

A. Identifying Data:

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

Current Address: 1071 Peninsular CT
Los Altos, CA 94024

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

Medical License: A71114 State of CA. 2000-present
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 2009-2013

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
08/01/98 - Present	Head Division in Human Gene Therapy, Pediatrics Stanford University School of Medicine
05/01/01 - Present	Professor, Departments of Pediatrics and Genetics, Stanford University
02/01/03- 2004	Co-Founder and Chief Scientific Advisor of Avocel
10/01/05-Present	Dennis Farrey Family Professor
04/2009 – 02/2019	Associate Chair for Basic Research (Department of Pediatrics)
06/2013	Scientific co-founder of Voyager Therapeutics
11/2014-11/2022	Co-founder, consultant, SAB and Board of Directors LogicBio Therapeutics

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)

Children's Health Initiative –Genetics Subcommittee

1998-present	Medical Scientist Training Program Steering Committee
1998-present	Search Committees for 7 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 Admissions Committee
2009-2024	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 1995-2005
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, 9/2007-2010
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 2013-2021
29. GDD NIH Study Section 2017-2020

Academic Honors and Awards

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| 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
- 2019 Michigan State University Distinguished Alumni Award
- 2020 Elected to the National Academy of Inventors

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 & 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
93. Fukuoka, Japan, Nov 2009
94. Keystone Meeting RNA Silencing: Mechanism, Biology and Application - Invited speaker. Lake Louise, Canada, Jan 2010
95. RNAi: Therapeutics & Mechanism University of Hong Kong - Plenary Speaker, Hong Kong, Nov 2009
96. 7th Annual Conference of the Israeli Society for Gene Therapy - Invited Speaker, Tel Aviv, May 2010
97. FASEB Meeting on Liver Biology - Invited Speaker. Snowmass, CO, August 2010
98. From the RNA World to the Clinic - Invited Speaker. HHMI. Janelia Farms, VA, September 2010
99. 17th Annual German Gene Therapy Society Meeting - Keynote Address, Munich, Germany, Oct 2010
100. Symposium of the SFB 455 Viral Offense and Immune Defense - Student Invited Speaker, Munich, Germany, October 2010
101. ASGCT Strategic Planning Meeting - Society Leadership, New Orleans, LA, January 2011

102. Beta Cell Biology Consortium - Invited Speaker, Washington DC, May 2011
103. Mammalian Genome Editing & Gene Therapy: Recent Developments, Current State of Play, US Defense Dept Washington DC, August 2011
104. ASGCT Plenary Session on Viral Vectors - Invited Speaker, Seattle, WA, May 2011
105. Washington University Translational Research Series, St. Louis, August 2011
106. 7th International Oligonucleotide Society - Co-organizer, Meeting Chair, and Plenary Speaker Copenhagen, Denmark, September 2011
107. Oregon Health Sciences University, Program in Molecular and Cellular Biosciences Graduate Student - Invited Seminar Speaker, Portland, OR, October 2011
108. University of Pennsylvania Gene Therapy Seminar Series - Invited Speaker, October 2011
109. Korean Society for Oligonucleotide Therapeutic Society - Invited Plenary Speaker, Seoul, Korea, November 2011
111. Keystone Symposia, Nucleic Acid Therapeutics: From Base Pairs to Bedsides - Co-Organizer, and Speaker, Santa Fe, NM, January 2012
112. Pugwash Purdue Student Biotechnology Symposium- Gene and RNAi based therapies. Invited Speaker, Purdue Univ. March 31, 2012
113. Association for Cancer Research (AACR) Annual Meeting – Invited Speaker. Can RNAi Cure Cancer? Chicago, IL, April 3, 2012
114. Inaugural Nanobiotechnology Conference at University of Illinois – Invited Speaker. Gene Therapy Vectors, April 5, 2012
115. Stem Cell Clonality and Genome Stability – Invited Speaker, Directing rAAV integration into the rDNA locus. Philadelphia, PA, May 15, 2012
116. European Science Foundation meeting on Antiviral RNAi. Invited Speaker on Targeting the HCV antigenome Pultusk, Poland June 11-15
117. Bill Gates Foundation Gene Therapy Technology Meeting-Speaker on AAV and non-viral gene transfer approaches. Seattle, WA, June 27, 2012
118. 22nd HCS/the 4th JARI Joint International Symposium on MicroRNAs in Cancer – Session Chair and Speaker, Hiroshima, Japan, August 30, 2012
119. Cold Spring Harbor – Oligonucleotide Therapeutics- Invited Speaker CSH, NY May 2013
120. GTCBio- 4th Annual RNAi Research and Therapeutics Conference- Keynote Speaker San Francisco CA June 20, 2013
121. ASGCT Outstanding Investigator Award Plenary Lecture May 2013 - Salt Lake City, UT
122. Oregon Health Sciences Gene Therapy Symposium- Keynote Speaker - November 20, 2013, Portland-Oregon
123. UC Santa Cruz RNA Biology International Meeting. Invited Speaker March 2014 Santa Cruz, CA
124. Case Western Reserve University Student Invitation Seminar Dept of Genetics April 23, 2014 Cleveland Ohio
125. Nature China Conference – Genomics and Stem Cell Based Therapies: Shaping the future of personalized medicine. Invited Speaker May 2014 Guangzhou China
126. Chinese Society of Gene and Cell Therapy Annual Meeting. Invited Speaker. June 2014 Chengdu China
127. American Biological Society Wedum Honorary Keynote Speaker. October 2014 San Diego CA

128. 10th Annual Oligonucleotide Therapeutic Society Session Chair and Invited Speaker October 2014 San Diego CA
129. European Society for Gene and Cell Therapy Plenary Speaker, The Hague, Netherlands October 2014
130. CRISPR Precision Gene Editing Conference Invited Speaker, Cambridge MA February 24, 2015
131. University of Iowa Internal Medicine Plenary Speaker Research Day, Iowa City, Iowa March 12, 2015
132. Australasian Gene and Cell Therapy Conference Invited Speaker on AAV vectors. April 2015
133. Nature Science Café (Sponsored by Nature Biotechnology) Invited Speaker and Panelist, June 6, 2015 San Diego California
134. 3rd Canton Nucleic Acids Forum. Plenary Speaker on Genome Editing. Nov 18-19th 2015 Guangzhou China
135. The Wellcome Trust Sanger Institute AstraZeneca CRISPR Conference. Plenary Speaker. Jan 17-19th , 2016. Cambridge, England
136. French Society for Gene and Cell Therapy Meeting. Plenary Speaker. March 8-9th 2016 Marseille France
137. British Society for Gene and Cell Therapy Annual Meeting. Plenary Speaker April 14-15, 2016
138. JASON Biodefense Meeting—CRISPR and Genome Editing. Invited Speaker. June 20, 2016 LaJolla CA
139. Gordon Conference. Post-transcriptional Gene Regulation. July 10-15, 2016. Invited Speaker. Stowe VT
140. Cell and Gene Therapy for HIV Cure. Invited Keynote speaker August 3-5, 2016. Seattle WA
141. 4th GRL International Conference on RNAi Therapeutics. Keynote speaker September 9, 2016 Seoul Korea
142. Medicine X Stanford University. Speaker and Session Conference Leader on Developing Viruses for Therapeutics. Sept. 17, 2016 Stanford University
143. European Society for Gene and Cell Therapy. Plenary Keynote Speaker. October 18-21, 2016.
144. Florence, Italy
145. Genome Editing for Gene and Cell Therapy (Nature Medicine Sponsored). Plenary Speaker. November 2-3, 2016. Hannover, Germany
146. 15th Annual Gene Therapy Symposium University of California at Davis. Plenary Speaker November 16-18, 2017. Sonoma, CA
147. Keystone Meeting Precision Genome Engineering. Invited Speaker. January 8-12, 2017. Breckenridge, CO
148. Medical Scientist Training Program Seminar Series Student Invitation. University of Kentucky. February 9, 2017. Lexington, KY
149. Fudan University Distinguished Faculty Lecture Shanghai China April 11, 2017
150. American Association for Pharmaceutical Sciences Keynote address San Diego CA May 1, 2017
151. American Association for the Advancement of Liver Disease. A New Era for Genome Editing, Plenary
152. Speaker October 22, 2017 Washington DC
153. China Nucleic Acids Platform. Plenary Speaker. tRNA derived small RNAs. Guangzhou China November 7-10, 2017
154. Rice University/Baylor College of Medicine Symposium on Gene Therapy. Keynote Speaker. Novel AAV vectors for classical and genome editing based therapeutics. Houston TX. Dec 5th 2017
155. Facilitate Cell and Gene Therapy World 2018. Two panel sessions—Viral vs Non-viral vectors and Genome Editing. Miami FL Jan 22-24 2018
156. Harvard University Seminar on tRNA derived small RNAs. Boston MA Feb 8, 2018
157. Cincinnati Childrens Hospital. Gene Therapy Seminar. Cincinnati OH Feb 22, 2018

158. National Hemophilia Foundation. Invited speaker – two talks AAV capsids and Genome Engineering. Washington DC Feb. 23-24, 2018
159. University of Nebraska Invited by MSTP Students for Seminar on Gene Therapeutics. April 4, 2018
160. University of North Dakota Invited Seminar Speaker on Gene Therapy. Fargo ND April 11, 2018
161. 27th International tRNA conference. Invited Speaker - tRNA derived small RNAs and their role in gene regulation. Sept 2018 Strasbourg France
162. Univ California San Diego. Seminar Series Invited Speak Jan 17, 2019. La Jolla CA
163. In vivo Gene Therapy and Genome Editing Summit May 18-19, 2019 Miami FL
164. Keystone Meeting Protein Replacement through Nucleic Acid Therapies Invited Speak April 8-12, 2019
165. Steamboat Springs CO
166. Translational Medicine and Therapeutics Series. Invited Speaker Univ Pennsylvania . May 15 2019 Philadelphia PA
167. Harvard-Broad Institute Workshop on Chemical Biology Therapeutic Workshop. August 23, 2019 Cambridge MA
168. Sanford Health Forum on Human Genetics. Plenary Speaker Sept 6, 2019. Sioux Falls SD
169. University of Georgia College of Pharmacy Series on Therapeutics Feb 5, 2020 Athens GA
170. China Nucleic Acids Forum Plenary Speaker Nov 2019 Guangzhou China
171. Peking University Seminar Speaker. Nov 2019 Beijing China
172. University of Georgia Seminar Speaker Feb 5, 2020 Athens GA
173. University of British Columbia Grand Rounds (Pediatrics) on Genome Editing (virtual) Feb 12, 2021
174. American Society. For Biochemistry and Molecular Biology. Invited speaker on tsRNA biology (virtual) April 27, 2001
175. ASGCT Virtual Workshop on AAV integration invited speaker and discussant Aug 21, 2021
176. 5th IGC conference Chinese Society of Biotechnology and Biomap Global. Keynote speaker (virtual) Oct 11, 2021
177. European Society for Gene and Cell Therapy. Invited plenary speaker in opening session (virtual) Oct 19th 2021
178. 12th National Hemophilia Foundation. Invited speaker on AAV transduction mechanisms. Wash DC Nov 2021
179. 10th Orphan Disease Symposium. Invited Speaker. AAV transduction mechanisms. Miami FL Nov 2021.
180. World Federation of Hemophilia World International Plenary Speaker on the State of Gene Therapy for Hemophilia. Montreal Canada May 2022
181. ASGCT co-workshop organizer/speaker on AAV integration. Washington DC May 2022
182. Sanford Symposium on Genetic Diseases. Speaker on Gene therapy for genetic diseases. Sept 2022
183. Genome Editing for Rare Diseases. Invited speaker on AAV epigenetics. Miami FL October 2022
184. Agency for Science Technology and Research. Invited speaker on AAV vectors for Gene Therapy. Singapore. Jan 2023
185. National University of Singapore Invited seminar speaker on Lnc122/miR122 non-coding RNAs. Singapore. Jan 2023
186. OPT Congress on Emerging mRNA therapeutics. Invited speaker on Lnc122/miR122 non-coding RNAs and session chair. Boston MA. March 2023
187. National Hemophilia Meeting on Gene Therapy. Invited speaker. AAV epigenetics. Washington DC March 2023

188. University of Pittsburgh Liver Center. Invited Seminar Speaker. Gene Transfer and liver biology. Pittsburgh PA March 2023
189. University of California San Diego- Pediatric Research Retreat. Invited speaker on Gene therapy. La Jolla CA. April 2023
190. ASGCT Workshop Speaker on How to become a site for AAV clinical trials. Los Angeles CA. May 2023.
191. Annual RNA consortium meeting meeting. Speaker Linking the Lnc122 RNA to MYC and cancer. City of Hope California. May 2023
192. China-America Advanced Therapy International Summit meeting. Invited speaker on The Difficulties in selecting an optimized AAV capsid for clinical trials (by zoom). Shanghai China. May 2023.
193. Genetics Therapies Conference. Invited speaker. AAV vector biology. Big Sky, MT June 23-25, 2023
194. Singapore Society of Gene Therapy. Invited Speaker. Choosing the best AAV vectors for clinical trials. Singapore. August 7-8, 2024
195. Chinese University of Hong Kong – Shenzhen Campus 10 year anniversary. Invited for 3 talks. Keynote Speaker: (1) AAV vector epigenetics. (2) miR122/Lnc122 and liver cancer. (3) Workings between Academic and Industry in Gene Based Therapeutics. Shenzhen, China. March 21-23, 2024
196. SAPA and Chinese Society for Cell and Gene Therapy. Keynote address on AAV vector biology. Suzhou China June 14-15, 2024
197. British Society for Gene and Cell Therapy Meeting. Keynote speaker on AAV vectors and clinical development. Oxford, England June 18-21, 2024
198. Miami in vivo gene therapy and genome editing summit. Invited speaker. Miami, FL Oct 14-16, 2024
199. World Federation of Hemophilia. Montreal Bleeding Disorders Workshop. Invited Speaker. Montreal Canada. Nov 7-9

F. Published Papers

1. Kay MA, Jacobs-Lorena M. 1985. Selective translational regulation of ribosomal protein gene expression during early development of *Drosophila melanogaster*. *Mol Cell Biol*.5:3583-92. 10.1128/mcb.5.12.3583; PMC369189
2. Kay MA, Jacobs-Lorena M. 1987. Developmental genetics of ribosome synthesis in *Drosophila* *Trends Genet*.3C:347-351. 10.1016/0168-9525(87)90295-2
3. Qian S, Zhang JY, Kay MA, Jacobs-Lorena M. 1987. Structural analysis of the *Drosophila* rpA1 gene, a member of the eucaryotic 'A' type ribosomal protein family. *Nucleic Acids Res*.15:987-1003. 10.1093/nar/15.3.987; PMC340503
4. Kay MA, Zhang JY, Jacobs-Lorena M. 1988. Identification and germline transformation of the ribosomal protein rp21 gene of *Drosophila*: complementation analysis with the Minute QIII locus reveals nonidentity. *Mol Gen Genet*.213:354-8. 10.1007/BF00339602
5. Kay MA, McCabe ED. 1990. Escherichia coli sepsis and prolonged hypophosphatemia following exertional heat stroke. *Pediatrics*.86:307-9
6. Kay MA, O'Brien W, Kessler B, McVie R, Ursin S, Dietrich K, McCabe ER. 1990. Transient organic aciduria and methemoglobinemia with acute gastroenteritis. *Pediatrics*.85:589-92
7. Kay MA, Baley P, Rothenberg S, Leland F, Fleming L, Ponder KP, Liu T, Finegold M, Darlington G, Pokorny W, et al. 1992. Expression of human alpha 1-antitrypsin in dogs after autologous

- transplantation of retroviral transduced hepatocytes. *Proc Natl Acad Sci U S A*.89:89-93. 10.1073/pnas.89.1.89; PMC48181
8. Kay MA, Li Q, Liu TJ, Leland F, Toman C, Finegold M, Woo SL. 1992. Hepatic gene therapy: persistent expression of human alpha 1-antitrypsin in mice after direct gene delivery in vivo. *Hum Gene Ther*.3:641-7. 10.1089/hum.1992.3.6-641
9. Kay MA, Ponder KP, Woo SL. 1992. Human gene therapy: present and future. *Breast Cancer Res Treat*.21:83-93. 10.1007/BF01836954
10. Liu TJ, Kay MA, Darlington GJ, Woo SL. 1992. Reconstitution of enzymatic activity in hepatocytes of phenylalanine hydroxylase-deficient mice. *Somat Cell Mol Genet*.18:89-96. 10.1007/BF01233451
11. Cristiano RJ, Smith LC, Kay MA, Brinkley BR, Woo SL. 1993. Hepatic gene therapy: efficient gene delivery and expression in primary hepatocytes utilizing a conjugated adenovirus-DNA complex. *Proc Natl Acad Sci U S A*.90:11548-52. 10.1073/pnas.90.24.11548; PMC48021
12. Kay MA. 1993. Hepatocyte transplantation for liver gene therapy. *Cell Transplant*.2:405-6. 10.1177/096368979300200506
13. Kay MA, Rothenberg S, Landen CN, Bellinger DA, Leland F, Toman C, Finegold M, Thompson AR, Read MS, Brinkhous KM, et al. 1993. In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs. *Science*.262:117-9. 10.1126/science.8211118
14. Kolodka TM, Finegold M, Kay MA, Woo SL. 1993. Hepatic gene therapy: efficient retroviral-mediated gene transfer into rat hepatocytes in vivo. *Somat Cell Mol Genet*.19:491-7. 10.1007/BF01233254
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