on Basic Cardiovascular Sciences (2001-present)
Chair, Membership Committee, Institute of Medicine (IOM) of the National Academy of Sciences Membership Committee, Section 02 (2002-2003); Vice Chair (2000-2001)
Membership Committee, American Academy of Arts and Sciences, Class II Biological Sciences, Section 2 (2001, 2002)
Scientific Planning Committee Member, Conseil Stratégique de l'Association Française contre les Myopathies (AFM) (2001-2003)
Scientific Coordinator, Stanford Interdisciplinary Institute for French Studies (2002-present)
Member, International Society for Stem Cell Research Education Subcommittee (2003-present)
Faculty Affiliate, Stanford Cardiovascular Institute (2003-present)

Member, International Society for Stem Cell Research Education Subcommittee (2003-2004)
Member, Institute of Medicine Nominating Committee (2004) & IOM Council Nominating Committee (2005)
Member, IOM Working Group on Physical and Basic Biomedical Sciences, Reorganization of sections (2004)
Faculty Affiliate, Stanford Center for Longevity (2004-present)
Member, Education Committee, Stanford Program in Regenerative Medicine (2005-present)

Selected Honorary Lectureship Invitations (last 5 years):

Plenary Speaker, Keystone Symposium: Molecular Biology of the Cardiovascular System (2000)
Invited Lecturer, NIH Director's Lecture Series, Bethesda, MD (2000)
Biosciences Distinguished Lecturer, Lawrence Berkeley National Laboratory, Berkeley, CA (2000)
Plenary Speaker, 5th Ben May Inst. for Cancer Research Symposium, Univ. Chicago (2000)
Plenary Speaker, Molecular Biology of Muscle Development and Disease, Asilomar, CA (2000)
Keynote Speaker, 25th Annual Meeting, Society of Cardiovascular and Interventional Radiology, San Diego, CA (2000)
Plenary Speaker, 3rd Annual Meeting, American Society of Gene Therapy, Denver, CO (2000)
Keynote Speaker, BAM'2000, The Sixth Abano Terme meeting on Rehabilitation, Padova, Italy (2000)
Invited Speaker, Department of Cell Biology, Biological Chemistry and Molecular Pharmacology and Institute of Chemistry and Cell Biology, Harvard University School of Medicine (2000)
Plenary Session Chair and Speaker, Gene Therapy 2001: A Gene Odyssey, Keystone Symposium, Utah (2001)
Co-Chair and Speaker, Stem Cell Minisymposium, 41st Meeting of the American Society for Cell Biology (ASCB), Washington, DC (2001)
Broadhurst Distinguished Lecturer, Harvard University, Cambridge, MA (2001)
Keynote Speaker, SBF 488 Symposium: Neural and Non-Neural Stem Cells, Heidelberg, Germany (2001)
Plenary Speaker, Gordon Research Conference on Angiogenesis and Microcirculation, Salve Regina University, Newport, RI (2001)
Plenary Speaker, Myogenesis, Gordon Research Conference, Il Ciocco, Italy (2001); Discussion Leader and Plenary Speaker (2004)
Mayer Lecture in the Life Sciences, Massachusetts Institute of Technology, Cambridge, MA (2002)
Mary Elizabeth Garrett Lecturer, Johns Hopkins University School of Medicine, Baltimore, MD (2002)
Catherine N. Stratton Lecturer on Critical Issues, Whitehead Institute, Massachusetts Institute of Technology, MA (2002)
President's (Harold Varmus) Research Seminar Series Lecturer, Memorial Sloan Kettering Cancer Center, NY (2002)
Plenary Speaker, British Society for Developmental Biology, Spring Meeting, University of Warwick, England (2003)
Plenary Speaker, Perlmutter Family Symposium on Neurodegenerative Disorders, Harvard University School of Medicine (2003)
2003 Annual Scientist in Medicine Lecture, University of Washington (2003)
Rolf-Sammet-Fonds Visiting Professorship, University of Frankfurt (2003)
Honorary Doctorate, University of Nijmegen, Holland (2003)
Speaker, Academie des Sciences de la France at Pontifical Academy, the Vatican in Rome, "Stem Cells and Cloning" (2003)
Plenary Speaker, XIIIth International Vascular Biology Meeting, Toronto, Canada (2004)
Plenary Speaker, 2nd Annual International Society for Stem Cell Research Meeting, Boston, MA (2004)
President's Lecture, 13th International Society of Differentiation Conference, Honolulu, HI (2004)

Keynote Speaker, Allergan Foundation Lectures in Modern Biology and the Howard A. Schneiderman Memorial Bioethics Lecture Series, University of California, Irvine (2004)
Louis A. Bloomfield Lecturer, Case Western Research University School of Medicine, Cleveland, OH (2005)

Research Accomplishments and Goals

ONGOING RESEARCH

- **Stem Cell Biology/Cell Differentiation**: We are elucidating the mechanisms underlying nuclear reprogramming critical to adult stem cell function in skeletal muscle and brain and testing their potential physiologic relevance in mouse models of disease.
- **Angiogenesis**: We are studying the interplay of pro- and anti-angiogenic factors to overcome limb and heart ischemia by growing new durable blood vessels (“well tempered vessels”).
- **Elucidation of signal transduction pathways**: Using technologies developed in our laboratory (restriction enzyme generated siRNAs (REGS) for loss of function analyses and β -galactosidase assays of protein complementation for monitoring intracellular protein translocation, we are determining the molecular bases (chromatin remodeling and signaling pathways) for changing the nuclear function of embryonic and adult stem cells.

OVERVIEW

The question of fundamental interest to my laboratory is how cells maintain their differentiated state, and how they can change in response to injury or other microenvironmental variables. Using muscle as a model system, we first demonstrated in tissue culture that a range of highly specialized human adult cells could be induced to express previously silent muscle genes. Recently, we have found that such changes in nuclear reprogramming also occur *in vivo* in adult mice and humans. Hematopoietic stem cells (HSCs) give rise to progeny (myelomonocytic precursors) that can fuse to adult skeletal muscle and Purkinje cells of the brain, activating tissue-specific genes typical of the cells with which they fuse. We have also shown that unpurified bone marrow derived cells give rise to satellite cells en route to becoming myofibers, a biological progression in response to two distinct types of injury. More study is required to prove that this event represents a bona fide change in cell fate in response to molecules in a niche, as has been elegantly shown in *Drosophila*. Very likely cells can also bypass the satellite cell stage and fuse directly with muscle fibers. However, irrespective of whether cells alter their patterns of gene expression in the absence or presence of fusion, in response to cell extrinsic or cell intrinsic signals, an understanding of the mechanisms underlying nuclear reprogramming is common to both. Our current focus is to understand the regulatory mechanisms that govern such changes at a molecular level using genetic mouse models of human disease and the novel technologies we have recently developed. Our prior history of using heterokaryons to study nuclear reprogramming *in vitro* (1980s) coupled with the tools we now have in hand (see below) should make it possible to obtain novel mechanistic insights regarding chromatin remodeling and critical cell signaling pathways in tissue culture. Identification of the molecules that recruit cells to damaged tissue and the fusion molecules that mediate the formation of specific heterokaryons *in vivo* are also of great interest. Through a better understanding of these mechanisms, we hope to significantly enhance the low frequency of the currently observed nuclear reprogramming (beyond that increased by physiological stimuli such as muscle exercise or over-use). Our results may shed light on mechanisms required for nuclear reprogramming in nuclear transplant experiments (cloning) by others, which also exhibit a low frequency of success. Our studies may also aid in the treatment of muscular dystrophies or brain disorders. Thus, although as we and others have shown, these reprogramming events are real, have a physiological basis, and are ongoing throughout mammalian life (adult HSC derivatives contribute to muscle, liver and brain), the efficiency remains low. That is the reason that it is now time to turn our attention to the molecular prerequisites in a manner that was not previously possible.

BACKGROUND: Heterokaryons

Years ago, our laboratory demonstrated in tissue culture that the differentiated state is not fixed and irreversible. Instead, it is dynamic, reversible, and largely determined by the balance of regulators within the cell at any given time. We showed this *in vitro* by fusing cells to form heterokaryons, non-dividing cell fusion products formed between mouse myoblasts and human primary cells derived from tissues of all three lineages (endoderm, ectoderm and mesoderm). The efficiency and time course of muscle gene activation following fusion differed among cell types, suggesting the existence of different regulatory mechanisms. Moreover, the relative levels and interplay of regulators were critical to cell fate. These findings have since been replicated in several laboratories using diverse cell types. They revealed that cell differentiation is determined largely by the stoichiometry of proteins in the cell and requires continuous regulation to be maintained. Taken together, they demonstrated a surprising potential to alter the differentiated state of adult human cells in tissue culture.

STEM CELLS: Bone Marrow Derived Stem Cells for Repair of Adult Tissues

Recently we have determined that the plasticity of nuclear gene expression observed in culture in heterokaryons, also occurs in the adult organism, and may comprise a key repair mechanism for injured tissues. We have found that hematopoietic stem cells (HSCs) within adult bone marrow give rise to myeloid precursors that can contribute to muscle and to brain. Damage appears to be the cue. In some rare cases, the mechanism of repair appears to be regeneration, or a cell fate change in response to a new microenvironment, or niche, as in *Drosophila*. In others, the

primary mechanism whereby this occurs is clearly fusion of an HSC derivative with a damaged cell. Since tissue-specific genes characteristic of the cell to which the myeloid cell fuses are activated, the primary mechanism whereby this occurs appears to be a natural *in vivo heterokaryon* in which a damaged cell is rescued. Our laboratory is now engaged in studies to discover methods for recruiting these cells efficiently to specific sites of damage: elucidating the molecules that attract them, the molecules involved in converting their patterns of gene expression to those of cell types of interest in brain and brawn, and showing that they can lead to improved function of the tissues to which they contribute. These studies are not only of fundamental biological significance, but also potentially have clinical potential for cell and gene therapy of muscular and neurodegenerative diseases.

ANGIOGENESIS: *The Development of Blood Vessels*

Angiogenesis is a critical component of stroke, head injury, cerebral vascular malformation development and brain tumor growth. An understanding of the mechanisms of adult cerebral angiogenesis is fundamental to therapeutic vessel modulation for these diseases. Many investigators, like we, have now shown that too much vascular endothelial growth factor (VEGF) can lead to hemangiomas (vascular tumors) and that regulating the levels of VEGF in a therapeutically efficacious manner is not feasible using the currently available delivery vectors. Recently we have found a means for enhancing VEGF's beneficial effects while abrogating its deleterious effects. Not only is the vasculature normal, but in ischemic limbs of mice, it is increased in density and blood flow is increased as well. Thus, our new approach overcomes previous problems and ameliorates both vascular morphology and function in hypoxic ischemic limbs. These discoveries were made possible using a model myoblast-mediated gene delivery method developed in our laboratory that allows careful control of diverse variables. Using this model, we showed that VEGF levels cannot be averaged, even over small distances of tissue *in vivo*, as the microenvironmental dose is critical and a mixture of aberrant and normal vascular structures will result. These results have been confirmed by others for VEGF delivered by adenoviral vectors and in transgenic mice. They pose a major problem for VEGF gene delivery for angiogenesis using clinically relevant gene therapy approaches. We recently tested hypotheses directed at counteracting or neutralizing the effects of VEGF. Our preliminary findings suggest that one such strategy achieves our goal in ischemic limbs of mice and may well be applicable to treating ischemic limb disease that is common in diabetes, and possibly cardiac ischemia as well. These findings underscore the need for attention to the interplay of molecules and cells critical to forming "Well Tempered Vessels" that are functional and durable over time.

NOVEL TECHNOLOGIES: *Applications Nuclear Reprogramming of Stem Cells*

Regulatable Gene Expression Vectors: We have developed several broadly useful technologies, such as tetracycline inducible retroviral vectors for rapid delivery and fine control of gene expression in mammalian cells in tissue culture. By engineering different dimerization domains into transcriptional activators and repressors, we can express both types of molecules in the same cell. As a result, gene expression can be completely repressed and then induced up to six orders of magnitude. We have found that increasing concentrations of extracellular signals leads to graded rheostat-like transcriptional responses when either activators or repressors are expressed alone. However, the expression of both activators and repressors together leads to an *all-or-none switch-like* response. These findings may explain why in nature, stripes with sharp borders are found during *Drosophila* development and why cells generally avoid neoplasia by shifting completely from quiescence to growth. Currently, dosage effects of growth factors on cell fate determination are being studied using this methodology.

Restriction Enzyme Generated siRNA (REGS) Vectors and Libraries: We developed a novel method for generating numerous siRNA constructs from any gene of interest or pool of genes using a combination of restriction enzyme digests and hairpin loop ligations. Small interfering

RNA (siRNA) technology greatly facilitates loss of gene function studies in mammalian cells. However, the generation of multiple unique siRNA expression vectors is slow, inefficient and costly. To test if the Restriction Enzyme Generated siRNAs (REGS) generated were functional, a transgene and two endogenous genes were silenced, resulting in the predicted phenotypes. REGS can generate approximately 34 unique siRNAs per kilobase of sequence with greater than 96% of the cloned inserts containing siRNAs with the appropriate structure. The efficiency of REGS enabled the creation of an siRNA library from double stranded cDNA containing approximately 415,000 independent clones. To our knowledge, the high yield of siRNAs per gene and the creation of a highly complex siRNA library using REGS is unprecedented. This loss of function technology will be invaluable in determining prerequisites for nuclear reprogramming.

Detection of inducible protein translocation in live intact mammalian cells: Recently we generated α -complementation strategies using β -galactosidase to monitor protein compartmentalization within cells and protein translocation in response to specific inducers. Enzymatic amplification of the signal generated provides high signal to noise ratios that are unparalleled in translocation assays to date. As a result, this method circumvents microscopic analyses and allows live cell-sorting and luminometer assays based on the intracellular location of specific proteins of interest in bulk populations of transduced cells. Specifically, mutation of the α peptide created a low affinity complementation system that was capable of detecting protein movements such as translocation from the cytosol to the plasma membrane. As proof of principle, we showed translocation of the C1 domain of PKC gamma to the plasma membrane in response to PMA and showed that the enzymatic assay for membrane translocation is 10-100 times more sensitive than GFP based detection systems, and attains 10-fold signal to noise ratios. We expanded this technique using the PH domain of AKT to monitor the creation of phosphorylated lipids in the plasma membrane in response to peptide growth factors and cellular stress. Signal transduction pathways critical to stem cell differentiation are currently being elucidated using this methodology.

Current Active Grants:

<u>Source (all as P.I.)</u>	<u>Title</u>	<u>Total Period</u>
NIH AG09521 (R37) (MERIT – 24 years)	Activators of Human Muscle Genes	4/1/86-3/31/2010
NIH HD18179 (R01) (24 years)	Regulation of Muscle Development	7/1/83-12/31/2007
NIH AG20961 (R01)	Stem Cells for Brain and Brawn	5/1/02-4/30/2007
NIH AG24987 (R01)	Regulators of Adult Stem Cell Fate	10/1/04-9/30/2009
Novartis Institutes for Biomedical Research	Stem Cell Differentiation: Generating Human Muscle from Human Blood via “Mouse Incubators”	7/1/04-6/30/2007

Patents:

- (1) 1989: Isolation, growth, differentiation and genetic engineering of human muscle cells, Patent No. 5,538,722 – Stanford Docket S89-054 (issued 7/23/96).

- (2) 1997: Detection of molecular interactions by reporter subunit complementation, U.S. Patent No. 6,342,345 B1 - International Application No. PCT/US98/06648 – Stanford Docket S96-125 (issued 1/29/02).
- (3) 1997: RetroTet ART: Retroviral Tetracycline regulatable vector system in which Activators of Repressors are expressed Together (pending).
- (4) 2003: Methods and compositions for use in preparing shRNAs (pending).
- (5) 2004: Biological Sensors for Protein Interactions (pending).
- (6) 2004: Methods for treating disorders of neuronal deficiency with bone marrow-derived cells (pending).

Training Grants (Active and Former):

NICHHD HD07249 (T32) (P.I.: D.K. Stevenson, Assoc. Program Director: H.M. Blau)	Developmental and Neonatal Biology Training Grant	5/1/04-4/30/2009
NIA AG00259 (T32) (P.I.: H. M. Blau)	Molecular Pharmacology of Diseases of Aging Predoctoral Training Grant	9/1/98-8/31/2004
NIGMS GM07149 (T32) (P.I.: H. M. Blau)	Predoctoral Pharmacology Training Grant	7/1/98-6/30/2003

Teaching:

- 1969-74: Teaching Fellow, Harvard University: courses in Introductory Biology, Cell Biology, and Human Genetics
- 1972-74: Resident Tutor in Biology, Adams House, Harvard University
- 1973-74: Human Genetics: Undergraduate level seminar course taught by HMB at Harvard University
- 1980 Spring: Pharmacology 203: Pharmacology for Medical Students, Course coordinator, Stanford University
- 1981 Winter: Pharmacology 209: Developmental Biology: Genetic and Environmental Influences, Stanford University (graduate level course directed by HMB)
- 1984, 87, 90: Pharmacology 231: Regulation of Gene Expression During Differentiation and Development, Stanford University (graduate level course directed by HMB)
- 1994, 95, Spring: Pharmacology 240: Course and Seminar Series on “Drug Discovery”, Stanford University (graduate level course, lecture by HMB on gene therapy)
- 1998, 2000-01, Spring: Pharmacology 231: Course and Seminar Series on “Stem Cells and Gene Therapy”, Stanford University (graduate level course co-directed by HMB and Garry Nolan)

- 2000, Spring Pharmacology 241: Course and Seminar Series on "Gene Therapy for Diseases of Aging" (graduate level course co-directed by HMB, Ellen Porzig and Garry Nolan)
- 1980-present: Guest lecturer at Stanford University in *Department of Biology*: in Regulatory Biochemistry in Higher Eukaryotes (248) and in Organismal Development (108); in *Department of Genetics*: in Human Genetics (202) and Somatic Cell Genetics (212); in *Department of Cell Biology*: in Cell Motility (225); in *Department of Molecular and Cellular Physiology*: in Pathophysiology (206); in *Cancer Biology Program*: in Signal Transduction Pathways in Development and Cancer (251) and Gene Regulation and Cell Differentiation (252); and in the *Human Biology Program*: in Human Biology
- 1978-2001: Pharmacology 201, 202: Pharmacology for Medical Students, Stanford University (taught by entire Pharmacology Department faculty; lectures by HMB on anticancer agents, anticoagulants, pharmacogenetics, teratogenesis, gout, chemical contraception, and gene therapy)
- 2001, Winter: Bio 2: Sophomore Lecture Series (faculty members lecture to undergraduates to inform them about careers in sciences and areas of research)
- 2002-03, Spring: Microbiology & Immunology 231: Course and Seminar Series on "Stem Cells and Gene Therapy," Stanford University (graduate level course co-directed by HMB and Garry Nolan)
- 2004, Winter Microbiology & Immunology 210: Pathogenesis of Bacteria, Viruses and Eukaryotic Parasites (taught by Microbiology and Immunology Department faculty; lecture by HMB on retroviruses in gene therapy)

Publications:

Papers published in refereed scientific journals

1. Blau, H.M. and Kafatos, F.C. (1978) Secretory kinetics in the follicular cells of silkmoths during eggshell formation. *J. Cell Biol.* **78**:131-151.
2. Blau, H.M. and Kafatos, F.C. (1979) Morphogenesis of the silkmoth chorion: Patterns of distribution and insolubilization of the structural proteins. *Devel. Biol.* **72**:211-225.
3. Blau, H.M. and Epstein, C.J. (1979) Manipulation of myogenesis *in vitro*: Reversible inhibition by DMSO. *Cell* **17**:95-108.
4. Blau, H. M. and Webster, C. (1981) Isolation and characterization of human muscle cells. *Proc. Natl. Acad. Sci., USA* **78**:5623-5627.
5. Blau, H.M., Kaplan, I., Tao, T.W. and Kriss, J.P. (1983) Thyroglobulin-independent, cell-mediated cytotoxicity of human eye muscle cells in tissue culture by lymphocytes of a patient with Graves' Ophthalmopathy. *Life Sci.* **32**:45-53.
6. Blau, H.M., Webster, C., Chiu, C.-P., Guttman, S. and Chandler, F. (1983) Differentiation properties of pure populations of human dystrophic muscle cells. *Exp. Cell Res.* **144**:495-503.

7. Gunning, P., Ponte, P., Okayama, H., Engel, J., Blau, H.M. and Kedes, L. (1983) Isolation and characterization of full-length cDNA clones for human alpha-, beta-, and gamma-actin mRNAs: Skeletal but not cytoplasmic actins have an amino terminal cysteine that is subsequently removed. Mol. Cell. Biol. 3:787-795.
8. Blau, H.M., Chiu, C.-P. and Webster, C. (1983) Cytoplasmic activation of human nuclear genes in stable heterokaryons. Cell 32:1171-1180.
9. Blau, H.M., Webster, C. and Pavlath, G.K. (1983) Defective myoblasts identified in Duchenne muscular dystrophy. Proc. Natl. Acad. Sci., USA 80:4856-4860.
10. Ponte, P., Gunning, P., Blau, H.M. and Kedes, L. (1983) Human actin genes are single copy for α -skeletal and α -cardiac actin but multicopy for β - and γ -cytoskeletal genes: 3' untranslated regions are isotype specific, but are conserved in evolution. Mol. Cell. Biol. 3:1783-1791.
11. Gunning, P., Ponte, P., Blau, H.M. and Kedes, L. (1983) Alpha-skeletal and alpha-cardiac actin genes are co-expressed in adult human skeletal muscle and heart. Mol. Cell. Biol. 3:1985-1995.
12. Chiu, C.P. and Blau, H.M. (1984) Reprogramming cell differentiation in the absence of DNA synthesis. Cell 37:879-887.
13. Bains, W., Ponte, P., Blau, H. and Kedes, L. (1984) Cardiac actin is the major actin gene product in skeletal muscle cell differentiation *in vitro*. Mol. Cell. Biol. 4:1449-1453.
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Books

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Committee on Ethics Publications

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Invited Chapters

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5. Blau, H.M., Chiu, C.-P., Pavlath, G.K. and Webster, C. (1985) Muscle gene expression in heterokaryons. In Gene Expression in Muscle (R.C. Strohman and S. Wolf, eds.), Plenum Press, New York, pp. 231-247.

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35. Ozawa, C.R., Springer, M.L. and Blau, H.M. (2000). A novel means of drug delivery: myoblast-mediated gene therapy and regulatable retroviral vectors. In Annual Review of Pharmacology and Toxicology 40. (A.K. Cho, ed.) Annual Reviews, Inc., Palo Alto, pp. 295-317.
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40. Brazelton, T.R. and Blau, H.M. (2004) Plasticity of circulating adult stem cells. In Lung Development and Regeneration, (D. Massaro, ed.) Marcel Dekker, Inc., New York, NY, pp. 217-246.
41. LaBarge, M.A. and Blau, H.M. (2004) Skeletal muscle stem cells. In Handbook of Stem Cells, Vol. 2: Adult and Fetal Stem Cells, (R. Lanza, H. Blau, et al., eds.) Academic Press, Elsevier, Burlington, MA, pp. 395-403.
42. Blau, H.M. (2005) Adult stem cells: prospects for reprogramming bone marrow derivatives in stable heterokaryons. In Commemorative volume of the 400th Anniversary of the Pontifical Academy of Sciences, *in press*.

Invited Presentations (1990-present):

- 1990** Special Lecture in Molecular and Cellular Approaches to Cardiovascular Diseases, Program of Excellence in Molecular Biology, University of California, San Francisco, CA
Plenary Speaker, Inaugural Symposium MGH Cancer Center on Cell Cell Interactions and Cell Fate Decisions, Boston, MA
Seminar, Department of Cellular and Developmental Biology, Harvard University, Cambridge, MA
Seminar, Department of Biology, Princeton University, Princeton, NJ
Plenary Speaker, The Fifth ICN-UCI Symposium on Molecular Aspects of Development, University of California, Irvine, CA
Plenary Speaker, UCLA/Keystone Symposium on Tissue Engineering Session Chair, Muscle Replacement and Maintenance, Keystone, CO
Special Lecture, 15th International Cancer Congress, Hamburg, Germany
Co-Chair with Michael Karin, FASEB Meeting, Regulation of Tissue Specific Gene Expression, Copper Mountain, CO
Plenary Speaker, Symposium on Molecular Genetics and Future Concepts of Therapy, VII International Congress on Neuromuscular Diseases, Munich, Germany
Jacques Monod Conference, Cell Interactions and Cell Differentiation, Toulon, France
Chair and Speaker, Minisymposium, American Society for Cell Biology, Control of Developmental Decisions in Muscle, San Diego, CA
- 1991** Co-organizer with Alan Kelly, and Plenary Speaker, ICN-UCLA Symposium: Gene Expression in Neuromuscular Development, Keystone, CO
Plenary Speaker, Graduate Student Symposium, Department of Molecular, Cellular and Developmental Biology, Boulder, CO
Distinguished Lecture Series on Molecular Biology in Medicine, Rutgers University, Piscataway, NJ
Bing Luncheon, Stanford Medical Development Lecture Series, Los Angeles, CA
Gene Therapy Program, Howard Hughes Medical Institute, Departments of Internal Medicine and Human Genetics, University of Michigan Medical Center, Ann Arbor, MI
Seminar, Department of Developmental and Cell Biology, University of California, Irvine, CA
Biochemistry Department Mini-course on the Mouse as an Experimental System: New Methods of Manipulation, University of California, San Francisco, CA
Plenary Speaker, International Symposium: Biotechnology of Growth Factors, Milano, Italy
Seminar, Department of Anatomy and Cell Biology, Harvard Medical School, Boston, MA
Seminar, Department of Biology, Harvard University, Cambridge, MA
Keynote Address, Gordon Conference on Neural Plasticity, Heniker, NH
Plenary Speaker, Symposium of Society of Experimental Biology, Birmingham, England
Gene Therapy Conference, Muscular Dystrophy Association, Tucson, AZ
Stanford Centennial, Stanford University, Stanford, CA
Delegation of U.S. National Academy of Sciences; Plenary Speaker, Joint Symposium on Gene Expression and Gene Regulation, Shanghai, China
Plenary Speaker, International Conference on the Molecular and Cellular Biology of the Cardiac Myocyte, American Heart Association, Asilomar, CA
Plenary Speaker, The First IMSUT International Symposium for Biomedical Research: Growth factors, signal transduction, and development, Tokyo, Japan
- 1992** Plenary Speaker, Keystone Symposium: Transcriptional Regulation in Development, Differentiation and Disease, Tamarron, CO

Seminar, Cell and Molecular Biology, Lawrence Berkeley Laboratory, Berkeley, CA
Plenary Speaker, Keystone Symposium: Growth and Differentiation Factors in Vertebrate Development, Keystone, CO
Pediatric Grand Rounds, Stanford University, Stanford, CA
Medicine Grand Rounds, Stanford University, Stanford, CA
Plenary Speaker, Women in Medicine Conference, Stanford University, Stanford, CA
Plenary Speaker FASEB Meeting: Transcriptional Regulation: Differentiation, Development and Disease, Copper Mountain, CO
Plenary Speaker, 5th International Congress on Cell Biology, Madrid, Spain
Plenary Speaker, EMBO Workshop-Molecular Biology and Pathology of Skeletal and Cardiac Myogenesis, Sardinia, Italy
Plenary Speaker, Gene Therapy, IBC Conference, Washington, D.C.
Plenary Speaker, Henry Harris Symposium: The legacy of cell fusion, Oxford, U.K.
Plenary Speaker, Symposium on Modern Research, Convocation of Queen Lane Building, Medical College of Pennsylvania, PA
Plenary Speaker, Oncogene Science Transcription Symposium, Cold Spring Harbor, NY
Plenary Speaker, Johnson & Johnson Gene Therapy Bio-Science Advisory Committee Meeting, New Brunswick, NJ

1993 Plenary Speaker, Keystone Symposium: Transcription Factors, Regulation and Differentiation, Keystone, CO
Plenary Speaker, Developmental Biology of the Cardiovascular System, Taos, NM
Dean's Distinguished Speaker, University of Colorado Health Sciences Center, Denver, CO
Plenary Speaker, Keystone Symposium: Gene Therapy, Keystone, CO
Gordon Conference on Biological Structure and Gene Expression, Volterra, Italy
Plenary Speaker, 4th International Symposium, Center for Biotechnology, Gene Transfer and Gene Therapy, Huddinge, Sweden
Plenary Speaker, ALZA 25th Anniversary Symp: Biological Delivery Systems, Stanford, CA
Gordon Conference: Developmental Biology, Proctor Academy, Andover, NH
Chair and Plenary Speaker, Symposium on Cell-Mediated Gene Therapy: A Novel Form of Drug Delivery, American Society for Pharmacology and Experimental Therapeutics, Annual Meeting, San Francisco, CA
Plenary Speaker, Conference on Delivery of Protein Drugs, The Next Ten Years, Kyoto, Japan (Represented by postdoctoral fellow, Dr. J. Dhawan)
Plenary Speaker, University of Chicago Cardiovascular Research Institute Symposium on Gene Therapy, Chicago, IL
Plenary Speaker, Gene Diagnosis and Gene Therapy, German Cancer Research Center, Heidelberg, Germany
Plenary Speaker, The Robert Steel Foundation International Symposium, Memorial Sloan-Kettering Cancer Center, New York, NY
Seminar, La Jolla Cancer Research Foundation, La Jolla, CA
3rd Annual University of California, San Diego American Heart Association Bugher Foundation Seminar Series, La Jolla, CA

1994 Seminars in Biology, University of California, San Francisco, CA
Seminar, Gladstone Institute Seminar Series, San Francisco, CA
Plenary Speaker, Keystone Symposium: Molecular Biology of Human Genetic Disease and Gene Therapy, Copper Mountain, CO
Keynote Speaker, West Coast Developmental Biology Conference, Marshall, CA
Seminar, University of Chicago Seminar Series, Dept. of Molecular Genetics & Cell Biology and Biochemistry & Molecular Biology, Chicago, IL
Plenary Speaker, 16th Annual Symposium: La Jolla Cancer Research Foundation, Stem Cells: Biology and Clinical Utility, La Jolla, CA

Session Chair and Plenary Speaker, Keystone Symposium: Molecular Biology of Muscle Development, Snowbird, UT
Plenary Speaker, American Association for Cancer Research 85th Annual Meeting: Transcriptional Control of Cell Proliferation and Differentiation, San Francisco, CA
McClintock Lecture, Cell and Molecular Biology, University of British Columbia, Canada
Plenary Speaker, 52nd Annual Meeting of the Society for Developmental Biology, University of Wisconsin, Madison, WI
Seminar, Neuromuscular Research Centre, St. Vincent's Hospital, Melbourne, Australia
Plenary Speaker, Gene Therapy Symposium, Cold Spring Harbor, NY
Keynote Address, Society for Basic Urologic Research, Stanford, CA
Seminar, Interdepartmental Seminar Series, UCLA School of Medicine, Los Angeles, CA
Seminar, Cardiovascular Research Center, Massachusetts General Hospital, Charlestown, MA
Seminar, Department of Cell Biology, Harvard Medical School, Boston, MA
Seminar, Department of Cell Biology, Univ. of Massachusetts Medical Center, Worcester, MA
Plenary Speaker, Cellular Disease Mechanisms and Therapeutic Strategies, American Society for Cell Biology 34th Annual Meeting, San Francisco, CA

- 1995** Seminar: Department of Biochemistry and Molecular Biology, MD Anderson Cancer Center, Houston, TX
Plenary Speaker, Gene Therapy: Moving From The Lab To The Clinic, Strategic Research Institute Conference, San Diego, CA
Session Chair and Plenary Speaker, Keystone Symposium: Gene Therapy and Molecular Medicine, Steamboat Springs, CO (Represented by postdoctoral fellow, Dr. M. Springer)
Special Lectures, Marie Curie Amphitheatre, Institut Curie, Paris, France (series of four lectures (1) gene regulation of the MyoD family of bHLH regulators, (2) gene and cell therapy for Duchenne Muscular Dystrophy, (3) myoblast-mediated gene therapy for nonmuscle disorders, (4) the role of riboregulators in development)
Special Lecture, Institut Pasteur, Paris, France
Special Lecture, Rhone Poulenc Rorer, Inc., Paris, France
Special Lecture, College de France, Institut d'Embryologie Cellulaire et Moleculaire, Nogent sur-Marne, Paris, France
Special Lecture, College de France, Laboratoire de Biochimie Cellulaire, Il Place Marcelin Berthelot, Paris, France
Plenary Speaker, 1st European Molecular Biology Laboratory Conference (ECBO): Cell Biology of Development, Heidelberg, Germany
Seminar, Differentiation Programm, EMBL, Heidelberg, Germany
Co-organizer with N. Rosenthal and Plenary Speaker, 54th Annual Meeting of the Society for Developmental Biology: Genes, Development, Cancer, San Diego, CA
Visiting Professor and Special Lectureship, Southwestern Medical Foundation For Women in Science and Medicine, University of Texas, Dallas, TX
Plenary Speaker, San Francisco Symposium 95: Translation and Stability of mRNA, San Francisco, CA
19th Annual Mildred Trotter Lecture, Washington University School of Medicine, St. Louis, MO
Plenary Speaker, Pennsylvania Muscle Institute Retreat and Symposium, University of Pennsylvania Medical Center, Philadelphia, PA
Organizer, ALZA Gene Therapy Workshop, Palo Alto, CA
Special Seminar, Wellcome/CRC Institute, Cambridge, England
Medical Nobel Committee, Special Lectures, Karolinska Research Series, Stockholm, Sweden

- 1996** Session Chair and Plenary Speaker, Keystone Symposium: Molecular Biology of the Cardiovascular System, Keystone, CO
Banbury Conference Meeting: Cellular and Molecular Biology of Mesenchyme, Cold Spring Harbor, NY
Distinguished Lecture Series, Division of Biological Sciences, Harvard School of Public Health, Boston, MA
Seminar, Cancer Center, Massachusetts Institute of Technology, Boston, MA
Seminar, Cell Biology Department, Duke University Medical Center, Durham, NC
Office of Medical Development Special Program: Gene Therapy - Challenge and Promise of a New Frontier, Stanford University, Stanford, CA
Seminar, Genetics and Molecular Medicine Seminar Series, Emory University, Atlanta, GA
Honors Program Lecture, New York University School of Medicine, New York, NY
Plenary Speaker, Stanford's Beckman Center 7th Annual Program in Molecular Genetic Medicine Symposium: Genetics to Gene Therapy for Complex Cardiovascular and Metabolic Disease, Stanford University, Stanford, CA
Plenary Speaker, Conference on Gene Therapy, Genomic Science Series, Hilton Head, SC
Seminar, UCLA School of Medicine, Los Angeles, CA
Plenary Speaker on Gene Therapy, Oncology Conference, Institut Curie, Paris, France
Basement Membrane Gordon Conference, Henniker, NH
Plenary Speaker, Human Gene Therapy Meeting, Cold Spring Harbor, NY (Represented by postdoctoral fellow, Dr. A. Hofmann)
Plenary Speaker, Third International Congress of the Cell Transplant Society, Miami, FL (Represented by postdoctoral fellow, Dr. M. Springer)
Session Co-chair and Plenary Speaker of the "RNA World", Sixth International Congress on Cell Biology, San Francisco, CA
- 1997** Co-Organizer with James Wilson, Keystone Meeting: Molecular and Cellular Biology of Gene Therapy, Snowbird, UT
Plenary Speaker, Keystone Meeting: Molecular Biology of Muscle Development, Snowmass, CO
Plenary Speaker, Jacques Monod Conference, Aussois, France (Represented by postdoctoral fellow, Dr. A. Spicher)
EMBO Workshop on Viral Vectors in Basic Biology, Heidelberg, Germany (Represented by postdoctoral fellow, Dr. O. Guicherit)
Invited Speaker, Bellagio Study Center of Rockefeller Foundation, Lake Como, Italy
Plenary Speaker, Presidential Symposium, American College of Neuropsychopharmacology, Hawaii
- 1998** Plenary Speaker, Keystone Meeting: Molecular and Cellular Biology of Gene Therapy, Keystone, CO
Plenary Speaker, Symposium for Molecular Medicine: The Road Toward Human Gene Therapy, University of California, San Diego, CA
Plenary Speaker, Barbara H. Bowman Memorial Symposium, The University of Texas Health Science Center at San Antonio, TX
Moderator and Session Chair, First Annual Meeting of the American Society of Gene Therapy, Seattle, WA
Plenary Speaker, 57th Annual Meeting, American Society for Developmental Biology, Stanford, CA
Invited Speaker, Gordon Research Conference on Myogenesis, Tilton, NH
Plenary Speaker, Molecular Interaction Technologies '98, San Francisco, CA
Special Invited Lecture, Amgen, Inc., Thousand Oaks, CA

- 1999** Plenary Speaker, Keystone Symposium on Molecular and Cellular Biology of Gene Therapy, Salt Lake City, UT
Invited Speaker, Chair Search Committee for Pharmacology Department, Cancer Center, Yale University, New Haven, CT
Invited Speaker, Cardiovascular Medicine Research & Clinical Conferences (CVRC), “Vascular Endothelial Growth Factor (VEGF): Problems and Prospects for Gene Therapy”, Stanford University
Invited Speaker, Conference on Stem Cells, Banbury Center, Cold Spring Harbor Laboratory, NY
Medical Scientist Training Program Invited Lecturer, Medical College of Wisconsin, Milwaukee, WI
Invited Speaker, American Society for Pharmacology and Experimental Therapeutics (ASPET) Teaching Institute on Pharmacologists of the Future, Washington, DC
Invited Speaker, American Society for Pharmacology and Experimental Therapeutics (ASPET) Women in Pharmacology Special Lecture (FASEB Excellence in Science Award), Wash., DC
Plenary Speaker, North American Vascular Biology Organization (NAVBO), Tissue Remodeling in Angiogenesis Symposium, Washington, DC
Distinguished Woman in Medicine and Science Lecturer, Northwestern University, Chicago, IL
Keynote Speaker, Student Research Forum, Oregon Health Sciences University, Portland, Oregon
Invited Speaker, Molecular, Cellular and Developmental Biology Department, California Institute of Technology, Pasadena, CA
Invited Speaker, Prescription for Discovery, Pharmaceutical Seminar Series, Perkin Elmer Applied Biosystems, San Francisco, CA
Plenary Speaker, Symposium on Cardiovascular Diseases, American Society of Gene Therapy (ASGT), Washington, DC
Chair, Symposium on Integrating Viral Vectors, American Society of Gene Therapy (ASGT), Washington, DC
Invited Speaker, Gordon Research Conference on Wound Repair, Colby-Sawyer College, New London, NH
Invited Speaker, Institute of Medicine (IOM) Distinguished Lecturer, Ann Arbor, MI
Plenary Speaker, 7th Annual Retreat, Institute for Human Gene Therapy, Atlantic City, NJ
FASEB Excellence in Science Award Lecture, American Society for Cell Biology (ASCB) Annual Meeting, Washington, DC
Plenary Speaker, Gene Therapy: Delivering the Medicines of the 21st Century Conference, Nature Biotechnology Conference, Washington, DC
Invited Speaker, Division of Cell and Molecular Medicine Seminar, UCSD, CA
- 2000** Plenary Speaker, New Opportunities in Aging Research in 2000 and Beyond, Gerontology Society of America, San Francisco, CA
Plenary Speaker, 22nd Princeton Conference on Cerebrovascular Disease, Redwood City, CA
Invited Speaker, NIH Director’s Wednesday Afternoon Lecture Series for 1999/2000, Washington, DC
Plenary Speaker, 8th European Society of Gene Therapy (ESGT), Stockholm, Sweden
Plenary Speaker, Keystone Symposia: Gene Therapy: The Next Millennium
Plenary Speaker, Keystone Symposia: Molecular Biology of the Cardiovascular System
Plenary Speaker, 22nd Princeton Conference on Cerebrovascular Disease, Redwood City, CA
Biosciences Distinguished Lecturer, Lawrence Berkeley National Laboratory, Berkeley, CA

- Plenary Speaker, 5th Ben May Inst. for Cancer Research Symposium, Univ. Chicago
Plenary Speaker, Molecular Biology of Muscle Development and Disease, Asilomar, CA
Keynote Speaker, 25th Annual Meeting, Society of Cardiovascular and Interventional Radiology, San Diego, CA
Plenary Speaker, 3rd Annual Meeting, American Society of Gene Therapy, Denver, CO
Keynote Speaker, BAM'2000, The Sixth Abano Terme Meeting on Rehabilitation, Padova, Italy
Invited Speaker, Department of Cell Biology, Biological Chemistry and Molecular Pharmacology and Institute of Chemistry and Cell Biology, Harvard University School of Medicine (2000)
- 2001** Plenary Session Chair and Speaker, Gene Therapy 2001: A Gene Odyssey, Keystone Symposium, Utah
Co-Chair and Speaker, Stem Cell Minisymposium, 41st Meeting of the American Society for Cell Biology (ASCB), Washington, DC
Broadhurst Distinguished Lecturer, Harvard University, Cambridge, MA
Keynote Speaker, SBF 488 Symposium: Neural and Non-Neural Stem Cells, Heidelberg, Germany
Plenary Speaker, Gordon Research Conference on Angiogenesis and Microcirculation, Salve Regina University, Newport, RI
Plenary Speaker, Myogenesis, Gordon Research Conference, Il Ciocco, Italy
- 2002** Mayer Lecture in the Life Sciences, Massachusetts Institute of Technology, Cambridge, MA
Mary Elizabeth Garrett Lecturer, Johns Hopkins University School of Medicine, Baltimore, MD
Catherine N. Stratton Lecturer on Critical Issues, Whitehead Institute, Massachusetts Institute of Technology, MA
President's (Harold Varmus) Research Seminar Series Lecturer, Memorial Sloan Kettering Cancer Center, NY
- 2003** Plenary Speaker, British Society for Developmental Biology, Spring Meeting, University of Warwick, England
Plenary Speaker, Perlmutter Family Symposium on Neurodegenerative Disorders, Harvard University School of Medicine
2003 Annual Scientist in Medicine Lecture, University of Washington
Rolf-Sammet-Fonds Visiting Professorship, University of Frankfurt
Honorary Doctorate, University of Nijmegen, Holland
Speaker, Academie des Sciences de la France at Pontifical Academy, the Vatican in Rome, "Stem Cells and Cloning"
- 2004** Plenary Speaker, XIIIth International Vascular Biology Meeting, Toronto, Canada
Plenary Speaker, 2nd Annual International Society for Stem Cell Research Meeting, Boston, MA
Discussion Leader and Plenary Speaker, Myogenesis, Gordon Research Conference, Il Ciocco, Italy
President's Lecture, 13th International Society of Differentiation Conference, Honolulu, HI
Keynote Speaker, Allergan Foundation Lectures in Modern Biology and the Howard A. Schneiderman Memorial Bioethics Lecture Series, University of California, Irvine
- 2005** Louis A. Bloomfield Lecturer, Case Western Research University School of Medicine, Cleveland, OH