

A. Identifying Data:

Name: Mark Allan Kay
Nationality: U.S.A.
Born: January 9, 1958

B. Academic History:

Education

1976-1980 B.S. Michigan State University
Physical Sciences
1980-1986 Ph.D. Case Western Reserve University
Developmental Genetics
1980-1987 M.D. Case Western Reserve University

Postgraduate Training

1987-1990 Internship and Residency, Baylor College of Medicine,
Houston, TX - Department of Pediatrics
1990-1993 Medical Genetics Clinical Fellowship, Baylor College of Medicine.
Post-doctoral research - Laboratory Director, Savio Woo, Ph.D.
Project - Gene Therapy for Hepatic Deficiencies

Educational Scholarships and Honors

Phi Kappa Phi Honorary Society - 1980
Arthur F. Hughes Memorial Award for Outstanding Research in Developmental Biology - 1986
The Upjohn Achievement Award - Excellence in Clinical Pharmacology – 1987
Henry Christian Award for Excellence in Research - American Federation for Clinical Research – 1992,
American Society of Human Genetics - student award for best paper in category of post-doctoral, basic
sciences - 1992

Board Certification

Diplomate of the American Board of Pediatrics - 1990-1997
Diplomate of the American Board of Medical Genetics in:

- 1) Clinical Biochemical Genetics -1993-2003
- 2) Clinical Genetics - 1993-2003

Clinical Trials

Phase I/II AAV-human factor IX mediated gene transfer into skeletal muscle 1998-1999 Co-PI;
1999-2001 Scientific Advisor
Phase I/II AAV-human factor IX mediated gene transfer into liver IND BB-9398 Holder 1/2001-1/2002;
Scientific advisor 2002-2005

C. Employment History:
Faculty Appointments

- 03/01/93- 06/30/93 Acting Assistant Professor, Department of Medicine
University of Washington
- 06/01/93 - 1994 Assistant Professor, Department of Medicine
Investigator, Markey Molecular Medicine Center
University of Washington
- 1994 Adjunct Assistant Professor, Department of Pediatrics
University of Washington
- 1995 Adjunct Assistant Professor, Department of Biochemistry
University of Washington
- 1995 Adjunct Assistant Professor, Department of Pathology
University of Washington
- 1997 - 07/31/98 Associate Professor of Medicine with adjuncts in Pediatrics, Biochemistry and Pathology, University of Washington
- 8/01/98 (with tenure) Associate Professor, Departments of Pediatrics and Genetics,
Stanford University School of Medicine
- 8/01/98 - present Director, Program in Human Gene Therapy,
Stanford University School of Medicine
- 5/01/01 - present Professor, Departments of Pediatrics and Genetics,
Stanford University
- 2/01/03 - 2004 Co-Founder and Chief Scientific Advisor of Avocel
- 10/01/05 - present Dennis Farrey Family Professor
- 4/2009 – present Associate Chair for Basic Research- Dept of Pediatrics

D. Public and Professional Service:
University Committees

University of Washington

- 1994-1998 Medical Scientist Training Program Steering Committee
- 1995-1997 Medical School Admissions

Stanford University

- 1998-1999 Children's Health Initiative - Genetics Subcommittee
- 1998-present Medical Scientist Training Program Steering Committee
- 1998-present Search Committees for 4 separate faculty positions
- 1999-9/2004 Dean's Fellowship Committee
- 1999-present Administrative Panel on Biosafety Committee

2000-present	Children's Health Initiative Grant Review Committee
2001-9/2003	Dean's Committee on Post-doctoral Affairs
2003-present	Department of Genetics Graduate School Admissions
2001-present	Berry Foundation Committee
2002-9/2005	Stanford University Faculty Senate
2002-9/2005	Faculty Senate Executive Committee
2006-present	Chairman, Berry Fellowship Committee

E. Honors and Awards:

Memberships in Professional Associations and Learned Societies

American Society of Human Genetics

American Academy of Pediatrics

American Association for the Advancement of Science

Western Society for Clinical Investigation

American Society of Gene Therapy

American Society of Microbiology

Japanese Society of Inherited Metabolic Disease- honorary member

Editorial Boards/Editorships

1. Editorial Board, *Gene Therapy*, March 1995-2007
2. Editorial Board, *Human Gene Therapy*, September 1995-present
3. Editorial Board, *Molecular Therapy*, August 1999-2003
4. Associate Editor, *Human Gene Therapy*, 2000-present
5. Associate Editor, *Molecular Therapy*, 2006-present
6. Associate Editor, *Silence* 2009-present

Other Scientific Leadership Roles

1. National Gene Vector Laboratory Scientific Review Board, March 1996-2002
2. Advisory Board for the Max Delbrück Center Sixth International Symposia of Gene Therapy, 1997-1998
3. Scientific planning board of the German-American Frontiers of Science sponsored by the National Academy of Science, 1997-1998
4. American Society of Gene Therapy - Board of Directors, 1997-2000
5. Ad hoc reviewer for the NIH, 1997-2000
6. Founding Board of Directors, American Society for Gene Therapy, 1997-2000
7. Co-organizer, 1999 Keystone Symposium on Gene Therapy

8. FDA-AAV working group related to planning platform studies and a shared drug master file for rare diseases, 1999-2000
9. European Society for Gene Therapy, Committee on Gene Therapy for Genetic Diseases, November 2000-November 2001
10. NIH Study Section Member - Medical Biochemistry, February 2000-January 2004
11. Co-organizer of 2001 Keystone meeting on Gene Therapy
12. IND Holder BB-9398 Intrahepatic AAV Gene Transfer for Hemophilia B, January-December 2001
13. Co-Organizer American Society of Microbiology Meeting on Viral Vectors, April 2001
14. Gene Therapy Working Group-National Hemophelia Foundation, June 2001-2003
15. Chair, Committee on Genetic Diseases-American Society for Gene Therapy, 2001-2003
16. Co-Organizer American Society of Microbiology Meeting on Viral Vectors, February 2002
17. Chair, Organizing Committee of the Gordon Conference on Viral Vectors for Gene Therapy, 2003-2004
18. Vice President of the American Society of Gene Therapy, 2003-2004.
19. President Elect of the American Society of Gene Therapy, 2004-2005.
20. President of the American Society of Gene Therapy, 2005-2006.
21. Vice President of Oligonucleotide Therapeutic Society 2009-2011.

Academic Honors and Awards

Western Society for Clinical Investigation, Young Investigator Award - February 1996
Arosenius Swedish Honorary Lectureship - 1997
American Society for Clinical Investigation-elected member - 1997
E. Mead Johnson Award for Pediatric Researcher of the Year - 2000
National Hemophelia Foundation Researcher of the Year - 2000
Named Professorship-Dennis Farrey Family Professor -2005

Invited Addresses

(Selected-excluding seminars at academic/industrial institutions – over 300 total)

1. Cold Spring Harbor Human Gene Therapy, Cold Spring Harbor, NY, October 1992.
2. Human Gene Therapy and Mutant Annual Models, Max-Delbrück Center for Molecular Medicine Berlin-Buch, Berlin, Germany, March 1993.
3. American Society of Human Genetics - Workshop on Human Gene Therapy, New Orleans, LA, October 1993.
4. International Conference on Coagulation Inhibitors, Chapel Hill, NC, November 1993.
5. Hemophilia Today, Poitiers, France, March 1994.

6. Immuno Hemophilia Update, St. Thomas, VI, March 1994.
7. American Pediatric Society and Society of Pediatric Research, National Pediatric Blood Club Symposium, Seattle, WA, May 1994
8. International Conference of the American Thoracic Society, Boston, MA, May 1994.
9. Advances in the Treatment of Hemophilia and von Willebrand's Disease, Oakland, CA, June 1994.
10. International Symposium on Gene Therapy, Valencia, Spain, November 1994.
11. Science in Medicine Lecture, University of Washington, WA, February 1995.
12. American Association for the Advancement of Science (AAAS) plenary session of Gene Therapy, Atlanta, GA, February 1995.
13. Third Annual Conference on Gene Therapy, Berlin, Germany, April 1995.
14. NIH Panel to Assess the NIH Investment in Research on Gene Therapy, San Francisco, CA, August 1995.
15. National Hemophilia Foundation Meeting, Philadelphia, PA, October 1995.
16. Tenth Anniversary: Vascular Gene Transfer: Models of Disease and Therapy, Bethesda, MD, March 1996.
17. Region IX Hemophilia Foundation Meeting, Napa Valley, CA, March 1996.
18. Organizer and Chair of session on Gene Therapy and Animal Models for the XXII International Congress of the World Federation of Hemophilia, Dublin, Ireland, June 1996.
19. Eighth Japanese-American Conference of Pharmacokinetics and Biopharmaceutics, Seattle, WA, July 1996.
20. National Hemophilia Meeting, San Diego, CA, September 1996.
21. Organizing committee of the 10th Annual Cystic Fibrosis Conference, Orlando, FL, October 1996.
22. 39th Meeting of the Japanese Society of Inherited Metabolic Disease, Tokyo, Japan, November, 1996.
23. Third Japanese Workshop on Gene Therapy, Tokyo, Japan, November, 1996.
24. American Association for the Advancement of Science (AAAS) plenary session on Human Genetics, Seattle, WA February, 1997.
25. Keystone Meeting, Cellular and Molecular Basis for Gene Therapy, Snowbird, UT, April 1997.
26. Muscular Dystrophy Association DMD Gene Therapy Workshop, Tucson, AZ, May 1997.
27. Williamsburg Cystic Fibrosis Meeting on Recent Advances in Gene Therapy, Williamsburg, VA, June 1997.
28. 3rd Annual Symposium on German-American Frontiers of Science, Munich, Germany, June 1997.
29. International Conference on Gene Therapy for Hemophilia, Chapel Hill, NC, September 1997.
30. The 11th Annual Cystic Fibrosis Conference, speaker and session chair Nashville, TN, October 1997.
31. International Society for Liver Transplantation, Seattle, WA, October 1997.
32. American Society for Human Genetics-Educational Session speaker, Baltimore, MD, October 1997.
33. European Workshop on Gene Therapy, Milan, Italy, November 1997.

34. Arosenius Honorary Lecture on Gene Therapy for Hemophilia, Stockholm, Sweden, November 1997.
35. Keystone Symposium on the Molecular and Cellular Biology of Gene Therapy, Keystone, CO, January 1998.
36. Society for Pediatric Research, State-of-the-Art Lecture on Gene Therapy for Genetic Diseases, New Orleans, LA, May 1998.
37. XXIII International Congress of the World Federation of Hemophilia-State-of-the-Art Plenary Session and Chair of Plenary distinguished lecture, The Hague, Netherlands, May 1998.
38. FASEB meeting on Mechanisms of Liver Growth and Differentiation in Health and Disease - Chair and speaker on Liver Gene Therapy and Cellular Transplantation, Snowmass, CO, July 1998.
39. NHF Workshop on Gene Therapy for Hemophilia, San Diego, CA, November 1998.
40. International Conference on Gene Therapy and Molecular Biology, Redwood City, CA, April 1999.
41. NIH/FDA Workshop on Non-Clinical Toxicology Study, Design Issues for Development of AAV-Based Gene Therapeutics, Bethesda, MD, May 1999.
42. 8th Biennial International Congress on Liver Development, Gene Regulation and Disease, Orvieto, Italy, June 1999
43. Williamsburg CF Meeting on Recent Progress in Gene Therapy, Williamsburg, VA, June 1999.
44. American Heart Failure Society, San Francisco, CA, September 1999.
45. American Society of Human Genetics: Symposia on Gene Therapy, San Francisco, CA, October 1999.
46. National Hemophilia Foundation, Presymposia on Gene Therapy for Hemophilia, Dallas, TX, November 1999.
47. Gene Therapy Approaches for Diabetes and Its Complications, Rockville, MD, November 1999.
48. Keystone Symposium on Gene Therapy 2000, Keystone, CO, January 2000.
49. FASEB Liver Regeneration, Snowmass, CO, July 2000.
50. National Hemophilia Foundation, Workshop on Gene Therapy for Hemophilia, San Diego, CA, April 2001.
51. American Academy of Pediatrics-Educational Session-Gene Therapy: Pitfalls and Promises, San Francisco, CA, October 2001.
52. American Society of Hematology, Symposia on Gene Therapy for Hemophilia, A phase 1 liver-based clinical trial for hemophilia B, Orlando, FL, December 2001.
55. Gordon Conference on Hemostasis and Thrombosis, Colby, ME, July 2002.
56. World Congress of International Society of Hematology - Plenary Speaker, Seoul, Korea, August 2002.
57. 10th Annual European Society for Gene Therapy - Plenary Speaker, Niece, France, October 2002.
58. American Society for Microbiology - Speaker, Banff, Alberta, Canada, March 2003
59. American Society for Human Gene Therapy - Speaker, Workshop on RNAi, Washington DC, June 2003.
60. 1st Annual International Conference on Transposition and Animal Biotechnology - Speaker, Minneapolis, MN, July 2003.

61. Falk Symposium - Speaker, Germany, October, 2003.
62. Gordon Conference on Viral Vectors for Gene Therapy - Speaker, Santa Barbara, CA, February 2004.
63. American Chemical Society Annual Meeting - Speaker, Anaheim, CA, March 2004.
64. Keystone Symposium on siRNAs and miRNAs - Speaker, Keystone, CO, April 2004.
65. RNAi Conference - Speaker, Boston, MA, May 2004.
66. American Society for Gene Therapy - Education Session, Gene Transfer in Liver, Minneapolis, MN, June 2004.
67. CHI RNAi Conference - Speaker, San Francisco, CA June 2004.
68. FASEB Meeting on Liver Biology - Speaker, Snowmass, CO, August 2004.
69. European Society of Gene Therapy Annual Meeting - Speaker, Edinburgh, Scotland, November 2004.
70. European Society for Gene Therapy - Speaker, Finland, November 2004.
71. Spanish Society for Gene Therapy - Keynote Speaker, Pamplona, Spain, January 2005.
72. Bari International Hemophilia Conference - Pizzomunno, Italy, May 2005.
73. American Society of Gene Therapy Symposia - Speaker, St. Louis, MO, June 2005.
74. Japanese Society of Gene Therapy Plenary Session - Speaker, Tokyo, Japan, July 2005.
75. Rennebohm Symposium, University of Wisconsin, Milwaukee, WI, September 2005.
76. Memorial Sloan Kettering Harold Varmus Presidential Symposium - Speaker, September 2005.
77. Conference on Cell and Gene Therapy - Speaker, Barcelona, Spain, October 2005.
78. University of Toronto Langdon Hall Conference-Gene Therapy - Speaker, Toronto, Canada, May 2006.
79. Mary Crowley Gene Therapy for Cancer - Speaker, Dallas, TX, September 2006.
80. Keystone meeting on RNAi/microRNA - Speaker, Keystone, CO, January 2007.
81. International Society for Heart and Lung Transplantation Plenary overview on RNAi, San Francisco, CA, March 2007.
82. Gordon Conference on Human Genomics and Genetics - Speaker, Newport, RI, July 2007.
83. Oligotherapeutics Society 13th annual meeting - Speaker, Berlin, Germany, October 2007.
84. 50th Anniversary Reunion for University of Washington Medical Genetics, October 2007.
85. American Society of Hematology, Educational Session on Micro RNA/RNAi, December 2007.
86. Gordon Research Conference, Science of Viral Vectors, Ventura, California, March, 2008.
87. Keystone meeting on RNAi/microRNA - Speaker, Keystone, CO, March, 2008.
88. Gene Therapy & Vaccines - Student invitee, University of Pennsylvania, May 2008.
89. Drug Delivery and Translational Research Conference, New York City, May 2008
90. American Society of Gene Therapy, 11th annual meeting, two plenary talks - Speaker, May 2008.
91. FASEB Liver meeting, Snowmass, Colorado, August 2008.

92. Keystone Meeting on RNA Therapeutics Lake Louise, CA. Session Chair and Plenary speaker. Feb. 2009
93. ASGT Meeting, San Diego, California. Session co-chair. May 2009
94. Keystone Meeting, Keystone, Colorado, Plenary speaker. June 2009.

F. Bibliography:

Peer-reviewed articles

1. **Kay, M.A.**, M. Jacobs-Lorena. 1985, Selective translational regulation of ribosomal protein gene expression during early development of *Drosophila Melanogaster*. *Mol Cell Biology*, 5:3583-3592.
2. Qian, S., J.-Y. Zhang, **M.A. Kay**, M. Jacobs-Lorena. 1987, Structure analysis of the *Drosophila* rpA1 gene, a member of the eucaryotic 'A' type ribosomal protein family. *Nucleic Acids Res*, 15:987-1003.
3. **Kay, M.A.**, M. Jacobs-Lorena. 1987, Developmental genetics of ribosome synthesis in *Drosophila*. *Trends Genet*, 3:347-351.
4. **Kay, M.A.**, J.-Y. Zhang, M. Jacobs-Lorena. 1988, Identification and germ line transformation of the ribosomal protein rp21 gene of *Drosophila*: Complementation analysis with the *Minute* QIII locus reveals nonidentity. *Mol Gen Genet*, 213:354-358.
5. **Kay, M.A.**, W. O'Brien, B. Kessler, R. McVie, S. Ursin, K. Dietrich, E.R.B. McCabe. 1990, Transient organic aciduria and methemoglobinemia with acute gastroenteritis. *Pediatrics*, 85:589-592.
6. **Kay, M.A.**, E.R.B. McCabe. 1990, *E. coli* Sepsis and Prolonged Hypophosphatemia Following Exertional Heat Stroke. *Pediatrics*, 86:307-309.
7. **Kay, M.A.**, P. Baley, S. Rothenberg, F. Leland, L. Fleming, K. Ponder, T.J. Liu, M. Finegold, G. Darlington, W. Pokorny, S.L.C. Woo. 1992, Expression of human alpha-1-antitrypsin in dogs after autologous transplantation of retroviral transduced hepatocytes. *Proc Natl Acad Sci USA*, 89:89-93.
8. Liu, T.J., **M.A. Kay**, G. Darlington, S.L.C. Woo. 1992, Reconstitution of Enzymatic Activity in Hepatocytes of Phenylalanine Hydroxylase-Deficient Mice. *Somat Cell Mol Genet*, 18:89-96.
9. **Kay, M.A.**, Q.T. Li, T.J. Liu, F. Leland, M. Finegold, S.L.C. Woo. 1992, Hepatic Gene Therapy: Persistent Expression of Human α 1-Antitrypsin in Mice after Direct Gene Delivery *In Vivo*. *Hum Gene Ther*, 3:641-647.
10. Li, Q.T., **M.A. Kay**, L. S. Perricaudet, M. Finegold, S.L.C. Woo. 1993, Assessment of Recombinant Adenoviral Vectors for Hepatic Gene Therapy. *Hum Gene Ther*, 4:403-409.
11. Kolodka, T.M., M. Finegold, **M.A. Kay**, S.L.C. Woo. 1993, Hepatic Gene Therapy: Efficient Retroviral-Mediated Gene Transfer into Rat Hepatocytes *In Vivo*. *Somat Cell Mol Genet*, 19:491-497 erratum appears in vol 20:3 (1994).
12. **Kay, M.A.**, S. Rothenberg, C. Landon, D. Bellinger, F. Leland, C. Toman, A. Thompson, M. Read, K. Brinkhous, S.L.C. Woo. 1993, *In Vivo* Gene Therapy of Hemophilia B: Sustained Partial Correction in Factor IX-Deficient Dogs. *Science*, 262:117-119.
13. Cristiano, R.J., L.C. Smith, **M.A. Kay**, B. Brinkley, S.L.C. Woo. 1993, Hepatic gene therapy: Efficient gene delivery expression in primary hepatocytes utilizing a conjugated adenovirus-DNA complex. *Proc Natl Acad Sci USA*, 90:11548-11552.
14. **Kay, M.A.**, C.N. Lander, S.R. Rothenberg, L.A. Taylor, F. Leland, S. Wiehle, B. Fang, D. Bellenger, M. Finegold, A.R. Thompson, M.S. Read, K.M. Brinkhous, S.L.C. Woo. 1994, *In vivo* hepatic gene therapy: Complete albeit transient correction of factor IX deficiency in hemophilia B dogs. *Proc Natl Acad Sci USA*, 91:2353-2357.

15. Jones, D., **M.A. Kay**, W. Craigen, E. McCabe, H. Hawkins, A. Dominey. 1995, Coal-black hyperpigmentation at birth in a child with congenital adrenal hypoplasia. *J Am Acad Dermatol*, 33:323-326.
16. **Kay, M.A.**, F. Graham, F. Leland, S.L.C. Woo. 1995, Therapeutic serum concentrations of human alpha 1-antitrypsin after adenoviral-mediated gene transfer into mouse hepatocytes. *Hepatology*, 21:515-519.
17. Barr, D., J. Tubb, D. Ferguson, A. Scaria, A. Lieber, C. Wilson, J. Perkins, **M.A. Kay**. 1995, Strain related variations in adenoviral-mediated transgene expression from mouse hepatocytes *in vivo*: Comparisons between immunocompetent and immunodeficient inbred strains. *Gene Ther*, 2:151-156.
18. Lieber, A., M.J. Vrancken-Peeters, **M.A. Kay**. 1995, Adenovirus-Mediated Transfer of the Rat Retrovirus Amphotropic Receptor cDNA Increases Retrovirus Transduction in Cultured Cells. *Hum Gene Ther*, 6:5-11.
19. Scaria, A., D.T. Curiel, **M.A. Kay**. 1995, Complementation of a human adenovirus early region 4 deletion mutant in 293 cells using adenovirus-polylysine-DNA complexes. *Gene Ther*, 2:295-298.
20. Lieber, A., M.J. Vrancken Peeters, L. Meuse, N. Fausto, J. Perkins, **M.A. Kay**. 1995, Adenovirus mediated urokinase gene transfer induces liver regeneration and allows for efficient retrovirus transduction of hepatocytes *in vivo*. *Proc Natl Acad Sci USA*, 92:6210-6214.
21. Lieber, A., M.J. Vrancken Peeters, A. Gown, J. Perkins, **M.A. Kay**. 1995, A Modified Urokinase Plasminogen Activator Induces Liver Regeneration without Bleeding. *Hum Gene Ther*, 6:1029-1037.
22. Fang, B., R.C. Eisensmith, H. Wang, **M.A. Kay**, G. Zhao, R.E. Cross, C.N. Landen, G. Gavin, D.A. Bellinger, M.S. Read, P.C. Hu, K.M. Brinkhous, S.L.C. Woo. 1995, Gene Therapy for Hemophilia B: Host Immunosuppression Prolongs the Therapeutic Effect of Adenovirus-Mediated Gene Transfer. *Hum Gene Ther*, 6:1039-1044.
23. **Kay, M.A.**, A.X. Holterman, L. Meuse, A. Gown, H. Ochs, P.S. Linsley, C.B. Wilson. 1995, Long-term hepatic adenovirus-mediated gene expression in mice following CTLA4Ig administration. *Nat Genet*, 11:191-197.
24. Vrancken-Peeters, M.J., A. Lieber, J. Perkins, **M.A. Kay**. 1996, Method for Multiple Portal Vein Infusions in Mice: Quantitation of Adenovirus-Mediated Hepatic Gene Transfer. *BioTechniques*, 20:278-285.
25. Liu, M.L., B. Winther, **M.A. Kay**. 1996, Pseudotransduction of Hepatocytes by Using Concentrated Pseudotyped Vesicular Stomatitis Virus G Glycoprotein (VSV-G)-Moloney Murine Leukemia Virus-Derived Retrovirus Vectors: Comparison of VSV-G and Amphotropic Vectors for Hepatic Gene Transfer. *J Virol*, 70:2497-2502.
26. Lieber, A., **M.A. Kay**. 1996, Adenovirus-Mediated Expression of Ribozymes in Mice. *J Virol*, 70:3153-3158.
27. Deshane, J., G.P. Siegal, R.D. Alvarez, M. Wang, M. Feng, G. Cabrera, T. Liu, **M.A. Kay**, D.T. Curiel. 1995, A strategy to accomplish gene therapy for ovarian carcinoma based upon targeted tumor killing via an intracellular antibody directed against the erbB-2 oncoprotein. *J Clin Invest*, 96:2980-2989.
28. Grim, J., J. Deshane, M. Feng, A. Lieber, **M.A. Kay**, D.T. Curiel. 1996, ErbB-2 knockout employing an intracellular single chain antibody (sFv) accomplishes specific toxicity in erbB-2 expressing lung cancer cells. *Am J Respir Cell Mol Biol*, 15:348-354.
29. Lieber, A., C.Y. He, S. Polyack, D. Gretsch, D. Barr, **M.A. Kay**. 1996, Elimination of Hepatitis C Virus RNA in Infected Human Hepatocytes by Adenovirus-Mediated Expression of Ribozymes. *J Virol*, 70:8782-8791.
30. Brand, K., W. Arnold, T. Bartels, A. Lieber, **M.A. Kay**, M. Strauss, B. Dorken. 1997, Toxicity associated with the treatment of liver tumors using the HSV-tk/GCV system and adenoviruses as vectors. *Cancer Gene Ther*, 4:9-16.

31. Sandig, V., P. Liser, A. Lieber, **M.A. Kay**, M. Strauss. 1996, HBV-derived promoters direct liver-specific expression of an adenovirus transduced LDL receptor gene. *Gene Ther*, 3:1002-1009.
32. **Kay, M.A.**, N. Fausto. 1997, Liver regeneration: prospects for therapy based on new technologies. *Mol Med Today*, 3:108-115.
33. Vrancken Peeters, M.J., G. Patijn, A. Lieber, L. Meuse, **M.A. Kay**. 1996, Adenovirus-Mediated Hepatic Gene Transfer in Mice: Comparison of Intravascular and Biliary Administration. *Hum Gene Ther*, 7:1693-1699.
34. Murry, C.E., **M.A. Kay**, S.D. Hauschka, S.M. Schwartz. 1996, Muscle differentiation during repair of myocardial necrosis via gene transfer with MyoD. *J Clin Invest*, 98:2209-2217.
35. Schowalter, D.B., J.C. Tubb, M. Liu, C.B. Wilson, **M.A. Kay**. 1997, Heterologous expression of adenovirus E3-gp19K in an E1a deleted adenovirus vector inhibits MHC I expression in vitro but does not prolong transgene expression in vivo. *Gene Ther*, 4:351-360.
36. Lieber, A. C.Y. He, I. Krillova, **M.A. Kay**. 1996, Recombinant Adenoviruses with Large Deletions Generated by Cre-Mediated Excision Exhibit Different Biological Properties Compared with First generation vectors in vitro and in vivo. *J Virol*, 70:8944-8960.
37. Vrancken Peeters, M.J., G.A. Patijn, A. Lieber, J. Perkins, **M.A. Kay**. 1997, Expansion of donor Hepatocytes after Recombinant Adenovirus-Induced Liver Regeneration in Mice. *Hepatology*, 25:884-888.
38. Bethune, C., T. Bui, M.L. Liu, **M.A. Kay**, R.J.Y. Ho. 1997, Development of a High-Performance Liquid Chromatographic Assay for G418 Sulfate (Geneticin). *Antimicrob Agents and Chemother*, 41:661-664.
39. Overturf, K., M. Al-Dhalimy, C.N. Ou, M. Finegold, R. Tanguay, A. Lieber, **M.A. Kay**, M. Grompe. 1997, Adenovirus-Mediated Gene Therapy in a Mouse Model of Hereditary Tyrosinemia Type 1. *Hum Gene Ther*, 8:513-521.
40. **Kay, M.A.**, L. Meuse, A.M. Gown, P. Linsley, D. Hollenbaugh, A. Aruffo, H. Ochs, C.B. Wilson. 1997, Transient immunomodulation with anti-CD40 ligand and CTLA4Ig enhances persistence and secondary adenovirus-mediated gene transfer into mouse liver. *Proc Natl Acad Sci USA*, 94:4686-4691.
41. Snyder, R.O., C.H. Miao, G.J. Patijn, S.K. Pratt, O. Danos, A.M. Gown, B. Winther, L. Meuse, L.K. Cohen, A.R. Thompson, **M.A. Kay**. 1997, Persistent and therapeutic concentrations of human factor IX in mice after hepatic gene transfer of recombinant AAV vectors. *Nat Genet*, 16:270-276.
42. Schowalter, D.B., L. Meuse, C.B. Wilson, P. Linsley, **M.A. Kay**. 1997, Constitutive expression of murine CTLA4Ig from a recombinant adenovirus vector results in prolonged transgene expression. *Gene Ther*, 4:853-860.
43. Bennett, R.L., M. Karayiorgou, C.A. Sabin, T.H. Norwood, **M.A. Kay**. 1997, Identification of an interstitial deletion in an adult female with schizophrenia, mental retardation, and dysmorphic features: Further support for a putative schizophrenia susceptibility locus at 5q21-23.1. *Am J Hum Genet*, 61:1450-1454.
44. Nelson, J.E., **M.A. Kay**. 1997, Persistence of recombinant adenovirus in vivo is not dependent on vector DNA replication. *J Virol*, 71:8902-8907.
45. Lieber, A., C.Y. He, L. Meuse, D. Schowalter, I. Krillova, B. Winther, **M.A. Kay**. 1997, The role of Kupffer cell activation and viral gene expression in early liver toxicity following infusion of recombinant adenoviral vectors. *J Virol*, 71:8798-8807.
46. Lieber, A., C.Y. He, **M.A. Kay**. 1997, Adenoviral preterminal protein stabilizes mini-adenoviral genomes in vitro and in vivo. *Nat Biotechnol*, 15:1383-1387.

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48. Patijn, G.A., A. Lieber, L. Meuse, B. Winther, **M.A. Kay**. 1998, High-efficiency retrovirus-mediated gene transfer into the livers of mice. *Hum Gene Ther*, 9:1449-1456.
49. Patijn, G.A., O. Terpstra, **M.A. Kay**. 1998, Method for continuous perfusion into the livers of mice. *Lab Anim Sci*, 48:379-383.
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51. Miao, C.H., R. Snyder, D.B. Schowalter, G.A. Patijn, B. Donahue, B. Winther, **M.A. Kay**. 1998, The kinetics of rAAV integration in the liver. *Nat Genet*, 19:13-15.
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Invited Manuscripts

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22. Nakai, H., **M.A. Kay**. 2003, Looking into the safety of AAV vectors. *Nature*, 17:424: 251.
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Book Chapters

1. Barr, D. and **M.A. Kay**. Methods for delivery of genes to hepatocytes in vivo using recombinant adenovirus vectors in Gene Therapy Protocols, ed. Paul Robbins. Methods in Molecular Medicine. 1997 Humana Press: Totowa, N.J.
2. **Kay, M.A.** Hepatic Gene Therapy for Hemophilia B. in Inhibitors to Coagulation Factors. Advances in Experimental Medicine and Biology, vol. 386. L.M. Aledort, L.W. Hoyer, J.M. Lusher, H.M. Reisner and G.C. White II. Plenum Press: NY pp 229-234.
3. **Kay, M.A.** and D. Russell Chapter 69 Gene Therapeutics for Harrison's Textbook of Medicine (2001) 15th edition, Braunwald, Fauci, Hauser, Longo, Jameson (ed). McGraw Hill: pp. 412-418
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5. Grimm D., K. Pandey, **M.A. Kay**. Adeno-associated virus vectors for short hairpin RNA expression. A Chapter in RNA Interference, (Volume 392 of Methods in Enzymology), edited by J. Rossi and D.R. Engelke: An imprint of Elsevier Science. Chapter 23.

Ongoing Research Support

17-2006-1028 (Kay) 12/01/06 - 11/30/09

Juvenile Diabetes Research Foundation
Using Liver Cells to Treat Diabetes

Dr. Kay is a **Co-PI with Dr. Markus Grompe** at Oregon Health & Science University. The funds are shared between the two laboratories. The major goal of this project is to study pancreatic transcription factor-induced transdifferentiation of liver cells to beta cells and develop a preclinical model for treatment of diabetes mellitus.

R01 DK078424 (HL086425) (Kay) 09/30/06 - 08/31/10

National Institutes of Health
Studies on RNAi Based Delivery in Vivo

This is a **shared grant with John Rossi at the City of Hope**. The major goal of this project is to offer a synergistic approach such that in the proposed consortium, they will work together to further unravel the mechanisms involved in shRNA based toxicities as well as develop new expression paradigms for *in vivo* shRNA-based therapeutics.

R01 AI071068 (Kay) 08/01/06 - 07/31/11
National Institutes of Health
RNAi for the Treatment of Viral Hepatitis
The major goal of this project is to develop an RNAi gene therapeutic that would be useful for treating hepatitis virus infection.

R01 HL092096-01A2 (Kay) 05/01/09 – 4/30/13
National Institutes of Health
Molecular Evolution Strategies to Derive New Recombinant AAV Vectors
The goal of this project is to develop a novel class of human gene transfer vectors based on adeno-associated virus (AAV) that combines high efficiency *in vivo* transduction of target tissues with the ability to circumvent or overcome host immunologic responses in humans.

Pending Research Support

R01 HL64274 (Kay)
National Institutes of Health
Gene Therapies for Hemophilias A & B
The major goal of this project is to (1) further study the mechanisms of AAV transduction *in vivo*, (2) build AAV vectors with improved expression cassettes for treating hemophilia; (3) development of robust DNA vectors for hepatic gene transfer.

RFA-OD-09-003 (Kay)
National Institutes of Health
Cell Reprogramming to Treat Diabetes
The major goal of this project is to further define the cell type and mechanism of liver cell reprogramming responsible for correcting diabetes mellitus after pancreatic transcription factor gene transfer into the liver.

R03 HL096222 (Longaker) 12/01/08 - 05/31/10
National Institutes of Health
Novel Strategies for Reprogramming iPS Derived Cardiac Cells
The goal of this project is to investigate the following aspects: (1) improve yield of induced pluripotent stem cells using human mesenchymal cells instead of fibroblasts; (2) use safe nonviral and nonimmunogenic minicircle plasmid as vector; (3) develop novel reprogramming strategies using microRNAs; (4) characterize functionality of iPS cell-derived cardiomyocytes compared to human embryonic stem cells; (5) define the network of genes involved in maintaining pluripotency and regulating differentiation using a systems biology approach; and (6) apply state-of-the-art molecular imaging technology to track the fate and function of transplanted cells.

Completed Research Support (last 5 years)

RS1-00236-1 (Kay) 07/01/07 - 06/30/09
California Institute for Regenerative Medicine
Novel Vectors for Gene Transfer into Human ES

The major goal of this project is to develop a more efficient and reliable gene transfer vector for transferring nucleic acids into embryonic stem cells than those in existence.

R01 HL64274 (Kay)

04/01/05 - 03/31/09

National Institutes of Health

Hepatic Gene Transfer for the Treatment of Hemophilias A&B

The major goal of this project is to (1) further study the mechanisms of AAV transduction *in vivo*, (2) build AAV vectors with improved expression cassettes for treating hemophilia; (3) development of robust DNA vectors for hepatic gene transfer.

R01 EB004657 (Davis)

02/01/05 - 01/31/09

California Institute of Technology

"Design of Gene Delivery System to Target Hepatocytes." To evaluate the hypothesis that a properly designed and engineered, synthetic, nonviral delivery vehicle bearing galactose-based targeting moieties can effectively deliver nucleic acids to hepatocytes in mice.

NIH/NIDDK RO1 DK49022 Kay (PI)

04/01/03 - 03/30/07

"Improved Adenoviral Vectors for Hepatic Gene Therapy" (To produce a high titer adenovirus vector devoid of all adenovirus coding sequences.)

NIH 2PO1 AR44012 (Khavari, P., Director/Kay, M., PI)

07/01/02 - 06/30/07

Molecular Therapeutics for Epithelial Disorders

Project 3: Transposon-Based Gene Therapeutics

NIH/NHLBI U01 HL66948 Kay (Director)

09/29/00 - 08/31/06

"Program of Excellence in Gene Therapy: Gene Therapy for Hemophilia."

To carry out clinical studies aimed at the goal of curing hemophilia.

NIH U19 AI40334 (Rice, C., Director/Kay, M., PI)

02/01/01 - 07/31/05

Hepatitis C: Studies of Immunity and Pathogenesis

Project 3: A mouse model for HCV infection.