

EXHIBIT 1

A. Identifying Data:

Name: Mark Allan Kay
Nationality: U.S.A.
Born: January 9th, 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 Delbruck 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 Hemophilia 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 Hemophilia 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 Animal Models, Max-Delbruck 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-Genes 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, Nice, 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 Feb. 2009. Session Chair and Plenary speaker
93. Keystone Meeting MicroRNAs in Cancer Keystone, CO June 2009. Plenary speaker
94. Oligonucleotide Therapeutic Society and Nucleic Acid Society of Japan. Speaker and Chair
Nov, 2009. Fukuoka , Japan

95. RNAi: Therapeutics and Mechanism University of Hong Kong. Plenary Speaker Nov 2009. Hong Kong,

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. *Somati 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. Gretch, 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. Patijin, 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. Kirillova, 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. Sobin, 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. Kirillova, 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.

47. Patijn, G.A., A. Lieber, D.B. Schowalter, R. Schwall, M.A. Kay. 1998, Hepatocyte Growth Factor (HGF) induces hepatocyte proliferation in vivo and allows for efficient retroviral-mediated gene transfer in mice. *Hepatology*, 28:707-716.
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.
50. Wilson, C.B., L.J. Embree, D. Schowalter, R. Albert, A. Aruffo, D. Hollenbaugh, P. Linsley, M.A. Kay. 1998, Transient inhibition of CD28 and CD40 ligand interactions prolongs adenoviral mediated transgene expression in the lung and facilitates expression after secondary vector administration. *J Virol*, 72:7542-7550.
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.
52. Stempien-Otero, A., A. Karsan, C.J. Cornejo, H. Xiang, T. Eunson, R. Morrison, M.A. Kay, C.B. Wilson, R. Winn, J. Harlan. 1999, Mechanisms of hypoxia-induced endothelial cell death in vitro: role of p53 in apoptosis. *J Biol Chem*, 274:8039-8045.
53. Lieber, A., C.Y. He, L. Meuse, C. Himeda, C. Wilson, M.A. Kay. 1998, Inhibition of NF-kappaB activation in combination with Bcl-2 expression allows for persistence of first generation adenoviral vectors in mouse liver. *J Virol*, 72:9267-9277.
54. Mizuguchi, H., M.A. Kay. 1998, Efficient Construction of a Recombinant Adenovirus Vector by an Improved In Vitro Ligation Method. *Hum Gene Ther*, 9:2577-2583.
55. Snyder, R.O., C. Miao, L. Meuse, J. Tubb, B.A. Donahue, H.F. Lin, D. Stafford, S. Patel, R. Thompson, T. Nichols, M. Read, D. Bellinger, M. Brinkhous, M.A. Kay. 1999, Correction of Hemophilia B in Canine and Murine Models using Recombinant Adeno-Associated Viral Vectors. *Nat Med*, 5:64-70.
56. Nakai, H., Y. Iwaki, M.A. Kay, L.B. Couto. 1999, Isolation of Recombinant Adeno-Associated Virus (rAAV) Vector-Cellular DNA Junctions from Mouse Liver. *J Virol*, 73:5438-5447.
57. Schowalter, D.B., C.L. Himeda, B.L. Winther, C.B. Wilson, M.A. Kay. 1999, Implication of Interfering Antibody Formation and Apoptosis as Two Different Mechanisms Leading to Variable Duration of Adenovirus-Mediated Transgene Expression in Immune-Competent Mice. *J Virol*, 73:4755-4766.
58. Mizuguchi, H., M.A. Kay. 1999, A Simple Method for Constructing E1 and E1/E4 Deleted Recombinant Adenoviral Vectors. *Hum Gene Ther*, 10:2013-2017.
59. Zen, K., A. Karsan, A. Stempien-Otero, E. Yee, J. Tupper, X. Li, T. Eunson, M.A. Kay, C.B. Wilson, R.K. Winn, J.M. Harlan. 1999, NF-kappa B activation is required for human endothelial survival during exposure to tumor necrosis factor-alpha but not to interleukin 1beta or lipopolysaccharide. *J Biol Chem*, 274:28808-28815.
60. Lieber, A., D. S. Steinwaerder, C.A. Carlson, M.A. Kay. 1999, Integrating Adeno-AAV Hybrid Vector Devoid of All Genes. *J Virol*, 73:9314-9324.
61. Lieber, A., M.A. Kay, Z-Y. Li. 2000, Nuclear Import of Molony Murine Leukemia Virus DNA by Adenoviral Pre-Terminal Protein Is Not Sufficient for Efficient Retroviral Transduction in Nondividing Cells. *J Virol*, 74:721-734.
62. Vilain, E., C. Lecointre, F. Desangles, M.A. Kay, P. Maroteaux, M.L. Merrer, and E.R.B. McCabe. 1999, IMAGE, a new clinical association of intrauterine growth retardation, metaphyseal dysplasia, adrenal hypoplasia congenita and genital anomalies. *J Clin Endocrinol Metab*, 84:4335-4340.

63. Park, F., K. Ohashi, W. Chiu, M.A. Kay. 2000, Efficient lentiviral transduction of liver requires cell cycling in vivo. *Nat Genet*, 24:49-52.
64. Ohashi, K., P. Marion, H. Nakai, L. Meuse, J.M. Cullen, B.B. Bordier, R. Schwall, H.B. Greenberg, J.S. Glenn, M.A. Kay. 2000, Sustained survival of human hepatocytes: A model for in vivo infection with human hepatitis B and hepatitis delta viruses. *Nat Med*, 6:327-331.
65. Kay, M.A., C.S. Manno, M.V. Ragni, P.J. Larson, L.B. Couto, A. McClelland, B. Glader, A.J. Chew, S.J. Tai, R.W. Herzog, V. Arruda, F. Johnson, C. Scallan, E. Skarsgard, A.W. Flake, K.A. High. 2000, Evidence for gene transfer and expression of blood coagulation factor IX in patients with severe hemophilia B treated with an AAV vector. *Nat Genet*, 24:257-261.
66. Miao, C.H., H. Nakai, A.R. Thompson, T.A. Storm, W. Chiu, R.O. Snyder, M.A. Kay. 2000, Non-Random Transduction of Recombinant Adeno-Associated Viral Vectors in Mouse Hepatocytes in Vivo: Cell Cycling Is Not Required for Transduction. *J Virol*, 74:3793-3803.
67. Park, F., K. Ohashi, M.A. Kay. 2000, Therapeutic levels of human factors VIII and IX using HIV-1 based lentiviral vectors in mouse liver. *Blood*, 96:1173-1176.
68. Yant, S.R., L. Meuse, W. Chiu, Z. Ivics, Z. Izsvak, M.A. Kay. 2000, Somatic integration and long-term therapeutic transgene expression in normal and hemophilic mice using plasmid DNA encoding a DNA transposon system. *Nat Genet*, 25:35-41.
69. Nakai, H., T.A. Storm, M.A. Kay. 2000, Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors. *Nat Biotechnol*, 18:527-532.
70. Miao, C.H., K. Ohashi, G.A. Patijn, L. Meuse, A.R. Thompson, M.A. Kay. 2000, Inclusion of the Hepatic Locus Control Region, an Intron and Untranslated Region Increases and Stabilizes Hepatic Factor IX Gene Expression in Vivo but Not in Vitro. *Mol Ther*, 1:432-442.
71. Nakai, H., T. Storm, M.A. Kay. 2000, Recruitment of Single-Stranded Recombinant Adeno-Associated Virus Vector Genomes and Intermolecular Recombination Are Responsible for Stable Transduction of Liver In Vivo. *J Virol*, 74:9451-9463.
72. Ohashi, K., L. Meuse, R. Schwall, M.A. Kay. 2001, cMet Activation Allows Persistent Engraftment of Ectopically Transplanted Xenogenic Human Hepatocytes in Mice. *Transplant Proc*, 33:587-588.
73. Mizuguchi, H., M.A. Kay, T. Hayakawa. 2001, A simplified system for constructing recombinant adenoviral vectors containing heterologous peptides in the HI loop of their fiber knob. *Gene Ther*, 8:730-735.
74. Mizuguchi, H., M.A. Kay, T. Hayakawa. 2001, In Vitro Ligation-Based Cloning of Foreign DNAs into the E3 and E1 Deletion Regions for Generation of Recombinant Adenovirus Vectors. *BioTechniques*, 30:1112-1116.
75. Stoll, S.M., C.R. Scimanti, E.J. Baba, L. Meuse, M.A. Kay, M.P. Calos. 2001, Epstein-Barr Virus/Human Vector Provides High-Level, Long-Term Expression of α -1-Antitrypsin in Mice. *Mol Ther*, 4:122-129.
76. Chen, Z.Y., S.R. Yant, C.Y. He, L. Meuse, S. Shen, M.A. Kay. 2001, Linear DNAs Concatemere in Vivo and Result in Sustained Transgene Expression in Mouse Liver. *Mol Ther*, 3:403-410.
77. Park, F., M.A. Kay. 2001, Modified HIV-1 Based Lentiviral Vectors Have an Effect on Viral Transduction Efficiency and Gene Expression In Vitro and In Vivo. *Mol Ther*, 4:164-73.

78. Nakai, H., S.R. Yant, T.A. Storm, S. Fuess, L. Meuse, M.A. Kay. 2001, Extrachromosomal Recombinant Adeno-Associated Virus Vector Genomes Are Primarily Responsible for Stable Liver Transduction in Vivo. *J Virol*, 75:6969-76.
79. Vollrath, D., W. Feng, J.L. Duncan, D. Yasumura, P.M. D'Cruz, A. Chappelow, M.T. Matthes, M.A. Kay, N.M. LaVail. 2001, Correction of the retinal dystrophy phenotype of the RCS rat by viral gene transfer of Mertk. *Proc Natl Acad Sci USA*, 98:12584-12589.
80. Arruda, V.R., P.A. Fields, R. Milner, L. Wainwright, M.P. De Miguel, P.J. Donovan, R.W. Herzog, T.C. Nichols, J.A. Biegel, M. Razavi, M. Dake, D. Huff, A.W. Flake, L. Couto, M.A. Kay, K.A. High. 2001, Lack of Germline Transmission of Vector Sequences Following Systemic Administration of Recombinant AAV-2 Vector in Males. *Mol Ther*, 4:586-592.
81. Ohashi, K., F. Park, M.A. Kay. 2002, Role of Hepatocyte Direct Hyperplasia on Lentiviral-Mediated Liver Transduction In Vivo. *Hum Gene Ther*, 13:653-663.
82. Ehrhardt, A., M.A. Kay. 2002, A new adenoviral helper-dependent vector results in sustained therapeutic levels of human coagulation factor IX at low doses in vivo. *Blood*, 99:3923-3930.
83. Bordier, B.B., P.L. Marion, K. Ohashi, M.A. Kay, H.B. Greenberg, J. L. Casey, J. S. Glenn. 2002, A Prenylation Inhibitor Prevents Production of Infectious Hepatitis Delta Virus Particles. *J Virol*, 76:10465-10472.
84. Ohashi, K., F. Park, R.H. Schwall, M.A. Kay. 2002, Efficient Gene Transduction to Cultured Hepatocytes by HIV-1 Derived Lentiviral Vector. *Transplant Proc*, 24:1431-1433.
85. McCaffrey, A.P., K. Ohashi, L. Meuse, S. Shen, A.M. Lancaster, P.J. Lukavsky, P. Sarnow, M.A. Kay. 2002, Determinants of Hepatitis C Translational Initiation In Vitro, in Cultured Cells and Mice. *Mol Ther*, 5:676-684.
86. McCaffrey, A.P., L. Meuse, T.T. Pham, D.S. Conklin, G.J. Hannon, M.A. Kay. 2002, Gene expression: RNA interference in adult mice. *Nature*, 418:38-39.
87. Nakai, H., C.E. Thomas, T.A. Storm, S. Fuess, S. Powell, J.F. Wright, M.A. Kay. 2002, A Limited Number of Transducible Hepatocytes Restricts a Wide-Range Linear Vector Dose Response in Recombinant Adeno-Associated Virus-Mediated Liver Transduction. *J Virol*, 76:11343-11349.
88. Yant, S., A. Ehrhardt, J.G. Mickelson, L. Meuse, T. Pham, M.A. Kay. 2002, Transposition from a gutless adeno-transposon vector stabilizes transgene expression in vivo. *Nat Biotechnol*, 20:999-1005.
89. Olivares, E.C., R.P. Hollis, T.W. Chalberg, L. Meuse, M.A. Kay, M.P. Calos. 2002, Site-specific integration produces therapeutic Factor IX levels in mice. *Nat Biotechnol*, 20:1124-1128.
90. Montini, E., P.K. Held, M. Noll, N. Morcinek, M. Al-Dhalimy, M. Finegold, S.R. Yant, M.A. Kay, M. Grompe. 2002, In vivo Correction of Murine Tyrosinemia Type I by DNA-Mediated Transposition. *Mol Ther*, 6:759-769.
91. Nakai, H., E. Montini, S. Fuess, T.A. Storm, L. Meuse, M. Finegold, M. Grompe, M.A. Kay. 2003, Helper-Independent and AAV-ITR-Independent Chromosomal Integration of Double-Stranded Linear DNA Vectors in Mice. *Mol Ther*, 1:101-111.
92. Nakai, H., S. Fuess, T.A. Storm, L.A. Meuse, M.A. Kay. 2003, Free DNA Ends Are Essential for Concatemerization of Synthetic Double-Stranded Adeno-Associated Virus Vector Genomes Transfected into Mouse Hepatocytes in Vivo. *Mol Ther*, 1:112-121.

93. Ehrhardt, A., P. Peng, H. Xu, L. Meuse, M.A. Kay. 2003, Optimization of CIS-Acting Elements for Gene Expression from Nonviral Vectors In Vivo. *Hum Gene Ther*, 14:215-225.
94. Manno, C.S., A.J. Chew, S. Hutchinson, P.J. Larsen, R.W. Herzog, V.R. Arruda, S.J. Tai, M.V Ragni, A. Thompson, M. Ozelo, L.B. Couto, D. Leonard, F.A. Johnson, A. McClelland, C. Scallan, E. Skarsgard, A.W. Flake, M.A. Kay, K.A. High, B. Glader. 2003, AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. *Blood*, 101:2963-2972.
95. Ortiz-Urda, S., Q. Lin, S.R. Yant, D. Keene, M.A. Kay, P.A. Khavari. 2003, Sustainable correction of junctional epidermolysis bullosa via transposon-mediated nonviral gene transfer. *Gene Ther*, 10:1099-1104.
96. McCaffrey, A.P., H. Nakai, K. Pandey, Z. Huang, F.H. Salazar, H. Xu, S.F. Wieland, P.L. Marion, M.A. Kay. 2003, Inhibition of hepatitis B virus replication in mice by RNA interference. *Nat Biotechnol*, 21:639-644.
97. Ehrhardt, A., H. Xu, M.A. Kay. 2003, Episomal Persistence of Recombinant Adenoviral Vector Genomes During Cell Cycle In Vivo. *J Virol*, 77: 7689-7695.
98. McCaffrey, A.P., L. Meuse, M. Karimi, C.H. Contag, M.A. Kay. 2003, A Potent and Specific Morpholino Antisense Inhibitor of Hepatitis C Translation in Mice. *Hepatology*, 38:503-508.
99. Chen, Z.Y., C.Y. HE, A. Ehrhardt, M.A. Kay. 2003, Minicircle DNA Vectors Devoid of Bacterial DNA Result in Persistent and High Level Transgene Expression in Vivo. *Mol Ther*, 8:495-500.
100. Grimm, D., M.A. Kay, J. Kleinschmidt. 2003, Helper Virus-Free, Optically Controllable, and Two-Plasmid-Based Production of Adeno-associated Virus Vectors of Serotypes 1 to 6. *Mol Ther*, 7:839-850.
101. Nakai, H., E. Montini, S. Fuess, T.A. Storm, M. Grompe, M.A. Kay. 2003, AAV serotype 2 vectors preferentially integrate into active genes in mice. *Nat Genetics*, 34:297-302.
102. Nakai, H., T.A. Storm, S. Fuess, M.A. Kay. 2003, Pathways of Removal of Free DNA Vector Ends in Normal and DNA-PKcs-Deficient SCID Mouse Hepatocytes Transduced with rAAV Vectors. *Hum Gene Ther*, 14:871-881.
103. Park, F., K. Ohashi, M.A. Kay. 2003, The Effect of Age on Hepatic Gene Transfer with Self-Inactivating Lentiviral Vectors in Vivo. *Mol Ther*, 8:314-323.
104. Grimm, D., S. Zhou, H. Nakai, C.E. Thomas, T.A. Storm, S. Fuess, T. Matsushita, J. Allen, R. Surosky, M. Lochrie, L. Meuse, A. McClelland, P. Colosi, M.A. Kay. 2003, Preclinical in vivo evaluation of pseudotyped adeno-associated virus vectors for liver gene therapy. *Blood*, 102:2412-2419.
105. Mikkelsen, J.G., S.R. Yant, L. Meuse, Z. Huang, H. Xu, M.A. Kay. 2003, Helper-Independent Sleeping Beauty Transposon-Transposase Vectors for Efficient Nonviral Gene Delivery and Persistent Gene Expression In Vivo. *Mol Ther*, 8:654-665.
106. Ehrhardt, A., H. Xu, A. M. Dillow, D.A. Bellinger, T.C. Nichols, M.A. Kay. 2003, A gene-deleted adenoviral vector results in phenotypic correction of canine Hemophilia B without liver toxicity or thrombocytopenia. *Blood*, 102:2403-2411.
107. Chyung, Y.H., P. Peng, M.A. Kay. 2003, System for Simultaneous Tissue-Specific and Disease-Specific Regulation of Therapeutic Gene Expression. *Hum Gene Ther*, 14:1255-1264.
108. Bordier, B.B., J. Ohkanda, P. Liu, S.Y. Lee, F.H. Salazar, P.L. Marion, K. Ohashi, L. Meuse, M.A. Kay, J.L. Casey, S.M. Sebti, A.D. Hamilton, J.S. Glenn. 2003, In vivo antiviral efficacy of prenylation inhibitors against hepatitis delta virus (HDV). *J Clin Invest*, 112:407-414.

109. Yant, S.R., M.A. Kay. 2003, Nonhomologous-End-Joining Factors Regulate DNA Repair Fidelity During Sleeping Beauty Element Transposition in Mammalian Cells. *Mol Cell Biol*, 23:8505-8518.
110. Stoll, S., L. Meuse, M.A. Kay, M.P. Calos. 2003, The role of EBV and genomic sequences in gene expression from extrachromosomal gene therapy vectors in mouse liver. *Gene Ther Mol Biol*, 7:211-219.
111. Scimienti, C.R., A.S. Neviasser, E.J. Baba, L. Meuse, M.A. Kay, M.P. Calos. 2003, Epstein-Barr virus provide prolonged robust factor IX expression in mice. *Biotechnol Prog*, 19:144-151.
112. Thomas, C.E., T.A. Storm, Z. Huang, M.A. Kay. 2004, Rapid uncoating of vector genomes is the key to efficient liver transduction with AAV pseudotyped vectors. *J Virol*, 78:3110-3122.
113. Chen, Z.Y., C.Y. He, L. Meuse, S. Shen, and M.A. Kay. 2004, Silencing of episomal transgene expression by plasmid bacterial DNA in vivo. *Gene Ther*, 11:856-864.
114. Wang, A.Y., P. Peng, A. Ehrhardt, T.A. Storm, M.A. Kay. 2004, Comparison of Adenoviral and Adeno-Associated Viral Vectors for Pancreatic Gene Delivery In Vivo. *Hum Gene Ther*, 15:405-413.
115. Layzer, J.M., A.P. McCaffrey, A.K. Tanner, Z. Huang, M.A. Kay, B.A. Sullenger. 2004, In vivo activity of nuclease-resistant siRNAs. *RNA*, 10:766-771.
116. Jenkins, D.D., K. Streetz, M. Tataria, D. Sahar, M. Kurobe, M.T. Longaker, M.A. Kay, K.G. Sylvester. 2004, Donor-Derived, Liver-Specific Protein Expression after Bone Marrow Transplantation. *Transplantation*, 78:530-6.
117. Yant, S.R., J. Park, Y. Huang, J.G. Mikkelsen, M.A. Kay. 2004, Mutational Analysis of the N-Terminal DNA-Binding Domain of Sleeping Beauty Transposase: Critical Residues for DNA Binding and Hyperactivity in Mammalian Cells. *Mol Cell Biol*, 24:9239-9247.
118. Ganaha, F., K. Ohashi, Y.S. Do, J. Lee, K. Sugimoto, H. Minamiguchi, C.J. Elkins, D. Sameni, S. Modanlou, M. Ali, E.Y. Kao, M.A. Kay, J.M. Waugh, M.D. Dake. 2004, Efficient inhibition of in-stent restenosis by controlled stent-based inhibition of elastase: a pilot study. *J Vasc Interv Radiol*, 15:1287-93.
119. Ohashi, K., J.M. Waugh, M.D. Dake, T. Yokoyama, H. Kuge, Y. Nakajima, M. Yamanouchi, H. Naka, A. Yoshioka, M.A. Kay. 2005, Liver Tissue Engineering at Extrahepatic Sites in Mice as a Potential New Therapy for Genetic Liver Diseases. *Hepatology*, 41:132-140.
120. Nakai, H., S. Fuess, T.A. Storm, S. Muramatsu, Y. Nara, M.A. Kay. 2005, Unrestricted Hepatocyte Transduction with Adeno-Associated Virus Serotype 8 Vectors in Mice. *J Virol*, 79:214-224.
121. Chen, Z.Y., C.Y. He, M.A. Kay. 2005, Improved production and purification of minicircle DNA vector free of plasmid bacterial sequences and capable of persistent transgene expression in vivo. *Hum Gene Ther*, 16:126-131.
122. Nakai, H., X. Wu, S. Fuess, T.A. Storm, D. Munroe, E. Montini, S.M. Burgess, M. Grompe, M.A. Kay. 2005, Large-Scale Molecular Characterization of Adeno-Associated Virus Vector Integration in Mouse Liver. *J Virol*, 79:3606-3614.
123. Yant, S.R., X. Wu, Y. Huang, B. Garrison, S.M. Burgess, M.A. Kay. 2005, High-Resolution Genome-Wide Mapping of Transposon Integration in Mammals. *Mol Cell Biol*, 25:2085-2094.
124. Ehrhardt, A., H. Xu, Z. Huang, J.H. Engler, M.A. Kay. 2005, A Direct Comparison of Two Nonviral Gene Therapy Vectors for Somatic Integration: In Vivo Evaluation of the Bacteriophage Integrase phiC31 and the Sleeping Beauty Transposase. *Mol Ther*, 11:695-706.
125. Ohashi, K., H. Nakai, L.B. Couto, M.A. Kay. 2005, Modified Infusion Procedures Affect Recombinant Adeno-Associated Virus Vector Type 2 Transduction in the Liver. *Hum Gene Ther*, 16:299-306.

126. Tward, A.D., K.D. Jones, S. Yant, M.A. Kay, R. Wang, J.M. Bishop. 2005, Genomic progression in mouse models for liver tumors. *Cold Spring Harb Symp Quant Biol*, 70:217-24.
127. Ohashi, K., M.A. Kay, T. Yokoyama, H. Kuge, H. Kanehiro, M. Hisanaga, S. Ko, Y. Nakajima. 2005, Stability and Repeat Regeneration Potential of the Engineered Liver Tissues under the Kidney Capsule in Mice. *Cell Transplant*, 14:621-7.
128. Ohashi, K., M.A. Kay, H. Kuge, T. Yokoyama, H. Kanehiro, M. Hisanaga, S. Ko, M. Nagao, M. Sho, Y. Nakajima. 2005, Heterotopically Transplanted Hepatocyte Survival Depends on Extracellular Matrix Components. *Transplant Proc*, 37:4587-8.
129. Riu, E., D. Grimm, Z. Huang, M.A. Kay. 2005, Increased Maintenance and Persistence of Transgenes by Excision of Expression Cassettes from Plasmid Sequences In Vivo. *Hum Gene Ther*, 16:558–570.
130. Tolar, J., M. Osborn, S. Bell, R. McElmurry, L. Xia, M. Riddle, A. Panoskaltis-Mortari, Y. Jiang, R.S. McIvor, C.H. Contag, S.R. Yant, M.A. Kay, C.M. Verfaillie, B.R. Blazar. 2005, Real-time in Vivo Imaging of Stem Cells Following Transgenesis by Transposition. *Mol Ther*, 12: 42-48.
131. Grimm, D., K. Pandey, H. Nakai, T.A. Storm, M.A. Kay. 2006, Liver Transduction with Recombinant Adeno-Associated Virus Is Primarily Restricted by Capsid Serotype Not Vector Genotype. *J Virol*, 80:426-439.
132. Grimm, D., M.A. Kay. 2006, Therapeutic short hairpin RNA expression in the liver: viral targets and vectors. *Gene Ther*, 13:563-75.
133. Manno, C.S, G.F. Pierce, V.R. Arruda, B. Glader, M. Ragni, J. Rasko, M.C. Ozelo, K. Hoots, P. Blatt, B. Konkle, M. Dake, R. Kaye, M. Razavi, A. Zajko, J. Zehnder, P.K. Rustag, H. Nakai, A. Chew, D. Leonard, J.F. Wright, J. Sommer, M. Tigges, D. Sabatino, A. Luk, H. Jiang, L. Couto, H. Ertl, K.A. High, M.A. Kay. 2006, AAV-mediated, liver directed gene transfer for severe hemophilia B: successful transduction and limitations imposed by the immune response. *Nat Med*, 12:342-7.
134. Grimm, D., K.L. Streetz, C.L. Jopling, T.A. Storm, K. Pandey, C. Davis, P.L. Marion, M.A. Kay. 2006, Fatality in mice due to oversaturation of cellular microRNA/short hairpin RNA pathways. *Nature*, 441:537-41.
135. Tolar, J., M.J. O'Shaughnessy, A. Panoskaltis-Mortari, R.T. McElmurry, S. Bell, M. Riddle, R.S. McIvor, S.R. Yant, M.A. Kay, D. Krause, C.M. Verfaillie, B.R. Blazar. 2006, Host factors that impact the biodistribution and persistence of multipotent adult progenitor cells. *Blood*, 107:4182-8.
136. Inagaki, K., S. Fuess, T.A. Storm, G.A. Gibson, C.F. Mctiernan, M.A. Kay, H. Nakai. 2006, Robust Systemic Transduction with AAV9 Vectors in Mice: Efficient Global Cardiac Gene Transfer Superior to That of AAV8. *Mol Ther*, 14:45-53.
137. Akache, B., D. Grimm, K. Pandey, S.R. Yant, H. Xu, M.A. Kay. 2006, The 37/67-Kilodalton Laminin Receptor Is a Receptor for Adeno-Associated Virus Serotypes 8, 2, 3, and 9. *J Virol*, 80:9831-6.
138. Tolar, J., A.J. Nauta, M.J. Osborn, A. Panoskaltis-Mortari, R.T. McElmurry, S. Bell, L. Xia, N. Zhou, M. Riddle, T.M. Schroeder, J.J. Westendorf, R.S. McIvor, P.C. Hogendoorn, K. Szuhai, L. Oseth, B. Hirsch, S.R. Yant, M.A. Kay, A. Peister, D.J. Prockop, W.E. Fibbe, B.R. Blazar. 2006, Sarcoma Derived from Cultured Mesenchymal Stem Cells. *Stem Cells*, 25(2):371-9.
139. Ehrhardt, A., J.A. Engler, H. Xu, A.M. Cherry, M.A. Kay. 2006, Molecular Analysis of Chromosomal Rearrangements in Mammalian Cells after phiC31-Mediated Integration. *Hum Gene Ther*, 17:1077-94.

140. Ehrhardt, A., S.R. Yant, J.C. Giering, H. Xu, J.A. Engler, M.A. Kay. 2007, Somatic Integration from an Adenoviral Hybrid Vector into a Hot Spot in Mouse Liver Results in Persistent Transgene Expression Levels In Vivo. *Mol Ther*, 15:146-56.
141. Wang, A.Y., A. Ehrhardt, H. Xu, M.A. Kay. 2007, Adenovirus Transduction Is Required for the Correction of Diabetes Using Pdx-1 or Neurogenin-3 in the Liver. *Mol Ther*, 15:255-63.
142. Akache, B., D. Grimm, X. Shen, S. Fuess, S.R. Yant, D.S. Glazer, J. Park, M.A. Kay. 2007, A Two-Hybrid Screen Identifies Cathepsins B and L as Uncoating Factors for Adeno-Associated Virus 2 and 8. *Mol Ther*, 15:330-9.
143. Grimm, D., M.A. Kay. 2007, Combinatorial RNAi: A Winning Strategy for the Race against Evolving Targets? *Mol Ther*, 15:878-88.
144. Yant, S.R., Y. Huang, B. Akache, M.A. Kay. 2007, Site-directed Transposon Integration in Human Cells. *Nucleic Acids Res*, 35:e50.
145. Zayed, H., L. Xia, A. Yerich, S.R. Yant, M.A. Kay, M. Puttaraju, G.J. McGarrity, D.L. Wiest, R.S. McIvor, J. Tolar, B.R. Blazar. 2007, Correction of DNA Protein Kinase Deficiency by Spliceosome-mediated RNA Trans-splicing and Sleeping Beauty Transposon Delivery. *Mol Ther*, 15:1273-79.
146. Riu, E., Z.Y. Chen, H. Xu, C.Y. He, M.A. Kay. 2007, Histone Modifications are Associated with the Persistence or Silencing of Vector-mediated Transgene Expression In Vivo. *Mol Ther*, 15:1348-55.
147. Azuma, H., N. Paulk, A. Ranade, C. Dorrell, M. Al-Dhalimy, E. Ellis, S. Storm, M.A. Kay, M. Finegold, M. Grompe. 2007, Robust expansion of human hepatocytes in *Fah(-)/Rag2(-)/Il2rg(-)* mice. *Nat Biotechnol*, 25:903-10.
148. Inagaki, K., S.M. Lewis, X. Wu, C. Ma, D.J. Munroe, S. Fuess, T.A. Storm, M.A. Kay, H. Nakai. 2007, DNA Palindromes with a Modest Arm Length of ≥ 20 Base Pairs Are a Significant Target for Recombinant Adeno-Associated Virus Vector Integration in the Liver, Muscles, and Heart in Mice. *J Virol*, 81:11290-303.
149. Inagaki, K., C. Ma, T.A. Storm, M.A. Kay, H. Nakai. 2007, The Role of DNA-PKcs and Artemis in Opening Viral DNA Hairpin Termini in Various Tissues in Mice. *J Virol*, 81:11304-21.
150. Shen, X., T. Storm, M.A. Kay. 2007, Characterization of the Relationship of AAV Capsid Domain Swapping to Liver Transduction Efficiency. *Mol Ther*, 15:1955-62.
151. Tward, A.D., K.D. Jones, S. Yant, S.T. Cheung, S.T. Fan, X. Chen, M.A. Kay, R. Wang, J.M. Bishop. 2007, Distinct pathways of genomic progression to benign and malignant tumors of the liver. *Proc Natl Acad Sci USA*, 104:14771-14776.
152. Paskowitz, D.M., K.P. Greenberg, D. Yasumura, D. Grimm, H. Yang, J.L. Duncan, M.A. Kay, M.M. Lavail, J.G. Flannery, D. Vollrath. 2007. Rapid and Stable Knockdown of an Endogenous Gene in Retinal Pigment Epithelium. *Hum Gene Ther*, 18:871-80.
153. Garrison, B.S., S.R. Yant, J.G. Mikkelsen, M.A. Kay. 2007, Postintegrative Gene Silencing Within the Sleeping Beauty Transposition System. *Mol Cell Biol*, 27:8824-33.
154. Moldt, B., S.R. Yant, P.R. Andersen, M.A. Kay, J.G. Mikkelsen. 2007, Cis-Acting Gene Regulatory Activities in the Terminal Regions of Sleeping Beauty DNA Transposon-Based Vectors. *Hum Gene Ther*, 18:1193-1204.
155. Grimm, D., M.A. Kay. 2007, Therapeutic application of RNAi: is mRNA targeting finally ready for prime time? *J Clin Invest*, 117:3633-41.

156. Streetz, K.L., R. Doyonnas, D. Grimm, D.D. Jenkins, S. Fuess, S. Perryman, J. Lin, C. Trautwein, J. Shizuru, H. Blau, K.G. Sylvester, M.A. Kay. 2008, Hepatic Parenchymal Replacement in Mice by Transplanted Allogenic Hepatocytes Is Facilitated by Bone Marrow Transplantation and Mediated by CD4 Cells. *Hepatology*, 47:706-18.
157. Chen, Z.Y., E. Riu, C.Y. He, H. Xu, M.A. Kay. 2008, Silencing of Episomal Transgene Expression in Liver by Plasmid Bacterial Backbone DNA Is Independent of CpG Methylation. *Mol Ther*, 16:548-56.
158. Grimm, D., J.S. Lee, L.Wang, T. Desai, B. Akache, T.A. Storm, M.A. Kay. 2008, In Vitro and In Vivo Gene Therapy Vector Evolution via Multispecies Interbreeding and Retargeting of Adeno-Associated Viruses. *J Virol*, 87:5887-5911.
159. Haussecker, D., D. Cao, Y. Huang, P. Parameswaran, A. Fire, M.A. Kay. 2008, Capped small RNAs and MOV10 in human hepatitis delta virus replication. *Nat Struct Mol Biol*, 15:714-21.
160. McCaffrey, A.P., P. Fawcett, H. Nakai, R.L. McCaffrey, A. Ehrhardt, T.T. Pham, K. Pandey, H. Xu, S. Feuss, T.A. Storm, M.A. Kay. 2008, The host response to adenovirus, helper-dependent adenovirus, and adeno-associated virus in mouse liver. *Mol Ther*, 16:931-41.
161. Zhang, X., M.W. Epperly, M.A. Kay, Z.Y. Chen, T. Smith, D. Franicola, B. Greenberger, P. Komanduri, J.S. Greenberger. 2008 Radioprotection In Vitro and In Vivo by Minicircle Plasmid Carrying the Human Manganese Superoxide Dismutase (MnSOD) Transgene. *Hum Gene Ther*, 19:820-6.
162. Doege, H., D. Grimm, A. Falcon, B. Tsang, T.A. Storm, H. Xu, A.M. Ortegon, M. Kazantzis, M.A. Kay, A. Stahl. 2008, Silencing of Hepatic Fatty Acid Transporter Protein 5 in Vivo Reverses Diet-induced Non-alcoholic Fatty Liver Disease and Improves Hyperglycemia. *J Biol Chem*, 283:22186-92.
163. Giering, J.C., D. Grimm, T.A. Storm, M.A. Kay. 2008, Expression of shRNA from a Tissue-specific pol II Promoter Is an Effective and Safe RNAi Therapeutic. *Mol Ther*, 16:1630-6.
164. Stenler, S., A. Andersson, O.E. Simonson, K.E. Lundin, Z.Y. Chen, M.A. Kay, C.I. Smith, C Sylven, P.Blomberg 2009. Gene Transfer to Mouse Heart and Skeletal Muscles Using a Minicircle Expressing Human Vascular Endothelial Growth Factor 2009. *J Cardiovasc Pharmacol*. Jan 6. [Epub ahead of print]
165. Gu, S., L. Jin, F. Zhang, P. Sarnow, M.A. Kay. 2009, Biological Basis for restriction of microRNA targets to the 3'untranslated region in mammalian mRNAs . *Nat Struct Mol Biol*, 16:144-150.
166. Jager L, M.A. Hausl, C. Rauschhuber, N.M. Wolf, M.A. Kay, A Ehrhardt. 2009. A rapid protocol for construction and production of high-capacity adenoviral vectors. *Nature Protocols* 4:547-564.
167. Cao, D. D. Haussecker, Y. Huang, M.A. Kay. Combined Proteomic-RNAi Screen for host factors involved in Hepatitis Delta Virus Replication. *RNA* 15:1971-1999
168. Huang, M., Z. Chen, S. Hu, F. Jia, G. Hoyt, R.C. Robbins, M.A.Kay, J.C. Wu. 2009 Novel minicircle vector for gene therapy in murine myocardial infarction *Circulation* 120 (11 suppl) S230-237..
169. Beer, S., D.I. Bellovin, J.S. Lee, K.Komatsubara, L.S. Wang, L.S. Koh, K. Borner, T.A. Storm, C.R. Davis, M.A. Kay, D.W. Felsher, D. Grimm 2009. Low-level shRNA Cytotoxicity Can Contribute to MYC-induced Hepatocellular Carcinoma in Adult Mice. *Molecular Therapy* online early publication.
170. Paulk, N., K. Wursthorn, Z. Wang, M.J. Finegold, M.A. Kay, M.Grompe 2009. Adeno-associated virus gene repair corrects a mouse model of hereditary tyrosinemia in vivo. *Hepatology* in press
171. Parameswaran, P., T. Burgon, M. Samuel, C. Wilkins, R.Lu, M. Ansel, V. Heissmeyer, S. Einav, W. Jackson, T. Doukas, S. Paranjape, C. Polacek, F. Barreto dos Santos, R. Jalili, B. Gharizadeh, D.Grimm¹⁶, M.A., Kay, P.Sarnow¹, M. Ronaghi¹⁸, S. Ding⁶, E. Harris¹², M. Chow, M. Diamond, K.

Kirkegaard, J. Glenn⁹, A. Fire 2009. 6 RNA Viruses and 41 Hosts: vs RNAs AND viral modulation of small RNA repertoire in vertebrate and invertebrate systems PLOS Pathogens in press

172. Haussecker, D., Y. Huang, A. Lau, P. Parameswaran, A. Fire, M.A. Kay. Human tRNA-derived small RNAs in RNA silencing RNA in press

Invited Manuscripts

1. Kay, M.A., K.P. Ponder, S.L.C. Woo. 1992, Human gene therapy: Present and future. *Breast Cancer Res Treat*, 21:83-92.
2. Kay, M.A. 1993, Hepatocyte transplantation for liver gene therapy. *Cell Transplantation*, 2:405-406.
3. Kay, M.A., S.L.C. Woo. 1994, Gene therapy for metabolic disorders. *Trends Genet*, 10:253-257.
4. Wilson, C., M.A. Kay. 1995, Immunomodulation to enhance gene therapy. *News and Views. Nat Med*, 1:887-889
5. Kay, M.A. 1995, Hepatic gene therapy for hemophilia B. *Adv Exp Med Biol*, 386: 229-334.
6. Thompson, A., M.A. Kay. 1996, Non-viral gene transfer to the liver. *Hepatology*, 24: 1541-1542
7. Kay, M.A. 1997, Adenoviral Vectors for Hepatic Gene Transfer in Animals. *Chest*, 111:138S-142S.
8. Schowalter, D.B, M.A. Kay. 1997, Gene Therapy: A Status Report. *Pediatric Annals*, 26:562-568.
9. Kay, M.A., D. Liu, P.M. Hoogerbrugge. 1998, Gene Therapy. *Proc Natl Acad Sci USA*, 94:12744-12746
10. Russell, D.W., M.A. Kay. 1999, Adeno-associated Virus Vectors and Hematology. *Blood*, 94:864- 874.
11. Patijn ,G.A., M.A. Kay. 1999, Hepatic Gene Therapy using Adeno-Associated Virus (AAV) vectors. *Semin Liver Dis*, 19:61-69.
12. Kay, M.A., K. High. 1999, Gene therapy for the Hemophilias. *Proc Natl Acad Sci USA*, 96: 9973-9975.
13. Kay, M.A., J. Glorioso, L. Naldini. 2001, Viral Vectors: The art of turning infectious agents into vehicles of therapeutics. *Nat Med*, 24:49-52.
14. Ohashi, K., F. Park, M.A. Kay. 2002, Hepatocyte Transplantation: Clinical and Experimental Application. *J Mol Med*, 79:617-30.
15. Mizuguchi, H., M.A. Kay, T. Hayakawa. 2001, Approaches for generating recombinant adenoviral vectors. *Adv Drug Deliv Rev*, 52:165-176.
16. McCaffrey, A.P., M.A. Kay. 2002, A story of mice and men. *Gene Ther*, 9(23):1563.
17. Thomas C., A. Ehrhardt, M.A. Kay. 2003, Progress and problems with the use of viral vectors for gene therapy. *Nat Rev Genet*, 4:346-358.
18. Grimm, D., M.A. Kay. 2003, From virus evolution to vector revolution: use of naturally occurring serotypes of adeno-associated virus (AAV) as novel vectors for human gene therapy. *Curr Gene Ther*, 3:281-305.
19. McCaffrey A.P., M.A. Kay, C.H. Contag, 2003, Advancing molecular therapies through in vivo bioluminescent imaging. *Mol Imaging*, 2:75-86.
20. McCaffrey A.P., M.A. Kay. 2003, RNA Interference gets infectious. *Gene Ther*, 10:1205.
21. Nakai, H., M.A. Kay. 2003, Looking into the safety of AAV vectors. *Nature*, 17:424: 251.
22. Ohashi, K., M.A. Kay. 2004, Extracellular matrix component cotransplantation prolongs survival of heterotopically transplanted human hepatocytes in mice. *Transplant Proc*, 36(8):2469-70.

23. Grimm, D., K. Pandey, M.A. Kay. 2005, Adeno-associated virus vectors for short hairpin RNA expression. *Methods Enzymol*, 392:381-405.
24. Ehrhardt, A., M.A. Kay. 2005, Gutted adenovirus: a rising star on the horizon? *Gene Ther*, 12:1540-1541.
25. Grimm, D., M.A. Kay. 2006, Therapeutic short hairpin RNA expression in the liver: viral targets and vectors. *Gene Ther*, 13(6):563-75.
26. Kay, M.A. 2007, AAV vectors and tumorigenicity. *Nat Biotechnol*, 25(10):1111-3.
27. Gu, S and M.A. Kay How do microRNAs repress genes? *Silence* in press
28. Haussecker, D. , and M.A. Kay MiR-122 Continues to Blaze the Trail for MicroRNA Therapeutics *Mol. Therapy* in Press

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
4. Kay, M.A., Chapter 71, *Gene Therapy. Nelson Textbook of Pediatrics*, (2003)17th edition. R.E. Behrman, R.M. Kliegman, H.B. Jenson. Saunders: An imprint of Elsevier Science: PA: pp 391-395.
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.