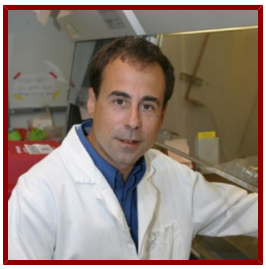
Stanford



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

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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

Yuqian Jiang, Ryan Setten, Guanhua Xun

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

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

Publications

PUBLICATIONS

- **Potential effects of endogenous RNA/DNA hybrids on CRISPR-Cas9-mediated homology-directed repair** *MOLECULAR THERAPY NUCLEIC ACIDS*
  Puzzo, F., Bayram, B., Macaubas, C., Lin, A., Jang, H., Zhang, F., Mellins, E., Kay, M. A.
  2026; 37 (2)

- **Potential effects of endogenous RNA/DNA hybrids on CRISPR-Cas9-mediated homology-directed repair.** *Molecular therapy. Nucleic acids*
  Puzzo, F., Bayram, B., Macaubas, C., Lin, A., Jang, H., Zhang, F., Mellins, E., Kay, M. A.
  2026; 37 (2): 102880
- **First-in-human nuclease-free homologous recombination-dependent gene editing in pediatric patients with methylmalonic acidemia: results of a phase 1/2 study.** *Gene therapy*
  Bedoyan, J. K., Morgan, T., Sun, A., Li, H., Gruskin, D., Payton, M., Chereau, F., Swenson, E. S., Lin, Q., Kay, M. A., Vockley, J.
  2026
- **Reprogramming AAV tropism with tumor-associated targeting ligands** *MOLECULAR THERAPY ONCOLOGY*
  Trisno, S. L., Kay, M. A.
  2025; 33 (4)
- **Reprogramming AAV tropism with tumor-associated targeting ligands.** *Molecular therapy. Oncology*
  Trisno, S. L., Kay, M. A.
  2025; 33 (4): 201103
- **Species-specific AAVR dominates species-tropism of adeno-associated virus (AAV) vectors.** *Research square*
  Kay, M., Jiang, Y., Liu, Y., Jing, Y., Zhang, F., Lin, A.
  2025
- **Integrated AAV optimization enables efficient gene delivery to kidney in murine and human tissue.** *Research square*
  Garcia, F. G., Moreno, M. R., Chetty, S., Johnson, N., An, A., Sostena, J., Thompson, L., Wang, J., Charu, V., Lee, J., Meyer, N., Stoller, M., Yang, et al
  2025
- **Stereo-random oligonucleotides enable efficient recruitment of ADAR in vitro and in vivo.** *Nature communications*
  Pfeiffer, L. S., Merkle, T., Vogel, P., Jarmoskaite, I., Geisinger, J. M., Latifi, N., Herrera-Barrera, M., Zhang, F., Groß, L., Schlitz, C., Hofacker, D. T., Lochmann, C., Fumagalli, et al
  2025; 16 (1): 8849
- **NEAT1 lncRNA Structure-Mediated Functional Modulation as a Novel Therapeutic Approach for MYC-Driven Cancers.**
  Jang, H., Kay, M., Suh, B., Zhang, F.
  CELL PRESS.2025
- **Conjugation of Peptide Ligands to AAV Towards Pancreatic Cell AAV Gene Delivery**
  Trisno, S., Kay, M.
  CELL PRESS.2025
- **Tales from the R-loops: how RNA/DNA hybrids may influence CRISPR/Cas9-mediated homologous recombination gene editing outcomes**
  Puzzo, F., Jang, H., Zhang, F., Kay, M.
  CELL PRESS.2025
- **Epigenetic Blueprint: Understanding how the AAV Capsid influences the Vector Epigenome**
  Vamva, E., Jiang, Y., Pekrun, K., Gonzalez-Sandoval, A., Kay, M.
  CELL PRESS.2025
- **The deLIVERed promises of gene therapy: past, present and future of liver-directed gene therapy.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Puzzo, F., Kay, M. A.
  2025
- **Development of a novel gene editing lexicon for hemophilia: methodology and results** *RESEARCH AND PRACTICE IN THROMBOSIS AND HAEMOSTASIS*
  Kessler, C. M., Valentino, L. A., Thornburg, C. D., Unzu, C., Kay, M. A., Peyvandi, F., Smith, P., Miesbach, W., Mckeown, W., Pierce, G. F., Khair, K., Starcevic, K., Pillai, et al
  2025; 9 (2)
- **Development of a novel gene editing lexicon for hemophilia: methodology and results.** *Research and practice in thrombosis and haemostasis*

Kessler, C. M., Valentino, L. A., Thornburg, C. D., Unzu, C., Kay, M. A., Peyvandi, F., Smith, P., Miesbach, W., McKeown, W., Pierce, G. F., Khair, K., Starcevic, K., Pillai, et al
2025; 9 (2): 102710

- **Operationalising a Haemophilia Gene Editing Lexicon for Practical Use.** *Haemophilia : the official journal of the World Federation of Hemophilia*
  McKeown, W., Hermans, C., Unzu, C., Kay, M. A., Peyvandi, F., Smith, P., Miesbach, W., Pierce, G. F., Khair, K., Valentino, L. A., Pipe, S. W., Pillai, M., Jones, et al
  2025
- **Functional analysis of tRNA-derived small translational regulation.** *Methods in enzymology*
  Kim, D., Kim, H. K., Kay, M. A.
  2025; 711: 336-355
- **Ruvkun and Ambros recognized for miRNAs.** *Molecular therapy. Nucleic acids*
  Kay, M. A.
  2024; 35 (4): 102379
- **Ruvkun and Ambros recognized for miRNAs** *MOLECULAR THERAPY NUCLEIC ACIDS*
  Kay, M. A.
  2024; 35 (4)
- **A novel gene editing lexicon strategy for the haemophilia community: Research plan for development and preliminary results.** *Haemophilia : the official journal of the World Federation of Hemophilia*
  Hermans, C., Valentino, L. A., Thornburg, C. D., Unzu, C., Kay, M. A., Peyvandi, F., Smith, P., Miesbach, W., McKeown, W., Pierce, G. F., Khair, K., Pipe, S. W., Starcevic, et al
  2024
- **AAV-mediated genome editing is influenced by the formation of R-loops.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Puzzo, F., Crossley, M. P., Goswami, A., Zhang, F., Pekrun, K., Garzon, J. L., Cimprich, K. A., Kay, M. A.
  2024
- **AAV-mediated genome editing is influenced by the formation of R-loops.** *bioRxiv : the preprint server for biology*
  Puzzo, F., Crossley, M. P., Goswami, A., Zhang, F., Pekrun, K., Garzon, J. L., Cimprich, K. A., Kay, M. A.
  2024
- **Correlation of antigen expression with epigenetic modifications after rAAV delivery of a hyperactive human Factor IX variant in mice and rhesus macaques.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Pekrun, K., Stephens, C. J., Gonzalez-Sandoval, A., Goswami, A., Zhang, F., Tarantal, A. F., Blouse, G., Kay, M. A.
  2024
- **Correlation of Antigen Expression with Epigenetic Modifications After rAAV Delivery of a Hyperactive Human Factor IX Variant in Mice and Rhesus Macaques**
  Pekrun, K., Stephens, C., Gonzalez-Sandoval, A., Goswami, A., Zhang, F., Tarantal, A. F., Blouse, G., Kay, M. A.
  CELL PRESS.2024: 111
- **Unwanted Concatemeric Knock-Ins Occur Frequently with Cas9/AAV-Mediated Gene-Editing: Detection and Prevention**
  Suchy, F. P., Karigane, D., Nakauchi, Y., Higuchi, M., Zhang, J., Pekrun, K., Hsu, I., Fan, A. C., Nishimura, T., Charlesworth, C. T., Bhadury, J., Nishimura, T., Wilkinson, et al
  CELL PRESS.2024: 211-212
- **The Efficacy of AAV-Mediated Genome Editing, and Undesired Vector Integrations Are Influenced by R-Loops Formation**
  Puzzo, F., Crossley, M., Goswami, A., Pekrun, K., Zhang, F., Cimprich, K., Kay, M. A.
  CELL PRESS.2024: 574
- **Screening for Cellular Factors Underlying Mechanisms of AAV Tropism**
  Jang, H., Du, Q., Kay, M. A.
  CELL PRESS.2024: 37-38
- **Epigenetic Blueprint: Understanding How the AAV Capsid Influences the Vector Epigenome**
  Vamva, E., Pekrun, K., Jiang, Y., Gonzalez, A., Gu, N., Dean, D. A., Kay, M. A.

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- **Genome engineering with Cas9 and AAV repair templates generates frequent concatemeric insertions of viral vectors.** *Nature biotechnology*
  Suchy, F. P., Karigane, D., Nakauchi, Y., Higuchi, M., Zhang, J., Pekrun, K., Hsu, I., Fan, A. C., Nishimura, T., Charlesworth, C. T., Bhadury, J., Nishimura, T., Wilkinson, et al
  2024

- **Selection of viral capsids and promoters affects the efficacy of rescue of Tmprss3-deficient cochlea.** *Molecular therapy. Methods & clinical development*
  Aaron, K. A., Pekrun, K., Atkinson, P. J., Billings, S. E., Abitbol, J. M., Lee, I. A., Eltawil, Y., Chen, Y. S., Dong, W., Nelson, R. F., Kay, M. A., Cheng, A. G.
  2023; 30: 413-428

- **Generation of DNA Aptamers Against Pancreatic Surface Receptors Towards AAV Cell-Specific Targeting**
  Trisno, S. L., Puzzo, F., Kay, M. A.
  CELL PRESS.2023: 228

- **Should You Judge an AAV by Its Cover? The Role the AAV Capsid Plays in Setting up the Vector Epigenome**
  Vamva, E., Gonzalez-Sandoval, A., Kay, M. A.
  CELL PRESS.2023: 138

- **The Role of DNA/RNA Hybrids on the Efficiency of Nuclease-Free Genome Editing and AAV Integration In Vivo**
  Puzzo, F., Crossley, M. P., Pekrun, K., Goswami, A., Zhang, F., Cimprich, K., Kay, M. A.
  CELL PRESS.2023: 518

- **Promoterless AAV Gene Targeting Vectors Favor Integration into the Albumin Locus Regardless of Homology Arm Sequences**
  Pekrun, K., Stephens, C., Zhang, F., Jang, H., Puzzo, F., Liehr, P., Goswami, A., Kay, M. A.
  CELL PRESS.2023: 738-739

- **Evaluation of Transcytosis Receptor Expression Between Human Brain Endothelial Cells In Vitro and In Vivo with Respect to Adeno-Associated Virus Capsid Selection**
  Rubin, J. D., Goswami, A., Kay, M. A.
  CELL PRESS.2023: 752

- **The AAV capsid can influence the epigenetic marking of rAAV delivered episomal genomes in a species dependent manner.** *Nature communications*
  Gonzalez-Sandoval, A., Pekrun, K., Tsuji, S., Zhang, F., Hung, K. L., Chang, H. Y., Kay, M. A.
  2023; 14 (1): 2448

- **A strategy for high antibody expression with low anti-drug antibodies using AAV9 vectors.** *Frontiers in immunology*
  Davis-Gardner, M. E., Weber, J. A., Xie, J., Pekrun, K., Alexander, E. A., Weisgrau, K. L., Furlott, J. R., Rakasz, E. G., Kay, M. A., Gao, G., Farzan, M., Gardner, M. R.
  2023; 14: 1105617

- **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

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- **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.
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- **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.
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- **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
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- **An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging** *NATURE CHEMICAL BIOLOGY*
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