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## BIOGRAPHICAL SKETCH

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NAME <b>Helen M. Blau, Ph.D.</b>		POSITION TITLE Donald E. and Delia B. Baxter Professor Director, Baxter Laboratory for Stem Cell Biology	
eRA COMMONS USER NAME <b>BLAU.HELEN</b>			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
University of York, York, England	B.A.	1969	Biology
Harvard University, Cambridge, MA	M.A.	1970	Biology
Harvard University, Cambridge, MA	Ph.D.	1975	Biology
University of California, San Francisco, CA	Postdoctoral	1975-1978	Medical Genetics/ Biochem. & Biophysics

### A. Positions and Honors.

1969-1975 Predoctoral Fellow, Department of Biology, Harvard University, Cambridge, MA  
1975-1978 Postdoctoral Fellow, Dept. of Biochem. & Biophysics, Divis. of Medical Genetics, U.C.S.F., CA  
1978-1986 Assistant Professor, Molecular Pharmacology, Stanford University School of Medicine, CA  
1986-1991 Associate Professor, Molecular Pharmacology, Stanford University School of Medicine, CA  
1991-2002 Professor, Molecular Pharmacology, Stanford University School of Medicine, CA  
1997-2002 Chair, Molecular Pharmacology, Stanford University School of Medicine, CA  
1997-present Director, Gene Therapy Technology, Stanford University School of Medicine, CA  
2002-present Professor, Microbiology and Immunology, Stanford University School of Medicine, Stanford, CA  
2002-present Director, Baxter Laboratory for Stem Cell Biology, Stanford University School of Med., CA  
2005-present Faculty Affiliate/Member, Bio-X Program, Cancer Center, Stanford Institute for Stem Cell Biology and Regenerative Medicine, Stanford University School of Medicine, Stanford, CA

### Honors:

Basil O'Connor Starter Research Award (1978-81); Mellon Foundation Faculty Fellow (1979-80); William M. Hume Faculty Scholar (1981-84); Research Career Development Award, National Institutes of Health (1984-89); SmithKline and Beecham Junior Faculty Scholar Award (1989-1991); election as Fellow of the American Association for the Advancement of Science (1991); Senior WICB Career Recognition Award of the American Society for Cell Biology (1992); President, Society for Developmental Biology (1994-1995); Yvette Mayent-Rothschild Visiting Professorship, Instituts Curie and Pasteur, Paris (1995); NIH MERIT Award (1995-2005); election to Institute of Medicine of the National Academy of Sciences (1995); Nobel Forum Lecture, Karolinska Inst., Sweden (1995); election to American Academy of Arts and Sciences (1996); National Advisory Council, NIH Institute on Aging (1996-2000); FASEB Excellence in Science Award (1999); Donald E. and Delia B. Baxter Endowed Professor (1999-present); NIH Director's Lecture (2000); McKnight Technological Innovations for Neuroscience Award (2001); Council Member, American Society for Cell Biology (2002-2004); President, International Society of Differentiation (2002-2004); Honorary Doctorate, University of Nijmegen, Holland (2003); Council Member, Institute of Medicine (IOM) (2003-2009); Harvard Board of Overseers (2004-2010); Member, NIH Committee to Review Biology of Aging Program for the National Institute of Aging (summer and fall, 2005); Scientific Advisory Board, Ellison Medical Foundation (2007-2011); Fulbright Senior Scholar Award (2007); NIH Challenge Grant (2009-2011).

### B. Selected publications (from +200 publications).

LaBarge, M.A. and Blau, H.M. (2002) Biological progression from adult bone marrow to mononucleate muscle stem cell to multinucleate muscle fiber in response to injury. *Cell* 111:589-601.  
Weimann, J.M., Charlton, C.A., Brazelton, T.R., Hackman, R.C. and Blau, H.M. (2003) Contribution of transplanted bone marrow cells to purkinje neurons in human adult brains. *Proc. Natl. Acad. Sci.* 100:2088-2093. PMID: PMC149963.

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- Springer, M.L., Ozawa, C.R., Banfi, A., Kraft, P.E., Ip, T.K., Brazelton, T.R. and Blau, H.M. (2003) Localized arteriole formation directly adjacent to the site of VEGF-induced angiogenesis in muscle. Mol. Therapy **7**:441-9.
- von Degenfeld, G., Banfi, A., Springer, M.L. and Blau, H.M. (2003) Myoblast-mediated gene transfer for therapeutic angiogenesis and arteriogenesis. Brit. J. Pharm. **140**:620-626. PMID: PMC1574078.
- Weimann, J.M., Johansson, C.B., Trejo, A. and Blau, H.M. (2003) Stable reprogrammed heterokaryons form spontaneously in Purkinje neurons in adult brain after BMT. Nature Cell Biol. **5**(11):959-966.
- Corbel, S.Y., Lee, A., Yi, L., Duenas, J., Brazelton, T.R., Blau, H.M. and Rossi, F.M.V. (2003) Contribution of hematopoietic stem cells to skeletal muscle. Nature Medicine **9**(12):1528-1532.
- Jacobi, J., Tam, B.Y.Y., Sundram, U., von Degenfeld, G., Blau, H.M. Kuo, C. J. and Cooke, J.P. (2004) Discordant effects of a soluble VEGF receptor on wound healing and angiogenesis. Gene Therapy **11**:302-309.
- Jacobi, J., Sydow K., von Degenfeld, G., Zhang, Y., Hayan Dayoub, H., Bingyin Wang B., Patterson, A. J., Kimoto, M., Blau, H.M., and Cooke, J.P. (2004) Overexpression of dimethylarginine dimethylaminohydrolase (DDAH) reduces tissue ADMA levels and enhances angiogenesis. Circulation **111**(11):1421-1438.
- Ozawa, C.R., Banfi, A., Glazer, N., Thurston, G., Springer, M.L., Kraft, P.E., McDonald, D.M. and Blau, H.M. (2004) Microenvironmental VEGF concentration, not total dose, determines a threshold between normal and aberrant angiogenesis. J. Clin. Invest. **113**(4):516-527. PMID: PMC338257.
- Pomerantz, J. and Blau, H.M. (2004) Nuclear reprogramming: A key to regenerative medicine. Nature Cell Biol. **6**:810-816.
- Sen, G., Wehrman, T.S., Myers, J. and Blau, H.M. (2004) Restriction enzyme-generated siRNA (REGS) vectors and libraries. Nature Genetics **36**(2):183-189.
- Palermo, A.T., LaBarge, M.A., Doyonnas, R., Pomerantz, J. and Blau, H.M. (2005) Bone marrow contribution to skeletal muscle: a physiological response to stress. Devel. Biol. **279**:336-344.
- Sen, G. and Blau, H.M. (2005) Argonaute 2/RISC resides in cytoplasmic bodies, sites of mammalian mRNA decay. Nature Cell Biol. **7**(6):633-636.
- Doyonnas, R., LaBarge, M.A., Sacco, A., Charlton, C. and Blau, H.M. (2004) Hematopoietic contribution to skeletal muscle regeneration by myelomonocytic precursors. PNAS **101**(37):13507-12. PMID: PMC518787.
- Banfi, A., von Degenfeld, G. and Blau, H.M. (2005) Critical role of microenvironmental factors in angiogenesis. Curr. Atheroscler. Rep. **7**(3):227-234.
- Wehrman, T.S., Casipit, C.L., Gewertz, N.M. and Blau, H.M. (2005) Enzymatic detection of protein translocation. Nature Methods **2**(7):521-527.
- Sacco, A., Doyonnas, R., LaBarge, M.A., Hammer, M.M., Kraft, P. and Blau, H.M. (2005) IGF-I increases bone marrow contribution to adult skeletal muscle and enhances the fusion of myelomonocytic precursors. J. Cell Biol. **171**(3):483-492. PMID: PMC2171272.
- Wehrman, T.S., von Degenfeld, G., Krutzik, P. O., Nolan, G.P. and Blau, H.M. (2006) Luminescent imaging of  $\beta$ -galactosidase activity in living subjects using sequential reporter-enzyme luminescence. Nature Methods **3**(4):295-301.
- Kutschka, I., Chen, I.Y., Kofidis, T., Arai, T., von Degenfeld, G., Sheikh, A.Y., Hendry, S.L., Pearl, J., Hoyt, G., Sista, R., Yang, P.C., Blau, H.M., Gambhir, S.S. and Robbins R.C. (2006) Collage matrices enhance survival of transplanted cardiomyoblasts and contribute to the improvement of ischemic rat hearts. Circulation **114**(1 Suppl):1167-1173.
- Kutschka, I., Kofidis, T., Chen, I.Y., von Degenfeld, G., Zwierzchoniewska, M., Hoyt, G., Arai, T., Lebl, D.R., Hendry, S.L., Sheikh, A.Y., Cooke, D.T., Connolly, A., Blau, H.M., Gambhir, S.S. and Robbins R.C. (2006) Adenoviral human BCL-2 transgene expression attenuates early donor cell death after cardiomyoblast transplantation into ischemic rat hearts. Circulation **114** (1 Suppl):1174-1180.
- Sen, G.L. and Blau, H.M. (2006) A brief history of RNAi: the silence of the genes. FASEB J. **20**(9):1293-1299. PMC Journal – In Process.
- von Degenfeld, G., Banfi, A., Springer, M.L., Wagner, R.A., Jacobi, J., Ozawa, C.R., Merchant, M.J., Cooke, J.P. and Blau, H.M. (2006) Microenvironmental VEGF distribution is critical for stable and functional vessel growth in ischemia. FASEB J. **20**(14):2657-9. PMC Journal – In Process.
- Wehrman, T.S., Raab, W.J., Casipit, C.L., Doyonnas, R., Pomerantz, J.H. and Blau, H.M. (2006) A system for quantifying dynamic protein interactions defines a role for Herceptin in modulating ErbB2 interactions. Proc. Natl. Acad. Sci. USA, **103**(50):19063-19068. PMID: PMC1748177.
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- Springer, M.L., Banfi, A., Ye, J., von Degenfeld, G., Kraft, P.E., Saini, S.A., Kapasi, N.K., and Blau, H.M. (2007) Localization of vascular response to VEGF is not dependent on heparin binding. FASEB J. 21 (Express article doi:10.1096/fj.06-7700com:1-12). PMC Journal – In Process.
- Kutschka, I., Chen, I.Y., Kofidis, T., von Degenfeld, G., Sheikh, A.Y., Hendry, S.L., Hoyt, G., Pearl, J., Blau, H.M., Gambhir, S.S. and Robbins, R.C. (2007) *In vivo* optical bioluminescence imaging of collagen-supported cardiac cell graphs. J. Heart and Lung Transplant. 26:3:273-280.
- Zhang, F., Pomerantz, J.H., Sen, G., Palermo, A.T., and Blau, H.M. (2007) Active tissue-specific DNA demethylatin conferred by somatic cell nuclei in stable heterokaryons. Proc. Natl. Acad. Sci. USA 104(11):4395-4400. PMID: PMC1838613.
- Blum, G., von Degenfeld, G., Merchant, M.J., Blau, H.M., Bogyo, M. (2007) Noninvasive optical imaging of cysteine protease activity using fluorescently quenched activity-based probes. Nature Chemical Biol. 3(10): 668-77.
- von Degenfeld, G., Wehrman, T.S., Hammer, M. and Blau, H.M. (2007) A universal technology for monitoring GPCR activation *in vitro* and non-invasively in live animals. FASEB J. 21:3819-3826. PMC Journal – In Process.
- Hammer, M.M., Wehrman, T.S., Blau, H.M. (2007) Novel enzyme complementation-based assays for G protein-coupled receptor signaling. FASEB J. 21:3827-3834. PMC Journal – In Process.
- Pajcini, K.V., Pomerantz, J.H., Alkan, O., Doyonnas, R. and Blau, H.M. (2008) Myoblasts and macrophages share molecular components that contribute to cell-cell fusion. J. Cell Biol 180:1005-1019. PMID: PMC2265408.
- Johansson, C.B., Youssef, S., Koleckar, K., Holbrook, C., Doyonnas, R., Corbel, S.Y., Steinman, L., Rossi, F.M.V. and Blau, H.M. (2008) Extensive fusion of haematopoietic cells with Purkinje neurons in response to chronic inflammation. Nature Cell Biol. 10(5): 575-583.
- Sacco, A., Doyonnas, R., Kraft, P., Vitorovic, S. and Blau H.M. (2008) Self-renewal and expansion of single transplanted muscle stem cells. Nature 456:502-506.
- Blau, H.M. (2008) Clinical implications for basic research: Cell therapies for Muscular Dystrophy (invited review). N. Engl. J. Med. 359(13):1410-1412.
- Lutolf, M.P., Doyonnas, R., Havenstrite, K., Koleckar, K. and Blau, H.M. (2009) Perturbation of single hematopoietic stem cell fates in artificial niches. Integrative Biology 1:59-69.
- Pomerantz, J.H., Mukherjee, S., Palermo, A.T. and Blau, H.M. (2009) Reprogramming to a muscle fate by fusion recapitulates differentiation. J. Cell Science 122(7):1045-1053.
- Palermo, A., Doyonnas, R., Bhutani, N., Pomerantz, J., Alkan, O. and Blau, H.M. (2009) Nuclear reprogramming in heterokaryons is rapid, extensive and bidirectional. FASEB J. 23:1431-1440. PMID: PMC Journal – In Process.

## C. Research Support

### Ongoing Research Support (Blau, H. M.)

NIH R37 AG009521-21 (Role: PI)

04/01/86-03/31/10

MERIT AWARD “Activators of Human Muscle Genes” The major goal of this grant is directed at a characterization of muscle gene activation at the molecular level. The focus is the cloning and characterization of genes encoding products that act at different points in the myogenic regulatory hierarchy.

NIH 5R01HL096113-01A1 (Blau)

04/01/09-02/28/14

“Regulation of Hematopoietic Stem Cell Fate in Bioengineered Niches” The specific aims of this proposal are 1) to study the influence of single extrinsic factors on HSC maintenance; 2) to investigate the mechanisms of action of candidate extrinsic factors on HSC self-renewal; and 3) to identify *in vitro* predictive parameters that correlate with HSC expansion and to use these parameters to screen a small protein library.

MDA4320 (Role: PI)

07/01/07-6/30/10

“A Novel Mouse Model for Duchenne Muscular Dystrophy” A limitation in the development of therapies for Duchenne muscular dystrophy (DMD) is the lack of mouse models that have the functional and pathological deficits typical of the human disease. In this proposal we test whether the DMD phenotype is not evident in the mouse model (mdx) because telomere function is not substantially impaired during the lifetime of the mouse.

Juvenile Diabetes Research Fdn. (Role: co-PI) 09/01/08-08/31/11  
"Directly Observed Development of Purified Human Pancreatic Progenitor Cells" Project 2 (PI Blau) The major goal of this project is to investigate mechanisms of regulating proliferation and multipotency of isolated multipotent human progenitor cells with the aim of discovering mechanisms that allow expansion of multipotent pancreatic progenitor cells.

Leukemia and Lymphoma Society TRP (Role: co-PI) 10/01/08-09/30/11  
"Bioengineered Niches to Control Human Follicular Lymphoma Cells" The major goals of this proposal are to identify extrinsic factor that influence FL cancer cell survival and propagation *in vitro* and to test the cancer stem cell hypothesis.

CIRM RT1-01001-1 (Role: PI) 04/01/09-03/31/11  
"Regulation of Stem Cell Fate in Bioengineered Arrays of Hydrogel Microwells" The goal of this proposal is to identify extrinsic factors with a role in muscle stem cell fate using a novel bioengineered culture platform *in vitro* and to monitor muscle stem cell function using a non-invasive bioluminescence *in vivo* assay.

2R01AG020961-06A2 (Blau) 07/01/09-06/30/11  
NIH/NIA  
"Regulation of Muscle Stem Cell Fate" The specific aims of this proposal are 1) to identify microenvironmental factors that control stem cell fate *in vitro*; 2) to elucidate the mechanisms of stem cell self-renewal; and 3) to assess muscle stem cell function using a non-invasive *in vivo* assay.

RB1-01292-1 (Blau) 09/01/09-08/31/12  
California Institute of Regenerative Medicine  
"Molecular Mechanisms of Reprogramming towards Pluripotency" The goal of this proposal is to elucidate the molecular events underlying reprogramming of adult somatic cells toward a stem cell fate using a novel cell fusion based heterokaryon platform.

1RC1 AR059365-01 (Blau, co-P.I.: J. Santiago) 09/30/09-09/29/11  
NIH/NIGMS Challenge Grant  
"Remote-control Mouse-implantable Micropumps for Establishment of Regenerative Capability" Our mouse-implantable remote-control micropumps enable drug delivery with physiologic temporal profiles.

1U01 HL100397-01 (P.I.: J. Cooke) 10/01/09-09/30/16  
NIH/NHLBI  
"Basic and Translational Research of iPSC-based Hematologic and Vascular Therapies" Dr. Blau is PI of Project 2: "Discovery of Novel Regulators to Enhance iPSC Derivation and Differentiation of EC" of this Consortium grant. The goal is to study nuclear reprogramming of somatic cells toward a human arterial endothelial cell (EC) fate; the regulators discovered in these experiments will be delivered as proteins to enhance iPSC generation and will be used in deriving arterial EC from iPSC more efficiently.

### **Completed Research Support**

SCL08-03 (Blau, co P.I.: J. Santiago) 11/1/08-10/31/09  
"Drug Delivery Micropump for Rejuvenation of Muscle Stem Cell Function in Old Mice"

NIH R01 AG024987-03 (Role: PI) 09/30/04-07/31/09  
"Regulators of Adult Stem Cell Fate"

NIH R01 EB005011 (Role: Co-Investigator) 09/30/05-07/31/09  
"Small Molecular Imaging of Cysteine Protease Activity"

Stanford Bio-X IIP3-34 (Role: PI) 10/01/06-09/30/08  
Bio-X Interdisciplinary Initiative Prog. - "Determination of Muscle Stem Cells in Bioengineered Artificial Niches"

NIH CCNE-TR Award (Role: PI on subaward) 12/1/07-4/30/08  
"Bioengineered Protein Nanoenvironments for Growth and Characterization of Human Lymphoma"

NIH R01 AG020961-04 (Role: PI) 05/15/02-04/30/08  
"Stem Cells for Brain and Brawn"

NIH R01 HD018179-24 (Role: PI) 01/24/03-12/31/07  
"Regulation of Muscle Development"

Novartis Institutes for Biomedical Research (Role: PI) 07/19/04-10/19/06  
"Stem Cell Differentiation"

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