

Mark A Kay

List of Publications by Year in descending order

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240
papers

36,399
citations

3515

90
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3173

186
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. <i>Nature Reviews Genetics</i> , 2003, 4, 346-358.	7.7	2,213
2	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. <i>Nature Medicine</i> , 2006, 12, 342-347.	15.2	1,865
3	Adenovirus-Associated Virus Vector-Mediated Gene Transfer in Hemophilia B. <i>New England Journal of Medicine</i> , 2011, 365, 2357-2365.	13.9	1,606
4	Fatality in mice due to oversaturation of cellular microRNA/short hairpin RNA pathways. <i>Nature</i> , 2006, 441, 537-541.	13.7	1,518
5	Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics. <i>Nature Medicine</i> , 2001, 7, 33-40.	15.2	1,205
6	Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B. <i>New England Journal of Medicine</i> , 2014, 371, 1994-2004.	13.9	1,063
7	RNA interference in adult mice. <i>Nature</i> , 2002, 418, 38-39.	13.7	1,043
8	Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector. <i>Nature Genetics</i> , 2000, 24, 257-261.	9.4	971
9	Robust expansion of human hepatocytes in Fah ^{-/-} /Rag2 ^{-/-} /Il2rg ^{-/-} mice. <i>Nature Biotechnology</i> , 2007, 25, 903-910.	9.4	729
10	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. <i>Blood</i> , 2003, 101, 2963-2972.	0.6	707
11	A nonviral minicircle vector for deriving human iPS cells. <i>Nature Methods</i> , 2010, 7, 197-199.	9.0	658
12	Sarcoma Derived from Cultured Mesenchymal Stem Cells. <i>Stem Cells</i> , 2007, 25, 371-379.	1.4	601
13	Inhibition of hepatitis B virus in mice by RNA interference. <i>Nature Biotechnology</i> , 2003, 21, 639-644.	9.4	595
14	Persistent and therapeutic concentrations of human factor IX in mice after hepatic gene transfer of recombinant AAV vectors. <i>Nature Genetics</i> , 1997, 16, 270-276.	9.4	589
15	State-of-the-art gene-based therapies: the road ahead. <i>Nature Reviews Genetics</i> , 2011, 12, 316-328.	7.7	587
16	Human tRNA-derived small RNAs in the global regulation of RNA silencing. <i>Rna</i> , 2010, 16, 673-695.	1.6	583
17	In Vitro and In Vivo Gene Therapy Vector Evolution via Multispecies Interbreeding and Retargeting of Adeno-Associated Viruses. <i>Journal of Virology</i> , 2008, 82, 5887-5911.	1.5	546
18	Robust systemic transduction with AAV9 vectors in mice: efficient global cardiac gene transfer superior to that of AAV8. <i>Molecular Therapy</i> , 2006, 14, 45-53.	3.7	527

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19	Somatic integration and long-term transgene expression in normal and haemophilic mice using a DNA transposon system. <i>Nature Genetics</i> , 2000, 25, 35-41.	9.4	491
20	In vivo activity of nuclease-resistant siRNAs. <i>Rna</i> , 2004, 10, 766-771.	1.6	483
21	Minicircle DNA vectors devoid of bacterial DNA result in persistent and high-level transgene expression in vivo. <i>Molecular Therapy</i> , 2003, 8, 495-500.	3.7	435
22	Extrachromosomal Recombinant Adeno-Associated Virus Vector Genomes Are Primarily Responsible for Stable Liver Transduction In Vivo. <i>Journal of Virology</i> , 2001, 75, 6969-6976.	1.5	417
23	Correction of hemophilia B in canine and murine models using recombinant adeno-associated viral vectors. <i>Nature Medicine</i> , 1999, 5, 64-70.	15.2	414
24	Biological basis for restriction of microRNA targets to the 3' untranslated region in mammalian mRNAs. <i>Nature Structural and Molecular Biology</i> , 2009, 16, 144-150.	3.6	383
25	Fate tracing of mature hepatocytes in mouse liver homeostasis and regeneration. <i>Journal of Clinical Investigation</i> , 2011, 121, 4850-4860.	3.9	376
26	Selection and evaluation of clinically relevant AAV variants in a xenograft liver model. <i>Nature</i> , 2014, 506, 382-386.	13.7	376
27	A transfer-RNA-derived small RNA regulates ribosome biogenesis. <i>Nature</i> , 2017, 552, 57-62.	13.7	366
28	AAV serotype 2 vectors preferentially integrate into active genes in mice. <i>Nature Genetics</i> , 2003, 34, 297-302.	9.4	359
29	The 37/67-Kilodalton Laminin Receptor Is a Receptor for Adeno-Associated Virus Serotypes 8, 2, 3, and 9. <i>Journal of Virology</i> , 2006, 80, 9831-9836.	1.5	356
30	Rapid Uncoating of Vector Genomes Is the Key to Efficient Liver Transduction with Pseudotyped Adeno-Associated Virus Vectors. <i>Journal of Virology</i> , 2004, 78, 3110-3122.	1.5	333
31	Efficient Construction of a Recombinant Adenovirus Vector by an Improved <i>In Vitro</i> Ligation Method. <i>Human Gene Therapy</i> , 1998, 9, 2577-2583.	1.4	329
32	Assessment of Recombinant Adenoviral Vectors for Hepatic Gene Therapy. <i>Human Gene Therapy</i> , 1993, 4, 403-409.	1.4	327
33	In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs. <i>Science</i> , 1993, 262, 117-119.	6.0	315
34	Helper virus-free, optically controllable, and two-plasmid-based production of adeno-associated virus vectors of serotypes 1 to 6. <i>Molecular Therapy</i> , 2003, 7, 839-850.	3.7	311
35	Unrestricted Hepatocyte Transduction with Adeno-Associated Virus Serotype 8 Vectors in Mice. <i>Journal of Virology</i> , 2005, 79, 214-224.	1.5	299
36	Long-term hepatic adenovirus-mediated gene expression in mice following CTLA4Ig administration. <i>Nature Genetics</i> , 1995, 11, 191-197.	9.4	298

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37	High-Resolution Genome-Wide Mapping of Transposon Integration in Mammals. <i>Molecular and Cellular Biology</i> , 2005, 25, 2085-2094.	1.1	298
38	A robust system for production of minicircle DNA vectors. <i>Nature Biotechnology</i> , 2010, 28, 1287-1289.	9.4	288
39	Efficient lentiviral transduction of liver requires cell cycling in vivo. <i>Nature Genetics</i> , 2000, 24, 49-52.	9.4	278
40	The Loop Position of shRNAs and Pre-miRNAs Is Critical for the Accuracy of Dicer Processing In Vivo. <i>Cell</i> , 2012, 151, 900-911.	13.5	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. <i>Molecular Therapy</i> , 2000, 1, 522-532.	3.7	230
42	A bright cyan-excitable orange fluorescent protein facilitates dual-emission microscopy and enhances bioluminescence imaging in vivo. <i>Nature Biotechnology</i> , 2016, 34, 760-767.	9.4	221
43	Preclinical in vivo evaluation of pseudotyped adeno-associated virus vectors for liver gene therapy. <i>Blood</i> , 2003, 102, 2412-2419.	0.6	196
44	Human COL7A1-corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa. <i>Science Translational Medicine</i> , 2014, 6, 264ra163.	5.8	194
45	Distinct pathways of genomic progression to benign and malignant tumors of the liver. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2007, 104, 14771-14776.	3.3	193
46	Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors. <i>Nature Biotechnology</i> , 2000, 18, 527-532.	9.4	191
47	Transposition from a gutless adeno-transposon vector stabilizes transgene expression in vivo. <i>Nature Biotechnology</i> , 2002, 20, 999-1005.	9.4	184
48	Expression of shRNA From a Tissue-specific pol II Promoter Is an Effective and Safe RNAi Therapeutic. <i>Molecular Therapy</i> , 2008, 16, 1630-1636.	3.7	183
49	Hepatic Gene Therapy: Persistent Expression of Human α 1-Antitrypsin in Mice after Direct Gene Delivery In Vivo. <i>Human Gene Therapy</i> , 1992, 3, 641-647.	1.4	182
50	The kinetics of rAAV integration in the liver. <i>Nature Genetics</i> , 1998, 19, 13-15.	9.4	181
51	Linear DNAs Concatemerize in Vivo and Result in Sustained Transgene Expression in Mouse Liver. <i>Molecular Therapy</i> , 2001, 3, 403-410.	3.7	179
52	Isolation of Recombinant Adeno-Associated Virus Vector-Cellular DNA Junctions from Mouse Liver. <i>Journal of Virology</i> , 1999, 73, 5438-5447.	1.5	178
53	Recruitment of Single-Stranded Recombinant Adeno-Associated Virus Vector Genomes and Intermolecular Recombination Are Responsible for Stable Transduction of Liver In Vivo. <i>Journal of Virology</i> , 2000, 74, 9451-9463.	1.5	174
54	Sustained survival of human hepatocytes in mice: A model for in vivo infection with human hepatitis B and hepatitis delta viruses. <i>Nature Medicine</i> , 2000, 6, 327-331.	15.2	172

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55	Improved Production and Purification of Minicircle DNA Vector Free of Plasmid Bacterial Sequences and Capable of Persistent Transgene Expression In Vivo. <i>Human Gene Therapy</i> , 2005, 16, 126-131.	1.4	168
56	Large-Scale Molecular Characterization of Adeno-Associated Virus Vector Integration in Mouse Liver. <i>Journal of Virology</i> , 2005, 79, 3606-3614.	1.5	164
57	FATP2 is a hepatic fatty acid transporter and peroxisomal very long-chain acyl-CoA synthetase. <i>American Journal of Physiology - Endocrinology and Metabolism</i> , 2010, 299, E384-E393.	1.8	161
58	Argonaute proteins are key determinants of RNAi efficacy, toxicity, and persistence in the adult mouse liver. <i>Journal of Clinical Investigation</i> , 2010, 120, 3106-3119.	3.9	161
59	Lack of Germline Transmission of Vector Sequences Following Systemic Administration of Recombinant AAV-2 Vector in Males. <i>Molecular Therapy</i> , 2001, 4, 586-592.	3.7	152
60	Genome Editing of Isogenic Human Induced Pluripotent Stem Cells Recapitulates Long QT Phenotype for Drug Testing. <i>Journal of the American College of Cardiology</i> , 2014, 64, 451-459.	1.2	149
61	Therapeutic levels of human factor VIII and IX using HIV-1-based lentiviral vectors in mouse liver. <i>Blood</i> , 2000, 96, 1173-1176.	0.6	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. <i>Molecular and Cellular Biology</i> , 2004, 24, 9239-9247.	1.1	142
63	Silencing of Episomal Transgene Expression in Liver by Plasmid Bacterial Backbone DNA Is Independent of CpG Methylation. <i>Molecular Therapy</i> , 2008, 16, 548-556.	3.7	141
64	How do miRNAs mediate translational repression?. <i>Silence: A Journal of RNA Regulation</i> , 2010, 1, 11.	8.0	140
65	Helper-Independent sleeping beauty Transposon-based Transposase vectors for efficient nonviral gene delivery and persistent gene expression in vivo. <i>Molecular Therapy</i> , 2003, 8, 654-665.	3.7	138
66	In Vivo Correction of Murine Tyrosinemia Type I by DNA-Mediated Transposition. <i>Molecular Therapy</i> , 2002, 6, 759-769.	3.7	137
67	A new adenoviral helper-dependent vector results in long-term therapeutic levels of human coagulation factor IX at low doses in vivo. <i>Blood</i> , 2002, 99, 3923-3930.	0.6	133
68	Silencing of Hepatic Fatty Acid Transporter Protein 5 in Vivo Reverses Diet-induced Non-alcoholic Fatty Liver Disease and Improves Hyperglycemia. <i>Journal of Biological Chemistry</i> , 2008, 283, 22186-22192.	1.6	133
69	Therapeutic application of RNAi: is mRNA targeting finally ready for prime time?. <i>Journal of Clinical Investigation</i> , 2007, 117, 3633-3641.	3.9	132
70	Bioengineered AAV Capsids with Combined High Human Liver Transduction In Vivo and Unique Humoral Seroreactivity. <i>Molecular Therapy</i> , 2018, 26, 289-303.	3.7	130
71	Site-directed transposon integration in human cells. <i>Nucleic Acids Research</i> , 2007, 35, e50-e50.	6.5	129
72	Nonrandom Transduction of Recombinant Adeno-Associated Virus Vectors in Mouse Hepatocytes In Vivo: Cell Cycling Does Not Influence Hepatocyte Transduction. <i>Journal of Virology</i> , 2000, 74, 3793-3803.	1.5	124

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73	Histone Modifications are Associated with the Persistence or Silencing of Vector-mediated Transgene Expression In Vivo. <i>Molecular Therapy</i> , 2007, 15, 1348-1355.	3.7	123
74	Novel NanoLuc substrates enable bright two-population bioluminescence imaging in animals. <i>Nature Methods</i> , 2020, 17, 852-860.	9.0	123
75	Hepatocyte transplantation: clinical and experimental application. <i>Journal of Molecular Medicine</i> , 2001, 79, 617-630.	1.7	122
76	Adeno-associated virus gene repair corrects a mouse model of hereditary tyrosinemia in vivo. <i>Hepatology</i> , 2010, 51, 1200-1208.	3.6	121
77	Liver tissue engineering at extrahepatic sites in mice as a potential new therapy for genetic liver diseases. <i>Hepatology</i> , 2005, 41, 132-140.	3.6	120
78	Hepatocyte growth factor induces hepatocyte proliferation in vivo and allows for efficient retroviral-mediated gene transfer in mice. <i>Hepatology</i> , 1998, 28, 707-716.	3.6	112
79	Adenovirus Transduction is Required for the Correction of Diabetes Using Pdx-1 or Neurogenin-3 in the Liver. <i>Molecular Therapy</i> , 2007, 15, 255-263.	3.7	111
80	Recombinant AAV as a Platform for Translating the Therapeutic Potential of RNA Interference. <i>Molecular Therapy</i> , 2014, 22, 692-701.	3.7	111
81	Method for Multiple Portal Vein Infusions in Mice: Quantitation of Adenovirus-Mediated Hepatic Gene Transfer. <i>BioTechniques</i> , 1996, 20, 278-285.	0.8	104
82	Liver Transduction with Recombinant Adeno-Associated Virus Is Primarily Restricted by Capsid Serotype Not Vector Genotype. <i>Journal of Virology</i> , 2006, 80, 426-439.	1.5	104
83	Minicircle DNA Vectors Achieve Sustained Expression Reflected by Active Chromatin and Transcriptional Level. <i>Molecular Therapy</i> , 2013, 21, 131-138.	3.7	103
84	Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid α -glucosidase. <i>Science Translational Medicine</i> , 2017, 9, .	5.8	103
85	Genome Editing of Human Embryonic Stem Cells and Induced Pluripotent Stem Cells With Zinc Finger Nucleases for Cellular Imaging. <i>Circulation Research</i> , 2012, 111, 1494-1503.	2.0	99
86	Modified HIV-1 Based Lentiviral Vectors Have an Effect on Viral Transduction Efficiency and Gene Expression in Vitro and in Vivo. <i>Molecular Therapy</i> , 2001, 4, 164-173.	3.7	98
87	Adeno-Associated Virus Vectors and Hematology. <i>Blood</i> , 1999, 94, 864-874.	0.6	97
88	Therapeutic serum concentrations of human alpha-1-antitrypsin after adenoviral-mediated gene transfer into mouse hepatocytes. <i>Hepatology</i> , 1995, 21, 815-819.	3.6	94
89	The Host Response to Adenovirus, Helper-dependent Adenovirus, and Adeno-associated Virus in Mouse Liver. <i>Molecular Therapy</i> , 2008, 16, 931-941.	3.7	93
90	Integrating Adenovirus-Adeno-Associated Virus Hybrid Vectors Devoid of All Viral Genes. <i>Journal of Virology</i> , 1999, 73, 9314-9324.	1.5	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. <i>Journal of Virology</i> , 2002, 76, 11343-11349.	1.5	92
92	Novel Minicircle Vector for Gene Therapy in Murine Myocardial Infarction. <i>Circulation</i> , 2009, 120, S230-7.	1.6	91
93	Minicircle DNA-based Gene Therapy Coupled With Immune Modulation Permits Long-term Expression of β -L-Iduronidase in Mice With Mucopolysaccharidosis Type I. <i>Molecular Therapy</i> , 2011, 19, 450-460.	3.7	86
94	Determinants of Hepatitis C Translational Initiation in Vitro, in Cultured Cells and Mice. <i>Molecular Therapy</i> , 2002, 5, 676-684.	3.7	85
95	Episomal Persistence of Recombinant Adenoviral Vector Genomes during the Cell Cycle In Vivo. <i>Journal of Virology</i> , 2003, 77, 7689-7695.	1.5	84
96	Nonhomologous-End-Joining Factors Regulate DNA Repair Fidelity during Sleeping Beauty Element Transposition in Mammalian Cells. <i>Molecular and Cellular Biology</i> , 2003, 23, 8505-8518.	1.1	79
97	A potent and specific morpholino antisense inhibitor of hepatitis C translation in mice. <i>Hepatology</i> , 2003, 38, 503-508.	3.6	78
98	Multiplexed in vivo homology-directed repair and tumor barcoding enables parallel quantification of Kras variant oncogenicity. <i>Nature Communications</i> , 2017, 8, 2053.	5.8	78
99	A gene-deleted adenoviral vector results in phenotypic correction of canine hemophilia B without liver toxicity or thrombocytopenia. <i>Blood</i> , 2003, 102, 2403-2411.	0.6	76
100	A direct comparison of two nonviral gene therapy vectors for somatic integration: in vivo evaluation of the bacteriophage integrase β -C31 and the Sleeping Beauty transposase. <i>Molecular Therapy</i> , 2005, 11, 695-706.	3.7	75
101	Host factors that impact the biodistribution and persistence of multipotent adult progenitor cells. <i>Blood</i> , 2006, 107, 4182-4188.	0.6	75
102	RNAi and Gene Therapy: A Mutual Attraction. <i>Hematology American Society of Hematology Education Program</i> , 2007, 2007, 473-481.	0.9	75
103	Hyperactive Sleeping Beauty Transposase Enables Persistent Phenotypic Correction in Mice and a Canine Model for Hemophilia B. <i>Molecular Therapy</i> , 2010, 18, 1896-1906.	3.7	75
104	Drugging RNAi. <i>Science</i> , 2015, 347, 1069-1070.	6.0	74
105	Capped small RNAs and MOV10 in human hepatitis delta virus replication. <i>Nature Structural and Molecular Biology</i> , 2008, 15, 714-721.	3.6	72
106	Thermodynamic stability of small hairpin RNAs highly influences the loading process of different mammalian Argonautes. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2011, 108, 9208-9213.	3.3	71
107	A rapid protocol for construction and production of high-capacity adenoviral vectors. <i>Nature Protocols</i> , 2009, 4, 547-564.	5.5	66
108	Evaluating the state of the science for adeno-associated virus integration: An integrated perspective. <i>Molecular Therapy</i> , 2022, 30, 2646-2663.	3.7	65

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109	Using a barcoded AAV capsid library to select for clinically relevant gene therapy vectors. JCI Insight, 2019, 4, .	2.3	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.	3.7	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.	1.5	61
112	Radioprotection <i>In Vitro</i> and <i>In Vivo</i> by Minicircle Plasmid Carrying the Human Manganese Superoxide Dismutase Transgene. Human Gene Therapy, 2008, 19, 820-826.	1.4	60
113	Somatic Correction of Junctional Epidermolysis Bullosa by a Highly Recombinogenic AAV Variant. Molecular Therapy, 2014, 22, 725-733.	3.7	60
114	Looking into the safety of AAV vectors. Nature, 2003, 424, 251-251.	13.7	58
115	Transfer RNA-Derived Small RNAs: Another Layer of Gene Regulation and Novel Targets for Disease Therapeutics. Molecular Therapy, 2020, 28, 2340-2357.	3.7	57
116	The Extragenic Spacer Length Between the 5' and 3' Ends of the Transgene Expression Cassette Affects Transgene Silencing From Plasmid-based Vectors. Molecular Therapy, 2012, 20, 2111-2119.	3.7	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.	2.9	52
118	A Mini-intronic Plasmid (MIP): A Novel Robust Transgene Expression Vector <i>In Vivo</i> and <i>In Vitro</i> . Molecular Therapy, 2013, 21, 954-963.	3.7	51
119	Novel codon-optimized mini-intronic plasmid for efficient, inexpensive and xeno-free induction of pluripotency. Scientific Reports, 2015, 5, 8081.	1.6	51
120	A Modified Urokinase Plasminogen Activator Induces Liver Regeneration Without Bleeding. Human Gene Therapy, 1995, 6, 1029-1037.	1.4	49
121	Role of Hepatocyte Direct Hyperplasia in Lentivirus-Mediated Liver Transduction <i>In Vivo</i> . Human Gene Therapy, 2002, 13, 653-663.	1.4	49
122	Helper-independent and AAV-ITR-independent chromosomal integration of double-stranded linear DNA vectors in mice. Molecular Therapy, 2003, 7, 101-111.	3.7	48
123	Increased Maintenance and Persistence of Transgenes by Excision of Expression Cassettes from Plasmid Sequences <i>In Vivo</i> . Human Gene Therapy, 2005, 16, 558-570.	1.4	48
124	DNA Palindromes with a Modest Arm Length of ~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.	1.5	48
125	miR-122 removal in the liver activates imprinted microRNAs and enables more effective microRNA-mediated gene repression. Nature Communications, 2018, 9, 5321.	5.8	48
126	Promoterless gene targeting without nucleases rescues lethality of a Crigler-Najjar syndrome mouse model. EMBO Molecular Medicine, 2017, 9, 1346-1355.	3.3	46

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127	Adenoviral preterminal protein stabilizes mini-adenoviral genomes in vitro and in vivo. <i>Nature Biotechnology</i> , 1997, 15, 1383-1387.	9.4	43
128	Combined proteomicâ€“RNAi screen for host factors involved in human hepatitis delta virus replication. <i>Rna</i> , 2009, 15, 1971-1979.	1.6	43
129	The effect of age on hepatic gene transfer with self-inactivating lentiviral vectors in vivo. <i>Molecular Therapy</i> , 2003, 8, 314-323.	3.7	42
130	Somatic Integration From an Adenoviral Hybrid Vector into a Hot Spot in Mouse Liver Results in Persistent Transgene Expression Levels In Vivo. <i>Molecular Therapy</i> , 2007, 15, 146-156.	3.7	41
131	Future of rAAV Gene Therapy: Platform for RNAi, Gene Editing, and Beyond. <i>Human Gene Therapy</i> , 2017, 28, 361-372.	1.4	40
132	Cis-Acting Gene Regulatory Activities in the Terminal Regions of Sleeping Beauty DNA Transposon-Based Vectors. <i>Human Gene Therapy</i> , 2007, 18, 1193-1204.	1.4	39
133	Low-level shRNA Cytotoxicity Can Contribute to MYC-induced Hepatocellular Carcinoma in Adult Mice. <i>Molecular Therapy</i> , 2010, 18, 161-170.	3.7	39
134	Evolution of a Human-Specific Tandem Repeat Associated with ALS. <i>American Journal of Human Genetics</i> , 2020, 107, 445-460.	2.6	39
135	AAV vectors and tumorigenicity. <i>Nature Biotechnology</i> , 2007, 25, 1111-1113.	9.4	38
136	A universal system to select gene-modified hepatocytes in vivo. <i>Science Translational Medicine</i> , 2016, 8, 342ra79.	5.8	38
137	An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging. <i>Nature Chemical Biology</i> , 2019, 15, 433-436.	3.9	37
138	Optimization of Cis-Acting Elements for Gene Expression from Nonviral Vectors In Vivo. <i>Human Gene Therapy</i> , 2003, 14, 215-225.	1.4	36
139	Stability and Repeat Regeneration Potential of the Engineered Liver Tissues under the Kidney Capsule in Mice. <i>Cell Transplantation</i> , 2005, 14, 621-627.	1.2	36
140	Real-Time in Vivo Imaging of Stem Cells Following Transgenesis by Transposition. <i>Molecular Therapy</i> , 2005, 12, 42-48.	3.7	36
141	AAV Vectors Containing rDNA Homology Display Increased Chromosomal Integration and Transgene Persistence. <i>Molecular Therapy</i> , 2012, 20, 1902-1911.	3.7	36
142	Promoterless, Nucleaseâ€“Free Genome Editing Confers a Growth Advantage for Corrected Hepatocytes in Mice With Methylmalonic Acidemia. <i>Hepatology</i> , 2021, 73, 2223-2237.	3.6	36
143	RNA structure probing reveals the structural basis of Dicer binding and cleavage. <i>Nature Communications</i> , 2021, 12, 3397.	5.8	36
144	Expression determinants of mammalian argonaute proteins in mediating gene silencing. <i>Nucleic Acids Research</i> , 2012, 40, 3704-3713.	6.5	35

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145	rAAV-Mediated Tumorigenesis: Still Unresolved After an AAV Assault. <i>Molecular Therapy</i> , 2012, 20, 2014-2017.	3.7	33
146	RNA interference-induced hepatotoxicity results from loss of the first synthesized isoform of microRNA-122 in mice. <i>Nature Medicine</i> , 2016, 22, 557-562.	15.2	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. <i>Journal of Virology</i> , 1999, 73, 4755-4766.	1.5	32
148	Regulated complex assembly safeguards the fidelity of Sleeping Beauty transposition. <i>Nucleic Acids Research</i> , 2017, 45, 311-326.	6.5	31
149	Organ Size Control Is Dominant over Rb Family Inactivation to Restrict Proliferation In Vivo. <i>Cell Reports</i> , 2014, 8, 371-381.	2.9	30
150	Translational Data from Adeno-Associated Virus-Mediated Gene Therapy of Hemophilia B in Dogs. <i>Human Gene Therapy Clinical Development</i> , 2015, 26, 5-14.	3.2	29
151	Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases. <i>JCI Insight</i> , 2019, 4, .	2.3	28
152	Ribosomal DNA Integrating rAAV-rDNA Vectors Allow for Stable Transgene Expression. <i>Molecular Therapy</i> , 2012, 20, 1912-1923.	3.7	27
153	Weak base pairing in both seed and 3' regions reduces RNAi off-targets and enhances si/shRNA designs. <i>Nucleic Acids Research</i> , 2014, 42, 12169-12176.	6.5	27
154	Pathways of Removal of Free DNA Vector Ends in Normal and DNA-PKcs Deficient SCID Mouse Hepatocytes Transduced with rAAV Vectors. <i>Human Gene Therapy</i> , 2003, 14, 871-881.	1.4	26
155	Allele-Specific Silencing Ameliorates Restrictive Cardiomyopathy Attributable to a Human Myosin Regulatory Light Chain Mutation. <i>Circulation</i> , 2019, 140, 765-778.	1.6	26
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