A. Identifying Data:

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

B. Academic History:

Education

1976-1980 BS	Michigan State University Physical Sciences
1980-1986 Ph.D.	Case Western Reserve University Developmental Genetics
1980-1987 MD	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

Phase I/II AAV-2/8-human factor IX mediated gene transfer into the liver. Co-investigator 2009-current

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	Head Division in Human Gene Therapy, Pediatrics 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 (Department 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 (selective examples)

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	Children's Health Initiative Grant Review Committee
2001-9/2003	Dean's Committee on Post-doctoral Affairs
2001-present	Berry Foundation Committee
2002-9/2005	Stanford University Faculty Senate
2002-9/2005	Faculty Senate Executive Committee
7/2006-present	Chairman, Berry Fellowship Committee
2006-present	Dept of Genetics Admissions Committee
2009-present	MSTP Task Force Committee
2009-present	Chairman, Research Advisory Committee Pediatrics

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
American Society for Gene and Cell Therapy

Editorial Boards/Editorships

1. Editorial Board, *Gene Therapy*, March 1995-present
2. Editorial Board, *Human Gene Therapy*, September 1995-2000
3. Editorial Board, *Molecular Therapy***,** August 1999-2003
4. Associate Editor, *Human Gene Therapy*, 2000-2013
5. Associate Editor, *Molecular Therapy*, 2006-2009
6. Editorial Board, *Molecular Therapy*, 2009-present
7. Associate Editor, *Silence* 2009- 2013
8. Senior Editor, Nucleic Acid Therapeutics (formerly Oligonucleotides) 2011-present
9. Editor, *Human Gene Therapy* 2013-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 meeting 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 of the 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. American Society of Cell and Gene Therapy Advisory Council, 2006-2010
22. American Society of Cell and Gene Therapy Chairman of Advisory Council, 2010-2011
23. Board of Directors, Oligonucleotide Society, 9/2007-present
24. Vice President of Oligonucleotide Therapeutic Society, 2009-2010
25. College of CSR Reviewers- NIH, 2010-2012
26. Beta Cell Consortium Executive Committee –NIH-NIDDK 2010-2012
27. Planning Committee ASGCT 2013
28. Organizing Committee for the Canton Nucleic Acids Forum 2013-current
29. GDD NIH Study Section 2017-2021

Academic Honors and Awards

1996 Western Society for Clinical Investigation, Young Investigator Award
1997 Arosenius Swedish Honorary Lectureship
1997 American Society for Clinical Investigation-elected member
2000 E. Mead Johnson Award for Pediatric Researcher of the Year
2000 National Hemophilia Foundation Researcher of the Year
2005 Named Professorship-Dennis Farrey Family Professor
2010 Association for American Physicians elected member
2011 Samuel Rosenthal Prize in Pediatrics
2013 Outstanding Investigator Award- American Society of Cell and Gene Therapy
2015 Stanford OTL Outstanding Inventor Award
2017 Michigan State University Lyman Briggs College Outstanding Alumni Award and Graduation Commencement Speaker
2017 Case Western Reserve School of Medicine Outstanding Alumni Award for Academic Achievement

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-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 Pharmocokinetics 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 & 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 meeting 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.
53. Gordon Conference on Hemostasis and Thrombosis, Colby, ME, July 2002.
54. World Congress of International Society of Hematology- Plenary Speaker, Seoul, Korea, August 2002.
55. 10th Annual European Society for Gene Therapy- Plenary Speaker, Niece, France, October 2002.
56. American Society for Microbiology- Speaker, Banff, Alberta, Canada, March 2003.
57. American Society for Human Gene Therapy- Speaker, Workshop on RNAi, Washington DC, June 2003.
58. 1st Annual International Conference on Transposition and Animal Biotechnology- Speaker, Minneapolis, MN, July 2003.
59. Falk Symposium- Speaker, Germany, October, 2003.
60. Gordon Conference on Viral Vectors for Gene Therapy- Speaker, Santa Barbara, CA, February 2004.
61. American Chemical Society Annual Meeting- Speaker, Anaheim, CA, March 2004.
62. Keystone Symposium on siRNAs and miRNAs- Speaker, Keystone, CO, April 2004.
63. RNAi Conference- Speaker, Boston, MA, May 2004.
64. American Society for Gene Therapy- Education Session, Gene Transfer in Liver, Minneapolis, MN, June 2004.
65. CHI RNAi Conference- Speaker, San Francisco, CA June 2004.

66. FASEB Meeting on Liver Biology- Speaker, Snowmass, CO, August 2004.
67. European Society of Gene Therapy Annual Meeting- Speaker, Edinburgh, Scotland, November 2004.
68. European Society for Gene Therapy- Speaker, Finland, November 2004.
69. Spanish Society for Gene Therapy- Keynote Speaker, Pamplona, Spain, January 2005.
70. Bari International Hemophilia Conference – Pizzomunno, Italy, May 2005.
71. American Society of Gene Therapy Symposia- speaker, St. Louis, MO, June 2005.
72. Japanese Society of Gene Therapy Plenary Invited Plenary- Speaker, Tokyo, Japan, July 2005.
73. Rennebohm Symposium, University of Wisconsin, WI, September 2005.
74. Memorial Sloan Kettering Harold Varmus Presidential Symposium- Speaker, September 2005.
75. Conference on Cell and Gene Therapy- Speaker, Barcelona, Spain, October 2005.
76. Univ Toronto Langdon Hall Conference-Gene Therapy- Speaker, Toronto, Canada, May 2006.
77. Crowley Gene Therapy for Cancer- Speaker, Dallas, TX, September 2006.
78. Keystone meeting on RNAi/microRNA- Speaker, Keystone, CO, January 2007.
79. Intl Soc for Heart & Lung Transplantation Plenary overview on RNAi. San Francisco, CA, March 2007.
80. Gordon Conference on Human Genomics and Genetics- Speaker, Newport, RI, July 2007.
81. Oligotherapeutics Society 13th annual meeting- Speaker, Berlin, Germany, October 2007.
82. 50th Anniversary Reunion for University of Washington Medical Genetics, October 2007.
83. American Society of Hematology, Educational Session on Micro RNA/RNAi, December 2007.
84. Gordon Research Conference, Science of Viral Vectors, Ventura, California, March, 2008.
85. Keystone meeting on RNAi/microRNA - Speaker, Keystone, CO, March, 2008.
86. Gene Therapy & Vaccines - Student invitee, University of Pennsylvania, May 2008.
87. Drug Delivery and Translational Research Conference, New York City, May 2008
88. American Society of Gene Therapy, 11th annual meeting, two plenary talks - Speaker, May 2008.
89. FASEB Liver meeting, Snowmass, Colorado, August 2008.
90. Keystone Meeting on RNA therapeutics - Chair and Plenary speaker, Lake Louise, CA, Feb 2009.
91. Keystone Meeting MicroRNAs in Cancer - Plenary speaker, Keystone, CO, June 2009.
92. Oligonucleotide Therapeutic Society & Nucleic Acid Society of Japan - Speaker & Chair
Fukuoka, Japan, Nov 2009.

93. Keystone Meeting RNA Silencing: Mechanism, Biology and Application - Invited speaker. Lake Louise, Canada, Jan 2010.

94. RNAi: Therapeutics & Mechanism University of Hong Kong - Plenary Speaker, Hong Kong, Nov 2009.

95. 7th Annual Conference of the Israeli Society for Gene Therapy - Invited Speaker, Tel Aviv, May 2010.

96. FASEB Meeting on Liver Biology - Invited Speaker. Snowmass, CO, August 2010.

97. From the RNA World to the Clinic - Invited Speaker. HHMI. Janelia Farms,VA, September 2010.

98. 17th Annual German Gene Therapy Society Meeting - Keynote Address, Munich, Germany, Oct 2010.

99. Symposium of the SFB 455 Viral Offense and Immune Defense - Student Invited Speaker, Munich, Germany, October 2010.

100. ASGCT Strategic Planning Meeting - Society Leadership, New Orleans, LA, January 2011.

101. Beta Cell Biology Consortium - Invited Speaker, Washington DC, May 2011.

102. Mammalian Genome Editing & Gene Therapy: Recent Developments, Current State of Play, US Defense Dept Washington DC, August 2011.

103. ASGCT Plenary Session on Viral Vectors - Invited Speaker, Seattle, WA, May 2011.

104. Washington University Translational Research Series, St. Louis, August 2011.

105. 7th International Oligonucleotide Society - Co-organizer, Meeting Chair, and Plenary Speaker Copenhagen, Denmark, September 2011.

106. Oregon Health Sciences University, Program in Molecular and Cellular Biosciences Graduate Student - Invited Seminar Speaker, Portland, OR, October 2011.

107. University of Pennsylvania Gene Therapy Seminar Series - Invited Speaker, October 2011.

108. Korean Society for Oligonucleotide Therapeutic Society - Invited Plenary Speaker, Seoul, Korea, November 2011.

109. Keystone Symposia, Nucleic Acid Therapeutics: From Base Pairs to Bedsides - Co-Organizer, and Speaker, Santa Fe, NM, January 2012

110. Pugwash Purdue Student Biotechnology Symposium- Gene and RNAi based therapies. Invited Speaker, Purdue Univ. March 31, 2012.

111. American Association for Cancer Research (AACR) Annual Meeting – Invited Speaker. Can RNAi Cure Cancer? Chicago, IL, April 3, 2012.

112. Inaugural Nanobiotechnology Conference at University of Illinois – Invited Speaker. Gene Therapy Vectors, April 5, 2012.

113. Stem Cell Clonality and Genome Stability – Invited Speaker, Directing rAAV integration into the rDNA locus. Philadelphia, PA, May 15, 2012.

114. Bill Gates Foundation Gene Therapy Technology Meeting-Speaker on AAV and non-viral gene transfer approaches. Seattle, WA, June 27, 2012.

115. 22nd HCS/the 4th JARI Joint International Symposium on MicroRNAs in Cancer – Session Chair and Speaker, Hiroshima, Japan, August 30, 2012.

116. Cold Spring Harbor – Oligonucleotide Therapeutics- Invited Speaker CSH, NY May 2013

117. GTCBio- 4th Annual RNAi Research and Therapeutics Conference- Keynote Speaker San Francisco CA June 20, 2013

118. ASGCT Outstanding Investigator Award Plenary Lecture May 2013 - Salt Lake City, UT

119. Oregon Health Sciences Gene Therapy Symposium- Keynote Speaker - November 20, 2013, Portland-Oregon

120. UC Santa Cruz RNA Biology International Meeting. Invited Speaker March 2014 Santa Cruz, CA

121. Case Western Reserve University Student Invitation Seminar Dept of Genetics April 23, 2014 Cleveland Ohio

122. Nature China Conference – Genomics and Stem Cell Based Therapies: Shaping the future of personalized medicine. Invited Speaker May 2014 Guangzhou China.

123. Chinese Society of Gene and Cell Therapy Annual Meeting. Invited Speaker. June 2014 Chengdu China.

124. American Biological Society Wedum Honorary Keynote Speaker. October 2014 San Diego CA

125. 10th Annual Oligonucleotide Therapeutic Society Session Chair and Invited Speaker October 2014 San Diego CA.

126. European Society for Gene and Cell Therapy Plenary Speaker, The Hague, Netherlands October 2014

127. CRISPR Precision Gene Editing Conference Invited Speaker, Cambridge MA February 24, 2015

128. University of Iowa Internal Medicine Plenary Speaker Research Day, Iowa City, Iowa March 12, 2015

129. Australasian Gene and Cell Therapy Conference Invited Speaker on AAV vectors. April 2015.

130. Nature Science Café (Sponsored by Nature Biotechnology) Invited Speaker and Panelist, June 6, 2015 San Diego California
131. 3rd Canton Nucleic Acids Forum. Plenary Speaker on Genome Editing. Nov 18-19th 2015 Guangzhou China.
132. The Wellcome Trust Sanger Institute AstraZeneca CRISPR Conference. Plenary Speaker. Jan 17-19th , 2016. Cambridge, England.
133. French Society for Gene and Cell Therapy Meeting. Plenary Speaker. March 8-9th 2016 Marseille France.
134. British Society for Gene and Cell Therapy Annual Meeting. Plenary Speaker April 14-15, 2016
135. JASON Biodefense Meeting—CRISPR and Genome Editing. Invited Speaker. June 20, 2016 LaJolla CA
136. Gordon Conference. Post-transcriptional Gene Regulation. July 10-15, 2016. Invited Speaker. Stowe VT
137. Cell and Gene Therapy for HIV Cure. Invited Keynote speaker August 3-5, 2016. Seattle WA
138. 4th GRL International Conference on RNAi Therapeutics. Keynote speaker September 9, 2016 Seoul Korea
139. Medicine X Stanford University. Speaker and Session Conference Leader on Developing Viruses for Therapeutics. Sept. 17, 2016 Stanford University.
140. European Society for Gene and Cell Therapy. Plenary Keynote Speaker. October 18-21, 2016. Florence, Italy
141. Genome Editing for Gene and Cell Therapy (Nature Medicine Sponsored). Plenary Speaker. November 2-3, 2016. Hannover, Germany
142. 15th Annual Gene Therapy Symposium University of California at Davis. Plenary Speaker November 16-18, 2017. Sonoma, CA
143. Keystone Meeting Precision Genome Engineering. Invited Speaker. January 8-12, 2017. Breckenridge, CO.
144. Medical Scientist Training Program Seminar Series Student Invitation. University of Kentucky. February 9, 2017. Lexington, KY
145. Fudan University Distinguished Faculty Lecture Shanghai China April 11, 2017
146. American Association for Pharmaceutical Sciences Keynote address San Diego CA May 1, 2017
147. American Association for the Advancement of Liver Disease. A New Era for Genome Editing, Plenary Speaker October 22, 2017 Washington DC

F. Published Papers

1 **Kay MA**, Jacobs-Lorena M. Selective translational regulation of ribosomal protein gene expression during early development of Drosophila melanogaster. Molecular and cellular biology 1985; 5(12): 3583-3592. PMCID: PMC369189.

2 **Kay MA**, Jacobs-Lorena M. Developmental genetics of ribosome synthesis in Drosophila. Trends in Genetics 1987; 3347-351.

3 Qian S, Zhang JY, **Kay MA**, Jacobs-Lorena M. Structural analysis of the Drosophila rpA1 gene, a member of the eucaryotic 'A' type ribosomal protein family. Nucleic acids research 1987; 15(3): 987-1003. PMCID: PMC340503.
4 **Kay MA**, Zhang JY, Jacobs-Lorena M. Identification and germline transformation of the ribosomal protein rp21 gene of Drosophila: complementation analysis with the Minute QIII locus reveals nonidentity. Molecular & general genetics : MGG 1988; 213(2-3): 354-358.
5 **Kay MA**, McCabe ED. Escherichia coli sepsis and prolonged hypophosphatemia following exertional heat stroke. Pediatrics 1990; 86(2): 307-309.
6 **Kay MA**, O'Brien W, Kessler B, McVie R, Ursin S, Dietrich K, McCabe ER. Transient organic aciduria and methemoglobinemia with acute gastroenteritis. Pediatrics 1990; 85(4): 589-592.
7 **Kay MA**, Baley P, Rothenberg S, Leland F, Fleming L, Ponder KP, Liu T, Finegold M, Darlington G, Pokorny W, et al. Expression of human alpha 1-antitrypsin in dogs after autologous transplantation of retroviral transduced hepatocytes. Proceedings of the National Academy of Sciences of the United States of America 1992; 89(1): 89-93. PMCID: PMC48181.
8 **Kay MA**, Li Q, Liu TJ, Leland F, Toman C, Finegold M, Woo SL. Hepatic gene therapy: persistent expression of human alpha 1-antitrypsin in mice after direct gene delivery in vivo. Human gene therapy 1992; 3(6): 641-647.
9 **Kay MA**, Ponder KP, Woo SL. Human gene therapy: present and future. Breast cancer research and treatment 1992; 21(2): 83-93.
10 Liu TJ, **Kay MA**, Darlington GJ, Woo SL. Reconstitution of enzymatic activity in hepatocytes of phenylalanine hydroxylase-deficient mice. Somatic cell and molecular genetics 1992; 18(1): 89-96.
11 Cristiano RJ, Smith LC, **Kay MA**, Brinkley BR, Woo SL. Hepatic gene therapy: efficient gene delivery and expression in primary hepatocytes utilizing a conjugated adenovirus-DNA complex. Proceedings of the National Academy of Sciences of the United States of America 1993; 90(24): 11548-11552. PMCID: PMC48021.
12 **Kay MA**. Hepatocyte transplantation for liver gene therapy. Cell transplantation 1993; 2(5): 405-406.
13 **Kay MA**, Rothenberg S, Landen CN, Bellinger DA, Leland F, Toman C, Finegold M, Thompson AR, Read MS, Brinkhous KM, et al. In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs. Science (New York, NY) 1993; 262(5130): 117-119.
14 Kolodka TM, Finegold M, **Kay MA**, Woo SL. Hepatic gene therapy: efficient retroviral-mediated gene transfer into rat hepatocytes in vivo. Somatic cell and molecular genetics 1993; 19(5): 491-497.
15 Li Q, **Kay MA**, Finegold M, Stratford-Perricaudet LD, Woo SL. Assessment of recombinant adenoviral vectors for hepatic gene therapy. Human gene therapy 1993; 4(4): 403-409.
16 **Kay MA**, Landen CN, Rothenberg SR, Taylor LA, Leland F, Wiehle S, Fang B, Bellinger D, Finegold M, Thompson AR, et al. In vivo hepatic gene therapy: complete albeit transient correction of factor IX deficiency in hemophilia B dogs. Proceedings of the National Academy of Sciences of the United States of America 1994; 91(6): 2353-2357. PMCID: PMC43369.
17 **Kay MA**, Woo SL. Gene therapy for metabolic disorders. Trends in genetics : TIG 1994; 10(7): 253-257.
18 Barr D, Tubb J, Ferguson D, Scaria A, Lieber A, Wilson C, Perkins J, **Kay MA**. Strain related variations in adenovirally mediated transgene expression from mouse hepatocytes in vivo: comparisons between immunocompetent and immunodeficient inbred strains. Gene therapy 1995; 2(2): 151-155.
19 Fang B, Eisensmith RC, Wang H, **Kay MA**, Cross RE, Landen CN, Gordon G, Bellinger DA, Read MS, Hu PC, et al. Gene therapy for hemophilia B: host immunosuppression prolongs the therapeutic effect of adenovirus-mediated factor IX expression. Human gene therapy 1995; 6(8): 1039-1044.

20 **Kay MA**. Hepatic gene therapy for hemophilia B. Advances in experimental medicine and biology 1995; 386229-234.
21 **Kay MA**, Graham F, Leland F, Woo SL. Therapeutic serum concentrations of human alpha-1-antitrypsin after adenoviral-mediated gene transfer into mouse hepatocytes. Hepatology (Baltimore, Md) 1995; 21(3): 815-819.
22 **Kay MA**, Holterman AX, Meuse L, Gown A, Ochs HD, Linsley PS, Wilson CB. Long-term hepatic adenovirus-mediated gene expression in mice following CTLA4Ig administration. Nature genetics 1995; 11(2): 191-197.
23 Lieber A, Peeters MJ, Gown A, Perkins J, **Kay MA**. A modified urokinase plasminogen activator induces liver regeneration without bleeding. Human gene therapy 1995; 6(8): 1029-1037.
24 Lieber A, Vrancken Peeters MJ, **Kay MA**. Adenovirus-mediated transfer of the amphotropic retrovirus receptor cDNA increases retroviral transduction in cultured cells. Human gene therapy 1995; 6(1): 5-11.
25 Lieber A, Vrancken Peeters MJ, Meuse L, Fausto N, Perkins J, **Kay MA**. Adenovirus-mediated urokinase gene transfer induces liver regeneration and allows for efficient retrovirus transduction of hepatocytes in vivo. Proceedings of the National Academy of Sciences of the United States of America 1995; 92(13): 6210-6214. PMCID: PMC41672.
26 Scaria A, Curiel DT, **Kay MA**. Complementation of a human adenovirus early region 4 deletion mutant in 293 cells using adenovirus-polylysine-DNA complexes. Gene therapy 1995; 2(4): 295-298.
27 Wilson C, **Kay MA**. Immunomodulation to enhance gene therapy. Nature medicine 1995; 1(9): 887-889.
28 Lieber A, He CY, Kirillova I, **Kay MA**. Recombinant adenoviruses with large deletions generated by Cre-mediated excision exhibit different biological properties compared with first-generation vectors in vitro and in vivo. Journal of virology 1996; 70(12): 8944-8960. PMCID: PMC190992.
29 Lieber A, He CY, Polyak SJ, Gretch DR, Barr D, **Kay MA**. Elimination of hepatitis C virus RNA in infected human hepatocytes by adenovirus-mediated expression of ribozymes. Journal of virology 1996; 70(12): 8782-8791. PMCID: PMC190975.
30 Lieber A, **Kay MA**. Adenovirus-mediated expression of ribozymes in mice. Journal of virology 1996; 70(5): 3153-3158. PMCID: PMC190178.
31 Liu ML, Winther BL, **Kay MA**. 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. Journal of virology 1996; 70(4): 2497-2502. PMCID: PMC190094.
32 Murry CE, **Kay MA**, Bartosek T, Hauschka SD, Schwartz SM. Muscle differentiation during repair of myocardial necrosis in rats via gene transfer with MyoD. The Journal of clinical investigation 1996; 98(10): 2209-2217. PMCID: PMC507669.
33 Peeters MJ, Patijn GA, Lieber A, Meuse L, **Kay MA**. Adenovirus-mediated hepatic gene transfer in mice: comparison of intravascular and biliary administration. Human gene therapy 1996; 7(14): 1693-1699.
34 Sandig V, Loser P, Lieber A, **Kay MA**, Strauss M. HBV-derived promoters direct liver-specific expression of an adenovirally transduced LDL receptor gene. Gene therapy 1996; 3(11): 1002-1009.
35 Thompson AR, **Kay MA**. Nonviral gene transfer to the liver. Hepatology (Baltimore, Md) 1996; 24(6): 1541-1542.
36 Vrancken Peeters MJ, Perkins AL, **Kay MA**. Method for multiple portal vein infusions in mice: quantitation of adenovirus-mediated hepatic gene transfer. BioTechniques 1996; 20(2): 278-285.
37 Barr D, **Kay MA**. Methods for delivery of genes to hepatocytes in vivo using recombinant adenovirus vectors. Methods in molecular medicine 1997; 7205-212.

38 Bennett RL, Karayiorgou M, Sobin CA, Norwood TH, **Kay MA**. 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. American journal of human genetics 1997; 61(6): 1450-1454. PMCID: PMC1716062.
39 Bethune C, Bui T, Liu ML, **Kay MA**, Ho RJ. Development of a high-performance liquid chromatographic assay for G418 sulfate (Geneticin). Antimicrobial agents and chemotherapy 1997; 41(3): 661-664. PMCID: PMC163768.
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Book Chapters

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Other Support

Mark Kay

ACTIVE

R01 AI071068 (Kay) 08/01/2016 – 07/31/2021 **2.4** calendar
National Institutes of Health $302,507
"Acute/chronic limitations to transcriptional RNAi therapies for infectious and other liver diseases"
The goal of this project is to develop an RNAi gene therapeutic for treating hepatitis viral infection.

U19 AI109662 (Glenn) 03/01/2014 – 03/31/2019 **1.0** calendar
National Institutes of Health $150,000
"*Advancing Broad Spectrum Host-Targeting Antiviral Strategies to the Clinic"*
The goal of this project is to identify the key determinants of pathogenesis and design novel antiviral strategies for selected viral pathogens

U01 DK089569 (Grompe) 09/30/2014 – 06/30/2019 **1.0** calendar
Prime Sponsor: National Institutes of Health $173,607
Oregon Health Sciences University
"In vivo targeting of diabetes-relevant human cell types with rAAV vectors"
The goal of this project is to establish and develop rAAV vectors that transduce cells near or in the pancreas for re-programming non-beta to beta cells for the treatment of diabetes.

R01 AI116698 (Kay) 03/01/2015 – 03/31/2020 **1.9** calendar
National Institutes of Health $371,896
"Selection of new rAAV Vectors using Replicating Viral Capsids Libraries"
The goal of this project is to generate new AAV capsid libraries and perform high throughput screens to isolate viruses with unique infection/transduction properties. These capsids will then be used in creating new AAV vectors and evaluated for their potential in preclinical animal models.

R01 HL064274 (Kay) 08/01/2015 – 05/31/2019 **2.3** calendar
National Institutes of Health $347,965
"Hepatic Gene Transfer for Treatment of Hemophilias A & B"
The goal of this project is to continue our studies on non-viral gene transfer vectors including establishing how they maintain their activity in vivo long-term and improving their efficacy. Our studies will advance gene therapeutic approaches for many conditions especially those efforts towards achieving a cure for the hemophilias.

OPP1154293 (Kay) 09/01/2016 – 02/28/2018 **1.0** calendar
Bill & Melinda Gates Foundation $651,878
"Establishing comparative transduction efficiencies and dosing requirements for AAV1, AAV-NP22 and AAV-NP66 in nonhuman primates to inform future passive vaccine trials"
The goal of this project is to allow us to perform the final pre-clinical validation comparing head-to-head AAV1 with our new variants AAV-NP22 and NP66 in a large cohort of rhesus macaques.

R01 DK114483 (Kay) 08/01/2017 – 07/31/2021 **1.8** calendar
National Institutes of Health $338,115
"The role of small RNA derived tRNAs in gene regulation: Mechanism and Therapeutic Applications"
tRNA derived small RNAs (tsRNAs) represent a class of non-coding RNAs that play important yet not well defined roles in gene regulation. We further define a novel mechanism by which a specific tsRNA regulates ribosome biogenesis. We will establish if other tsRNA species have similar models of regulation. Finally, we will manipulate these RNAs using gene therapy and oiigonucleotide delivery and establish their therapeutic potential in human disease states

Falk Medical Research Trust Catalyst Awards Program (Kay) 11/30/2017 – 11/29/2018 **0.6** calendar
$273,368
Dr. Ralph & Marian Falk Medical Research Trust
"Enhancement of therapeutic AAV-mediated gene targeting without nucleases"
The goal of this project is to develop a safe mean to deliver a therapeutic gene into a specific and preselected region of the human genome to supply a gene product in patients.

OVERLAP
None

At no time during the year is the total effort over 100% FTE