


## 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

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#### 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

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### 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

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### 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

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### 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., Shekaly, 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., Konkright, 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., Valdmans, 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., Wosczyzna, 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 Luminal 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., Wosczyzna, 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., Wosczyzna, 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.  
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