Mark A Kay

List of Publications by Year
in descending order

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241 papers	36,399 citations	3531 90 h-index	3106 187 g-index
251 all docs	251 docs citations	251 times ranked	27790 citing authors

#	Article	IF	Citations
1	Progress and problems with the use of viral vectors for gene therapy. Nature Reviews Genetics, 2003, 4, 346-358.	16.3	2,213
2	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. Nature Medicine, 2006, 12, 342-347.	30.7	1,865
3	Adenovirus-Associated Virus Vectorâ€“Mediated Gene Transfer in Hemophilia B. New England Journal of Medicine, 2011, 365, 2357-2365.	27.0	1,606
4	Fatality in mice due to oversaturation of cellular microRNA/short hairpin RNA pathways. Nature, 2006, 441, 537-541.	27.8	1,518
5	Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics. Nature Medicine, 2001, 7, 33-40.	30.7	1,205
6	Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B. New England Journal of Medicine, 2014, 371, 1994-2004.	27.0	1,063
7	RNA interference in adult mice. Nature, 2002, 418, 38-39.	27.8	1,043
8	Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector. Nature Genetics, 2000, 24, 257-261.	21.4	971
9	Robust expansion of human hepatocytes in Fahâ^'/â^'/Rag2â^'/â^'/Il2rgâ^'/â^' mice. Nature Biotechnology, 2007, 25, 903-910.	17.5	729
10	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. Blood, 2003, 101, 2963-2972.	1.4	707
11	A nonviral minicircle vector for deriving human iPS cells. Nature Methods, 2010, 7, 197-199.	19.0	658
12	Sarcoma Derived from Cultured Mesenchymal Stem Cells. Stem Cells, 2007, 25, 371-379.	3.2	601
13	Inhibition of hepatitis B virus in mice by RNA interference. Nature Biotechnology, 2003, 21, 639-644.	17.5	595
14	Persistent and therapeutic concentrations of human factor IX in mice after hepatic gene transfer of recombinant AAV vectors. Nature Genetics, 1997, 16, 270-276.	21.4	589
15	State-of-the-art gene-based therapies: the road ahead. Nature Reviews Genetics, 2011, 12, 316-328.	16.3	587
16	Human tRNA-derived small RNAs in the global regulation of RNA silencing. Rna, 2010, 16, 673-695.	3.5	583
17	In Vitro and In Vivo Gene Therapy Vector Evolution via Multispecies Interbreeding and Retargeting of Adeno-Associated Viruses. Journal of Virology, 2008, 82, 5887-5911.	3.4	546
18	Robust systemic transduction with AAV9 vectors in mice: efficient global cardiac gene transfer superior to that of AAV8. Molecular Therapy, 2006, 14, 45-53.	8.2	527

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19	Somatic integration and long-term transgene expression in normal and haemophilic mice using a DNA transposon system. Nature Genetics, 2000, 25, 35-41.	21.4	491
20	In vivo activity of nuclease-resistant siRNAs. Rna, 2004, 10, 766-771.	3.5	483
21	Minicircle DNA vectors devoid of bacterial DNA result in persistent and high-level transgene expression in vivo. Molecular Therapy, 2003, 8, 495-500.	8.2	435
22	Extrachromosomal Recombinant Adeno-Associated Virus Vector Genomes Are Primarily Responsible for Stable Liver Transduction In Vivo. Journal of Virology, 2001, 75, 6969-6976.	3.4	417
23	Correction of hemophilia B in canine and murine models using recombinant adeno-associated viral vectors. Nature Medicine, 1999, 5, 64-70.	30.7	414
24	Biological basis for restriction of microRNA targets to the 3â€² untranslated region in mammalian mRNAs. Nature Structural and Molecular Biology, 2009, 16, 144-150.	8.2	383
25	Fate tracing of mature hepatocytes in mouse liver homeostasis and regeneration. Journal of Clinical Investigation, 2011, 121, 4850-4860.	8.2	376
26	Selection and evaluation of clinically relevant AAV variants in a xenograft liver model. Nature, 2014, 506, 382-386.	27.8	376
27	A transfer-RNA-derived small RNA regulates ribosome biogenesis. Nature, 2017, 552, 57-62.	27.8	366
28	AAV serotype 2 vectors preferentially integrate into active genes in mice. Nature Genetics, 2003, 34, 297-302.	21.4	359
29	The 37/67-Kilodalton Laminin Receptor Is a Receptor for Adeno-Associated Virus Serotypes 8, 2, 3, and 9. Journal of Virology, 2006, 80, 9831-9836.	3.4	356
30	Rapid Uncoating of Vector Genomes Is the Key toEfficient Liver Transduction with Pseudotyped Adeno-Associated VirusVectors. Journal of Virology, 2004, 78, 3110-3122.	3.4	333
31	Efficient Construction of a Recombinant Adenovirus Vector by an ImprovedIn VitroLigation Method. Human Gene Therapy, 1998, 9, 2577-2583.	2.7	329
32	Assessment of Recombinant Adenoviral Vectors for Hepatic Gene Therapy. Human Gene Therapy, 1993, 4, 403-409.	2.7	327
33	In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs. Science, 1993, 262, 117-119.	12.6	315
34	Helper virus-free, optically controllable, and two-plasmid-based production of adeno-associated virus vectors of serotypes 1 to 6. Molecular Therapy, 2003, 7, 839-850.	8.2	311
35	Unrestricted Hepatocyte Transduction with Adeno-Associated Virus Serotype 8 Vectors in Mice. Journal of Virology, 2005, 79, 214-224.	3.4	299
36	Longâ€“term hepatic adenovirusâ€“mediated gene expression in mice following CTLA4Ig administration. Nature Genetics, 1995, 11, 191-197.	21.4	298

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37	High-Resolution Genome-Wide Mapping of Transposon Integration in Mammals. Molecular and Cellular Biology, 2005, 25, 2085-2094.	2.3	298
38	A robust system for production of minicircle DNA vectors. Nature Biotechnology, 2010, 28, 1287-1289.	17.5	288
39	Efficient lentiviral transduction of liver requires cell cycling in vivo. Nature Genetics, 2000, 24, 49-52.	21.4	278
40	The Loop Position of shRNAs and Pre-miRNAs Is Critical for the Accuracy of Dicer Processing InÂVivo. Cell, 2012, 151, 900-911.	28.9	266
41	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, 2000, 1, 522-532.	8.2	230
42	A bright cyan-excitable orange fluorescent protein facilitates dual-emission microscopy and enhances bioluminescence imaging in vivo. Nature Biotechnology, 2016, 34, 760-767.	17.5	221
43	Preclinical in vivo evaluation of pseudotyped adeno-associated virus vectors for liver gene therapy. Blood, 2003, 102, 2412-2419.	1.4	196
44	Human COL7A1 -corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa. Science Translational Medicine, 2014, 6, 264ra163.	12.4	194
45	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, 2007, 104, 14771-14776.	7.1	193
46	Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors. Nature Biotechnology, 2000, 18, 527-532.	17.5	191
47	Transposition from a gutless adeno-transposon vector stabilizes transgene expression in vivo. Nature Biotechnology, 2002, 20, 999-1005.	17.5	184
48	Expression of shRNA From a Tissue-specific pol II Promoter Is an Effective and Safe RNAi Therapeutic. Molecular Therapy, 2008, 16, 1630-1636.	8.2	183
49	Hepatic Gene Therapy: Persistent Expression of Human Î±1-Antitrypsin in Mice after Direct Gene Delivery In Vivo. Human Gene Therapy, 1992, 3, 641-647.	2.7	182
50	The kinetics of rAAV integration in the liver. Nature Genetics, 1998, 19, 13-15.	21.4	181
51	Linear DNAs Concatemerize in Vivo and Result in Sustained Transgene Expression in Mouse Liver. Molecular Therapy, 2001, 3, 403-410.	8.2	179
52	Isolation of Recombinant Adeno-Associated Virus Vector-Cellular DNA Junctions from Mouse Liver. Journal of Virology, 1999, 73, 5438-5447.	3.4	178
53	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, 2000, 74, 9451-9463.	3.4	174
54	Sustained survival of human hepatocytes in mice: A model for in vivo infection with human hepatitis B and hepatitis delta viruses. Nature Medicine, 2000, 6, 327-331.	30.7	172

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55	Improved Production and Purification of Minicircle DNA Vector Free of Plasmid Bacterial Sequences and Capable of Persistent Transgene ExpressionIn Vivo. Human Gene Therapy, 2005, 16, 126-131.	2.7	168
56	Large-Scale Molecular Characterization of Adeno-Associated Virus Vector Integration in Mouse Liver. Journal of Virology, 2005, 79, 3606-3614.	3.4	164
57	FATP2 is a hepatic fatty acid transporter and peroxisomal very long-chain acyl-CoA synthetase. American Journal of Physiology - Endocrinology and Metabolism, 2010, 299, E384-E393.	3.5	161
58	Argonaute proteins are key determinants of RNAi efficacy, toxicity, and persistence in the adult mouse liver. Journal of Clinical Investigation, 2010, 120, 3106-3119.	8.2	161
59	Lack of Germline Transmission of Vector Sequences Following Systemic Administration of Recombinant AAV-2 Vector in Males. Molecular Therapy, 2001, 4, 586-592.	8.2	152
60	Genome Editing of Isogenic Human Induced Pluripotent Stem Cells Recapitulates Long QT Phenotype for Drug Testing. Journal of the American College of Cardiology, 2014, 64, 451-459.	2.8	149
61	Therapeutic levels of human factor VIII and IX using HIV-1â€“based lentiviral vectors in mouse liver. Blood, 2000, 96, 1173-1176.	1.4	144
62	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, 2004, 24, 9239-9247.	2.3	142
63	Silencing of Episomal Transgene Expression in Liver by Plasmid Bacterial Backbone DNA Is Independent of CpG Methylation. Molecular Therapy, 2008, 16, 548-556.	8.2	141
64	How do miRNAs mediate translational repression?. Silence: A Journal of RNA Regulation, 2010, 1, 11.	8.1	140
65	Helper-Independent sleeping beauty Transposonâ€“Transposase vectors for efficient nonviral gene delivery and persistent gene expression in vivo. Molecular Therapy, 2003, 8, 654-665.	8.2	138
66	In Vivo Correction of Murine Tyrosinemia Type I by DNA-Mediated Transposition. Molecular Therapy, 2002, 6, 759-769.	8.2	137
67	A new adenoviral helperâ€“dependent vector results in long-term therapeutic levels of human coagulation factor IX at low doses in vivo. Blood, 2002, 99, 3923-3930.	1.4	133
68	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, 2008, 283, 22186-22192.	3.4	133
69	Therapeutic application of RNAi: is mRNA targeting finally ready for prime time?. Journal of Clinical Investigation, 2007, 117, 3633-3641.	8.2	132
70	Bioengineered AAV Capsids with Combined High Human Liver Transduction InÂVivo and Unique Humoral Seroreactivity. Molecular Therapy, 2018, 26, 289-303.	8.2	130
71	Site-directed transposon integration in human cells. Nucleic Acids Research, 2007, 35, e50-e50.	14.5	129
72	Nonrandom Transduction of Recombinant Adeno-Associated Virus Vectors in Mouse Hepatocytes In Vivo: Cell Cycling Does Not Influence Hepatocyte Transduction. Journal of Virology, 2000, 74, 3793-3803.	3.4	124

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73	Histone Modifications are Associated with the Persistence or Silencing of Vector-mediated Transgene Expression In Vivo. Molecular Therapy, 2007, 15, 1348-1355.	8.2	123
74	Novel NanoLuc substrates enable bright two-population bioluminescence imaging in animals. Nature Methods, 2020, 17, 852-860.	19.0	123
75	Hepatocyte transplantation: clinical and experimental application. Journal of Molecular Medicine, 2001, 79, 617-630.	3.9	122
76	Adeno-associated virus gene repair corrects a mouse model of hereditary tyrosinemia in vivo. Hepatology, 2010, 51, 1200-1208.	7.3	121
77	Liver tissue engineering at extrahepatic sites in mice as a potential new therapy for genetic liver diseases. Hepatology, 2005, 41, 132-140.	7.3	120
78	Hepatocyte growth factor induces hepatocyte proliferationin vivo and allows for efficient retroviral-mediated gene transfer in mice. Hepatology, 1998, 28, 707-716.	7.3	112
79	Adenovirus Transduction is Required for the Correction of Diabetes Using Pdx-1 or Neurogenin-3 in the Liver. Molecular Therapy, 2007, 15, 255-263.	8.2	111
80	Recombinant AAV as a Platform for Translating the Therapeutic Potential of RNA Interference. Molecular Therapy, 2014, 22, 692-701.	8.2	111
81	Method for Multiple Portal Vein Infusions in Mice: Quantitation of Adenovirus-Mediated Hepatic Gene Transfer. BioTechniques, 1996, 20, 278-285.	1.8	104
82	Liver Transduction with Recombinant Adeno-Associated Virus Is Primarily Restricted by Capsid Serotype Not Vector Genotype. Journal of Virology, 2006, 80, 426-439.	3.4	104
83	Minicircle DNA Vectors Achieve Sustained Expression Reflected by Active Chromatin and Transcriptional Level. Molecular Therapy, 2013, 21, 131-138.	8.2	103
84	Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid Î±-glucosidase. Science Translational Medicine, 2017, 9, .	12.4	103
85	Genome Editing of Human Embryonic Stem Cells and Induced Pluripotent Stem Cells With Zinc Finger Nucleases for Cellular Imaging. Circulation Research, 2012, 111, 1494-1503.	4.5	99
86	Modified HIV-1 Based Lentiviral Vectors Have an Effect on Viral Transduction Efficiency and Gene Expression in Vitro and in Vivo. Molecular Therapy, 2001, 4, 164-173.	8.2	98
87	Adeno-Associated Virus Vectors and Hematology. Blood, 1999, 94, 864-874.	1.4	97
88	Therapeutic serum concentrations of human alpha-1-antitrypsin after adenoviral-mediated gene transfer into mouse hepatocytes. Hepatology, 1995, 21, 815-819.	7.3	94
89	The Host Response to Adenovirus, Helper-dependent Adenovirus, and Adeno-associated Virus in Mouse Liver. Molecular Therapy, 2008, 16, 931-941.	8.2	93
90	Integrating Adenovirusâ€“Adeno-Associated Virus Hybrid Vectors Devoid of All Viral Genes. Journal of Virology, 1999, 73, 9314-9324.	3.4	92

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91	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, 2002, 76, 11343-11349.	3.4	92
92	Novel Minicircle Vector for Gene Therapy in Murine Myocardial Infarction. Circulation, 2009, 120, S230-7.	1.6	91
93	Minicircle DNA-based Gene Therapy Coupled With Immune Modulation Permits Long-term Expression of Î±-L-Iduronidase in Mice With Mucopolysaccharidosis Type I. Molecular Therapy, 2011, 19, 450-460.	8.2	86
94	Determinants of Hepatitis C Translational Initiation in Vitro, in Cultured Cells and Mice. Molecular Therapy, 2002, 5, 676-684.	8.2	85
95	Episomal Persistence of Recombinant Adenoviral Vector Genomes during the Cell Cycle In Vivo. Journal of Virology, 2003, 77, 7689-7695.	3.4	84
96	Nonhomologous-End-Joining Factors Regulate DNA Repair Fidelity during Sleeping Beauty Element Transposition in Mammalian Cells. Molecular and Cellular Biology, 2003, 23, 8505-8518.	2.3	79
97	A potent and specific morpholino antisense inhibitor of hepatitis C translation in mice. Hepatology, 2003, 38, 503-508.	7.3	78
98	Multiplexed in vivo homology-directed repair and tumor barcoding enables parallel quantification of Kras variant oncogenicity. Nature Communications, 2017, 8, 2053.	12.8	78
99	A gene-deleted adenoviral vector results in phenotypic correction of canine hemophilia B without liver toxicity or thrombocytopenia. Blood, 2003, 102, 2403-2411.	1.4	76
100	A direct comparison of two nonviral gene therapy vectors for somatic integration: in vivo evaluation of the bacteriophage integrase Ï•C31 and the Sleeping Beauty transposase. Molecular Therapy, 2005, 11, 695-706.	8.2	75
101	Host factors that impact the biodistribution and persistence of multipotent adult progenitor cells. Blood, 2006, 107, 4182-4188.	1.4	75
102	RNAi and Gene Therapy: A Mutual Attraction. Hematology American Society of Hematology Education Program, 2007, 2007, 473-481.	2.5	75
103	Hyperactive Sleeping Beauty Transposase Enables Persistent Phenotypic Correction in Mice and a Canine Model for Hemophilia B. Molecular Therapy, 2010, 18, 1896-1906.	8.2	75
104	Drugging RNAi. Science, 2015, 347, 1069-1070.	12.6	74
105	Capped small RNAs and MOV10 in human hepatitis delta virus replication. Nature Structural and Molecular Biology, 2008, 15, 714-721.	8.2	72
106	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, 2011, 108, 9208-9213.	7.1	71
107	A rapid protocol for construction and production of high-capacity adenoviral vectors. Nature Protocols, 2009, 4, 547-564.	12.0	66
108	Evaluating the state of the science for adeno-associated virus integration: An integrated perspective. Molecular Therapy, 2022, 30, 2646-2663.	8.2	65

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109	Using a barcoded AAV capsid library to select for clinically relevant gene therapy vectors. JCI Insight, 2019, 4, .	5.0	64
110	Survival Advantage of Both Human Hepatocyte Xenografts and Genome-Edited Hepatocytes for Treatment of Î±-1 Antitrypsin Deficiency. Molecular Therapy, 2017, 25, 2477-2489.	8.2	62
111	The Role of DNA-PKcs and Artemis in Opening Viral DNA Hairpin Termini in Various Tissues in Mice. Journal of Virology, 2007, 81, 11304-11321.	3.4	61
112	Radioprotection In Vitro and In Vivo by Minicircle Plasmid Carrying the Human Manganese Superoxide Dismutase Transgene. Human Gene Therapy, 2008, 19, 820-826.	2.7	60
113	Somatic Correction of Junctional Epidermolysis Bullosa by a Highly Recombinogenic AAV Variant. Molecular Therapy, 2014, 22, 725-733.	8.2	60
114	Looking into the safety of AAV vectors. Nature, 2003, 424, 251-251.	27.8	58
115	Transfer RNA-Derived Small RNAs: Another Layer of Gene Regulation and Novel Targets for Disease Therapeutics. Molecular Therapy, 2020, 28, 2340-2357.	8.2	57
116	The Extragenic Spacer Length Between the 5â€² and 3â€² Ends of the Transgene Expression Cassette Affects Transgene Silencing From Plasmid-based Vectors. Molecular Therapy, 2012, 20, 2111-2119.	8.2	55
117	A tRNA-Derived Small RNA Regulates Ribosomal Protein S28 Protein Levels after Translation Initiation in Humans and Mice. Cell Reports, 2019, 29, 3816-3824.e4.	6.4	52
118	A Mini-intronic Plasmid (MIP): A Novel Robust Transgene Expression Vector In Vivo and In Vitro. Molecular Therapy, 2013, 21, 954-963.	8.2	51
119	Novel codon-optimized mini-intronic plasmid for efficient, inexpensive and xeno-free induction of pluripotency. Scientific Reports, 2015, 5, 8081.	3.3	51
120	A Modified Urokinase Plasminogen Activator Induces Liver Regeneration Without Bleeding. Human Gene Therapy, 1995, 6, 1029-1037.	2.7	49
121	Role of Hepatocyte Direct Hyperplasia in Lentivirus-Mediated Liver TransductionIn Vivo. Human Gene Therapy, 2002, 13, 653-663.	2.7	49
122	Helper-independent and AAV-ITR-independent chromosomal integration of double-stranded linear DNA vectors in mice. Molecular Therapy, 2003, 7, 101-111.	8.2	48
123	Increased Maintenance and Persistence of Transgenes by Excision of Expression Cassettes from Plasmid SequencesIn Vivo. Human Gene Therapy, 2005, 16, 558-570.	2.7	48
124	DNA Palindromes with a Modest Arm Length of â‰³20 Base Pairs Are a Significant Target for Recombinant Adeno-Associated Virus Vector Integration in the Liver, Muscles, and Heart in Mice. Journal of Virology, 2007, 81, 11290-11303.	3.4	48
125	miR-122 removal in the liver activates imprinted microRNAs and enables more effective microRNA-mediated gene repression. Nature Communications, 2018, 9, 5321.	12.8	48
126	Promoterless gene targeting without nucleases rescues lethality of a Criglerâ€Najjar syndrome mouse model. EMBO Molecular Medicine, 2017, 9, 1346-1355.	6.9	46

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127	Adenoviral preterminal protein stabilizes mini-adenoviral genomes in vitro and in vivo. Nature Biotechnology, 1997, 15, 1383-1387.	17.5	43
128	Combined proteomicâ€“RNAi screen for host factors involved in human hepatitis delta virus replication. Rna, 2009, 15, 1971-1979.	3.5	43
129	The effect of age on hepatic gene transfer with self-inactivating lentiviral vectors in vivo. Molecular Therapy, 2003, 8, 314-323.	8.2	42
130	Somatic Integration From an Adenoviral Hybrid Vector into a Hot Spot in Mouse Liver Results in Persistent Transgene Expression Levels In Vivo. Molecular Therapy, 2007, 15, 146-156.	8.2	41
131	Future of rAAV Gene Therapy: Platform for RNAi, Gene Editing, and Beyond. Human Gene Therapy, 2017, 28, 361-372.	2.7	40
132	Cis-Acting Gene Regulatory Activities in the Terminal Regions of Sleeping Beauty DNA Transposon-Based Vectors. Human Gene Therapy, 2007, 18, 1193-1204.	2.7	39
133	Low-level shRNA Cytotoxicity Can Contribute to MYC-induced Hepatocellular Carcinoma in Adult Mice. Molecular Therapy, 2010, 18, 161-170.	8.2	39
134	Evolution of a Human-Specific Tandem Repeat Associated with ALS. American Journal of Human Genetics, 2020, 107, 445-460.	6.2	39
135	AAV vectors and tumorigenicity. Nature Biotechnology, 2007, 25, 1111-1113.	17.5	38
136	A universal system to select gene-modified hepatocytes in vivo. Science Translational Medicine, 2016, 8, 342ra79.	12.4	38
137	An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging. Nature Chemical Biology, 2019, 15, 433-436.	8.0	37
138	Optimization of Cis-Acting Elements for Gene Expression from Nonviral VectorsIn Vivo. Human Gene Therapy, 2003, 14, 215-225.	2.7	36
139	Stability and Repeat Regeneration Potential of the Engineered Liver Tissues under the Kidney Capsule in Mice. Cell Transplantation, 2005, 14, 621-627.	2.5	36
140	Real-Time in Vivo Imaging of Stem Cells Following Transgenesis by Transposition. Molecular Therapy, 2005, 12, 42-48.	8.2	36
141	AAV Vectors Containing rDNA Homology Display Increased Chromosomal Integration and Transgene Persistence. Molecular Therapy, 2012, 20, 1902-1911.	8.2	36
142	Promoterless, Nucleaseâ€Free Genome Editing Confers a Growth Advantage for Corrected Hepatocytes in Mice With Methylmalonic Acidemia. Hepatology, 2021, 73, 2223-2237.	7.3	36
143	RNA structure probing reveals the structural basis of Dicer binding and cleavage. Nature Communications, 2021, 12, 3397.	12.8	36
144	Expression determinants of mammalian argonaute proteins in mediating gene silencing. Nucleic Acids Research, 2012, 40, 3704-3713.	14.5	35

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145	rAAV-Mediated Tumorigenesis: Still Unresolved After an AAV Assault. Molecular Therapy, 2012, 20, 2014-2017.	8.2	33
146	RNA interferenceâ€“induced hepatotoxicity results from loss of the first synthesized isoform of microRNA-122 in mice. Nature Medicine, 2016, 22, 557-562.	30.7	32
147	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, 1999, 73, 4755-4766.	3.4	32
148	Regulated complex assembly safeguards the fidelity ofSleeping Beautytransposition. Nucleic Acids Research, 2017, 45, 311-326.	14.5	31
149	Organ Size Control Is Dominant over Rb Family Inactivation to Restrict Proliferation InÂVivo. Cell Reports, 2014, 8, 371-381.	6.4	30
150	Translational Data from Adeno-Associated Virus-Mediated Gene Therapy of Hemophilia B in Dogs. Human Gene Therapy Clinical Development, 2015, 26, 5-14.	3.1	29
151	Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases. JCI Insight, 2019, 4, .	5.0	28
152	Ribosomal DNA Integrating rAAV-rDNA Vectors Allow for Stable Transgene Expression. Molecular Therapy, 2012, 20, 1912-1923.	8.2	27
153	Weak base pairing in both seed and 3â€² regions reduces RNAi off-targets and enhances si/shRNA designs. Nucleic Acids Research, 2014, 42, 12169-12176.	14.5	27
154	Pathways of Removal of Free DNA Vector Ends in Normal and DNA-PKcsâ€“Deficient SCID Mouse Hepatocytes Transduced with rAAV Vectors. Human Gene Therapy, 2003, 14, 871-881.	2.7	26
155	Allele-Specific Silencing Ameliorates Restrictive Cardiomyopathy Attributable to a Human Myosin Regulatory Light Chain Mutation. Circulation, 2019, 140, 765-778.	1.6	26
156	IMAGe, a New Clinical Association of Intrauterine Growth Retardation, Metaphyseal Dysplasia, Adrenal Hypoplasia Congenita, and Genital Anomalies. Journal of Clinical Endocrinology and Metabolism, 1999, 84, 4335-4340.	3.6	26
157	Correction of DNA Protein Kinase Deficiency by Spliceosome-mediated RNA Trans-splicing and Sleeping Beauty Transposon Delivery. Molecular Therapy, 2007, 15, 1273-1279.	8.2	24
158	Regulation of microRNA-mediated gene silencing by microRNA precursors. Nature Structural and Molecular Biology, 2014, 21, 825-832.	8.2	23
159	Free DNA ends are essential for concatemerization of synthetic Double-Stranded Adeno-Associated virus vector genomes transfected into mouse hepatocytes in vivo. Molecular Therapy, 2003, 7, 112-121.	8.2	22
160	Rapid and Stable Knockdown of an Endogenous Gene in Retinal Pigment Epithelium. Human Gene Therapy, 2007, 18, 871-880.	2.7	22
161	A 5â€² Noncoding Exon Containing Engineered Intron Enhances Transgene Expression from Recombinant AAV Vectors in vivo. Human Gene Therapy, 2017, 28, 125-134.	2.7	21
162	Bioengineered Viral Platform for Intramuscular Passive Vaccine Delivery to Human Skeletal Muscle. Molecular Therapy - Methods and Clinical Development, 2018, 10, 144-155.	4.1	21

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163	Osteosarcoma Derived from Cultured Mesenchymal Stem Cells.. Blood, 2006, 108, 2554-2554.	1.4	21
164	An in vitro-identified high-affinity nucleosome-positioning signal is capable of transiently positioning a nucleosome in vivo. Epigenetics and Chromatin, 2010, 3, 13.	3.9	20
165	Tracking Adeno-Associated Virus Capsid Evolution by High-Throughput Sequencing. Human Gene Therapy, 2020, 31, 553-564.	2.7	19
166	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. Blood, 2010, 116, 248-248.	1.4	19
167	Modified Infusion Procedures Affect Recombinant Adeno-Associated Virus Vector Type 2 Transduction in the Liver. Human Gene Therapy, 2005, 16, 299-306.	2.7	17
168	Development of a Clinical Protocol for Hepatic Gene Transfer: Lessons Learned in Preclinical Studies. Pediatric Research, 1993, 33, 313-320.	2.3	12
169	The anti-genomic (negative) strand of Hepatitis C Virus is not targetable by shRNA. Nucleic Acids Research, 2013, 41, 3688-3698.	14.5	12
170	RNA Interference Gene Therapy: A story of mice and men. Gene Therapy, 2002, 9, 1563-1563.	4.5	11
171	Selecting the Best AAV Capsid for Human Studies. Molecular Therapy, 2015, 23, 1800-1801.	8.2	11
172	Increased precursor microRNA-21 following status epilepticus can compete with mature microRNA-21 to alter translation. Experimental Neurology, 2016, 286, 137-146.	4.1	11
173	Hepatic parenchymal replacement in mice by transplanted allogeneic hepatocytes is facilitated by bone marrow transplantation and mediated by CD4 cells. Hepatology, 2008, 47, 706-718.	7.3	10
174	Sequence-Modified Antibiotic Resistance Genes Provide Sustained Plasmid-Mediated Transgene Expression in Mammals. Molecular Therapy, 2017, 25, 1187-1198.	8.2	10
175	The 3â€™tsRNAs are aminoacylated: Implications for their biogenesis. PLoS Genetics, 2021, 17, e1009675.	3.5	10
176	Engineering Cellular Resistance to HIV. New England Journal of Medicine, 2014, 370, 968-969.	27.0	8
177	AAV vectors engineered to target insulin receptor greatly enhance intramuscular gene delivery. Molecular Therapy - Methods and Clinical Development, 2020, 19, 496-506.	4.1	8
178	Promoterless Gene Targeting Approach Combined to CRISPR/Cas9 Efficiently Corrects Hemophilia B Phenotype in Neonatal Mice. Frontiers in Genome Editing, 2022, 4, 785698.	5.2	8
179	Fludarabine increases nuclease-free AAV- and CRISPR/Cas9-mediated homologous recombination in mice. Nature Biotechnology, 2022, 40, 1285-1294.	17.5	8
180	Extracellular matrix component cotransplantation prolongs survival of heterotopically transplanted human hepatocytes in mice. Transplantation Proceedings, 2004, 36, 2469-2470.	0.6	7

#	Article	IF	Citations
181	Donor-Derived, Liver-Specific Protein Expression after Bone Marrow Transplantation. Transplantation, 2004, 78, 530-536.	1.0	7
182	Improved Genome Editing through Inhibition of FANCM and Members of the BTR Dissolvase Complex. Molecular Therapy, 2021, 29, 1016-1027.	8.2	7
183	Advancing Molecular Therapies through In Vivo Bioluminescent Imaging. Molecular Imaging, 2003, 2, 153535002003031.	1.4	6
184	Evaluating the Genomic Parameters Governing rAAV-Mediated Homologous Recombination. Molecular Therapy, 2021, 29, 1028-1046.	8.2	6
185	Characterization of Vector-Based Delivery ofNeurogenin-3in Murine Diabetes. Human Gene Therapy, 2014, 25, 651-661.	2.7	5
186	Adeno-Associated Viral Vector Mediated Gene Transfer for Hemophilia B. Blood, 2011, 118, 5-5.	1.4	4
187	Escherichia coli Sepsis and Prolonged Hypophosphatemia Following Exertional Heat Stroke. Pediatrics, 1990, 86, 307-309.	2.1	4
188	Translational Data from AAV-Mediated Gene Therapy of Hemophilia B in Dogs. Human Gene Therapy Clinical Development, 2014, , 150127063140004.	3.1	2
189	Viral Vectors Take On HIV Infection. New England Journal of Medicine, 2015, 373, 770-772.	27.0	2
190	253. Expanded Packaging Capacity of AAV by Lumenal Charge Alteration. Molecular Therapy, 2016, 24, S99-S100.	8.2	2
191	Stable Factor IX Activity Following AAV-Mediated Gene Transfer in Patients with Severe Hemophilia B. Blood, 2012, 120, 752-752.	1.4	2
192	Methods for Delivery of Genes to Hepatocytes In Vivo Using Recombinant Adenovirus Vectors. , 1997, 7, 205-212.		1
193	Gene transfer for hemophilia and viral infection. International Journal of Hematology, 2002, 76, 147-147.	1.6	1
194	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, 2021, Publish Ahead of Print, .	1.4	1
195	RNAi in drug development: Practical considerations. , 2005, , 384-395.		0
196	374. Hepatitis Delta Virus-Mediated Amplification of Therapeutic RNAi. Molecular Therapy, 2006, 13, S142.	8.2	0
197	516. Development of a Minicircle Vector Free of Plasmid Bacterial DNA Sequences and Capable of Ã¸C31-Mediated Site-Specific Integration. Molecular Therapy, 2006, 13, S198-S199.	8.2	0
198	796. Pol II-Driven shRNA as an Effective Hepatitis B Virus Therapeutic. Molecular Therapy, 2006, 13, S308-S309.	8.2	0

#	Article	IF	Citations
199	9. Biology of rAAV8 in Mouse Liver Following Vector Administration at Birth. Molecular Therapy, 2006, 13, S4.	8.2	0
200	114. Revisiting rAAV Vector Integration in scid Mice: DNA-PKcs Deficiency Does Not Substantially Increase Integration Frequency in Hepatic and Non-Hepatic Tissues In Vivo. Molecular Therapy, 2006, 13, S47.	8.2	0
201	1099. RNAi-Based Therapy for the Treatment of HCV. Molecular Therapy, 2006, 13, S422-S423.	8.2	0
202	108. Study of an AAV-8 Capsid Mutant with Direct Heparin Binding Capability but Reduced Efficiency in Liver-Targeted Transduction. Molecular Therapy, 2006, 13, S44-S45.	8.2	0
203	11. Transposition from a Gene-Deleted Adenoviral Vector Results in Phenotypic Correction in a Canine Model for Hemophilia B. Molecular Therapy, 2006, 13, S5.	8.2	0
204	1066. A Novel Class of Miniature Stabilized Double-Stranded AAV (msdsAAV) Vectors for the In Vivo Expression of Short Hairpin RNAs. Molecular Therapy, 2006, 13, S409.	8.2	0
205	742. Molecular Evolution of Adeno-Associated Viral (AAV) Vectors Via DNA Family Shuffling of Primate and Non-Primate Serotypes. Molecular Therapy, 2006, 13, S287.	8.2	0
206	746. Localization of Structural Determinants in AAV Capsid for Efficient Liver Transduction by Domain Swapping between AAV-2 and AAV-8. Molecular Therapy, 2006, 13, S288.	8.2	0
207	489. Treatment for Hemophilia B Using Self- Complimentary AAV8 Vectors. Molecular Therapy, 2006, 13, S189-S190.	8.2	0
208	792. Post-Integrative Gene Silencing in the Sleeping Beauty Transposition System. Molecular Therapy, 2006, 13, S307.	8.2	0
209	1034. Expression of Short Hairpin RNAs by Liver and Non Liver Specific RNA Pol II Expression Cassettes: What Governs Activity?. Molecular Therapy, 2006, 13, S397.	8.2	0
210	680. Non-Viral Transposon Mediated Gene Transfer of Human Factor VIII to Hemophilia A Mice. Molecular Therapy, 2006, 13, S262.	8.2	0
211	805. Fatality in Mice Due to Oversaturation of Cellular Micro/Short Hairpin RNA Pathways. Molecular Therapy, 2006, 13, S312.	8.2	0
212	5. Mechanisms for Hairpin Loop Opening of â€œClosedâ€•AAV-ITRs by Specific Cellular Endonuclease Activities, a Prerequisite for rAAV Vector Genome Recombinations In Vivo. Molecular Therapy, 2006, 13, S2-S3.	8.2	0
213	539. Unraveling the Mechanisms Underlying Silencing/Activation of Episomal Vectors In Vivo. Molecular Therapy, 2006, 13, S207.	8.2	0
214	103. A Screen for Host Cellular Proteins That Interact with Adeno-Associated Virus Capsid Proteins Reveals Proteins Involved in AAV8 Transduction. Molecular Therapy, 2006, 13, S42-S43.	8.2	0
215	804. In Vivo Correction of a Metabolic Liver Disease by AAV8-Mediated Homologous Recombination. Molecular Therapy, 2006, 13, S311-S312.	8.2	0
216	microRNAs outwit immune limitations in gene therapy. Blood, 2007, 110, 4136-4137.	1.4	0

#	Article	IF	Citations
217	94. AAV Integration Site Determination Using Illumina Mate Pair Sequencing. Molecular Therapy, 2015, 23, S39-S40.	8.2	0
218	303. AAV Capsid Evolution for Enhanced Antibody Delivery To Human Muscle for Use in Next-Generation HIV Vaccines. Molecular Therapy, 2015, 23, S122-S123.	8.2	0
219	305. A Screening Strategy for Selecting Recombinant Adeno-Associated Viral Vectors That Selectively Transduce Viral Infected Cells. Molecular Therapy, 2015, 23, S123.	8.2	0
220	578. microRNA Inhibition Through Gapmer Activated RNase H-Mediated Degradation. Molecular Therapy, 2015, 23, S230.	8.2	0
221	683. In Vivo Expansion of Hepatocytes with Targeted rAAV Integration Results in a >100-Fold Increase of Transgene Expression. Molecular Therapy, 2015, 23, S272.	8.2	0
222	688. AAV8-Mediated Liver Gene Targeting Without Nucleases Rescues Lethality in a Mouse Model of the Crigler-Najjar Syndrome. Molecular Therapy, 2015, 23, S274.	8.2	0
223	129. Does Transcription Influence AAV-Mediated Homologous Recombination?. Molecular Therapy, 2016, 24, S53.	8.2	0
224	257. Selection of Next Generation AAV Gene Therapy Vectors for Specific and Precise Gene Delivery. Molecular Therapy, 2016, 24, S101.	8.2	0
225	737. RNAi Induced Hepatotoxicity Results from a Functional Depletion of the First Synthesized Isoform of miR-122. Molecular Therapy, 2016, 24, S290-S291.	8.2	0
226	48. Treatment of Methylmalonic Acidemia by Promoterless Gene-Targeting Using Adeno-Associated Viral (AAV) Mediated Homologous Recombination. Molecular Therapy, 2016, 24, S21-S22.	8.2	0
227	289. Sequence Modified Antibiotic Resistance Genes Provide Sustained Plasmid Mediated Transgene Expression in Mammals. Molecular Therapy, 2016, 24, S116.	8.2	0
228	539. Screening for Recombinant Adeno-Associated Viral Vectors That Selectively Transduce Hepatitis B Virus Infected Cells. Molecular Therapy, 2016, 24, S215.	8.2	0
229	722. AAV Capsid Evolution for Enhanced Antibody Delivery to Human Skeletal Muscle for Use in Next-Generation HIV Vaccines and Muscle Gene Therapies. Molecular Therapy, 2016, 24, S284-S285.	8.2	0
230	A Tribute to George Stamatoyannopoulos. Human Gene Therapy, 2016, 27, 280-286.	2.7	0
231	Dieter C. Gruenert, PhD (1949â€“2016). Nucleic Acid Therapeutics, 2016, 26, 266-267.	3.6	0
232	The Role of tRNA Derived Small RNAs in Gene Regulation in Normal Tissues and Cancer. FASEB Journal, 2021, 35, .	0.5	0
233	Transgenesis of Multipotent Adult Progenitor Cells (MAPC) with Sleeping Beauty Transposons to Determine MAPC Homing and Persistence in Real-Time In Vivo.. Blood, 2004, 104, 2099-2099.	1.4	0
234	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.. Blood, 2004, 104, 507-507.	1.4	0

#	Article	IF	Citations
235	Mesenchymal Cancer Cells Can Arise from Ex Vivo Modified Mesenchymal Stem Cells.. Blood, 2005, 106, 4326-4326.	1.4	0
236	Minicircle Plasmid Containing the Human Manganese Superoxide Dismutase (MnSOD) Transgene Confers Radioprotection to Hematopoietic Progenitor Cell Line 32Dcl3.. Blood, 2007, 110, 5138-5138.	1.4	0
237	Cellular Immune Responses To Vector In a Gene Therapy Trial For Hemophilia B Using An AAV8 Self-Complementary Factor IX Vector. Blood, 2013, 122, 717-717.	1.4	0
238	Abstract LB-390: A transfer RNA derived small RNA affects translation in rapidly dividing cells and a target for hepatocellular carcinoma. , 2018, , .		0
239	Abstract IA03: Functional lung cancer genomics through in vivo genome editing. , 2018, , .		0
240	The Role of tRNA Derived Small RNAs in Gene Regulation in Normal Tissues and Cancer. FASEB Journal, 2020, 34, 1-1.	0.5	0
241	Abstract LB-343: A Leu(CAG)-tRNA derived small RNA regulates ribosomal protein S28 after translation initiation in both human and mouse liver cancers. , 2020, , .		0