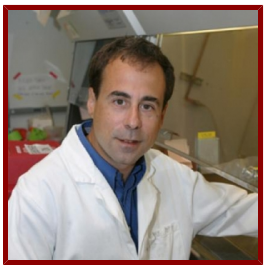
Stanford



Mark A. Kay, M.D., Ph.D.

Dennis Farrey Family Professor of Pediatrics, and Professor of Genetics

Pediatrics - Human Gene Therapy

NIH Biosketch available Online Curriculum Vitae available Online

CONTACT INFORMATION

- **Alternate Contact**

  Melinda Hing - Administrative Associate

  Email mhing@stanford.edu

  Tel 650-498-6532

Bio

ACADEMIC APPOINTMENTS

- Professor, Pediatrics - Human Gene Therapy
- Professor, Genetics
- Member, Bio-X
- Member, Cardiovascular Institute
- Member, Maternal & Child Health Research Institute (MCHRI)
- Member, Stanford Cancer Institute
- Member, Wu Tsai Neurosciences Institute

ADMINISTRATIVE APPOINTMENTS

- Co-Organizer, American Society of Microbiology Meeting on Viral Vectors, (2002- present)
- Executive Committee, Faculty Senate - Stanford, (2002- present)
- Chief Scientific Advisor, Benitec, LLC, (2003-2005)
- Vice President, ASGT, (2003-2004)
- Chair of Organizing Committee, Gordon Conference on Viral Vectors for Gene Therapy, (2003-2004)
- President Elect, American Society of Gene Therapy, (2004-2005)
- President, American Society of Gene Therapy, (2005-2006)
- Associate Chair for Basic Research, Department of Pediatrics, (2012- present)

HONORS AND AWARDS

- Board of Directors, Oligotherapeutics Society (2011-current)
- 2017 Distinguished Alumni and Commencement Speaker- Lyman Briggs College, Michigan State University (2017)
- 2017 Distinguished Alumni Award, Case Western Reserve University (2017)
- Young Investigator Award, Western Society for Clinical Investigation (1996)

- Arosenius Swedish Honorary Lectureship, - (1997)
- Elected Member, American Society for Clinical Investigation (1997)
- Pediatric Researcher of the Year, E. Mead Johnson Award (2000)
- Researcher of the Year, National Hemophilia Foundation (2000)
- Elected Member, AAP (2010)
- Outstanding Achievement/Investigator Award, American Society for Cell and Gene Therapy (2013)
- Sam Rosenthal Prize for Excellence in Pediatrics, Rosenthal Foundation (2011-2013)

PROFESSIONAL EDUCATION

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

LINKS

- Gene Therapy: http://med.stanford.edu/genetherapy
- Lab Website: http://web.stanford.edu/group/markkaylab/Home.html
- Kay photos: https://mark-kay-photography.smugmug.com

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

The goal of the Program in Human Gene Therapy is to develop gene transfer technologies and use them for hepatic gene therapy for the treatment of genetic and acquired diseases. The general approach is to develop new vector systems and delivery methods, test them in the appropriate animal models, uncover the mechanisms involved in vector transduction, and use the most promising approaches in clinical trials. Specifically, we work on a variety of viral and non-viral vector systems. Our major disease models are hemophilia, hepatitis C and B viral infections, and diabetes. The second major focus includes the role that small RNAs play in mammalian gene regulation.

Teaching

STANFORD ADVISEES

Postdoctoral Faculty Sponsor

Qianhui Du, Aranyak Goswami, Yuqian Jiang, Yuqing Jing, Yiming Liu, Francesco Puzzo, Tong Su, Eirini Vamva

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Cancer Biology (Phd Program)
- Genetics (Phd Program)
- Medical Genetics (Fellowship Program)

Publications

PUBLICATIONS

- **Aptamer-programmable adeno-associated viral vectors as a novel platform for cell-specific gene transfer.** *Molecular therapy. Nucleic acids*
  Puzzo, F., Zhang, C., Powell Gray, B., Zhang, F., Sullenger, B. A., Kay, M. A.
  2023; 31: 383-397

- **A standardized ontology for naming tRNA-derived RNAs based on molecular origin.** *Nature methods*
  Holmes, A. D., Chan, P. P., Chen, Q., Ivanov, P., Drouard, L., Polacek, N., Kay, M. A., Lowe, T. M.
  2023

- **Selection of rAAV vectors that cross the human blood-brain barrier and target the central nervous system using a transwell model** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
  Song, R., Pekrun, K., Khan, T. A., Zhang, F., Pasca, S. P., Kay, M. A.
  2022; 27: 73-88

- **Evaluating the State of the Science for Adeno-Associated Virus (AAV) Integration:An Integrated Perspective.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Sabatino, D. E., Bushman, C. F., Chandler, R. J., Crystal, R. G., Davidson, B. L., Dolmetsch, R., Eggan, K. C., Gao, G., Gil-Farina, I., Kay, M. A., McCarty, D. M., Montini, E., Ndu, et al
  2022

- **Fludarabine increases nuclease-free AAV- and CRISPR/Cas9-mediated homologous recombination in mice.** *Nature biotechnology*
  Tsuji, S., Stephens, C. J., Bortolussi, G., Zhang, F., Baj, G., Jang, H., de Alencastro, G., Muro, A. F., Pekrun, K., Kay, M. A.
  2022

- **Lnc122-The miR122 Precursor Has an Independent Role as a Tumor Suppressor in Liver**
  Jang, H., Chu, K., Zhang, F., Stephens, C., Kay, M. A.
  CELL PRESS.2022: 514

- **Programmable Adeno-Associated Viral Vectors for Cell Specific Targeting**
  Puzzo, F., Zhang, C., Zhang, F., Kay, M. A.
  CELL PRESS.2022: 66

- **Using Recombinant Adeno-Associated Viral Vectors for Long-Term Expression of a Hyperactive Human Factor IX Mutant in Hemophilic Mice and Comparison of AAV-LK03 and AAV-KP1 in Nonhuman Primates**
  Pekrun, K., Stephens, C. J., Zhang, F., Kelly, L., Le Moan, N., Tarantal, A. F., Blouse, G. E., Kay, M. A.
  CELL PRESS.2022: 330

- **Promoterless AAV Vectors with Homology Arms Can Integrate and Express from Transcriptionally Active Sites in Non-Targeted Loci**
  Stephens, C. J., Pekrun, K., Xu, J., Tsuji, S., Jing, Y., Puzzo, F., Zhang, F., Kay, M. A.
  CELL PRESS.2022: 391-392

- **The Primate Selective Transduction of rAAV-LK03 Vectors is Related to Variation in Histone and Histone Post-Translational Modifications on the Viral Genome in the Host Nucleus**
  Gonzalez-Sandoval, A., Tsuji, S., Hung, K. L., Zhang, F., Kay, M. A.
  CELL PRESS.2022: 408

- **Loops as Determinant of AAV Integration by Homologous Recombination**
  Puzzo, F., Zhang, F., Pekrun, K., Kay, M. A.
  CELL PRESS.2022: 90-91

- **Promoterless Gene Targeting Approach Combined to CRISPR/Cas9 Efficiently Corrects Hemophilia B Phenotype in Neonatal Mice.** *Frontiers in genome editing*
  Lisjak, M., De Caneva, A., Marais, T., Barbon, E., Biferi, M. G., Porro, F., Barzel, A., Zentilin, L., Kay, M. A., Mingozzi, F., Muro, A. F.
  2022; 4: 785698

- **Selective Microvascular Tissue Transfection Using Minicircle DNA for Systemic Delivery of Human Coagulation Factor IX in a Rat Model Using a Therapeutic Flap.** *Plastic and reconstructive surgery*
  Than, P. A., Davis, C. R., Rennert, R. C., Morrison, S. D., Findlay, M. W., Kay, M. A., Gurtner, G. C.
  2021

- **The 3'tsRNAs are aminoacylated: Implications for their biogenesis.** *PLoS genetics*
  Liu, Z., Kim, H. K., Xu, J., Jing, Y., Kay, M. A.
  2021; 17 (7): e1009675

- **Breaking Thru the Human Blood Brain Barrier: Discovering AAV Vectors Targeting the Central Nervous System Using a Transwell Model**
  Song, R., Pekrun, K., Khan, T. A., Zhang, F., Pasca, S., Kay, M. A.

CELL PRESS.2021: 26-27

- **The 3 ' tsRNAS Are Aminoacylated Further Implicating Their Role in Ribosome Biogenesis during Tissue Homeostasis and Cancer**
  Liu, Z., Kim, H., Xu, J. I., Kay, M. A.
  CELL PRESS.2021: 128

- **Improving the In Vivo Gene Targeting Efficiency of Liver-Directed rAAV Vector Using the Nucleotide Analog Class of Ribonucleotide Reductase Inhibitors**
  Tsuji, S., Stephens, C. J., Bortolussi, G., Pekrun, K., Zhang, F., de Alencastro, G., Baj, G., Muro, A. F., Kay, M. A.
  CELL PRESS.2021: 163

- **A Longitudinal Study of Juvenile Methylmalonic Acidemia (MMA) Mice Treated by Target Integration of MMUT into Albumin with a Promoterless AAV Vector**
  Venturoni, L. E., Chandler, R. J., Chau, N., Liao, J., Gordo, S., Kay, M., Barzel, A., Venditti, C.
  CELL PRESS.2021: 189

- **Evaluating the Genomic Parameters Governing rAAV-Mediated Homologous Recombination** *MOLECULAR THERAPY*
  Spector, L. P., Tiffany, M., Ferraro, N. M., Abell, N. S., Montgomery, S. B., Kay, M. A.
  2021; 29 (3): 1028–46

- **Improved Genome Editing through Inhibition of FANCM and Members of the BTR Dissolvase Complex.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  de Alencastro, G. n., Puzzo, F. n., Pavel-Dinu, M. n., Zhang, F. n., Pillay, S. n., Majzoub, K. n., Tiffany, M. n., Jang, H. n., Sheikali, A. n., Cromer, M. K., Meetei, R. n., Carette, J. E., Porteus, et al
  2021; 29 (3): 1016–27

- **RNA structure probing reveals the structural basis of Dicer binding and cleavage.** *Nature communications*
  Luo, Q. J., Zhang, J., Li, P., Wang, Q., Zhang, Y., Roy-Chaudhuri, B., Xu, J., Kay, M. A., Zhang, Q. C.
  2021; 12 (1): 3397

- **AAV vectors engineered to target insulin receptor greatly enhance intramuscular gene delivery.** *Molecular therapy. Methods & clinical development*
  Jackson, C. B., Richard, A. S., Ojha, A., Conkright, K. A., Trimarchi, J. M., Bailey, C. C., Alpert, M. D., Kay, M. A., Farzan, M., Choe, H.
  2020; 19: 496–506

- **Promoterless, nuclease-free genome editing confers a growth advantage for corrected hepatocytes in mice with methylmalonic acidemia.** *Hepatology (Baltimore, Md.)*
  Chandler, R. J., Venturoni, L. E., Liao, J., Hubbard, B. T., Schneller, J. L., Hoffmann, V., Gordo, S., Zang, S., Ko, C., Chau, N., Chiang, K., Kay, M. A., Barzel, et al
  2020

- **Transfer RNA-Derived Small RNAs: Another Layer of Gene Regulation and Novel Targets for Disease Therapeutics.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Kim, H. K., Yeom, J., Kay, M. A.
  2020

- **A Leu(CAG)-tRNA derived small RNA regulates ribosomal protein S28 after translation initiation in both human and mouse liver cancers**
  Kim, H., Xu, J., Chu, K., Park, H., jang, H., Li, P., Valdmanis, P., Zhang, Q., Kay, M.
  AMER ASSOC CANCER RESEARCH.2020

- **Novel NanoLuc substrates enable bright two-population bioluminescence imaging in animals.** *Nature methods*
  Su, Y., Walker, J. R., Park, Y., Smith, T. P., Liu, L. X., Hall, M. P., Labanieh, L., Hurst, R., Wang, D. C., Encell, L. P., Kim, N., Zhang, F., Kay, et al
  2020

- **CB 2679d-GT - A Novel Human Factor IX Variant Shows Enhanced Activity After Delivery Into Hemophilic Mice Using an AAV Capsid with High Liver Transduction**
  Pekrun, K., Blouse, G. E., Zhang, F., Le Moan, N., Knudsen, T., Landau, J., Kay, M. A.
  CELL PRESS.2020: 172–73

- **Treatment of Juvenile Mice with Methylmalonic Acidemia (MMA) by Targeted Integration of MMUT into Albumin Using a Promoterless AAV Vector**
  Venturoni, L. E., Chandler, R. J., Chau, N., Liao, J., Gordo, S., Kay, M. A., Barzel, A., Venditti, C. P.
  CELL PRESS.2020: 420–21

- **Investigation of the Hepatocyte Population Amenable to Gene Targeting by rAAV-Mediated Homologous Recombination in Mice**
  Tsuji, S., Stephens, C. J., Pekrun, K., Zhang, F., Zhang, A., Kay, M. A.
  CELL PRESS.2020: 80

- **Proteins Complex of the Fanconi Anemia Pathway as Determinant of AAV-Mediated Genomic Targeted Integration**
  Puzzo, F., de Alencastro, G., Pavel-Dinu, M., Zhang, F., Pillay, S., Majzoub, K., Tiffany, M., Jang, H., Sheikali, A., Cromer, K. M., Meetei, R., Carette, J. E., Porteus, et al
  CELL PRESS.2020: 459

- **Selection of Adeno-Associated Virus Vectors Targeting the Central Nervous System Usingan In Vitro Model of Human Blood-Brain Barrier**
  Song, R., Pekrun, K., Khan, T. A., Zhang, F., Pasca, S., Kay, M. A.
  CELL PRESS.2020: 75

- **Targeting Various Genomic Loci using AAV-GeneRide Results in Similar Genome Editing Efficiencies but May Affect Translation of the Chimeric mRNA Transcripts**
  Stephens, C. J., Pekrun, K., Tsuji, S., Zhang, F., Kay, M. A.
  CELL PRESS.2020: 455

- **The Primate Selective Transduction of rAAV-LK03 Vectors is Unrelated to Variation in Double-Stranded Viral Genome Formation in the Nucleus Between Species**
  Tsuji, S., Sandoval, A., Zhang, F., Xu, J., Kay, M. A.
  CELL PRESS.2020: 46

- **Transcriptional and Position Effect Contributions to rAAV-Mediated Gene Targeting**
  Spector, L. P., Tiffany, M., Ferraro, N. M., Abell, N. S., Montgomery, S. B., Kay, M. A.
  CELL PRESS.2020: 290

- **The Role of tRNA Derived Small RNAs in Gene Regulation in Normal Tissues and Cancer**
  Kay, M.
  WILEY.2020

- **Tracking adeno-associated virus capsid evolution by high-throughput sequencing.** *Human gene therapy*
  de Alencastro, G., Pekrun, K., Valdmanis, P., Tiffany, M., Xu, J., Kay, M. A.
  2020

- **Evaluating the genomic parameters governing rAAV-mediated homologous recombination.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Spector, L. P., Tiffany, M. n., Ferraro, N. M., Abell, N. S., Montgomery, S. B., Kay, M. A.
  2020

- **Evolution of a Human-Specific Tandem Repeat Associated with ALS.** *American journal of human genetics*
  Course, M. M., Gudsnuk, K. n., Smukowski, S. N., Winston, K. n., Desai, N. n., Ross, J. P., Sulovari, A. n., Bourassa, C. V., Spiegelman, D. n., Couthouis, J. n., Yu, C. E., Tsuang, D. W., Jayadev, et al
  2020

- **A tRNA-Derived Small RNA Regulates Ribosomal Protein S28 Protein Levels after Translation Initiation in Humans and Mice.** *Cell reports*
  Kim, H. K., Xu, J., Chu, K., Park, H., Jang, H., Li, P., Valdmanis, P. N., Zhang, Q. C., Kay, M. A.
  2019; 29 (12): 3816

- **Using a barcoded AAV capsid library to select for clinically relevant gene therapy vectors.** *JCI insight*
  Pekrun, K., De Alencastro, G., Luo, Q., Liu, J., Kim, Y., Nygaard, S., Galivo, F., Zhang, F., Song, R., Tiffany, M. R., Xu, J., Hebrok, M., Grompe, et al
  2019; 4 (22)

- **Allele-Specific Silencing Ameliorates Restrictive Cardiomyopathy Due to a Human Myosin Regulatory Light Chain Mutation.** *Circulation*
  Zaleta-Rivera, K., Dainis, A., Ribeiro, A. J., Sanchez Cordero, P., Rubio, G., Shang, C., Liu, J., Finsterbach, T., Parikh, V. N., Sutton, S., Seo, K., Sinha, N., Jain, et al
  2019

- **An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging** *NATURE CHEMICAL BIOLOGY*
  Oh, Y., Park, Y., Cho, J. H., Wu, H., Paulk, N. K., Liu, L., Kim, N., Kay, M. A., Wu, J. C., Lin, M. Z.

2019; 15 (5): 433-+

- **Efficient and Long-Term Correction of Liver Metabolic Diseases by Coupling AAV-Mediated Promoterless Gene Targeting to SaCas9 Nuclease**
  De Caneva, A., Porro, F., Bortolussi, G., Sola, R., Lisjak, M., Barzel, A., Giacca, M., Kay, M. A., Vlahovicek, K., Zentillin, L., Muro, A. F.
  CELL PRESS.2019: 461
- **A Novel Adeno Associated Virus Capsid Variant selected on Human Islets Shows Robust Transduction in Many Cell Types In Vitro and In Vivo**
  Pekrun, K., De Alencastro, G., Galivo, F., Kim, Y., Zhang, F., Song, R., Tiffany, M., Nygaard, S., Luo, Q., Liu, J., Xu, J., Hebrok, M., Grompe, et al
  CELL PRESS.2019: 25–26
- **Amino-Acylated LeuCAG3 ' tsRNA Mediates Translational Elongation of Ribosomal Protein S28 mRNA and is a Key Regulatory Step in Ribosome Biogenesis**
  Kim, H., Liu, Z., Xu, J., Chu, K., Park, H., Jang, H., Li, P., Valdmanis, P., Zhang, Q., Kay, M.
  CELL PRESS.2019: 302
- **Elucidating the Mechanism of Species Specificity of Recombinant AAV Capsid Vector-Mediated Transduction**
  Tsuji, S., Kay, M. A.
  CELL PRESS.2019: 220–21
- **Treatment of Methylmalonic Acidemia by Targeted Integration of MUT into Albumin Using an Optimized Promoterless AAV Vector**
  Venturoni, L. E., Chandler, R. J., Chau, N., Liao, J., Kay, M. A., Barzel, A., Venditti, C. P.
  CELL PRESS.2019: 367
- **Transcriptional and Position Effect Contributions to rAAV-Mediated Gene Targeting**
  Spector, L. P., Tiffany, M., Ferraro, N. M., Abell, N. S., Montgomery, S. B., Kay, M. A.
  CELL PRESS.2019: 294
- **Exploiting the Regenerative Capacity of Liver for Nuclease-Free Genome Editing**
  Puzzo, F., De Alencastro, G., Patijn, G., Zhang, F., Pekrun, K., Kay, M.
  CELL PRESS.2019: 463
- **An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging.** *Nature chemical biology*
  Oh, Y., Park, Y., Cho, J. H., Wu, H., Paulk, N. K., Liu, L. X., Kim, N., Kay, M. A., Wu, J. C., Lin, M. Z.
  2019
- **Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases.** *JCI insight*
  De Caneva, A. n., Porro, F. n., Bortolussi, G. n., Sola, R. n., Lisjak, M. n., Barzel, A. n., Giacca, M. n., Kay, M. A., Vlahovi#ek, K. n., Zentilin, L. n., Muro, A. F.
  2019; 5
- **miR-122 removal in the liver activates imprinted microRNAs and enables more effective microRNA-mediated gene repression.** *Nature communications*
  Valdmanis, P. N., Kim, H. K., Chu, K., Zhang, F., Xu, J., Munding, E. M., Shen, J., Kay, M. A.
  2018; 9 (1): 5321
- **miR-122 removal in the liver activates imprinted microRNAs and enables more effective microRNA-mediated gene repression** *NATURE COMMUNICATIONS*
  Valdmanis, P. N., Kim, H., Chu, K., Zhang, F., Xu, J., Munding, E. M., Shen, J., Kay, M. A.
  2018; 9
- **Functional lung cancer genomics through in vivo genome editing**
  Winters, I. P., Rogers, Z. N., McFarland, C. D., Lalgudi, P. V., Chiou, S., Kay, M. A., Petrov, D., Winslow, M. M.
  AMER ASSOC CANCER RESEARCH.2018
- **Bioengineered Viral Platform for Intramuscular Passive Vaccine Delivery to Human Skeletal Muscle** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
  Paulk, N. K., Pekrun, K., Charville, G. W., Maguire-Nguyen, K., Wosczyna, M. N., Xu, J., Zhang, Y., Lisowski, L., Yoo, B., Vilches-Moure, J. G., Lee, G. K., Shrager, J. B., Rando, et al
  2018; 10: 144–55
- **A transfer RNA derived small RNA affects translation in rapidly dividing cells and a target for hepatocellular carcinoma**
  Kim, H., Fuchs, G., Wang, S., Wei, W., Zhang, Y., Park, H., Roy-Chaudhuri, B., Li, P., Xu, J., Chu, K., Zhang, F., Chua, M., So, et al
  AMER ASSOC CANCER RESEARCH.2018

- **Transcriptional and Position Effect Contributions to rAAV-Mediated Homologous Recombination**
  Spector, L. P., Tiffany, M., Kay, M. A.
  CELL PRESS.2018: 368

- **Generide (TM), a Novel AAV Strategy to Treat Pediatric Patients with Methylmalonic Acidemia**
  Liao, J., Rais, Y., Hayon, Y., Barzel, A., Lisowski, L., Kay, M. A., Chiang, K., Chau, N.
  CELL PRESS.2018: 363

- **Promoterless Targeting without Nucleases of Hyperactive Factor IX Corrects the Bleeding Diathesis in Hemophilia B Mice**
  Hayon, Y., Reines, N., Kilovaty, I., Rais, Y., Chau, N., Chiang, K., Lisowski, L., Kay, M. A., Barzel, A.
  CELL PRESS.2018: 89–90

- **Alteration of AAV Capsid Lumenal Residues to Expand Vector Genome Packaging Capacity**
  Tiffany, M., Pekrun, K., Zhang, F., Kay, M. A.
  CELL PRESS.2018: 191

- **Disruption of the Heparin-Binding Site and Insertion of the PHB.P Peptide in AAV-DJ Improve Transduction of the Central Nervous System**
  Song, R., Kay, M. A.
  CELL PRESS.2018: 40–41

- **Improved Genome Editing through Inhibition of the FANCM Pathway**
  de Alencastro, G., Pekrun, K., Zhang, F., Pillay, S., Majzoub, K., Carette, J., Kay, M. A.
  CELL PRESS.2018: 433–34

- **Targeted Integration of MUT into the Albumin Locus Using a Promoterless AAV Vector (Generide (TM)) Confers a Hepatocellular Growth Advantage in Mice with Methylmalonic Acidemia**
  Chandler, R. J., Chau, N., Chiang, K., Kay, M. A., Barzel, A., Venditti, C. P.
  CELL PRESS.2018: 446–47

- **CORRECTION OF MURINE MODELS OF METHYLMALONIC ACIDEMIA USING ALBUMIN TARGETED HOMOLOGOUS RECOMBINATION WITH A PROMOTERLESS ADENO-ASSOCIATED VIRAL INTEGRATING VECTOR**
  Chandlers, R. J., Cahana, A., Kay, M. A., Barzel, A., Venditti, C. P.
  ACADEMIC PRESS INC ELSEVIER SCIENCE.2018: 206

- **Bioengineered AAV Capsids with Combined High Human Liver Transduction In Vivo and Unique Humoral Seroreactivity** *MOLECULAR THERAPY*
  Paulk, N. K., Pekrun, K., Zhu, E., Nygaard, S., Li, B., Xu, J., Chu, K., Leborgne, C., Dane, A. P., Haft, A., Zhang, Y., Zhang, F., Morton, et al
  2018; 26 (1): 289–303

- **Bioengineered Viral Platform for Intramuscular Passive Vaccine Delivery to Human Skeletal Muscle.** *Molecular therapy. Methods & clinical development*
  Paulk, N. K., Pekrun, K. n., Charville, G. W., Maguire-Nguyen, K. n., Wosczyna, M. N., Xu, J. n., Zhang, Y. n., Lisowski, L. n., Yoo, B. n., Vilches-Moure, J. G., Lee, G. K., Shrager, J. B., Rando, et al
  2018; 10: 144–55

- **Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid alpha-glucosidase** *SCIENCE TRANSLATIONAL MEDICINE*
  Puzzo, F., Colella, P., Biferi, M. G., Bali, D., Paulk, N. K., Vidal, P., Collaud, F., Simon-Sola, M., Charles, S., Hardet, R., Leborgne, C., Meliani, A., Cohen-Tannoudji, et al
  2017; 9 (418)

- **Survival Advantage of Both Human Hepatocyte Xenografts and Genome-Edited Hepatocytes for Treatment of alpha-1 Antitrypsin Deficiency** *MOLECULAR THERAPY*
  Borel, F., Tang, Q., Gernoux, G., Greer, C., Wang, Z., Barzel, A., Kay, M. A., Shultz, L. D., Greiner, D. L., Flotte, T. R., Brehm, M. A., Mueller, C.
  2017; 25 (11): 2477–89

- **Human and Baculovirus-Insect Manufacturing Platforms Generate Chemically and Functionally Distinct AAV Vectors with Sexually Dimorphic Liver Transduction.**
  Paulk, N. K., Rumachik, N., Malaker, S., Adams, C., Leib, R., Bertozzi, C. R., Kay, M.
  WILEY.2017: 373A

- **Promoterless gene targeting without nucleases rescues lethality of a Crigler-Najjar syndrome mouse model** *EMBO MOLECULAR MEDICINE*
  Porro, F., Bortolussi, G., Barzel, A., De Caneva, A., Iaconcig, A., Vodret, S., Zentilin, L., Kay, M. A., Muro, A. F.

2017; 9 (10): 1346–55

- **Sequence-Modified Antibiotic Resistance Genes Provide Sustained Plasmid-Mediated Transgene Expression in Mammals** *MOLECULAR THERAPY*
  Lu, J., Zhang, F., Fire, A. Z., Kay, M. A.
  2017; 25 (5): 1187-1198
- **CRISPR/Cas9 sgRNAs Do Not Exert the Same Competition with Liver microRNAs as shRNAs**
  Valdmanis, P. N., Chu, K., Zhang, F., Luo, Q., Kay, M. A.
  CELL PRESS.2017: 296
- **A 3 ' tRNA Derived Small RNA (tsRNA) Affects Translation in Rapidly Dividing Cells and a Target for Hepatocellular Carcinoma**
  Kim, H., Fuchs, G., Wang, S., Wei, W., Zhang, Y., Park, H., Roy-Chaudhuri, B., Zhang, F., Chua, M., So, S., Sarnow, P., Kay, M. A.
  CELL PRESS.2017: 34–35
- **Evolved AAV Capsids for Intramuscular Passive Vaccine Administration to Human Skeletal Muscle**
  Paulk, N. K., Pekrun, K., Charville, G., Maguire-Nguyen, K., Xu, J., Wosczyna, M., Lisowski, L., Lee, G., Shrager, J., Rando, T., Kay, M. A.
  CELL PRESS.2017: 96
- **Rescue of Mice with Methylmalonic Acidemia from Immediate Neonatal Lethality Using an Albumin Targeted, Promoterless Adeno-Associated Viral Integrating Vector**
  Chandler, R. J., Cahana, A., Hubbard, B. T., Kay, M. A., Barzel, A., Venditti, C. P.
  CELL PRESS.2017: 13
- **Small Hairpin RNAs Delivered in Human Cortical Spheroids Compete with Endogenous microRNAs**
  Valdmanis, P. N., Andersen, J., Chu, K., Pasca, S. P., Kay, M. A.
  CELL PRESS.2017: 37
- **Proof-of-Concept for Non-Nuclease-Mediated Genome Editing to Treat A-1 Antitrypsin Deficiency**
  Borel, F., Barzel, A., Kay, M. A., Mueller, C.
  CELL PRESS.2017: 29–30
- **Tracking Adeno-Associated Virus (AAV) Capsid Evolution by High-Throughput Sequencing**
  de Alencastro, G., Pekrun, K., Valdmanis, P. N., Xu, J., Kay, M. A.
  CELL PRESS.2017: 44–45
- **rAAV Is Extensively and Differentially Post-Translationally Modified in Human versus Insect Cell Line Production Methods**
  Paulk, N. K., Rumachik, N. G., Adams, C. M., Leib, R., Stamnes, S., Holt, K. H., Sinn, P. L., Kotin, R. M., Bertozzi, C. R., Kay, M. A.
  CELL PRESS.2017: 46
- **Alteration of AAV Capsid Lumenal Residues Expands Genome Packaging Capacity**
  Tiffany, M. R., Pekrun, K., Zhang, F., Kay, M. A.
  CELL PRESS.2017: 47
- **Transcriptional and Position Effect Contributions to rAAV-Mediated Homologous Recombination**
  Spector, L. P., Tiffany, M., Kay, M. A.
  CELL PRESS.2017: 84–85
- **Engineered AAV Capsids with Combined High Human Liver Transduction In Vivo and Unique Humoral Seroreactivity**
  Paulk, N. K., Pekrun, K., Lisowski, L., Zhu, E., Nygaard, S., Li, B., Xu, J., Chu, K., Leborgne, C., Dane, A., Haft, A., Morton, C., Valentine, et al
  CELL PRESS.2017: 188
- **Liver-Mediated Gene Therapy with an Engineered Secretable GAA Transgene Results in Whole-Body Correction of Pompe Disease**
  Ronzitti, G., Colella, P., Puzzo, F., Biferi, M., Bali, D., Paulk, N. K., Vidal, P., Collaud, F., Sola, M., Charles, S., Hardet, R., Leborgne, C., Meliani, et al
  CELL PRESS.2017: 244
- **Future of rAAV gene therapy: Platform for RNAi, Gene Editing and Beyond.** *Human gene therapy*
  Valdmanis, P., Kay, M. A.
  2017
- **Regulated complex assembly safeguards the fidelity of Sleeping Beauty transposition.** *Nucleic acids research*
  Wang, Y., Pryputniewicz-Dobrinska, D., Nagy, E. É., Kaufman, C. D., Singh, M., Yant, S., Wang, J., Dalda, A., Kay, M. A., Ivics, Z., Izsvák, Z.

2017; 45 (1): 311-326

- **A 5 ' Noncoding Exon Containing Engineered Intron Enhances Transgene Expression from Recombinant AAV Vectors in vivo** *HUMAN GENE THERAPY*
  Lu, J., Williams, J. A., Luke, J., Zhang, F., Chu, K., Kay, M. A.
  2017; 28 (1): 125-134
- **Multiplexed in vivo homology-directed repair and tumor barcoding enables parallel quantification of Kras variant oncogenicity.** *Nature communications*
  Winters, I. P., Chiou, S. H., Paulk, N. K., McFarland, C. D., Lalgudi, P. V., Ma, R. K., Lisowski, L. n., Connolly, A. J., Petrov, D. A., Kay, M. A., Winslow, M. M.
  2017; 8 (1): 2053
- **A transfer-RNA-derived small RNA regulates ribosome biogenesis.** *Nature*
  Kim, H. K., Fuchs, G. n., Wang, S. n., Wei, W. n., Zhang, Y. n., Park, H. n., Roy-Chaudhuri, B. n., Li, P. n., Xu, J. n., Chu, K. n., Zhang, F. n., Chua, M. S., So, et al
  2017; 552 (7683): 57–62
- **Increased precursor microRNA-21 following status epilepticus can compete with mature microRNA-21 to alter translation.** *Experimental neurology*
  Chak, K., Roy-Chaudhuri, B., Kim, H. K., Kemp, K. C., Porter, B. E., Kay, M. A.
  2016; 286: 137-146
- **Dieter C. Gruenert, PhD (1949-2016) In Memoriam** *NUCLEIC ACID THERAPEUTICS*
  Disterer, P., Kay, M. A., Parker, G. C.
  2016; 26 (4): 266–67
- **A bright cyan-excitable orange fluorescent protein facilitates dual-emission microscopy and enhances bioluminescence imaging in vivo.** *Nature biotechnology*
  Chu, J., Oh, Y., Sens, A., Ataie, N., Dana, H., Macklin, J. J., Laviv, T., Welf, E. S., Dean, K. M., Zhang, F., Kim, B. B., Tang, C. T., Hu, et al
  2016; 34 (7): 760-767
- **A universal system to select gene-modified hepatocytes in vivo** *SCIENCE TRANSLATIONAL MEDICINE*
  Nygaard, S., Barzel, A., Haft, A., Major, A., Finegold, M., Kay, M. A., Grompe, M.
  2016; 8 (342)
- **RNA interference-induced hepatotoxicity results from loss of the first synthesized isoform of microRNA-122 in mice** *NATURE MEDICINE*
  Valdmanis, P. N., Gu, S., Chu, K., Jin, L., Zhang, F., Munding, E. M., Zhang, Y., Huang, Y., Kutay, H., Ghoshal, K., Lisowski, L., Kay, M. A.
  2016; 22 (5): 557-562
- **AAV Capsid Evolution for Enhanced Antibody Delivery to Human Skeletal Muscle for Use in Next-Generation HIV Vaccines and Muscle Gene Therapies**
  Paulk, N. K., Charville, G., Maguire, K., Pekrun, K., Zhang, Y., Tiffany, M., Vilches-Moure, J., Lee, G., Shrager, J., Rando, T., Kay, M. A.
  NATURE PUBLISHING GROUP.2016: S284–S285
- **RNAi Induced Hepatotoxicity Results from a Functional Depletion of the First Synthesized Isoform of miR-122**
  Valdmanis, P. N., Gu, S., Chu, K., Jin, L., Zhang, F., Munding, E. M., Zhang, Y., Huang, Y., Kutay, H., Ghoshal, K., Lisowski, L., Kay, M. A.
  NATURE PUBLISHING GROUP.2016: S290–S291
- **Treatment of Methylmalonic Acidemia by Promoterless Gene-Targeting Using Adeno-Associated Viral (AAV) Mediated Homologous Recombination**
  Chandler, R. J., Barzel, A., Kay, M. A., Venditti, C. P.
  NATURE PUBLISHING GROUP.2016: S21–S22
- **Does Transcription Influence AAV-Mediated Homologous Recombination?**
  Spector, L. P., Kay, M. A.
  NATURE PUBLISHING GROUP.2016: S53
- **Selection of Next Generation AAV Gene Therapy Vectors for Specific and Precise Gene Delivery**
  Muench, R., Kay, M.
  NATURE PUBLISHING GROUP.2016: S101
- **Expanded Packaging Capacity of AAV by Lumenal Charge Alteration**
  Tiffany, M., Kay, M. A.
  NATURE PUBLISHING GROUP.2016: S99–S100
- **Sequence Modified Antibiotic Resistance Genes Provide Sustained Plasmid Mediated Transgene Expression in Mammals**

Lu, J., Zhang, F., Fire, A., Kay, M.
NATURE PUBLISHING GROUP.2016: S116

- **Screening for Recombinant Adeno-Associated Viral Vectors That Selectively Transduce Hepatitis B Virus Infected Cells**
de Alencastro, G., Pekrun, K., Kay, M.
NATURE PUBLISHING GROUP.2016: S215

- **A Tribute to George Stamatoyannopoulos** *HUMAN GENE THERAPY*
Srivastava, A., Kay, M. A., Athanasopoulos, T., Angastiniotis, M., Anagnostopoulos, A., Karponi, G., Yannaki, E., Zon, L. I., Lederer, C. W., Phylactides, M. S., Kleanthous, M.
2016; 27 (4): 280–86

- **Selecting the Best AAV Capsid for Human Studies** *MOLECULAR THERAPY*
Kay, M. A.
2015; 23 (12): 1800–1801

- **Viral Vectors Take On HIV Infection** *NEW ENGLAND JOURNAL OF MEDICINE*
Mellins, E. D., Kay, M. A.
2015; 373 (8): 770–72

- **NEW RAAV VECTORS AND APPROACHES FOR EPISOMAL AND INTEGRATION BASED GENE TRANSFER**
Kay, M. A.
WILEY-BLACKWELL.2015: 179

- **In Vivo Expansion of Hepatocytes with Targeted rAAV Integration Results in a > 100-Fold Increase of Transgene Expression**
Nygaard, S., Barzel, A., Haft, A., Kay, M. A., Grompe, M.
NATURE PUBLISHING GROUP.2015: S272

- **Directed Evolution of Improved AAV Capsids for the Ideal Human Liver Vector-Can Human Liver Tropism and Human Immune Evasion Be Achieved?**
Paulk, N. K., Pekrun, K., Lisowski, L., Zhang, Y., Chu, K., Kay, M. A.
NATURE PUBLISHING GROUP.2015: S104–S105

- **AAV Capsid Evolution for Enhanced Antibody Delivery To Human Muscle for Use in Next-Generation HIV Vaccines**
Paulk, N. K., Charville, G. W., Pekrun, K., Maguire, K., Rando, T. A., Kay, M. A.
NATURE PUBLISHING GROUP.2015: S122–S123

- **AAV Integration Site Determination Using Illumina Mate Pair Sequencing**
Tiffany, M. R., Kay, M. A.
NATURE PUBLISHING GROUP.2015: S39–S40

- **A Screening Strategy for Selecting Recombinant Adeno-Associated Viral Vectors That Selectively Transduce Viral Infected Cells**
de Alencastro, G., Paulk, N. K., Pekrun, K., Kay, M. A.
NATURE PUBLISHING GROUP.2015: S123

- **microRNA Inhibition Through Gapmer Activated RNase H-Mediated Degradation**
Munding, E. M., Kay, M. A.
NATURE PUBLISHING GROUP.2015: S230

- **AAV8-Mediated Liver Gene Targeting Without Nucleases Rescues Lethality in a Mouse Model of the Crigler-Najjar Syndrome**
Porro, F., Bortolussi, G., Barzel, A., Zentilin, L., Vodret, S., Bockor, L., Kay, M. A., Muro, A. F.
NATURE PUBLISHING GROUP.2015: S274

- **RNA interference. Drugging RNAi.** *Science*
Haussecker, D., Kay, M. A.
2015; 347 (6226): 1069-1070

- **Translational Data from Adeno-Associated Virus-Mediated Gene Therapy of Hemophilia B in Dogs** *HUMAN GENE THERAPY CLINICAL DEVELOPMENT*
Nichols, T. C., Whitford, M. H., Arruda, V. R., Stedman, H. H., Kay, M. A., High, K. A.
2015; 26 (1): 5-14

- **Promoterless gene targeting without nucleases ameliorates haemophilia B in mice.** *Nature*
  BARZEL, A., Paulk, N. K., Shi, Y., Huang, Y., Chu, K., Zhang, F., Valdmanis, P. N., Spector, L. P., Porteus, M. H., Gaensler, K. M., Kay, M. A.
  2015; 517 (7534): 360-364

- **Promoterless gene targeting without nucleases ameliorates haemophilia B in mice.** *Nature*
  BARZEL, A., Paulk, N. K., Shi, Y., Huang, Y., Chu, K., Zhang, F., Valdmanis, P. N., Spector, L. P., Porteus, M. H., Gaensler, K. M., Kay, M. A.
  2015; 517 (7534): 360-364

- **Upregulation of the microRNA cluster at the Dlkl-Dio3 locus in lung adenocarcinoma** *ONCOGENE*
  Valdmanis, P. N., Roy-Chaudhuri, B., Kim, H. K., Sayles, L. C., Zheng, Y., Chuang, C., Caswell, D. R., Chu, K., Zhang, Y., Winslow, M. M., Sweet-Cordero, E. A., Kay, M. A.
  2015; 34 (1): 94-103

- **Novel codon-optimized mini-intronic plasmid for efficient, inexpensive, and xeno-free induction of pluripotency.** *Scientific reports*
  Diecke, S., Lu, J., Lee, J., Termglinchan, V., Kooreman, N. G., Burridge, P. W., Ebert, A. D., Churko, J. M., Sharma, A., Kay, M. A., Wu, J. C.
  2015; 5: 8081-?

- **Novel codon-optimized mini-intronic plasmid for efficient, inexpensive, and xeno-free induction of pluripotency.** *Scientific reports*
  Diecke, S., Lu, J., Lee, J., Termglinchan, V., Kooreman, N. G., Burridge, P. W., Ebert, A. D., Churko, J. M., Sharma, A., Kay, M. A., Wu, J. C.
  2015; 5: 8081-?

- **Human COL7A1-corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa** *SCIENCE TRANSLATIONAL MEDICINE*
  Sebastiano, V., Zhen, H. H., Derafshi, B. H., Bashkirova, E., Melo, S. P., Wang, P., Leung, T. L., Siprashvili, Z., Tichy, A., Li, J., Ameen, M., Hawkins, J., Lee, et al
  2014; 6 (264)

- **Human COL7A1-corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa.** *Science translational medicine*
  Sebastiano, V., Zhen, H. H., Haddad, B., Bashkirova, E., Melo, S. P., Wang, P., Leung, T. L., Siprashvili, Z., Tichy, A., Li, J., Ameen, M., Hawkins, J., Lee, et al
  2014; 6 (264): 264ra163-?

- **Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B** *NEW ENGLAND JOURNAL OF MEDICINE*
  Nathwani, A. C., Reiss, U. M., Tuddenham, E. G., Rosales, C., Chowdary, P., Mcintosh, J., Della Peruta, M., Lheriteau, E., Patel, N., Raj, D., Riddell, A., Pie, J., Rangarajan, et al
  2014; 371 (21): 1994-2004

- **Novel rAAV vectors for episomal and integration based gene transfer**
  Kay, M. A.
  MARY ANN LIEBERT, INC.2014: A16

- **Weak base pairing in both seed and 3' regions reduces RNAi off-targets and enhances si/shRNA designs.** *Nucleic acids research*
  Gu, S., Zhang, Y., Jin, L., Huang, Y., Zhang, F., Bassik, M. C., Kampmann, M., Kay, M. A.
  2014; 42 (19): 12169-12176

- **Regulation of microRNA-mediated gene silencing by microRNA precursors.** *Nature structural & molecular biology*
  Roy-Chaudhuri, B., Valdmanis, P. N., Zhang, Y., Wang, Q., Luo, Q., Kay, M. A.
  2014; 21 (9): 825-832

- **Regulation of microRNA-mediated gene silencing by microRNA precursors.** *Nature structural & molecular biology*
  Roy-Chaudhuri, B., Valdmanis, P. N., Zhang, Y., Wang, Q., Luo, Q., Kay, M. A.
  2014; 21 (9): 825-832

- **Genome Editing of Isogenic Human Induced Pluripotent Stem Cells Recapitulates Long QT Phenotype for Drug Testing.** *Journal of the American College of Cardiology*
  Wang, Y., Liang, P., Lan, F., Wu, H., Lisowski, L., Gu, M., Hu, S., Kay, M. A., Urnov, F. D., Shinnawi, R., Gold, J. D., Gepstein, L., Wu, et al
  2014; 64 (5): 451-459

- **Organ Size Control Is Dominant over Rb Family Inactivation to Restrict Proliferation In Vivo** *CELL REPORTS*
  Ehmer, U., Zmoos, A., Auerbach, R. K., Vaka, D., Butte, A. J., Kay, M. A., Sage, J.
  2014; 8 (2): 370-380

- **Organ Size Control Is Dominant over Rb Family Inactivation to Restrict Proliferation In Vivo.** *Cell reports*
  Ehmer, U., Zmoos, A., Auerbach, R. K., Vaka, D., Butte, A. J., Kay, M. A., Sage, J.
  2014; 8 (2): 371-381
- **Characterization of Vector-Based Delivery of Neurogenin-3 in Murine Diabetes** *HUMAN GENE THERAPY*
  Phillips, N., Kay, M. A.
  2014; 25 (7): 651-661
- **Precursor microRNA Can Act as a Post-Transcriptional Regulator of Mature microRNA Activity**
  Roy-Chaudhuri, B., Valdmanis, P. N., Zhang, Y., Wang, Q., Luo, Q., Kay, M. A.
  NATURE PUBLISHING GROUP.2014: S5
- **The Linkage of Backbone Transcription and Plasmid DNA Silencing**
  Lu, J., Zhang, F., Maniar, L., Zhang, W., Fire, A., Kay, M. A.
  NATURE PUBLISHING GROUP.2014: S4
- **In vivo Gene-Targeting Without Nucleases Facilitates Therapeutic Levels of hF-IX Following AAV8 Vector Injections of Either Neonate or Adult Mice**
  Barzel, A., Paulk, N. K., Shi, Y., Huang, Y., Chu, K., Zhang, F., Porteus, M. H., Gaensler, K. M., Kay, M. A.
  NATURE PUBLISHING GROUP.2014: S89
- **Mini-Intronic Plasmid (MIP) Vector Sequences Enhance AAV-Mediated Transgene Expression In Vitro and In Vivo**
  Lu, J., Williams, J., Luke, J., Zhang, F., Kay, M. A.
  NATURE PUBLISHING GROUP.2014: S112–S113
- **Utilizing DNA Shuffling Technologies To Generate AAV Libraries To Select for and Evolve Capsids With an Expanded Packaging Capacity**
  Paulk, N., Pekrun, K., Kay, M.
  NATURE PUBLISHING GROUP.2014: S116–S117
- **Somatic Correction of Junctional Epidermolysis Bullosa by a Highly Recombinogenic AAV Variant.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Melo, S. P., Lisowski, L., Bashkirova, E., Zhen, H. H., Chu, K., Keene, D. R., Marinkovich, M. P., Kay, M. A., Oro, A. E.
  2014; 22 (4): 725-733
- **Recombinant AAV as a Platform for Translating the Therapeutic Potential of RNA Interference** *MOLECULAR THERAPY*
  Borel, F., Kay, M. A., Mueller, C.
  2014; 22 (4): 692-701
- **Engineering cellular resistance to HIV.** *New England journal of medicine*
  Kay, M. A., Walker, B. D.
  2014; 370 (10): 968-969
- **Selection and evaluation of clinically relevant AAV variants in a xenograft liver model** *NATURE*
  Lisowski, L., Dane, A. P., Chu, K., Zhang, Y., Cunningham, S. C., Wilson, E. M., Nygaard, S., Grompe, M., Alexander, I. E., Kay, M. A.
  2014; 506 (7488): 382-?
- **Selection and evaluation of clinically relevant AAV variants in a xenograft liver model.** *Nature*
  Lisowski, L., Dane, A. P., Chu, K., Zhang, Y., Cunningham, S. C., Wilson, E. M., Nygaard, S., Grompe, M., Alexander, I. E., Kay, M. A.
  2014; 506 (7488): 382-386
- **The expanding repertoire of circular RNAs.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Valdmanis, P. N., Kay, M. A.
  2013; 21 (6): 1112-1114
- **A Species Restricted rAAV Vector Obtained from Viral Capsid Shuffling**
  Lisowski, L., Chu, K., Kay, M. A.
  NATURE PUBLISHING GROUP.2013: S3
- **A Mini-Intronic Plasmid (MIP): A Novel Robust Transgene Expression Vector In Vivo and In Vitro** *16th Annual Meeting of the American-Society-of-Gene-and-Cell-Therapy (ASGCT)*
  Lu, J., Zhang, F., Kay, M. A.

NATURE PUBLISHING GROUP.2013: S103–S103

- **AAV-Mediated Direct Somatic Genetic Correction of Epidermolysis Bullosa**
  Melo, S., Lisowski, L., Bashkirova, L., Kay, M. A., Oro, A. E.
  NATURE PUBLISHING GROUP.2013: S106

- **The Anti-Genomic (Negative) Strand of Hepatitis C Virus Is Not Targetable by shRNA**
  Lisowski, L., Elazar, M., Chu, K., Glenn, J. S., Kay, M. A.
  NATURE PUBLISHING GROUP.2013: S75

- **Upregulation of a Cluster of microRNAs on Mouse Chromosome 12qF1 in a Kras Mutant Mouse Model of Lung Adenocarcinoma** *16th Annual Meeting of the American-Society-of-Gene-and-Cell-Therapy (ASGCT)*
  Valdmanis, P. N., Roy-Chaudhuri, B., Kim, H. K., Sayles, L. C., Zheng, Y., Chuang, C., Caswell, D., Winslow, M., Sweet-Cordero, E. A., Kay, M. A.
  NATURE PUBLISHING GROUP.2013: S168–S168

- **Benchtop DNA Synthesizer: Oligo-Templated Polymerization (OTP)** *16th Annual Meeting of the American-Society-of-Gene-and-Cell-Therapy (ASGCT)*
  Barzel, A., Kay, M. A.
  NATURE PUBLISHING GROUP.2013: S132–S133

- **A Mini-intronic Plasmid (MIP): A Novel Robust Transgene Expression Vector In Vivo and In Vitro.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Lu, J., Zhang, F., Kay, M. A.
  2013; 21 (5): 954-963

- **MicroRNAs in Cancer: The 22nd Hiroshima Cancer Seminar/The 4th Japanese Association for RNA Interference Joint International Symposium, 30 August 2012, Grand Prince Hotel Hiroshima** *JAPANESE JOURNAL OF CLINICAL ONCOLOGY*
  Tahara, H., Kay, M. A., Yasui, W., Tahara, E.
  2013; 43 (5): 579–82

- **The anti-genomic (negative) strand of Hepatitis C Virus is not targetable by shRNA.** *Nucleic acids research*
  Lisowski, L., Elazar, M., Chu, K., Glenn, J. S., Kay, M. A.
  2013; 41 (6): 3688-3698

- **Minicircle DNA vectors achieve sustained expression reflected by active chromatin and transcriptional level.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Gracey Maniar, L. E., Maniar, J. M., Chen, Z., Lu, J., Fire, A. Z., Kay, M. A.
  2013; 21 (1): 131-138

- **Minicircle DNA Vectors Achieve Sustained Expression Reflected by Active Chromatin and Transcriptional Level** *MOLECULAR THERAPY*
  Maniar, L. E., Maniar, J. M., Chen, Z., Lu, J., Fire, A. Z., Kay, M. A.
  2013; 21 (1): 131-138

- **Genome Editing of Human Embryonic Stem Cells and Induced Pluripotent Stem Cells With Zinc Finger Nucleases for Cellular Imaging** *CIRCULATION RESEARCH*
  Wang, Y., Zhang, W. Y., Hu, S., Lan, F., Lee, A. S., Huber, B., Lisowski, L., Liang, P., Huang, M., de Almeida, P. E., Won, J. H., Sun, N., Robbins, et al
  2012; 111 (12): 1494-?

- **Stable Factor IX Activity Following AAV-Mediated Gene Transfer in Patients with Severe Hemophilia B** *54th Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
  Davidoff, A., Tuddenham, E. G., Rangarajan, S., Rosales, C., McIntosh, J., Chowdary, P., Riddell, A., Glader, B., Rustagi, P., Ng, C., Kay, M., Zhou, J., Spence, et al
  AMER SOC HEMATOLOGY.2012

- **The Loop Position of shRNAs and Pre-miRNAs Is Critical for the Accuracy of Dicer Processing In Vivo** *CELL*
  Gu, S., Jin, L., Zhang, Y., Huang, Y., Zhang, F., Valdmanis, P. N., Kay, M. A.
  2012; 151 (4): 900-911

- **The Extragenic Spacer Length Between the 5 ' and 3 ' Ends of the Transgene Expression Cassette Affects Transgene Silencing From Plasmid-based Vectors** *MOLECULAR THERAPY*
  Lu, J., Zhang, F., Xu, S., Fire, A. Z., Kay, M. A.
  2012; 20 (11): 2111-2119

- **rAAV-Mediated Tumorigenesis: Still Unresolved After an AAV Assault** *MOLECULAR THERAPY*
  Valdmanis, P. N., Lisowski, L., Kay, M. A.
  2012; 20 (11): 2014-2017

- **Ribosomal DNA Integrating rAAV-rDNA Vectors Allow for Stable Transgene Expression** *MOLECULAR THERAPY*
  Lisowski, L., Lau, A., Wang, Z., Zhang, Y., Zhang, F., Grompe, M., Kay, M. A.
  2012; 20 (10): 1912-1923

- **Optimization of liver gene transfer for hemophilia B** *Collaborative Congress of the European-Society-of-Gene-and-Cell-Therapy/French-Society-of-Cell-and-Gene-Therapy*
  Anguela, X. M., Chen, Y., Davidson, R. J., Zhou, S., Nichols, T. C., Kay, M. A., Mingozzi, F., High, K. A.
  MARY ANN LIEBERT INC.2012: A53–A53

- **AAV Vectors Containing rDNA Homology Display Increased Chromosomal Integration and Transgene Persistence** *MOLECULAR THERAPY*
  Wang, Z., Lisowski, L., Finegold, M. J., Nakai, H., Kay, M. A., Grompe, M.
  2012; 20 (10): 1902-1911

- **Human ES-cell-derived cardiomyocytes electrically couple and suppress arrhythmias in injured hearts** *NATURE*
  Shiba, Y., Fernandes, S., Zhu, W., Filice, D., Muskheli, V., Kim, J., Palpant, N. J., Gantz, J., Moyes, K. W., Reinecke, H., Van Biber, B., Dardas, T., Mignone, et al
  2012; 489 (7415): 322-?

- **Slicing-Independent RISC Activation Requires the Argonaute PAZ Domain** *CURRENT BIOLOGY*
  Gu, S., Jin, L., Huang, Y., Zhang, F., Kay, M. A.
  2012; 22 (16): 1536-1542

- **Targeting 2A-Fusions to Endogenous Genes** *15th Annual Meeting of the American-Society-of-Gene-and-Cell-Therapy (ASGCT)*
  Barzel, A., Voit, R., Porteus, M., Kay, M. A.
  NATURE PUBLISHING GROUP.2012: S234–S234

- **Effect of Argonaute-2 in Mediating Non-Coding RNA Expression**
  Valdmanis, P. N., Chaudhuri, B., Cao, D., Pouliot, Y., Kay, M. A.
  NATURE PUBLISHING GROUP.2012: S133

- **Novel, Shuffled, Human-Specific rAAV Vectors**
  Lisowski, L., Chu, K., Kay, M. A.
  NATURE PUBLISHING GROUP.2012: S140–S141

- **Expression determinants of mammalian argonaute proteins in mediating gene silencing** *NUCLEIC ACIDS RESEARCH*
  Valdmanis, P. N., Gu, S., Schuermann, N., Sethupathy, P., Grimm, D., Kay, M. A.
  2012; 40 (8): 3704-3713

- **Adenovirus-Associated Virus Vector-Mediated Gene Transfer in Hemophilia B** *NEW ENGLAND JOURNAL OF MEDICINE*
  Nathwani, A. C., Tuddenham, E. G., Rangarajan, S., Rosales, C., McIntosh, J., Linch, D. C., Chowdary, P., Riddell, A., Pie, A. J., Harrington, C., O'Beirne, J., Smith, K., Pasi, et al
  2011; 365 (25): 2357-2365

- **Fate tracing of mature hepatocytes in mouse liver homeostasis and regeneration** *JOURNAL OF CLINICAL INVESTIGATION*
  Malato, Y., Naqvi, S., Schuermann, N., Ng, R., Wang, B., Zape, J., Kay, M. A., Grimm, D., Willenbring, H.
  2011; 121 (12): 4850-4860

- **Adeno-Associated Viral Vector Mediated Gene Transfer for Hemophilia B** *53rd Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
  Nathwani, A. C., Tuddenham, E. G., Rangarajan, S., Rosales, C., McIntosh, J. H., Linch, D. C., Chowdary, P., Griffioen, A., Riddell, A., Pie, J., Harrington, C., O'Beirne, J., Smith, et al
  AMER SOC HEMATOLOGY.2011: 4–5

- **THE MECHANISM OF miRNA AND siRNA ARGONAUTE LOADING IN MAMMALS** *7th Annual Meeting of the Oligonucleotide-Therapeutics-Society*
  Kay, M. A., Gu, S., Jin, L.
  MARY ANN LIEBERT INC.2011: A50–A51

- **Double Knockdown of Prolyl Hydroxylase and Factor-Inhibiting Hypoxia-Inducible Factor With Nonviral Minicircle Gene Therapy Enhances Stem Cell Mobilization and Angiogenesis After Myocardial Infarction** *Annual Meeting of the American-Heart-Association*

Huang, M., Nguyen, P., Jia, F., Hu, S., Gong, Y., de Almeida, P. E., Wang, L., Nag, D., Kay, M. A., Giaccia, A. J., Robbins, R. C., Wu, J. C.
LIPPINCOTT WILLIAMS & WILKINS.2011: S46–S54

- **NOVEL ANTI-HCV THERAPY: SINGLE SHRNA TARGETING BOTH STRANDS OF HCV**
  Lisowski, L., Elazar, M., Chu, K., Glenn, J. S., Kay, M. A.
  WILEY-BLACKWELL.2011: 418–19

- **Thermodynamic stability of small hairpin RNAs highly influences the loading process of different mammalian Argonautes** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
  Gu, S., Jin, L., Zhang, F., Huang, Y., Grimm, D., Rossi, J. J., Kay, M. A.
  2011; 108 (22): 9208-9213

- **State-of-the-art gene-based therapies: the road ahead** *NATURE REVIEWS GENETICS*
  Kay, M. A.
  2011; 12 (5): 316-328

- **Enhanced transgene expression improves immune responses following DNA vaccination**
  Skinner, N., Dietz, W., Jund, M., Hwu, P., Chen, Z., Kay, M., Blazar, B., Pennell, C., Osborn, M.
  AMER ASSOC IMMUNOLOGISTS.2011

- **Minicircle DNA-based Gene Therapy Coupled With Immune Modulation Permits Long-term Expression of alpha-L-Iduronidase in Mice With Mucopolysaccharidosis Type I** *MOLECULAR THERAPY*
  Osborn, M. J., McElmurry, R. T., Lees, C. J., DeFeo, A. P., Chen, Z., Kay, M. A., Naldini, L., Freeman, G., Tolar, J., Blazar, B. R.
  2011; 19 (3): 450-460

- **Generation of adult human induced pluripotent stem cells using nonviral minicircle DNA vectors** *NATURE PROTOCOLS*
  Narsinh, K. H., Jia, F., Robbins, R. C., Kay, M. A., Longaker, M. T., Wu, J. C.
  2011; 6 (1): 78-88

- **A robust system for production of minicircle DNA vectors** *NATURE BIOTECHNOLOGY*
  Kay, M. A., He, C., Chen, Z.
  2010; 28 (12): 1287-U96

- **Early Clinical Trial Results Following Administration of a Low Dose of a Novel Self Complementary Adeno-Associated Viral Vector Encoding Human Factor IX In Two Subjects with Severe Hemophilia B** *52nd Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
  Nathwani, A., Tuddenham, E., Rosales, C., McIntosh, J., Riddell, A., Rustagi, P., Glader, B., Kay, M., Allay, J., Coleman, J., Sleep, S., High, K. A., Mingozzi, et al
  AMER SOC HEMATOLOGY.2010: 114–14

- **Hyperactive Sleeping Beauty Transposase Enables Persistent Phenotypic Correction in Mice and a Canine Model for Hemophilia B** *MOLECULAR THERAPY*
  Hausl, M. A., Zhang, W., Muether, N., Rauschhuber, C., Franck, H. G., Merricks, E. P., Nichols, T. C., Kay, M. A., Ehrhardt, A.
  2010; 18 (11): 1896-1906

- **Early clinical trial results following administration of a low dose of a novel self complementary adenohyphen;associated viral vector encoding human factor ix in two subjects with severe Haemophilia B** *18th Annual Congress of the European-Society-of-Gene-and-Cell-Therapy*
  Nathwani, A. C., Tuddenham, E. G., Rosales, C., Mcintosh, J., Riddell, A., Rustagi, P., Galder, B., Kay, M., ALLAY, J., Coleman, J., Sleep, S., High, K. A., Mingozzi, et al
  MARY ANN LIEBERT INC.2010: 1362–62

- **Argonaute proteins are key determinants of RNAi efficacy, toxicity, and persistence in the adult mouse liver** *JOURNAL OF CLINICAL INVESTIGATION*
  Grimm, D., Wang, L., Lee, J. S., Schuermann, N., Gu, S., Boerner, K., Storm, T. A., Kay, M. A.
  2010; 120 (9): 3106-3119

- **Gene Transfer Approaches for Gene Addition, Knockdown and Cellular Reprogramming In Vivo**
  Kay, M. A.
  MARY ANN LIEBERT INC.2010: 1172–73

- **FATP2 is a hepatic fatty acid transporter and peroxisomal very long-chain acyl-CoA synthetase** *AMERICAN JOURNAL OF PHYSIOLOGY-ENDOCRINOLOGY AND METABOLISM*
  Falcon, A., Doege, H., Fluitt, A., Tsang, B., Watson, N., Kay, M. A., Stahl, A.
  2010; 299 (3): E384-E393

- **An in vitro-identified high-affinity nucleosome-positioning signal is capable of transiently positioning a nucleosome in vivo** *EPIGENETICS & CHROMATIN*
  Gracey, L. E., Chen, Z., Maniar, J. M., Valouev, A., Sidow, A., Kay, M. A., Fire, A. Z.
  2010; 3

- **Prevention of spontaneous bleeding in dogs with haemophilia A and haemophilia B** *10th Novo Nordisk Symposium on Haemostasis Management*
  Nichols, T. C., Raymer, R. A., Franck, H. W., Merricks, E. P., Bellinger, D. A., Defriess, N., Margaritis, P., Arruda, V. R., Kay, M. A., High, K. A.
  WILEY-BLACKWELL.2010: 19–23

- **Human tRNA-derived small RNAs in the global regulation of RNA silencing** *RNA-A PUBLICATION OF THE RNA SOCIETY*
  Haussecker, D., Huang, Y., Lau, A., Parameswaran, P., Fire, A. Z., Kay, M. A.
  2010; 16 (4): 673-695

- **Adeno-Associated Virus Gene Repair Corrects a Mouse Model of Hereditary Tyrosinemia In Vivo** *HEPATOLOGY*
  Paulk, N. K., Wursthorn, K., Wang, Z., Finegold, M. J., Kay, M. A., Grompe, M.
  2010; 51 (4): 1200-1208

- **A nonviral minicircle vector for deriving human iPS cells** *NATURE METHODS*
  Jia, F., Wilson, K. D., Sun, N., Gupta, D. M., Huang, M., Li, Z., Panetta, N. J., Chen, Z. Y., Robbins, R. C., Kay, M. A., Longaker, M. T., Wu, J. C.
  2010; 7 (3): 197-U46

- **miR-122 Continues to Blaze the Trail for MicroRNA Therapeutics** *MOLECULAR THERAPY*
  Haussecker, D., Kay, M. A.
  2010; 18 (2): 240-242

- **Six RNA Viruses and Forty-One Hosts: Viral Small RNAs and Modulation of Small RNA Repertoires in Vertebrate and Invertebrate Systems** *PLOS PATHOGENS*
  Parameswaran, P., Sklan, E., Wilkins, C., Burgon, T., Samuel, M. A., Lu, R., Ansel, K. M., Heissmeyer, V., Einav, S., Jackson, W., Doukas, T., Paranjape, S., Polacek, et al
  2010; 6 (2)

- **Low-level shRNA Cytotoxicity Can Contribute to MYC-induced Hepatocellular Carcinoma in Adult Mice** *MOLECULAR THERAPY*
  Beer, S., Bellovin, D. I., Lee, J. S., Komatsubara, K., Wang, L. S., Koh, H., Boerner, K., Storm, T. A., Davis, C. R., Kay, M. A., Felsher, D. W., Grimm, D.
  2010; 18 (1): 161-170

- **How do miRNAs mediate translational repression?** *Silence*
  Gu, S., Kay, M. A.
  2010; 1 (1): 11-?

- **Enhancing HIF-1a by Double shRNA Knockdown in Murine Myocardial Infarction** *82nd National Conference and Exhibitions and Scientific Sessions of the American-Heart-Association*
  Huang, M., Jia, F., Hu, S., Wang, X., Kay, M. A., Robbins, R. C., Wu, J. C.
  LIPPINCOTT WILLIAMS & WILKINS.2009: S827–S827

- **Generation of Low-antigenicity MHC I Knock-down Human Embryonic Stem Cells Using Molecular Therapy**
  Deuse, T., Seifert, M., Phillips, N., Fire, A., Kay, M., Tsao, P., Reichenspurner, H., Robbins, R. C., Schrepfer, S.
  LIPPINCOTT WILLIAMS & WILKINS.2009: S594–S595

- **Combined proteomic-RNAi screen for host factors involved in human hepatitis delta virus replication** *RNA-A PUBLICATION OF THE RNA SOCIETY*
  Cao, D., Haussecker, D., Huang, Y., Kay, M. A.
  2009; 15 (11): 1971-1979

- **Novel Minicircle Vector for Gene Therapy in Murine Myocardial Infarction** *81st Annual Scientific Session of the American-Heart-Association*
  Huang, M., Chen, Z., Hu, S., Jia, F., Li, Z., Hoyt, G., Robbins, R. C., Kay, M. A., Wu, J. C.
  LIPPINCOTT WILLIAMS & WILKINS.2009: S230–S237

- **FACING THE BLEEDING OBVIOUS: AAV-MEDIATED GENE THERAPY FOR HAEMOPHILIA B** *6th Meeting of the Australasian-Gene-Therapy-Society*
  Rasko, J. E., High, K., Tigges, M., Manno, C., Sabatino, D., Dake, M., McDonnell, J. W., Razavi, M., Arruda, V., Herzog, R., Rustagi, P., Sommer, J., Ragni, et al
  JOHN WILEY & SONS LTD.2009: 843–43

- **AAV Based RNAi Therapies To Treat and/or Prevent HCV in Animal Models**
  Lisowski, L., Elazar, M., Grompe, M., Glenn, J. S., Kay, M. A.
  NATURE PUBLISHING GROUP.2009: S14

- **AAV-rDNA Mediated Site-Specifi c Integration in Human Cell and Safety Analysis in Long-Term AAV-rDNA Treated Mice**
  Wang, Z., Mathur, J., Lisowski, L., Kay, M., Grompe, M.
  NATURE PUBLISHING GROUP.2009: S174

- **The Best of Both Worlds - Integrating rAAV Vectors as a Safe Tool for Gene Therapy**
  Lisowski, L., Zhang, F., Grompe, M., Kay, M. A.
  NATURE PUBLISHING GROUP.2009: S182

- **A Hyperactive Transposase System Delivered by a High-Capacity Adenoviral Vector Results in Long-Term Phenotypic Correction of Canine Hemophilia B**
  Hausl, M. A., Zhang, W., Franck, H. W., Nichols, T. C., Kay, M. A., Ehrhardt, A.
  NATURE PUBLISHING GROUP.2009: S241–S242

- **Simplified Minicircle DNA Vector Production Using Genetically Modified E. coli: Towards a GMP Suitable Production Process**
  Chen, Z., He, C., Kay, M. A.
  NATURE PUBLISHING GROUP.2009: S286–S287

- **The Mechanism(s) Responsible for Plasmid DNA Silencing In Vivo** *12th Annual Meeting of the American Society of Gene Therapy*
  Lu, J., Chen, Z., Kay, M. A.
  NATURE PUBLISHING GROUP.2009: S285–S286

- **Gene Transfer to Mouse Heart - A Study of Mini-Circle Vector Efficiency**
  Stenler, S., Andersson, A., Simonson, O. E., Lundin, K. E., Chen, Z., Kay, M. A., Smith, E. I., Sylven, C., Blomberg, P.
  NATURE PUBLISHING GROUP.2009: S348

- **Biological basis for restriction of microRNA targets to the 3 ' untranslated region in mammalian mRNAs** *NATURE STRUCTURAL & MOLECULAR BIOLOGY*
  Gu, S., Jin, L., Zhang, F., Sarnow, P., Kay, M. A.
  2009; 16 (2): 144-150

- **Gene Transfer to Mouse Heart and Skeletal Muscles Using a Minicircle Expressing Human Vascular Endothelial Growth Factor** *JOURNAL OF CARDIOVASCULAR PHARMACOLOGY*
  Stenler, S., Andersson, A., Simonson, O. E., Lundin, K. E., Chen, Z., Kay, M. A., Smith, C. I., Sylven, C., Blomberg, P.
  2009; 53 (1): 18-23

- **A rapid protocol for construction and production of high-capacity adenoviral vectors** *NATURE PROTOCOLS*
  Jager, L., Hausl, M. A., Rauschhuber, C., Wolf, N. M., Kay, M. A., Ehrhardt, A.
  2009; 4 (4): 547-564

- **Novel Minicircle Vector for Gene Therapy In Murine Myocardial Infarction**
  Huang, M., Chen, Z., Jia, F., Li, Z., Hoyt, G., Robbins, R. C., Kay, M. A., Wu, J. C.
  LIPPINCOTT WILLIAMS & WILKINS.2008: S791

- **Expression of shRNA from a tissue-specific pol II promoter is an effective and safe RNAi therapeutic** *MOLECULAR THERAPY*
  Giering, J. C., Grimm, D., Storm, T. A., Kay, M. A.
  2008; 16 (9): 1630-1636

- **Silencing of hepatic fatty acid transporter protein 5 in vivo reverses diet-induced non-alcoholic fatty liver disease and improves Hyperglycemia** *JOURNAL OF BIOLOGICAL CHEMISTRY*
  Doege, H., Grimm, D., Falcon, A., Tsang, B., Storm, T. A., Xu, H., Ortegon, A. M., Kazantzis, M., Kay, M. A., Stahl, A.
  2008; 283 (32): 22186-22192

- **Radioprotection in vitro and in vivo by minicircle plasmid carrying the human manganese superoxide dismutase transgene** *HUMAN GENE THERAPY*
  Zhang, X., Epperly, M. W., Kay, M. A., Chen, Z., Dixon, T., Franicola, D., Greenberger, B. A., Komanduri, P., Greenberger, J. S.
  2008; 19 (8): 820-826

- **Capped small RNAs and MOV10 in human hepatitis delta virus replication** *NATURE STRUCTURAL & MOLECULAR BIOLOGY*
  Haussecker, D., Cao, D., Huang, Y., Parameswaran, P., Fire, A. Z., Kay, M. A.
  2008; 15 (7): 714-721

- **In vitro and in vivo gene therapy vector evolution via multispecies interbreeding and retargeting of adeno-associated viruses** *JOURNAL OF VIROLOGY*
  Grimm, D., Lee, J. S., Wang, L., Desai, T., Akache, B., Storm, T. A., Kay, M. A.
  2008; 82 (12): 5887-5911

- **Wandering eye for RNAi** *NATURE MEDICINE*
  Rossi, J., Zamore, P., Kay, M. A.
  2008; 14 (6): 611

- **The host response to adenovirus, helper-dependent adenovirus, and adeno-associated virus in mouse liver** *MOLECULAR THERAPY*
  McCaffrey, A. P., Fawcett, P., Nakai, H., McCaffrey, R. L., Ehrhardt, A., Pham, T. T., Pandey, K., Xu, H., Feuss, S., Storm, T. A., Kay, M. A.
  2008; 16 (5): 931-941

- **Silencing of episomal transgene expression in liver by plasmid bacterial backbone DNA is independent of CpG methylation** *MOLECULAR THERAPY*
  Chen, Z., Riu, E., He, C., Xu, H., Kay, M. A.
  2008; 16 (3): 548-556

- **Hepatic parenchymal replacement in mice by transplanted allogeneic hepatocytes is facilitated by bone marrow transplantation and mediated by CD4 cells** *HEPATOLOGY*
  Streetz, K. L., Doyonnas, R., Grimm, D., Jenkins, D. D., Fuess, S., Perryman, S., Lin, J., Trautwein, C., Shizuru, J., Blau, H., Sylvester, K. G., Kay, M. A.
  2008; 47 (2): 706-718

- **microRNAs outwit immune limitations in gene therapy** *BLOOD*
  Kay, M. A.
  2007; 110 (13): 4136–37

- **Therapeutic application of RNAi: is mRNA targeting finally ready for prime time?** *JOURNAL OF CLINICAL INVESTIGATION*
  Grimm, D., Kay, M. A.
  2007; 117 (12): 3633-3641

- **Cis-acting gene regulatory activities in the terminal regions of Sleeping Beauty DNA transposon-based vectors** *HUMAN GENE THERAPY*
  Moldt, B., Yant, S. R., Andersen, P. R., Kay, M. A., Mikkelsen, J. G.
  2007; 18 (12): 1193-1204

- **Postintegrative gene silencing within the Sleeping Beauty transposition system** *MOLECULAR AND CELLULAR BIOLOGY*
  Garrison, B. S., Yant, S. R., Mikkelsen, J. G., Kay, M. A.
  2007; 27 (24): 8824-8833

- **Minicircle plasmid containing the human manganese superoxide dismutase (MnSOD) transgene confers radioprotection to hematopoietic progenitor cell line 32Dcl3**
  Zhang, X., Epperly, M. W., Kay, M. A., Chen, Z., Smith, T., Franicola, D., Greenberger, B., Komanduri, P., Greenberger, J. S.
  AMER SOC HEMATOLOGY.2007: 367B

- **Characterization of the relationship of AAV capsid domain swapping to liver transduction efficiency** *MOLECULAR THERAPY*
  Shen, X., Storm, T., Kay, M. A.
  2007; 15 (11): 1955-1962

- **DNA palindromes with a modest arm length of greater than or similar to s20 base pairs are a significant target for recombinant adeno-associated virus vector integration in the liver, muscles, and heart in mice** *JOURNAL OF VIROLOGY*
  Inagaki, K., Lewis, S. M., Wu, X., Ma, C., Munroe, D. J., Fuess, S., Storm, T. A., Kay, M. A., Nakai, H.
  2007; 81 (20): 11290-11303

- **Rapid and stable knockdown of an endogenous gene in retinal pigment epithelium** *HUMAN GENE THERAPY*
  Paskowitz, D. M., Greenberg, K. P., Yasumura, D., Grimm, D., Yang, H., Duncan, J. L., Kay, M. A., LaVail, M. M., Flannery, J. G., Vollrath, D.
  2007; 18 (10): 871-880

- **Liver directed AAV-mediated homologous recombination is largely independent of serotype** *58th Annual Meeting of the American-Association-for-the-Study-of-Liver-Diseases*

Wursthorn, K., Paulk, N., Storm, T., Finegold, M. J., Kay, M., Grompe, M.
WILEY-BLACKWELL.2007: 887A–887A

- **AAV vectors and turnorigenicity** *NATURE BIOTECHNOLOGY*
  Kay, M. A.
  2007; 25 (10): 1111-1113

- **The role of DNA-PKcs and artemis in opening viral DNA hairpin termini in various tissues in mice** *JOURNAL OF VIROLOGY*
  Inagaki, K., Ma, C., Storm, T. A., Kay, M. A., Nakai, H.
  2007; 81 (20): 11304-11321

- **Distinct pathways of genomic progression to benign and malignant tumors of the liver** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
  Tward, A. D., Jones, K. D., Yant, S., Cheung, S. T., Fan, S. T., Chen, X., Kay, M. A., Wang, R., Bishop, J. M.
  2007; 104 (37): 14771-14776

- **Robust expansion of human hepatocytes in Fah(-/-)/Rag2(-/-)/Il2rg(-/-) mice** *NATURE BIOTECHNOLOGY*
  Azuma, H., Paulk, N., Ranade, A., Dorrell, C., Al-Dhalimy, M., Ellis, E., Strom, S., Kay, M. A., Finegold, M., Grompe, M.
  2007; 25 (8): 903-910

- **Histone modifications are associated with the persistence or silencing of vector-mediated transgene expression in vivo** *MOLECULAR THERAPY*
  Riu, E., Chen, Z., Xu, H., He, C., Kay, M. A.
  2007; 15 (7): 1348-1355

- **Correction of DNA protein kinase deficiency by spliceosome-mediated RNA trans-splicing and Sleeping Beauty transposon delivery** *MOLECULAR THERAPY*
  Zayed, H., Xia, L., Yerich, A., Yant, S. R., Kay, M. A., Puttaraju, M., McGarrity, G. J., Wiest, D. L., McIvor, R. S., Tolar, J., Blazar, B. R.
  2007; 15 (7): 1273-1279

- **Dual system of RNA trans-splicing element and sleeping beauty transposon corrects dna protein kinase mutation in severe combined immune deficiency** *12th Congress of the European-Hematology-Association*
  Tolar, J., Zayed, H., Xia, L., Yerich, A., Yant, S. R., Kay, M. A., Puttaraju, M., Mansfield, S. G., Wiest, D., McIvor, R. S., Blazar, B. R.
  FERRATA STORTI FOUNDATION.2007: 154–154

- **Combinatorial RNAi: A winning strategy for the race against evolving targets?** *MOLECULAR THERAPY*
  Grimm, D., Kay, M. A.
  2007; 15 (5): 878-888

- **Site-directed transposon integration in human cells** *NUCLEIC ACIDS RESEARCH*
  Yant, S. R., Huang, Y., Akache, B., Kay, M. A.
  2007; 35 (7)

- **Adenovirus transduction is required for the correction of diabetes using Pdx-1 or neurogenin-3 in the liver** *MOLECULAR THERAPY*
  Wang, A. Y., Ehrhardt, A., Xu, H., Kay, M. A.
  2007; 15 (2): 255-263

- **Sarcoma derived from cultured mesenchymal stem cells** *STEM CELLS*
  Tolar, J., Nauta, A. J., Osborn, M. J., Mortari, A. P., McElmurry, R. T., Bell, S., Xia, L., Zhou, N., Riddle, M., Schroeder, T. M., Westendorf, J. J., McIvor, R. S., Hogendoorn, et al
  2007; 25 (2): 371-379

- **A two-hybrid screen identifies cathepsins B and L as uncoating factors for adeno-associated virus 2 and 8** *MOLECULAR THERAPY*
  Akache, B., Grimm, D., Shen, X., Fuess, S., Yant, S. R., Glazer, D. S., Park, J., Kay, M. A.
  2007; 15 (2): 330-339

- **RNAi and gene therapy: a mutual attraction.** *Hematology / the Education Program of the American Society of Hematology. American Society of Hematology. Education Program*
  Grimm, D., Kay, M. A.
  2007: 473-481

- **Somatic integration from an adenoviral hybrid vector into a hot spot in mouse liver results in persistent transgene expression levels in vivo** *MOLECULAR THERAPY*
  Ehrhardt, A., Yant, S. R., Giering, J. C., Xu, H., Engler, J. A., Kay, M. A.
  2007; 15 (1): 146-156

- **AAV8 vector injection at birth significantly augments the progression of liver tumors in HBV transgenic mice predisposed to hepatocellular carcinoma**
  Inagaki, K., Monga, S. S., Storm, T. A., Fuess, S., Kay, M. A., Nakai, H.
  JOHN WILEY & SONS LTD.2006: 1445–46

- **Molecular analysis of chromosomal rearrangements in mammalian cells after phi C31-mediated integration** *HUMAN GENE THERAPY*
  Ehrhardt, A., Engler, J. A., Xu, H., Cherry, A. M., Kay, M. A.
  2006; 17 (11): 1077-1094

- **The 37/67-kilodalton laminin receptor is a receptor for adeno-associated virus serotypes 8, 2, 3, and 9** *JOURNAL OF VIROLOGY*
  Akache, B., Grimm, D., Pandey, K., Yant, S. R., Xu, H., Kay, M. A.
  2006; 80 (19): 9831-9836

- **In vivo correction of a metabolic liver disease by AAV8-mediated homologous recombination**
  Wursthorn, K., Storm, T., Kay, M., Feingold, M., Grompe, M.
  JOHN WILEY & SONS INC.2006: 433A

- **Correction of severe combined immune deficiency in multipotent adult progenitor cells by spliceosome-mediated RNA trans-splicing and Sleeping Beauty transposon delivery** *35th Annual Meeting of the International-Society-for-Experimental-Hematology*
  Zayed, H., Xia, L., Yerich, A., Yant, S. R., Kay, M. A., Puttaraju, M., Mansfield, G., Wiest, D. L., McIvor, R. S., Tolar, J., Blazar, B. R.
  ELSEVIER SCIENCE INC.2006: 49–49

- **Robust systemic transduction with AAV9 vectors in mice: Efficient global cardiac gene transfer superior to that of AAV8** *MOLECULAR THERAPY*
  Inagaki, K., Fuess, S., Storm, T. A., Gibson, G. A., McTiernan, C. F., Kay, M. A., Nakai, H.
  2006; 14 (1): 45-?

- **Fatality in mice due to oversaturation of cellular microRNA/short hairpin RNA pathways** *NATURE*
  Grimm, D., Streetz, K. L., Jopling, C. L., Storm, T. A., Pandey, K., Davis, C. R., Marion, P., Salazar, F., Kay, M. A.
  2006; 441 (7092): 537-541

- **Host factors that impact the biodistribution and persistence of multipotent adult progenitor cells** *BLOOD*
  Tolar, J., O'Shaughnessy, M. J., Panoskaltsis-Mortari, A., McElmurry, R. T., Bell, S., Riddle, M., McIvor, R. S., Yant, S. R., Kay, M. A., Krause, D., Verfaillie, C. M., Blazar, B. R.
  2006; 107 (10): 4182-4188

- **Molecular Evolution of Adeno-Associated Viral (AAV) Vectors Via DNA Family Shuffling of Primate and Non-Primate Serotypes**
  Grimm, D., Lee, J. S., Storm, T. A., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S287

- **Transposition from a Gene-Deleted Adenoviral Vector Results in Phenotypic Correction in a Canine Model for Hemophilia B**
  Ehrhardt, A., Xu, H., Dillow, A. M., Yant, S. R., Nichols, T. C., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S5

- **Pol II-Driven shRNA as an Effective Hepatitis B Virus Therapeutic**
  Giering, J. C., Grimm, D., Storm, T. A., Xu, H., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S308–S309

- **Localization of Structural Determinants in AAV Capsid for Efficient Liver Transduction by Domain Swapping between AAV-2 and AAV-8**
  Shen, X., Xu, H., Storm, T., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S288

- **Study of an AAV-8 Capsid Mutant with Direct Heparin Binding Capability but Reduced Efficiency in Liver-Targeted Transduction**
  Shen, X., Xu, H., Storm, T., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S44–S45

- **Development of a Minicircle Vector Free of Plasmid Bacterial DNA Sequences and Capable of (empty set)C31-Mediated Site-Specific Integration**

Chen, Z., He, C., Ehrhardt, A., Xu, H., Kay, M. A.
NATURE PUBLISHING GROUP.2006: S198–S199

- **A Novel Class of Miniature Stabilized Double-Stranded AAV (msdsAAV) Vectors for the In Vivo Expression of Short Hairpin RNAs**
  Grimm, D., Lee, J. S., Streetz, K. L., Storm, T. A., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S409
- **Chromatin Study of Adenovirus Vector DNA In Vivo**
  Cao, D., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S328
- **Biology of rAAV8 in Mouse Liver Following Vector Administration at Birth**
  Inagaki, K., Fuess, S., Storm, T. A., Kay, M. A., Nakai, H.
  NATURE PUBLISHING GROUP.2006: S4
- **Mechanisms for Hairpin Loop Opening of "Closed" AAV-ITRs by Specific Cellular Endonuclease Activities, a Prerequisite for rAAV Vector Genome Recombinations In Vivo**
  Inagaki, K., Storm, T. A., Kay, M. A., Nakai, H.
  NATURE PUBLISHING GROUP.2006: S2–S3
- **RNAi-Based Therapy for the Treatment of HCV**
  Couto, L. B., Parker, A. E., Haniff, G., Suhy, D. A., Kolykhalov, A. A., Roelvink, P. W., Garcia, L., Schroeder, R., Kay, M. A., Cunningham, S. M.
  NATURE PUBLISHING GROUP.2006: S422–S423
- **Expression of Short Hairpin RNAs by Liver and Non Liver Specific RNA Pol II Expression Cassettes: What Governs Activity?**
  Suhy, D. A., Kolykhalov, A. A., Couto, L. B., Garcia, L., Schroeder, A. R., Parker, A. E., Haniff, G., Kay, M. A., Roelvink, P. W.
  NATURE PUBLISHING GROUP.2006: S397
- **In Vivo Correction of a Metabolic Liver Disease by AAV8-Mediated Homologous Recombination**
  Wursthorn, K., Storm, T., Kay, M. A., Finegold, M., Grompe, M.
  NATURE PUBLISHING GROUP.2006: S311–S312
- **Fatality in Mice Due to Oversaturation of Cellular Micro/Short Hairpin RNA Pathways**
  Grimm, D., Streetz, K. L., Storm, T. A., Jopling, C. L., Lee, J. S., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S312
- **Hepatitis Delta Virus-Mediated Amplification of Therapeutic RNAi**
  Haussecker, D., Elazar, M., Glenn, J. S., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S142
- **Directed Transposon Integration in Human Cells**
  Yant, S. R., Huang, Y., Akache, B., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S148–S149
- **Post-Integrative Gene Silencing in the Sleeping Beauty Transposition System**
  Garrison, B. S., Yant, S. R., Mikkelsen, J. G., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S307
- **Non-Viral Transposon Mediated Gene Transfer of Human Factor VIII to Hemophilia A Mice**
  Xie, L., Kang, Y., Yant, S., Kay, M., McCray, P.
  NATURE PUBLISHING GROUP.2006: S262
- **Revisiting rAAV Vector Integration in scid Mice: DNA-PKcs Deficiency Does Not Substantially Increase Integration Frequency in Hepatic and Non-Hepatic Tissues In Vivo**
  Inagaki, K., Storm, T. A., Kay, M. A., Nakai, H.
  NATURE PUBLISHING GROUP.2006: S47
- **Unraveling the Mechanisms Underlying Silencing/Activation of Episomal Vectors In Vivo**
  Riu, E., Chen, Z., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S207

- **A Screen for Host Cellular Proteins That Interact with Adeno-Associated Virus Capsid Proteins Reveals Proteins Involved in AAV8 Transduction**
  Akache, B., Fuess, S., Grimm, D., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S42–S43

- **Treatment for Hemophilia B Using Self-Complimentary AAV8 Vectors**
  Hebert, M. L., Grimm, D., Storm, T. A., Kay, M. A.
  NATURE PUBLISHING GROUP.2006: S189–S190

- **Therapeutic short hairpin RNA expression in the liver: viral targets and vectors** *GENE THERAPY*
  Grimm, D., Kay, M. A.
  2006; 13 (6): 563-575

- **Successful transduction of liver in hemophilia by AAV-factor IX and limitations imposed by the host immune response** *NATURE MEDICINE*
  Manno, C. S., Arruda, V. R., Pierce, G. F., Glader, B., Ragni, M., Rasko, J., Ozelo, M. C., Hoots, K., Blatt, P., Konkle, B., Dake, M., Kaye, R., Razavi, et al
  2006; 12 (3): 342-347

- **Analysis of vector genome integration sites in various tissues following systemic administration of AAV serotype 8 vector in mice** *11th Annual Meeting of the Japan-Society-of-Gene-Therapy*
  Inagaki, K., Wu, X., Fuess, S., Storm, T. A., Kay, M. A., Nakai, H.
  WILEY-BLACKWELL.2006: 391–91

- **Liver transduction with recombinant adeno-associated virus is primarily restricted by capsid serotype not vector genotype** *JOURNAL OF VIROLOGY*
  Grimm, D., Pandey, K., Nakai, H., Storm, T. A., Kay, M. A.
  2006; 80 (1): 426-439

- **In vivo selection of primary and bone-marrow-derived hepatocytes after allogeneic transplantation in mice** *41st Annual Meeting of the European-Association-for-the-Study-of-the-Liver*
  Streetz, K. L., Doyonnas, R., Jenkins, D., Perryman, S., Fuess, S., Lin, S., Shizuru, J., Blau, H., Trautwein, C., Sylvester, K., Kay, M. A.
  ELSEVIER SCIENCE BV.2006: S33–S33

- **In vivo selection of transplanted allogeneic hepatocytes and bone-marrow derived hepatocytes after allogeneic bone-marrow transplantation in mice** *56th Annual Meeting of the American-Association-for-the-Study-of-Liver-Diseases*
  Streetz, K., Doyonnas, R., Jenkins, D., Lin, S., Shizuru, J., Blau, H., Sylvester, K., Kay, M.
  WILEY-BLACKWELL.2005: 370A–371A

- **Cell therapy for hepatocyte replacement through bone marrow derived myelomonocytic progenitors** *91st Annual Clinical Congress of the American-College-of-Surgeons*
  Sylvester, K. G., Jenkins, D., Streetz, K., Doyannis, R., Perryman, S., Kay, M., Blau, H.
  ELSEVIER SCIENCE INC.2005: S47–S48

- **Lessons from a clinical trial of liver-directed AAV gene transfer in hemophilia B** *4th Meeting of the Australasian-Gene-Therapy-Society*
  Rasko, J., High, K., TIGGES, M., Manno, C., Sabatino, D., Dake, M., Razavi, M., Arruda, V., Herzog, R., Rustagi, P., Sommer, J., Ragni, M., Konkle, et al
  WILEY-BLACKWELL.2005: 1117–18

- **Real-time in vivo imaging transgenesis by of stem cells following transposition** *MOLECULAR THERAPY*
  Tolar, J., Osborn, M., Bell, S., McElmurry, R., Xia, L., Riddle, M., Panoskaltsis-Mortari, A., Jiang, Y. H., McIvor, R. S., Contag, C. H., Yant, S. R., Kay, M. A., Verfaillie, et al
  2005; 12 (1): 42-48

- **Real-time in vivo imaging of stem cells following transgenesis by transposition.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Tolar, J., Osborn, M., Bell, S., McElmurry, R., Xia, L., Riddle, M., Panoskaltsis-Mortari, A., Jiang, Y., McIvor, R. S., Contag, C. H., Yant, S. R., Kay, M. A., Verfaillie, et al
  2005; 12 (1): 42-48

- **A direct comparison of two nonviral gene therapy vectors for somatic integration: In vivo evaluation of the bacteriophage integrase phi c31 and the Sleeping Beauty transposase** *MOLECULAR THERAPY*
  Ehrhardt, A., Xu, H., Huang, Z., Engler, J. A., Kay, M. A.
  2005; 11 (5): 695-706

- **Increased maintenance and persistence of transgenes by excision of expression cassettes from plasmid sequences in vivo** *HUMAN GENE THERAPY*
  Riu, E., Grimm, D., Huang, Z., Kay, M. A.

2005; 16 (5): 558-570

- **Allogeneic bone-marrow transplantation in mice provides tolerance and promotes massive in vivo selection of subsequently transplanted hepatocytes** *40th Annual Meeting of the European-Association-for-the-Study-of-the-Liver*
  Streetz, K. L., Doyonnas, R., Jenkins, D., Sylvester, K., Kay, M. A.
  ELSEVIER SCIENCE BV.2005: 6–6

- **Large-scale molecular characterization of adeno-associated virus vector integration in mouse liver** *JOURNAL OF VIROLOGY*
  Nakai, H., Wu, X. L., Fuess, S., Storm, T. A., Munroe, D., Montini, E., Burgess, S. M., Grompe, M., Kay, M. A.
  2005; 79 (6): 3606-3614

- **Modified infusion procedures affect recombinant adeno-associated virus vector type 2 transduction in the liver** *HUMAN GENE THERAPY*
  Ohashi, K., Nakai, H., Couto, L. B., Kay, M. A.
  2005; 16 (3): 299-306

- **High-resolution genome-wide mapping of transposon integration in mammals** *MOLECULAR AND CELLULAR BIOLOGY*
  Yant, S. R., Wu, X. L., Huang, Y., Garrison, B., Burgess, S. M., Kay, M. A.
  2005; 25 (6): 2085-2094

- **Liver tissue engineering at extrahepatic sites in mice as a potential new therapy for genetic liver diseases** *HEPATOLOGY*
  Ohashi, K., Waugh, J. M., Dake, M. D., Yokoyama, T., Kuge, H., Nakajima, Y., Yamanouchi, M., Naka, H., Yoshioka, A., Kay, M. A.
  2005; 41 (1): 132-140

- **Stability and repeat regeneration potential of the engineered liver tissues under the kidney capsule in mice** *3rd Annual Meeting of the Japanese-Society-for-Regenerative-Medicine*
  Ohashi, K., Kay, M. A., Yokoyama, T., Kuge, H., Kanehiro, H., Hisanaga, M., Ko, S., Nakajima, Y.
  COGNIZANT COMMUNICATION CORP.2005: 621–27

- **Unrestricted hepatocyte transduction with adeno-associated virus serotype 8 vectors in mice** *JOURNAL OF VIROLOGY*
  Nakai, H., Fuess, S., Storm, T. A., Muramatsu, S., Nara, Y., Kay, M. A.
  2005; 79 (1): 214-224

- **Genomic progression in mouse models for liver tumors** *70th Cold Spring Harbor Symposium on Quantitative Biology*
  Tward, A. D., Jones, K. D., Yant, S., Kay, M. A., Wang, R., BISHOP, J. M.
  COLD SPRING HARBOR LABORATORY PRESS.2005: 217–224

- **Improved production and purification of minicircle DNA vector free of plasmid bacterial sequences and capable of persistent transgene expression in vivo** *HUMAN GENE THERAPY*
  Chen, Z. Y., He, C. Y., Kay, M. A.
  2005; 16 (1): 126-131

- **Adeno-associated virus vectors for short hairpin RNA expression** *RNA INTERFERENCE*
  Grimm, D., Pandey, K., Kay, M. A.
  2005; 392: 381-405

- **Human immune responses to AAV-2 capsid may limit duration of expression in liver-directed gene transfer in humans with hemophilia B.** *46th Annual Meeting of the American-Society-of-Hematology*
  High, K., TIGGES, M., Manno, C., Sabatino, D., Arruda, V., Herzog, R., Rustagi, P., Rasko, J., Sommer, J., Jaworski, K., Ragni, M., Glader, B., Lessard, et al
  AMER SOC HEMATOLOGY.2004: 121A–121A

- **Transgenesis of multipotent adult progenitor cells (MAPC) with sleeping beauty transposons to determine MAPC homing and persistence in real-time in vivo.** *46th Annual Meeting of the American-Society-of-Hematology*
  Tolar, J., Osborn, M., Bell, S., Xia, L., Riddle, M., Panoskaltsis-Mortari, A., McIvor, S., Stephen, Y. R., Kay, M. A., Contag, C. H., Verfaillie, C. M., Blazar, B. R.
  AMER SOC HEMATOLOGY.2004: 577A–578A

- **Real-time in vivo biodistribution of multipotent adult progenitor cells (MAPC): Role of the immune system in MAPC resistance in non-transplanted and bone marrow transplanted mice.** *46th Annual Meeting of the American-Society-of-Hematology*
  Tolar, J., Bell, S., McElmurry, R., Xia, L. L., McIvor, R. S., Yam, S. R., Kay, M. A., Contag, C. H., Verfaillie, C. M., Blazar, B. R.
  AMER SOC HEMATOLOGY.2004: 147A–148A

- **Efficient inhibition of in-stent restenosis by controlled stent-based inhibition of elastase: A pilot study** *JOURNAL OF VASCULAR AND INTERVENTIONAL RADIOLOGY*

Ganaha, F., Ohashi, K., Do, Y. S., Lee, J., Sugimoto, K., Minamiguchi, H., Elkins, C. J., Sameni, D., Modanlou, S., Kao, E. Y., Kay, M. A., Waugh, J. M., Dake, et al

2004; 15 (11): 1287-1293

- **Extracellular matrix component cotransplantation prolongs survival of heterotopically transplanted human hepatocytes in mice** *8th Congress of the Asian-Society-of-Transplantation*
  Ohashi, K., Kay, M. A.
  ELSEVIER SCIENCE INC.2004: 2469–70
- **Mutational analysis of the N-terminal DNA-binding domain of Sleeping Beauty transposase: Critical residues for DNA binding and hyperactivity in mammalian cells** *MOLECULAR AND CELLULAR BIOLOGY*
  Yant, S. R., Park, J., Huang, Y., Mikkelsen, J. G., Kay, M. A.
  2004; 24 (20): 9239-9247
- **Donor-derived, liver-specific protein expression after bone marrow transplantation** *TRANSPLANTATION*
  Jenkins, D. D., Streetz, K., Tataria, M., Sahar, D., Kurobe, M., Longaker, M. T., Kay, M. A., Sylvester, K. G.
  2004; 78 (4): 530-536
- **Silencing of episomal transgene expression by plasmid bacterial DNA elements in vivo** *GENE THERAPY*
  Chen, Z. Y., He, C. Y., Meuse, L., Kay, M. A.
  2004; 11 (10): 856-864
- **Comparison of adenoviral and adeno-associated viral vectors for pancreatic gene delivery in vivo** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Wang, A. Y., Peng, P. D., Ehrhardt, A., Storm, T. A., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S66–S66
- **Mechanistic insights into the persistence of non-viral mediated gene transfer in vivo** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Riu, E., Huang, Z., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S398–S398
- **In vivo activity of nuclease-resistant siRNAs** *RNA-A PUBLICATION OF THE RNA SOCIETY*
  Layzer, J. M., McCaffrey, A. P., Tanner, A. K., Huang, Z., Kay, M. A., Sullenger, B. A.
  2004; 10 (5): 766-771
- **Hyperactive transposase mutants of the Sleeping Beauty transposon** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Yant, S. R., Park, J., Huang, Y., Mikkelsen, J. G., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S310–S310
- **Toward adenoviral mediated RNA interference for the treatment of hepatitis B virus infection** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  McCaffrey, A. P., Pandey, K., Ehrhardt, A., Huang, Z., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S24–S25
- **Immune responses to AAV and to Factor IX in a phase I study of AAV-mediated, liver-directed gene transfer for hemophilia B** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  High, K., Manno, C., Sabatino, D., Hutchison, S., Dake, M., Razavi, M., Kaye, R., Aruda, V., Herzog, R., Rustagi, P., Rasko, J., Hoots, K., Blatt, et al
  NATURE PUBLISHING GROUP.2004: S383–S384
- **A new model to functionally measure fusion events in the liver after bone-marrow transplantation over time** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Streetz, K. L., Jenkins, D. D., Longaker, M. T., Sylvester, K. G., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S118–S118
- **Hot spots for rAAV2 vector integration in mice** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Nakai, H., Wu, X. L., Fuess, S., Storm, T., Burgess, S., Grompe, M., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S6–S6
- **Complete inhibition of hepatitis B virus gene expression in vivo with short hairpin RNA expressed from a novel double-stranded, bi-cistronic adeno-associated virus pseudotype 8 vector** *7th Annual Meeting of the American-Society-of-Gene-Therapy*
  Grimm, D., Streetz, K. L., Storm, T. A., Nakai, H., McCaffrey, A. P., Huang, Z., Salazar, F. H., Marion, P. L., Kay, M. A.
  NATURE PUBLISHING GROUP.2004: S141–S142

- **Comparison of adenoviral and adeno-associated viral vectors for pancreatic gene delivery in vivo** *HUMAN GENE THERAPY*
  Wang, A. Y., Peng, P. D., Ehrhardt, A., Storm, T. A., Kay, M. A.
  2004; 15 (4): 405-413

- **Functional measurement of fusion in the liver after bone-marrow transplantation over time** *39th Annual Meeting of the European-Association-for-the-Study-of-the-Liver*
  Streetz, K. L., Jenkins, D., Longaker, G., Sylvester, K., Kay, M. A.
  ELSEVIER SCIENCE BV.2004: 109–109

- **Gene transfer: Bench to bedside.**
  Kay, M. A.
  AMER CHEMICAL SOC.2004: U132

- **Rapid uncoating of vector genomes is the key to efficient liver transduction with pseudotyped adeno-associated virus vectors** *JOURNAL OF VIROLOGY*
  Thomas, C. E., Storm, T. A., Huang, Z., Kay, M. A.
  2004; 78 (6): 3110-3122

- **Hepatic gene transfer** *12th International Falk Liver Week*
  Kay, M. A.
  SPRINGER.2004: 171–171

- **Nonhomologous-end-joining factors regulate DNA repair fidelity during Sleeping Beauty element transposition in mammalian cells** *MOLECULAR AND CELLULAR BIOLOGY*
  Yant, S. R., Kay, M. A.
  2003; 23 (23): 8505-8518

- **Preclinical in vivo evaluation of pseudotyped adeno-associated virus vectors for liver gene therapy** *BLOOD*
  Grimm, D., Zhou, S. Z., Nakai, H., Thomas, C. E., Storm, T. A., Fuess, S., Matsushita, T., Allen, J., Surosky, R., Lochrie, M., Meuse, L., McClelland, A., Colosi, et al
  2003; 102 (7): 2412-2419

- **A gene-deleted adenoviral vector results in phenotypic correction of canine hemophilia B without liver toxicity or thrombocytopenia** *BLOOD*
  Ehrhardt, A., Xu, H., Dillow, A. M., Bellinger, D. A., Nichols, T. C., Kay, M. A.
  2003; 102 (7): 2403-2411

- **Helper-independent Sleeping Beauty transposon-transposase vectors for efficient nonviral gene delivery and persistent gene expression in vivo** *MOLECULAR THERAPY*
  Mikkelsen, J. G., Yant, S. R., Meuse, L., Huang, Z., Xu, H., Kay, M. A.
  2003; 8 (4): 654-665

- **Minicircle DNA vectors devoid of bacterial DNA result in persistent and high-level transgene expression in vivo** *MOLECULAR THERAPY*
  Chen, Z. Y., He, C. Y., Ehrhardt, A., Kay, M. A.
  2003; 8 (3): 495-500

- **Hepatocyte targeting for quantifiable and functional cellular transplantation** *89th Annual Clinical Congress of the American-College-of-Surgeons*
  Jenkins, D. D., Streetz, K., Kay, M., Longaker, M., Sylvester, K.
  ELSEVIER SCIENCE INC.2003: S15–S15

- **System for simultaneous tissue-specific and disease-specific regulation of therapeutic gene expression** *HUMAN GENE THERAPY*
  Chyung, Y. H., Peng, P. D., Kay, M. A.
  2003; 14 (13): 1255-1264

- **A potent and specific morpholino antisense inhibitor of hepatitis C translation in mice** *HEPATOLOGY*
  McCaffrey, A. P., Meuse, L., Karimi, M., Contag, C. H., Kay, M. A.
  2003; 38 (2): 503-508

- **The effect of age on hepatic gene transfer with self-inactivating lentiviral vectors in vivo** *MOLECULAR THERAPY*
  Park, F., Ohashi, K., Kay, M. A.
  2003; 8 (2): 314-323

- **From virus evolution to vector revolution: use of naturally occurring serotypes of adeno-associated virus (AAV) as novel vectors for human gene therapy.** *Current gene therapy*
  Grimm, D., Kay, M. A.
  2003; 3 (4): 281-304

- **In vivo antiviral efficacy of prenylation inhibitors against hepatitis delta virus** *JOURNAL OF CLINICAL INVESTIGATION*
  Bordier, B. B., Ohkanda, J., Liu, P., Lee, S. Y., Salazar, F. H., Marion, P. L., Ohashi, K., Meuse, L., Kay, M. A., Casey, J. L., Sebti, S. M., Hamilton, A. D., Glenn, et al
  2003; 112 (3): 407-414

- **Looking into the safety of AAV vectors** *NATURE*
  Kay, M. A., Nakai, H.
  2003; 424 (6946): 251

- **Episomal persistence of recombinant adenoviral vector Genomes during the cell cycle in vivo** *JOURNAL OF VIROLOGY*
  Ehrhardt, A., Xu, H., Kay, M. A.
  2003; 77 (13): 7689-7695

- **AAV serotype 2 vectors preferentially integrate into active genes in mice** *NATURE GENETICS*
  Nakai, H., Montini, E., Fuess, S., Storm, T. A., Grompe, M., Kay, M. A.
  2003; 34 (3): 297-302

- **Sustainable correction of junctional epidermolysis bullosa via transposon-mediated nonviral gene transfer** *GENE THERAPY*
  Ortiz-Urda, S., Lin, Q., Yant, S. R., Keene, D., Kay, M. A., Khavari, P. A.
  2003; 10 (13): 1099-1104

- **Inhibition of hepatitis B virus in mice by RNA interference** *NATURE BIOTECHNOLOGY*
  McCaffrey, A. P., Nakai, H., Pandey, K., Huang, Z., Salazar, F. H., Xu, H., Wieland, S. F., Marion, P. L., Kay, M. A.
  2003; 21 (6): 639-644

- **Pathways of removal of free DNA vector ends in normal and DNA-PKcs-deficient SCID mouse Hepatocytes transduced with rAAV vectors** *HUMAN GENE THERAPY*
  Nakai, H., Storm, T. A., Fuess, S., Kay, M. A.
  2003; 14 (9): 871-881

- **Helper virus-free, optically controllable, and two-plasmid-based production of adeno-associated virus vectors of serotypes 1 to 6** *MOLECULAR THERAPY*
  Grimm, D., Kay, M. A., Kleinschmidt, J. A.
  2003; 7 (6): 839-850

- **Progress and problems with the use of viral vectors for gene therapy** *NATURE REVIEWS GENETICS*
  Thomas, C. E., Ehrhardt, A., Kay, M. A.
  2003; 4 (5): 346-358

- **Assessing the risk of inadvertant germline transmision of recombinant AAV-2 vector** *6th Annual Meeting of the American-Society-of-Gene-Therapy*
  Arruda, V. R., Schuettrumpf, J., Liu, J. H., Addya, K., Leonard, D., Couto, L., Chew, A., Zhen, Z., Sommer, J., Herzog, R. W., Kay, M. A., Bert, G., Manno, et al
  NATURE PUBLISHING GROUP.2003: S161–S162

- **Cleavage/excision of plasmid DNA in vivo leads to increased maintenance and persistence of transgenes expression in mouse** *6th Annual Meeting of the American-Society-of-Gene-Therapy*
  Riu, E., Huang, Z., Kay, M. A.
  NATURE PUBLISHING GROUP.2003: S9–S10

- **Australian patients in a multi-centre phase I/II trial of AAV-mediated gene transfer to the liver for severe hemophilia B** *3rd Meeting of the Australasian-Gene-Therapy-Society*
  Rasko, J. E., High, K., Kay, M. A., Glader, B., Manno, C. S., Hutchinson, S., Dake, M., Razavi, M., Kaye, R., Arruda, V. R., Herzog, R., McClelland, A., Pearce, et al
  WILEY-BLACKWELL.2003: S3–S4

- **Sleeping Beauty transposase-transposon cis-vectors for efficient nonviral gene delivery and persistent gene expression in vivo** *6th Annual Meeting of the American-Society-of-Gene-Therapy*
  Mikkelsen, J. G., Yant, S. R., Meuse, L., Huang, Z., Xu, H., Kay, M. A.

NATURE PUBLISHING GROUP.2003: S74–S74

- **Pre-clinical in vivo evaluation of pseudotyped adeno-associated virus (AAV) vectors for liver gene therapy** *6th Annual Meeting of the American-Society-of-Gene-Therapy*
  Grimm, D., Zhou, S. Z., Nakai, H., Thomas, C. E., Storm, T. A., Fuess, S., Matsushita, T., Surosky, R., Lochrie, M., Meuse, L., McClelland, A., Colosi, P., Kay, et al
  NATURE PUBLISHING GROUP.2003: S27–S27

- **AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B** *BLOOD*
  Manno, C. S., Chew, A. J., Hutchison, S., Larson, P. J., Herzog, R. W., Arruda, V. P., Tai, S. J., Ragni, M. V., Thompson, A., Ozelo, M., Couto, L. B., Leonard, D. G., Johnson, et al
  2003; 101 (8): 2963-2972

- **Advancing molecular therapies through in vivo bioluminescent imaging.** *Molecular imaging*
  McCaffrey, A., Kay, M. A., Contag, C. H.
  2003; 2 (2): 75-86

- **Optimization of cis-acting elements for gene expression from nonviral vectors in vivo** *HUMAN GENE THERAPY*
  Ehrhardt, A., Peng, P. D., Xu, H., Meuse, L., Kay, M. A.
  2003; 14 (3): 215-225

- **Helper-independent and AAV-ITR-independent chromosomal integration of double-stranded linear DNA vectors in mice** *MOLECULAR THERAPY*
  Nakai, H., Montini, E., Fuess, S., Storm, T. A., Meuse, L., Finegold, M., Grompe, M., Kay, M. A.
  2003; 7 (1): 101-111

- **Liver tissues engineered at the kidney capsule and their regeneration potential**
  Ohashi, K., Nakajima, Y., Kay, M. A.
  COGNIZANT COMMUNICATION CORP.2003: 150

- **Free DNA ends are essential for concatemerization of synthetic double-stranded adeno-associated virus vector genomes transfected into mouse hepatocytes in vivo** *MOLECULAR THERAPY*
  Nakai, H., Fuess, S., Storm, T. A., Meuse, L. A., Kay, M. A.
  2003; 7 (1): 112-121

- **Epstein-Barr virus vectors provide prolonged robust factor IX expression in mice** *BIOTECHNOLOGY PROGRESS*
  Sclimenti, C. R., Neviaser, A. S., Baba, E. J., Meuse, L., Kay, M. A., Calos, M. P.
  2003; 19 (1): 144-151

- **In vivo correction of murine tyrosinemia type I by DNA-mediated transposition** *MOLECULAR THERAPY*
  Montini, E., Held, P. K., Noll, M., Morcinek, N., Al-Dhalimy, M., Finegold, M., Yant, S. R., Kay, M. A., Grompe, M.
  2002; 6 (6): 759-769

- **A story of mice and men.** *Gene therapy*
  McCaffrey, A. P., Kay, M. A.
  2002; 9 (23): 1563-?

- **A phase I/II clinical trial for liver directed AAV-mediated gene transfer for severe hemophilia B.** *44th Annual Meeting of the American-Society-of-Hematology*
  Kay, M. A., High, K., Glader, B., Manno, C. S., Hutchinson, S., Dake, M., Razavi, M., Kaye, R., Arruda, V. R., Herzog, R., McClelland, A., Rustagi, P., Johnson, et al
  AMER SOC HEMATOLOGY.2002: 115A–115A

- **Assessing the risk of inadvertent germline transmission of vector DNA following intravascular delivery of rAAV vector.** *44th Annual Meeting of the American-Society-of-Hematology*
  Arruda, V. R., Schuettrumpf, J., Couto, L., Leonard, D., Addya, K., Liu, J. H., Sommer, J., Herzog, R. W., Kay, M. A., Glader, B., Manno, C. S., Chew, A., High, et al
  AMER SOC HEMATOLOGY.2002: 869A–869A

- **Site-specific genomic integration produces therapeutic Factor IX levels in mice** *NATURE BIOTECHNOLOGY*
  Olivares, E. C., Hollis, R. P., Chalberg, T. W., Meuse, L., Kay, M. A., Calos, M. P.
  2002; 20 (11): 1124-1128

- **A limited number of transducible hepatocytes restricts a wide-range linear vector dose response in recombinant adeno-associated virus-mediated liver transduction** *JOURNAL OF VIROLOGY*
  Nakai, H., Thomas, C. E., Storm, T. A., Fuess, S., Powell, S., Wright, J. F., Kay, M. A.
  2002; 76 (22): 11343-11349
- **A prenylation inhibitor prevents production of infectious hepatitis delta virus particles** *JOURNAL OF VIROLOGY*
  Bordier, B. B., Marion, P. L., Ohashi, K., Kay, M. A., Greenberg, H. B., Casey, J. L., Glenn, J. S.
  2002; 76 (20): 10465-10472
- **Transposition from a gutless adeno-transposon vector stabilizes transgene expression in vivo** *NATURE BIOTECHNOLOGY*
  Yant, S. R., Ehrhardt, A., Mikkelsen, J. G., Meuse, L., Pham, T., Kay, M. A.
  2002; 20 (10): 999-1005
- **Efficient gene transduction to cultured hepatocytes by HIV-1 derived lentiviral vector** *2nd International Congress on Immunosuppression*
  Ohashi, K., Park, F., Schwall, R. H., Kay, M. A.
  ELSEVIER SCIENCE INC.2002: 1431–33
- **RNA interference in adult mice.** *Nature*
  McCaffrey, A. P., Meuse, L., Pham, T. T., Conklin, D. S., Hannon, G. J., Kay, M. A.
  2002; 418 (6893): 38-39
- **Gene expression - RNA interference in adult mice** *NATURE*
  McCaffrey, A. P., Meuse, L., Pham, T. T., Conklin, D. S., Hannon, G. J., Kay, M. A.
  2002; 418 (6893): 38-39
- **A new adenoviral helper-dependent vector results in long-term therapeutic levels of human coagulation factor IX at low doses in vivo** *BLOOD*
  Ehrhardt, A., Kay, M. A.
  2002; 99 (11): 3923-3930
- **Determinants of hepatitis C translational initiation in vitro, in cultured cells and mice** *MOLECULAR THERAPY*
  McCaffrey, A. P., Hashi, K., Meuse, L., Shen, S. L., Lancaster, A. M., Lukavsky, P. J., Sarnow, P., Kay, M. A.
  2002; 5 (6): 676-684
- **Role of hepatocyte direct hyperplasia in lentivirus-mediated liver transduction in vivo** *HUMAN GENE THERAPY*
  Ohashi, K., Park, F., Kay, M. A.
  2002; 13 (5): 653-663
- **Lack of germline transmission of vector sequences following systemic administration of recombinant AAV-2 vector in males** *MOLECULAR THERAPY*
  Arruda, V. R., Fields, P. A., Milner, R., Wainwright, L., De Miguel, M. P., Donovan, P. J., Herzog, R. W., Nichols, T. C., Biegel, J. A., Razavi, M., Dake, M., Huff, D., Flake, et al
  2001; 4 (6): 586-592
- **Approaches for generating recombinant adenovirus vectors** *ADVANCED DRUG DELIVERY REVIEWS*
  Mizuguchi, H., Kay, M. A., Hayakawa, T.
  2001; 52 (3): 165-176
- **Hepatocyte transplantation: clinical and experimental application** *JOURNAL OF MOLECULAR MEDICINE-JMM*
  Ohashi, K., Park, F., Kay, M. A.
  2001; 79 (11): 617-630
- **Correction of the retinal dystrophy phenotype of the RCS rat by viral gene transfer of Mertk** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
  Vollrath, D., Feng, W., Duncan, J. L., Yasumura, D., D'Cruz, P. M., Chappelow, A., Matthes, M. T., Kay, M. A., LaVail, M. M.
  2001; 98 (22): 12584-12589
- **Modified HIV-1 based lentiviral vectors have an effect on viral transduction efficiency and gene expression in vitro and in vivo** *MOLECULAR THERAPY*
  Park, F., Kay, M. A.
  2001; 4 (3): 164-173
- **Extrachromosomal recombinant adeno-associated virus vector genomes are primarily responsible for stable liver transduction in vivo** *JOURNAL OF VIROLOGY*

Nakai, H., Yant, S. R., Storm, T. A., Fuess, S., Meuse, L., Kay, M. A.
2001; 75 (15): 6969-6976

- **Epstein-Barr virus/human vector provides high-level, long-term expression of alpha(1)-antitrypsin in mice** *MOLECULAR THERAPY*
  Stoll, S. M., Sclimenti, C. R., Baba, E. J., Meuse, L., Kay, M. A., Calos, M. P.
  2001; 4 (2): 122-129

- **A simplified system for constructing recombinant adenoviral vectors containing heterologous peptides in the Hl loop of their fiber knob** *GENE THERAPY*
  Mizuguchi, H., Koizumi, N., Hosono, T., Utoguchi, N., Watanabe, Y., Kay, M. A., Hayakawa, T.
  2001; 8 (9): 730-735

- **In vitro ligation-based cloning of foreign DNAs into the E3 and E1 deletion regions for generation of recombinant adenovirus vectors** *BIOTECHNIQUES*
  Mizuguchi, H., Kay, M. A., Hayakawa, T.
  2001; 30 (5): 1112-?

- **Linear DNAs concatemerize in vivo and result in sustained transgene expression in mouse liver** *MOLECULAR THERAPY*
  Chen, Z. Y., Yant, S. R., He, C. Y., Meuse, L., Shen, S., Kay, M. A.
  2001; 3 (3): 403-410

- **cMet activation allows persistent engraftment of ectopically transplanted xenogenic human hepatocytes in mice** *18th World Congress of the Transplantation-Society*
  Ohashi, K., Meuse, L., Schwall, R., Kay, M. A.
  ELSEVIER SCIENCE INC.2001: 587–88

- **Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics** *NATURE MEDICINE*
  Kay, M. A., Glorioso, J. C., Naldini, L.
  2001; 7 (1): 33-40

- **A phase I trial of AAV-mediated muscle directed gene transfer for hemophilia B.**
  Manno, C. S., Glader, B., Ragni, M. V., Thompson, A., Costa, F. F., Chew, A. J., Herzog, R. W., Arruda, V. R., Couto, L. B., Clelland, A. M., Johnson, F., Flake, A., Skarsgard, et al
  AMER SOC HEMATOLOGY.2000: 801A–801A

- **Persistent hF.IX expression in mouse hepatocytes from episomal rAAV circular intermediates does not rely on the presence of AAV-ITR but the structure of expression cassette itself.**
  Nakai, H., Fuess, S., Meuse, L., Storm, T., Kay, M. A.
  AMER SOC HEMATOLOGY.2000: 431A

- **A proposed rAAV-liver directed clinical trial for hemophilia B.**
  Nakai, H., Ohashi, K., Arruda, McClelland, A., Couto, L. B., Meuse, L., Storm, T., Dake, M. D., Manno, C. S., Glader, B., High, K. A., Kay, M. A.
  AMER SOC HEMATOLOGY.2000: 798A–799A

- **Recruitment of single-stranded recombinant adeno-associated virus vector genomes and intermolecular recombination are responsible for stable transduction of liver in vivo** *JOURNAL OF VIROLOGY*
  Nakai, H., Storm, T. A., Kay, M. A.
  2000; 74 (20): 9451-9463

- **Therapeutic levels of human factor VIII and IX using HIV-1-based lentiviral vectors in mouse liver** *BLOOD*
  Park, F., Ohashi, K., Kay, M. A.
  2000; 96 (3): 1173-1176

- **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** *MOLECULAR THERAPY*
  Miao, C. H., Ohashi, K., Patijn, G. A., Meuse, L., Ye, X., Thompson, A. R., Kay, M. A.
  2000; 1 (6): 522-532

- **Somatic integration and long-term transgene expression in normal and haemophilic mice using a DNA transposon system** *NATURE GENETICS*
  Yant, S. R., Meuse, L., Chiu, W., Ivics, Z., Izsvak, Z., Kay, M. A.
  2000; 25 (1): 35-41

- **Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors** *NATURE BIOTECHNOLOGY*

Nakai, H., Storm, T. A., Kay, M. A.
2000; 18 (5): 527-532

- **Nonrandom transduction of recombinant adeno-associated virus vectors in mouse hepatocytes in vivo: Cell cycling does not influence hepatocyte transduction** *JOURNAL OF VIROLOGY*
  Miao, C. H., Nakai, H., Thompson, A. R., Storm, T. A., Chiu, W., Snyder, R. O., Kay, M. A.
  2000; 74 (8): 3793-3803

- **Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector** *NATURE GENETICS*
  Kay, M. A., Manno, C. S., Ragni, M. V., Larson, P. J., Couto, L. B., McClelland, A., Glader, B., Chew, A. J., Tai, S. J., Herzog, R. W., Arruda, V., Johnson, F., Scallan, et al
  2000; 24 (3): 257-261

- **Sustained survival of human hepatocytes in mice: A model for in vivo infection with human hepatitis B and hepatitis delta viruses** *NATURE MEDICINE*
  Ohashi, K., Marion, P. L., Nakai, H., Meuse, L., Cullen, J. M., Bordier, B. B., Schwall, R., Greenberg, H. B., Glenn, J. S., Kay, M. A.
  2000; 6 (3): 327-331

- **Efficient lentiviral transduction of liver requires cell cycling in vivo** *NATURE GENETICS*
  Park, F., Ohashi, K., Chiu, W., Naldini, L., Kay, M. A.
  2000; 24 (1): 49-52

- **Nuclear import of moloney murine leukemia virus DNA mediated by adenovirus preterminal protein is not sufficient for efficient retroviral transduction in nondividing cells** *JOURNAL OF VIROLOGY*
  Lieber, A., Kay, M. A., Li, Z. Y.
  2000; 74 (2): 721-734

- **Commentary - IMAGe, a new clinical association of Intrauterine growth retardation, Mataphyseal dysplasia, Adrenal hypoplasia congenita, Genital anomalies** *JOURNAL OF CLINICAL ENDOCRINOLOGY & METABOLISM*
  Vilain, E., Le Merrer, M., Lecointre, C., Desangles, F., Kay, M. A., Maroteaux, P., McCabe, E. R.
  1999; 84 (12): 4335–40

- **A phase I trial of AAV-mediated muscle-directed gene therapy for hemophilia B.**
  Manno, C. S., Herzog, R. W., Arruda, V. R., Couto, L. B., Tai, S. J., McClelland, A., Flake, A. W., Chew, A. J., Fields, P. A., Armstrong, A. E., Leonard, D., Skarsgard, E. D., Glader, et al
  AMER SOC HEMATOLOGY.1999: 642A–642A

- **Optimization of factor IX gene expression in the liver of mice using cis-DNA elements.**
  Miao, C. H., Patijn, G. A., Meuse, L., Ohashi, K., Thompson, A. R., Kay, M. A.
  AMER SOC HEMATOLOGY.1999: 454A–455A

- **Integrating adenovirus-adeno-associated virus hybrid vectors devoid of all viral genes** *JOURNAL OF VIROLOGY*
  Lieber, A., Steinwaerder, D. S., Carlson, C. A., Kay, M. A.
  1999; 73 (11): 9314-9324

- **NF-kappa B activation is required for human endothelial survival during exposure to tumor necrosis factor-alpha but not to interleukin-1 beta or lipopolysaccharide** *JOURNAL OF BIOLOGICAL CHEMISTRY*
  Zen, K., Karsan, A., Stempien-Otero, A., Yee, E., Tupper, J., Li, X. W., Eunson, T., Kay, M. A., Wilson, C. B., Winn, R. K., Harlan, J. M.
  1999; 274 (40): 28808-28815

- **A simple method for constructing E1-and E1/E4-deleted recombinant adenoviral vectors** *HUMAN GENE THERAPY*
  Mizuguchi, H., Kay, M. A.
  1999; 10 (12): 2013-2017

- **Adeno-associated virus vectors and hematology** *BLOOD*
  Russell, D. W., Kay, M. A.
  1999; 94 (3): 864-?

- **Isolation of recombinant adeno-associated virus vector-cellular DNA junctions from mouse liver** *JOURNAL OF VIROLOGY*
  Nakai, H., Iwaki, Y., Kay, M. A., Couto, L. B.
  1999; 73 (7): 5438-5447

- **Implication of interfering antibody formation and apoptosis as two different mechanisms leading to variable duration of adenovirus-mediated transgene expression in immune-competent mice** *JOURNAL OF VIROLOGY*
  Schowalter, D. B., Himeda, C. L., Winther, B. L., Wilson, C. B., Kay, M. A.
  1999; 73 (6): 4755-4766

- **Mechanisms of hypoxia-induced endothelial cell death - Role of p53 in apoptosis** *JOURNAL OF BIOLOGICAL CHEMISTRY*
  Stempien-Otero, A., Karsan, A., Cornejo, C. J., Xiang, H., Eunson, T., Morrison, R. S., Kay, M., Winn, R., Harlan, J.
  1999; 274 (12): 8039-8045

- **Correction of hemophilia B in canine and murine models using recombinant adeno-associated viral vectors** *NATURE MEDICINE*
  Snyder, R. O., Miao, C., Meuse, L., Tubb, J., Donahue, B. A., Lin, H. F., Stafford, D. W., Patel, S., Thompson, A. R., Nichols, T., Read, M. S., Bellinger, D. A., Brinkhous, et al
  1999; 5 (1): 64-70

- **Hepatic gene therapy using adeno-associated virus vectors** *SEMINARS IN LIVER DISEASE*
  Patijn, G. A., Kay, M. A.
  1999; 19 (1): 61-69

- **Efficient construction of a recombinant adenovirus vector by an improved in vitro ligation method** *HUMAN GENE THERAPY*
  Mizuguchi, H., Kay, M. A.
  1998; 9 (17): 2577-2583

- **Inhibition of NF-kappa B activation in combination with Bcl-2 expression allows for persistence of first-generation adenovirus vectors in the mouse liver** *JOURNAL OF VIROLOGY*
  Lieber, A., He, C. Y., Meuse, L., Himeda, C., Wilson, C., Kay, M. A.
  1998; 72 (11): 9267-9277

- **Transient inhibition of CD28 and CD40 ligand interactions prolongs adenovirus-mediated transgene expression in the lung and facilitates expression after secondary vector administration** *JOURNAL OF VIROLOGY*
  Wilson, C. B., Embree, L. J., Schowalter, D., Albert, R., Aruffo, A., Hollenbaugh, D., Linsley, P., Kay, M. A.
  1998; 72 (9): 7542-7550

- **Method for continuous infusion into the portal vein of mice** *LABORATORY ANIMAL SCIENCE*
  Patijn, G. A., Terpstra, O. T., Kay, M. A.
  1998; 48 (4): 379-383

- **High-efficiency retrovirus-mediated gene transfer into the livers of mice** *HUMAN GENE THERAPY*
  Patijn, G. A., Lieber, A., Meuse, L., Winther, B., Kay, M. A.
  1998; 9 (10): 1449-1456

- **Adenoviral preterminal protein stabilizes mini-adenoviral genomes in vitro and in vivo** *NATURE BIOTECHNOLOGY*
  Lieber, A., He, C. Y., Kay, M. A.
  1997; 15 (13): 1383-1387

- **The role of Kupffer cell activation and viral gene expression in early liver toxicity after infusion of recombinant adenovirus vectors** *JOURNAL OF VIROLOGY*
  Lieber, A., He, C. Y., Meuse, L., Schowalter, D., Kirillova, I., Winther, B., Kay, M. A.
  1997; 71 (11): 8798-8807

- **Gene therapy: A status report** *PEDIATRIC ANNALS*
  Schowalter, D. B., Kay, M. A.
  1997; 26 (9): 562-568

- **Constitutive expression of murine CTLA4Ig from a recombinant adenovirus vector results in prolonged transgene expression** *GENE THERAPY*
  Schowalter, D. B., Meuse, L., Wilson, C. B., Linsley, P. S., Kay, M. A.
  1997; 4 (8): 853-860

- **Persistent and therapeutic concentrations of human factor IX in mice after hepatic gene transfer of recombinant AAV vectors** *NATURE GENETICS*
  Snyder, R. O., Miao, C. H., Patijn, G. A., Spratt, S. K., Danos, O., Nagy, D., GOWN, A. M., Winther, B., Meuse, L., Cohen, L. K., Thompson, A. R., Kay, M. A.
  1997; 16 (3): 270-276

- **Transient immunomodulation with anti-CD40 ligand antibody and CTLA4Ig enhances persistence and secondary adenovirus-mediated gene transfer into mouse liver** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
  Kay, M. A., Meuse, L., GOWN, A. M., Linsley, P., Hollenbaugh, D., Aruffo, A., Ochs, H. D., Wilson, C. B.
  1997; 94 (9): 4686-4691

- **Expansion of donor hepatocytes after recombinant adenovirus-induced liver regeneration in mice** *HEPATOLOGY*
  Peeters, M. J., Patijn, G. A., Lieber, A., Perkins, J., Kay, M. A.
  1997; 25 (4): 884-888

- **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 THERAPY*
  Schowalter, D. B., Tubb, J. C., Liu, M., Wilson, C. B., Kay, M. A.
  1997; 4 (4): 351-360

- **Adenovirus-mediated gene therapy in a mouse model of hereditary tyrosinemia type I** *HUMAN GENE THERAPY*
  Overturf, K., ALDHALIMY, M., Ou, C. N., Finegold, M., Tanguay, R., Lieber, A., Kay, M., Grompe, M.
  1997; 8 (5): 513-521

- **Liver regeneration: Prospects for therapy based on new technologies** *MOLECULAR MEDICINE TODAY*
  Kay, M. A., Fausto, N.
  1997; 3 (3): 108-115

- **Development of a high-performance liquid chromatographic assay for G418 sulfate (Geneticin)** *ANTIMICROBIAL AGENTS AND CHEMOTHERAPY*
  Bethune, C., Bui, T., Liu, M. L., Kay, M. A., Ho, R. J.
  1997; 41 (3): 661-664

- **Liver-associated toxicity of the HSV-tk/GCV approach and adenoviral vectors** *CANCER GENE THERAPY*
  Brand, K., Arnold, W., Bartels, T., Lieber, A., Kay, M. A., Strauss, M., Dorken, B.
  1997; 4 (1): 9-16

- **Methods for delivery of genes to hepatocytes in vivo using recombinant adenovirus vectors.** *Methods in molecular medicine*
  Barr, D., Kay, M. A.
  1997; 7: 205-212

- **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*
  Lieber, A., He, C. Y., Kirillova, I., Kay, M. A.
  1996; 70 (12): 8944-8960

- **Elimination of hepatitis C virus RNA in infected human hepatocytes by adenovirus-mediated expression of ribozymes** *JOURNAL OF VIROLOGY*
  Lieber, A., He, C. Y., Polyak, S. J., Gretch, D. R., Barr, D., Kay, M. A.
  1996; 70 (12): 8782-8791

- **HBV-derived promoters direct liver-specific expression of an adenovirally transduced LDL receptor gene** *GENE THERAPY*
  Sandig, V., Loser, P., Lieber, A., Kay, M. A., Strauss, M.
  1996; 3 (11): 1002-1009

- **Adenovirus-mediated hepatic gene transfer in mice: Comparison of intravascular and biliary administration** *HUMAN GENE THERAPY*
  Peeters, M. J., Patijn, G. A., Lieber, A., Meuse, L., Kay, M. A.
  1996; 7 (14): 1693-1699

- **erbB-2 knockout employing an intracellular single-chain antibody (sFv) accomplishes specific toxicity in erbB-2-expressing lung cancer cells** *AMERICAN JOURNAL OF RESPIRATORY CELL AND MOLECULAR BIOLOGY*
  Grim, J., Deshane, J., Feng, M. Z., Lieber, A., Kay, M., Curiel, D. T.
  1996; 15 (3): 348-354

- **Adenovirus-mediated expression of ribozymes in mice** *JOURNAL OF VIROLOGY*
  Lieber, A., Kay, M. A.
  1996; 70 (5): 3153-3158

- **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*
  Liu, M. L., Winther, B. L., Kay, M. A.
  1996; 70 (4): 2497-2502

- **Method for multiple portal vein infusions in mice: Quantitation of adenovirus-mediated hepatic gene transfer** *BIOTECHNIQUES*
  Peeters, M. J., Lieber, A., Perkins, J., Kay, M. A.
  1996; 20 (2): 278-?

- **LONG-TERM HEPATIC ADENOVIRUS-MEDIATED GENE-EXPRESSION IN MICE FOLLOWING CTLA4LG ADMINISTRATION** *NATURE GENETICS*
  Kay, M. A., Holterman, A. X., Meuse, L., Gown, A., Ochs, H. D., Linsley, P. S., Wilson, C. B.
  1995; 11 (2): 191-197

- **GENE-THERAPY FOR HEMOPHILIA-B - HOST IMMUNOSUPPRESSION PROLONGS THE THERAPEUTIC EFFECT OF ADENOVIRUS-MEDIATED FACTOR-IX EXPRESSION** *HUMAN GENE THERAPY*
  Fang, B., Eisensmith, R. C., Wang, H., Kay, M. A., Cross, R. E., Landen, C. N., Gordon, G., Bellinger, D. A., Read, M. S., Hu, P. C., Brinkhous, K. M., Woo, S. L.
  1995; 6 (8): 1039-1044

- **A MODIFIED UROKINASE PLASMINOGEN-ACTIVATOR INDUCES LIVER-REGENERATION WITHOUT BLEEDING** *HUMAN GENE THERAPY*
  Lieber, A., Peeters, M. J., Gown, A., Perkins, J., Kay, M. A.
  1995; 6 (8): 1029-1037

- **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*
  Lieber, A., Peeters, M. J., Meuse, L., Fausto, N., Perkins, J., Kay, M. A.
  1995; 92 (13): 6210-6214

- **STRAIN RELATED VARIATIONS IN ADENOVIRALLY MEDIATED TRANSGENE EXPRESSION FROM MOUSE HEPATOCYTES IN-VIVO - COMPARISONS BETWEEN IMMUNOCOMPETENT AND IMMUNODEFICIENT INBRED STRAINS** *GENE THERAPY*
  Barr, D., Tubb, J., Ferguson, D., Scaria, A., Lieber, A., Wilson, C., Perkins, J., Kay, M. A.
  1995; 2 (2): 151-155

- **THERAPEUTIC SERUM CONCENTRATIONS OF HUMAN ALPHA-1-ANTITRYPSIN AFTER ADENOVIRAL-MEDIATED GENE-TRANSFER INTO MOUSE HEPATOCYTES** *HEPATOLOGY*
  Kay, M. A., Graham, F., LELAND, F., Woo, S. L.
  1995; 21 (3): 815-819

- **ADENOVIRUS-MEDIATED TRANSFER OF THE AMPHOTROPIC RETROVIRUS RECEPTOR CDNA INCREASES RETROVIRAL TRANSDUCTION IN CULTURED-CELLS** *HUMAN GENE THERAPY*
  Lieber, A., Peeters, M. J., Kay, M. A.
  1995; 6 (1): 5-11

- **GENE-THERAPY FOR METABOLIC DISORDERS** *TRENDS IN GENETICS*
  Kay, M. A., Woo, S. L.
  1994; 10 (7): 253-257

- **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*
  Kay, M. A., Landen, C. N., ROTHENBERG, S. R., TAYLOR, L. A., LELAND, F., Wiehle, S., Fang, B. L., Bellinger, D., Finegold, M., Thompson, A. R., Read, M., Brinkhous, K. M., Woo, et al
  1994; 91 (6): 2353-2357

- **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*
  Cristiano, R. J., Smith, L. C., Kay, M. A., Brinkley, B. R., Woo, S. L.
  1993; 90 (24): 11548-11552

- **IN-VIVO GENE-THERAPY OF HEMOPHILIA-B - SUSTAINED PARTIAL CORRECTION IN FACTOR-IX-DEFICIENT DOGS** *SCIENCE*
  Kay, M. A., Rothenberg, S., Landen, C. N., Bellinger, D. A., LELAND, F., Toman, C., Finegold, M., Thompson, A. R., Read, M. S., Brinkhous, K. M., Woo, S. L.

1993; 262 (5130): 117-119

- **ASSESSMENT OF RECOMBINANT ADENOVIRAL VECTORS FOR HEPATIC GENE-THERAPY** *HUMAN GENE THERAPY*
  Li, Q. T., Kay, M. A., Finegold, M., STRATFORDPERRICAUDET, L. D., Woo, S. L.
  1993; 4 (4): 403-409
- **DEVELOPMENT OF A CLINICAL PROTOCOL FOR HEPATIC GENE-TRANSFER - LESSONS LEARNED IN PRECLINICAL STUDIES** *PEDIATRIC RESEARCH*
  Ledley, F. D., ADAMS, R. M., Soriano, H. E., Darlington, G., Finegold, M., Lanford, R., Carey, D., Lewis, D., BALEY, P. A., Rothenberg, S., Kay, M., Brandt, M., Moen, et al
  1993; 33 (4): 313-320
- **HEPATIC GENE-THERAPY - PERSISTENT EXPRESSION OF HUMAN ALPHA-1-ANTITRYPSIN IN MICE AFTER DIRECT GENE DELIVERY INVIVO** *HUMAN GENE THERAPY*
  Kay, M. A., Li, Q. T., Liu, T. J., LELAND, F., Toman, C., Finegold, M., Woo, S. L.
  1992; 3 (6): 641-647
- **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*
  Kay, M. A., BALEY, P., Rothenberg, S., LELAND, F., Fleming, L., Ponder, K. P., Liu, T. J., Finegold, M., Darlington, G., POKORNY, W., Woo, S. L.
  1992; 89 (1): 89-93
- **HUMAN GENE-THERAPY - PRESENT AND FUTURE** *BREAST CANCER RESEARCH AND TREATMENT*
  Kay, M. A., Ponder, K. P., Woo, S. L.
  1992; 21 (2): 83-93
- **RECONSTITUTION OF ENZYMATIC-ACTIVITY IN HEPATOCYTES OF PHENYLALANINE HYDROXYLASE-DEFICIENT MICE** *SOMATIC CELL AND MOLECULAR GENETICS*
  Liu, T. J., Kay, M. A., Darlington, G. J., Woo, S. L.
  1992; 18 (1): 89-96