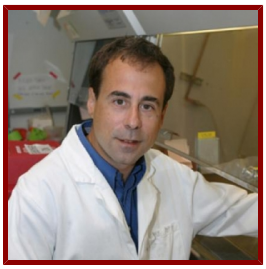
Stanford



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

Dennis Farrey Family Professor in 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, 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

Adriana Gonzalez Sandoval, Hagoon Jang, Yuqing Jing, Francesco Puzzo, Ren Song, Calvin Stephens

Doctoral Dissertation Advisor (AC)

Laura Spector

Postdoctoral Research Mentor

Adriana Gonzalez Sandoval

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

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

Publications

PUBLICATIONS

- **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., Porro, F., Bortolussi, G., Sola, R., Lisjak, M., Barzel, A., Giacca, M., Kay, M. A., Vlahovi#ek, K., Zentilin, L., 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

- **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.
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- **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
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- **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

- **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.
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- **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.
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- **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.
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- **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.
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- **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
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- **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., Connolly, A. J., Petrov, D. A., Kay, M. A., Winslow, M. M.
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- **A transfer-RNA-derived small RNA regulates ribosome biogenesis.** *Nature*
  Kim, H. K., Fuchs, G., Wang, S., Wei, W., Zhang, Y., Park, H., Roy-Chaudhuri, B., Li, P., Xu, J., Chu, K., Zhang, F., Chua, M. S., So, et al
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- **Dieter C. Gruenert, PhD (1949-2016) In Memoriam** *NUCLEIC ACID THERAPEUTICS*
  Disterer, P., Kay, M. A., Parker, G. C.
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- **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
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- **A universal system to select gene-modified hepatocytes in vivo** *SCIENCE TRANSLATIONAL MEDICINE*
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  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.
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- **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.
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- **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.
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- **Selecting the Best AAV Capsid for Human Studies** *MOLECULAR THERAPY*
  Kay, M. A.
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- **Viral Vectors Take On HIV Infection** *NEW ENGLAND JOURNAL OF MEDICINE*
  Mellins, E. D., Kay, M. A.
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- **NEW RAAV VECTORS AND APPROACHES FOR EPISOMAL AND INTEGRATION BASED GENE TRANSFER**
  Kay, M. A.
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- **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.
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- **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.
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- **microRNA Inhibition Through Gapmer Activated RNase H-Mediated Degradation**
  Munding, E. M., Kay, M. A.
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- **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.
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  Haussecker, D., Kay, M. A.
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- **Translational Data from Adeno-Associated Virus-Mediated Gene Therapy of Hemophilia B in Dogs** *HUMAN GENE THERAPY CLINICAL DEVELOPMENT*
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- **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.
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