Mark A Kay

List of Publications by Citations

195 papers	30,706 citations	85 h-index	175 g-index
250 ext. papers	33,741 ext. citations	14.2 avg, IF	6.89 L-index

#	Paper	IF	Citations
195	Progress and problems with the use of viral vectors for gene therapy. *Nature Reviews Genetics*, **2003**, 4, 346-58	30.1	1911
194	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. *Nature Medicine*, **2006**, 12, 342-7	50.5	1525
193	Fatality in mice due to oversaturation of cellular microRNA/short hairpin RNA pathways. *Nature*, **2006**, 441, 537-41	50.4	1358
192	Adenovirus-associated virus vector-mediated gene transfer in hemophilia B. *New England Journal of Medicine*, **2011**, 365, 2357-65	59.2	1271
191	Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics. *Nature Medicine*, **2001**, 7, 33-40	50.5	1066
190	RNA interference in adult mice. *Nature*, **2002**, 418, 38-9	50.4	924
189	Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector. *Nature Genetics*, **2000**, 24, 257-61	36.3	850
188	Long-term safety and efficacy of factor IX gene therapy in hemophilia B. *New England Journal of Medicine*, **2014**, 371, 1994-2004	59.2	810
187	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. *Blood*, **2003**, 101, 2963-72	2.2	607
186	Robust expansion of human hepatocytes in Fah-/-/Rag2-/-/Il2rg-/- mice. *Nature Biotechnology*, **2007**, 25, 903-10	44.5	599
185	A nonviral minicircle vector for deriving human iPS cells. *Nature Methods*, **2010**, 7, 197-9	21.6	590
184	Sarcoma derived from cultured mesenchymal stem cells. *Stem Cells*, **2007**, 25, 371-9	5.8	544
183	Inhibition of hepatitis B virus in mice by RNA interference. *Nature Biotechnology*, **2003**, 21, 639-44	44.5	539
182	Persistent and therapeutic concentrations of human factor IX in mice after hepatic gene transfer of recombinant AAV vectors. *Nature Genetics*, **1997**, 16, 270-6	36.3	538
181	State-of-the-art gene-based therapies: the road ahead. *Nature Reviews Genetics*, **2011**, 12, 316-28	30.1	515
180	Human tRNA-derived small RNAs in the global regulation of RNA silencing. *Rna*, **2010**, 16, 673-95	5.8	473
179	Robust systemic transduction with AAV9 vectors in mice: efficient global cardiac gene transfer superior to that of AAV8. *Molecular Therapy*, **2006**, 14, 45-53	11.7	465

178	Somatic integration and long-term transgene expression in normal and haemophilic mice using a DNA transposon system. *Nature Genetics*, **2000**, 25, 35-41	36.3	446
177	In vitro and in vivo gene therapy vector evolution via multispecies interbreeding and retargeting of adeno-associated viruses. *Journal of Virology*, **2008**, 82, 5887-911	6.6	423
176	In vivo activity of nuclease-resistant siRNAs. *Rna*, **2004**, 10, 766-71	5.8	421
175	Minicircle DNA vectors devoid of bacterial DNA result in persistent and high-level transgene expression in vivo. *Molecular Therapy*, **2003**, 8, 495-500	11.7	394
174	Correction of hemophilia B in canine and murine models using recombinant adeno-associated viral vectors. *Nature Medicine*, **1999**, 5, 64-70	50.5	373
173	Extrachromosomal recombinant adeno-associated virus vector genomes are primarily responsible for stable liver transduction in vivo. *Journal of Virology*, **2001**, 75, 6969-76	6.6	358
172	The 37/67-kilodalton laminin receptor is a receptor for adeno-associated virus serotypes 8, 2, 3, and 9. *Journal of Virology*, **2006**, 80, 9831-6	6.6	310
171	Biological basis for restriction of microRNA targets to the 3Tuntranslated region in mammalian mRNAs. *Nature Structural and Molecular Biology*, **2009**, 16, 144-50	17.6	308
170	Fate tracing of mature hepatocytes in mouse liver homeostasis and regeneration. *Journal of Clinical Investigation*, **2011**, 121, 4850-60	15.9	303
169	AAV serotype 2 vectors preferentially integrate into active genes in mice. *Nature Genetics*, **2003**, 34, 297-302	36.3	303
168	Rapid uncoating of vector genomes is the key to efficient liver transduction with pseudotyped adeno-associated virus vectors. *Journal of Virology*, **2004**, 78, 3110-22	6.6	301
167	Assessment of recombinant adenoviral vectors for hepatic gene therapy. *Human Gene Therapy*, **1993**, 4, 403-9	4.8	295
166	Efficient construction of a recombinant adenovirus vector by an improved in vitro ligation method. *Human Gene Therapy*, **1998**, 9, 2577-83	4.8	289
165	In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs. *Science*, **1993**, 262, 117-9	33.3	288
164	Selection and evaluation of clinically relevant AAV variants in a xenograft liver model. *Nature*, **2014**, 506, 382-6	50.4	279
163	Helper virus-free, optically controllable, and two-plasmid-based production of adeno-associated virus vectors of serotypes 1 to 6. *Molecular Therapy*, **2003**, 7, 839-50	11.7	272
162	High-resolution genome-wide mapping of transposon integration in mammals. *Molecular and Cellular Biology*, **2005**, 25, 2085-94	4.8	267
161	Efficient lentiviral transduction of liver requires cell cycling in vivo. *Nature Genetics*, **2000**, 24, 49-52	36.3	262

160	Unrestricted hepatocyte transduction with adeno-associated virus serotype 8 vectors in mice. *Journal of Virology*, **2005**, 79, 214-24	6.6	258
159	Long-term hepatic adenovirus-mediated gene expression in mice following CTLA4Ig administration. *Nature Genetics*, **1995**, 11, 191-7	36.3	257
158	A robust system for production of minicircle DNA vectors. *Nature Biotechnology*, **2010**, 28, 1287-9	44.5	223
157	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, 522-32	11.7	212
156	A transfer-RNA-derived small RNA regulates ribosome biogenesis. *Nature*, **2017**, 552, 57-62	50.4	204
155	The loop position of shRNAs and pre-miRNAs is critical for the accuracy of dicer processing in vivo. *Cell*, **2012**, 151, 900-911	56.2	198
154	Preclinical in vivo evaluation of pseudotyped adeno-associated virus vectors for liver gene therapy. *Blood*, **2003**, 102, 2412-9	2.2	172
153	Increasing the size of rAAV-mediated expression cassettes in vivo by intermolecular joining of two complementary vectors. *Nature Biotechnology*, **2000**, 18, 527-32	44.5	168
152	Expression of shRNA from a tissue-specific pol II promoter is an effective and safe RNAi therapeutic. *Molecular Therapy*, **2008**, 16, 1630-6	11.7	167
151	Linear DNAs concatemerize in vivo and result in sustained transgene expression in mouse liver. *Molecular Therapy*, **2001**, 3, 403-10	11.7	166
150	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, 14771-6	11.5	165
149	Hepatic gene therapy: persistent expression of human alpha 1-antitrypsin in mice after direct gene delivery in vivo. *Human Gene Therapy*, **1992**, 3, 641-7	4.8	164
148	Transposition from a gutless adeno-transposon vector stabilizes transgene expression in vivo. *Nature Biotechnology*, **2002**, 20, 999-1005	44.5	162
147	Isolation of recombinant adeno-associated virus vector-cellular DNA junctions from mouse liver. *Journal of Virology*, **1999**, 73, 5438-47	6.6	162
146	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, 9451-63	6.6	160
145	The kinetics of rAAV integration in the liver. *Nature Genetics*, **1998**, 19, 13-5	36.3	159
144	Human COL7A1-corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa. *Science Translational Medicine*, **2014**, 6, 264ra163	17.5	157
143	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, 327-31	50.5	152

142	Improved production and purification of minicircle DNA vector free of plasmid bacterial sequences and capable of persistent transgene expression in vivo. *Human Gene Therapy*, **2005**, 16, 126-31	4.8	151
141	Argonaute proteins are key determinants of RNAi efficacy, toxicity, and persistence in the adult mouse liver. *Journal of Clinical Investigation*, **2010**, 120, 3106-19	15.9	145
140	A bright cyan-excitable orange fluorescent protein facilitates dual-emission microscopy and enhances bioluminescence imaging in vivo. *Nature Biotechnology*, **2016**, 34, 760-7	44.5	143
139	Large-scale molecular characterization of adeno-associated virus vector integration in mouse liver. *Journal of Virology*, **2005**, 79, 3606-14	6.6	139
138	Lack of germline transmission of vector sequences following systemic administration of recombinant AAV-2 vector in males. *Molecular Therapy*, **2001**, 4, 586-92	11.7	136
137	Therapeutic levels of human factor VIII and IX using HIV-1Based lentiviral vectors in mouse liver. *Blood*, **2000**, 96, 1173-1176	2.2	135
136	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, 9239-47	4.8	130
135	Silencing of episomal transgene expression in liver by plasmid bacterial backbone DNA is independent of CpG methylation. *Molecular Therapy*, **2008**, 16, 548-56	11.7	129
134	Helper-Independent Sleeping Beauty transposon-transposase vectors for efficient nonviral gene delivery and persistent gene expression in vivo. *Molecular Therapy*, **2003**, 8, 654-65	11.7	124
133	In vivo correction of murine tyrosinemia type I by DNA-mediated transposition. *Molecular Therapy*, **2002**, 6, 759-69	11.7	124
132	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, 451-9	15.1	123
131	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, 3793-803	6.6	118
130	FATP2 is a hepatic fatty acid transporter and peroxisomal very long-chain acyl-CoA synthetase. *American Journal of Physiology - Endocrinology and Metabolism*, **2010**, 299, E384-93	6	117
129	Hepatocyte transplantation: clinical and experimental application. *Journal of Molecular Medicine*, **2001**, 79, 617-30	5.5	116
128	Therapeutic application of RNAi: is mRNA targeting finally ready for prime time?. *Journal of Clinical Investigation*, **2007**, 117, 3633-41	15.9	115
127	A new adenoviral helper-dependent vector results in long-term therapeutic levels of human coagulation factor IX at low doses in vivo. *Blood*, **2002**, 99, 3923-30	2.2	114
126	Liver tissue engineering at extrahepatic sites in mice as a potential new therapy for genetic liver diseases. *Hepatology*, **2005**, 41, 132-40	11.2	113
125	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, 22186-92	5.4	111

124	Histone modifications are associated with the persistence or silencing of vector-mediated transgene expression in vivo. *Molecular Therapy*, **2007**, 15, 1348-55	11.7	110
123	Site-directed transposon integration in human cells. *Nucleic Acids Research*, **2007**, 35, e50	20.1	108
122	Hepatocyte growth factor induces hepatocyte proliferation in vivo and allows for efficient retroviral-mediated gene transfer in mice. *Hepatology*, **1998**, 28, 707-16	11.2	101
121	Bioengineered AAV Capsids with Combined High Human Liver Transduction In Vivo and Unique Humoral Seroreactivity. *Molecular Therapy*, **2018**, 26, 289-303	11.7	97
120	Adeno-associated virus gene repair corrects a mouse model of hereditary tyrosinemia in vivo. *Hepatology*, **2010**, 51, 1200-8	11.2	97
119	Adenovirus transduction is required for the correction of diabetes using Pdx-1 or Neurogenin-3 in the liver. *Molecular Therapy*, **2007**, 15, 255-63	11.7	97
118	Minicircle DNA vectors achieve sustained expression reflected by active chromatin and transcriptional level. *Molecular Therapy*, **2013**, 21, 131-8	11.7	91
117	Method for multiple portal vein infusions in mice: quantitation of adenovirus-mediated hepatic gene transfer. *BioTechniques*, **1996**, 20, 278-85	2.5	91
116	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, 164-73	11.7	90
115	The host response to adenovirus, helper-dependent adenovirus, and adeno-associated virus in mouse liver. *Molecular Therapy*, **2008**, 16, 931-41	11.7	89
114	Recombinant AAV as a platform for translating the therapeutic potential of RNA interference. *Molecular Therapy*, **2014**, 22, 692-701	11.7	88
113	Liver transduction with recombinant adeno-associated virus is primarily restricted by capsid serotype not vector genotype. *Journal of Virology*, **2006**, 80, 426-39	6.6	86
112	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, 11343-9	6.6	86
111	Integrating adenovirus-adeno-associated virus hybrid vectors devoid of all viral genes. *Journal of Virology*, **1999**, 73, 9314-24	6.6	84
110	Genome editing of human embryonic stem cells and induced pluripotent stem cells with zinc finger nucleases for cellular imaging. *Circulation Research*, **2012**, 111, 1494-503	15.7	81
109	Novel minicircle vector for gene therapy in murine myocardial infarction. *Circulation*, **2009**, 120, S230-7	16.7	81
108	Determinants of hepatitis C translational initiation in vitro, in cultured cells and mice. *Molecular Therapy*, **2002**, 5, 676-84	11.7	81
107	Adeno-Associated Virus Vectors and Hematology. *Blood*, **1999**, 94, 864-874	2.2	81

106	Minicircle DNA-based gene therapy coupled with immune modulation permits long-term expression of □L-iduronidase in mice with mucopolysaccharidosis type I. *Molecular Therapy*, **2011**, 19, 450-60	11.7	77
105	Therapeutic serum concentrations of human alpha-1-antitrypsin after adenoviral-mediated gene transfer into mouse hepatocytes. *Hepatology*, **1995**, 21, 815-819	11.2	76
104	Episomal persistence of recombinant adenoviral vector genomes during the cell cycle in vivo. *Journal of Virology*, **2003**, 77, 7689-95	6.6	74
103	Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid □glucosidase. *Science Translational Medicine*, **2017**, 9,	17.5	72
102	Nonhomologous-end-joining factors regulate DNA repair fidelity during Sleeping Beauty element transposition in mammalian cells. *Molecular and Cellular Biology*, **2003**, 23, 8505-18	4.8	70
101	A gene-deleted adenoviral vector results in phenotypic correction of canine hemophilia B without liver toxicity or thrombocytopenia. *Blood*, **2003**, 102, 2403-11	2.2	69
100	Host factors that impact the biodistribution and persistence of multipotent adult progenitor cells. *Blood*, **2006**, 107, 4182-8	2.2	68
99	A potent and specific morpholino antisense inhibitor of hepatitis C translation in mice. *Hepatology*, **2003**, 38, 503-8	11.2	68
98	A direct comparison of two nonviral gene therapy vectors for somatic integration: in vivo evaluation of the bacteriophage integrase phiC31 and the Sleeping Beauty transposase. *Molecular Therapy*, **2005**, 11, 695-706	11.7	66
97	RNA interference. Drugging RNAi. *Science*, **2015**, 347, 1069-70	33.3	65
96	Hyperactive sleeping beauty transposase enables persistent phenotypic correction in mice and a canine model for hemophilia B. *Molecular Therapy*, **2010**, 18, 1896-906	11.7	65
95	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, 9208-13	11.5	64
94	Capped small RNAs and MOV10 in human hepatitis delta virus replication. *Nature Structural and Molecular Biology*, **2008**, 15, 714-21	17.6	64
93	RNAi and gene therapy: a mutual attraction. *Hematology American Society of Hematology Education Program*, **2007**, 473-81	3.1	64
92	A rapid protocol for construction and production of high-capacity adenoviral vectors. *Nature Protocols*, **2009**, 4, 547-64	18.8	56
91	Somatic correction of junctional epidermolysis bullosa by a highly recombinogenic AAV variant. *Molecular Therapy*, **2014**, 22, 725-33	11.7	54
90	Radioprotection in vitro and in vivo by minicircle plasmid carrying the human manganese superoxide dismutase transgene. *Human Gene Therapy*, **2008**, 19, 820-6	4.8	52
89	The role of DNA-PKcs and artemis in opening viral DNA hairpin termini in various tissues in mice. *Journal of Virology*, **2007**, 81, 11304-21	6.6	50

88	The extragenic spacer length between the 5Tand 3Tends of the transgene expression cassette affects transgene silencing from plasmid-based vectors. *Molecular Therapy*, **2012**, 20, 2111-9	11.7	47
87	Role of hepatocyte direct hyperplasia in lentivirus-mediated liver transduction in vivo. *Human Gene Therapy*, **2002**, 13, 653-63	4.8	47
86	DNA palindromes with a modest arm length of greater, similar 20 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, 11290-303	6.6	46
85	Novel NanoLuc substrates enable bright two-population bioluminescence imaging in animals. *Nature Methods*, **2020**, 17, 852-860	21.6	46
84	Multiplexed in vivo homology-directed repair and tumor barcoding enables parallel quantification of Kras variant oncogenicity. *Nature Communications*, **2017**, 8, 2053	17.4	44
83	Novel codon-optimized mini-intronic plasmid for efficient, inexpensive, and xeno-free induction of pluripotency. *Scientific Reports*, **2015**, 5, 8081	4.9	44
82	A mini-intronic plasmid (MIP): a novel robust transgene expression vector in vivo and in vitro. *Molecular Therapy*, **2013**, 21, 954-63	11.7	44
81	Helper-independent and AAV-ITR-independent chromosomal integration of double-stranded linear DNA vectors in mice. *Molecular Therapy*, **2003**, 7, 101-11	11.7	44
80	Looking into the safety of AAV vectors. *Nature*, **2003**, 424, 251	50.4	43
79	Increased maintenance and persistence of transgenes by excision of expression cassettes from plasmid sequences in vivo. *Human Gene Therapy*, **2005**, 16, 558-70	4.8	42
78	A modified urokinase plasminogen activator induces liver regeneration without bleeding. *Human Gene Therapy*, **1995**, 6, 1029-37	4.8	42
77	Survival Advantage of Both Human Hepatocyte Xenografts and Genome-Edited Hepatocytes for Treatment of 1 Antitrypsin Deficiency. *Molecular Therapy*, **2017**, 25, 2477-2489	11.7	41
76	The effect of age on hepatic gene transfer with self-inactivating lentiviral vectors in vivo. *Molecular Therapy*, **2003**, 8, 314-23	11.7	40
75	Cis-acting gene regulatory activities in the terminal regions of sleeping beauty DNA transposon-based vectors. *Human Gene Therapy*, **2007**, 18, 1193-204	4.8	38
74	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, 146-56	11.7	37
73	Low-level shRNA cytotoxicity can contribute to MYC-induced hepatocellular carcinoma in adult mice. *Molecular Therapy*, **2010**, 18, 161-70	11.7	35
72	Real-time in vivo imaging of stem cells following transgenesis by transposition. *Molecular Therapy*, **2005**, 12, 42-8	11.7	35
71	Adenoviral preterminal protein stabilizes mini-adenoviral genomes in vitro and in vivo. *Nature Biotechnology*, **1997**, 15, 1383-7	44.5	34

70	Optimization of cis-acting elements for gene expression from nonviral vectors in vivo. *Human Gene Therapy*, **2003**, 14, 215-25	4.8	34
69	miR-122 removal in the liver activates imprinted microRNAs and enables more effective microRNA-mediated gene repression. *Nature Communications*, **2018**, 9, 5321	17.4	34
68	Stability and repeat regeneration potential of the engineered liver tissues under the kidney capsule in mice. *Cell Transplantation*, **2005**, 14, 621-7	4	33
67	Future of rAAV Gene Therapy: Platform for RNAi, Gene Editing, and Beyond. *Human Gene Therapy*, **2017**, 28, 361-372	4.8	32
66	Expression determinants of mammalian argonaute proteins in mediating gene silencing. *Nucleic Acids Research*, **2012**, 40, 3704-13	20.1	32
65	Using a barcoded AAV capsid library to select for clinically relevant gene therapy vectors. *JCI Insight* , **2019**, 4,	9.9	32
64	A universal system to select gene-modified hepatocytes in vivo. *Science Translational Medicine*, **2016**, 8, 342ra79	17.5	31
63	Combined proteomic-RNAi screen for host factors involved in human hepatitis delta virus replication. *Rna*, **2009**, 15, 1971-9	5.8	31
62	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, 4755-66	6.6	31
61	Promoterless gene targeting without nucleases rescues lethality of a Crigler-Najjar syndrome mouse model. *EMBO Molecular Medicine*, **2017**, 9, 1346-1355	12	28
60	AAV vectors containing rDNA homology display increased chromosomal integration and transgene persistence. *Molecular Therapy*, **2012**, 20, 1902-11	11.7	28
59	Translational data from adeno-associated virus-mediated gene therapy of hemophilia B in dogs. *Human Gene Therapy Clinical Development*, **2015**, 26, 5-14	3.2	25
58	RNA interference-induced hepatotoxicity results from loss of the first synthesized isoform of microRNA-122 in mice. *Nature Medicine*, **2016**, 22, 557-62	50.5	25
57	Organ size control is dominant over Rb family inactivation to restrict proliferation in vivo. *Cell Reports*, **2014**, 8, 371-81	10.6	24
56	Ribosomal DNA integrating rAAV-rDNA vectors allow for stable transgene expression. *Molecular Therapy*, **2012**, 20, 1912-23	11.7	24
55	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	10.6	21
54	rAAV-mediated tumorigenesis: still unresolved after an AAV assault. *Molecular Therapy*, **2012**, 20, 2014-7	11.7	20
53	Pathways of removal of free DNA vector ends in normal and DNA-PKcs-deficient SCID mouse hepatocytes transduced with rAAV vectors. *Human Gene Therapy*, **2003**, 14, 871-81	4.8	20

52	Regulation of microRNA-mediated gene silencing by microRNA precursors. *Nature Structural and Molecular Biology*, **2014**, 21, 825-32	17.6	19
51	Correction of DNA protein kinase deficiency by spliceosome-mediated RNA trans-splicing and sleeping beauty transposon delivery. *Molecular Therapy*, **2007**, 15, 1273-9	11.7	19
50	Rapid and stable knockdown of an endogenous gene in retinal pigment epithelium. *Human Gene Therapy*, **2007**, 18, 871-80	4.8	19
49	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, 112-21	11.7	19
48	Transfer RNA-Derived Small RNAs: Another Layer of Gene Regulation and Novel Targets for Disease Therapeutics. *Molecular Therapy*, **2020**, 28, 2340-2357	11.7	19
47	An in vitro-identified high-affinity nucleosome-positioning signal is capable of transiently positioning a nucleosome in vivo. *Epigenetics and Chromatin*, **2010**, 3, 13	5.8	18
46	Regulated complex assembly safeguards the fidelity of Sleeping Beauty transposition. *Nucleic Acids Research*, **2017**, 45, 311-326	20.1	17
45	Weak base pairing in both seed and 3Tregions reduces RNAi off-targets and enhances si/shRNA designs. *Nucleic Acids Research*, **2014**, 42, 12169-76	20.1	17
44	Modified infusion procedures affect recombinant adeno-associated virus vector type 2 transduction in the liver. *Human Gene Therapy*, **2005**, 16, 299-306	4.8	17
43	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	2.2	17
42	Coupling AAV-mediated promoterless gene targeting to SaCas9 nuclease to efficiently correct liver metabolic diseases. *JCI Insight*, **2019**, 5,	9.9	15
41	Evolution of a Human-Specific Tandem Repeat Associated with ALS. *American Journal of Human Genetics*, **2020**, 107, 445-460	11	15
40	An orange calcium-modulated bioluminescent indicator for non-invasive activity imaging. *Nature Chemical Biology*, **2019**, 15, 433-436	11.7	14
39	Allele-Specific Silencing Ameliorates Restrictive Cardiomyopathy Attributable to a Human Myosin Regulatory Light Chain Mutation. *Circulation*, **2019**, 140, 765-778	16.7	14
38	Tracking Adeno-Associated Virus Capsid Evolution by High-Throughput Sequencing. *Human Gene Therapy*, **2020**, 31, 553-564	4.8	12
37	A 5TNoncoding Exon Containing Engineered Intron Enhances Transgene Expression from Recombinant AAV Vectors in vivo. *Human Gene Therapy*, **2017**, 28, 125-134	4.8	11
36	Bioengineered Viral Platform for Intramuscular Passive Vaccine Delivery to Human Skeletal Muscle. *Molecular Therapy - Methods and Clinical Development*, **2018**, 10, 144-155	6.4	11
35	The anti-genomic (negative) strand of Hepatitis C Virus is not targetable by shRNA. *Nucleic Acids Research*, **2013**, 41, 3688-98	20.1	11

34	Promoterless, Nuclease-Free Genome Editing Confers a Growth Advantage for Corrected Hepatocytes in Mice With Methylmalonic Acidemia. *Hepatology*, **2021**, 73, 2223-2237	11.2	11
33	Osteosarcoma Derived from Cultured Mesenchymal Stem Cells.. *Blood*, **2006**, 108, 2554-2554	2.2	10
32	Hepatic parenchymal replacement in mice by transplanted allogeneic hepatocytes is facilitated by bone marrow transplantation and mediated by CD4 cells. *Hepatology*, **2008**, 47, 706-18	11.2	9
31	Development of a clinical protocol for hepatic gene transfer: lessons learned in preclinical studies. *Pediatric Research*, **1993**, 33, 313-20	3.2	9
30	Sequence-Modified Antibiotic Resistance Genes Provide Sustained Plasmid-Mediated Transgene Expression in Mammals. *Molecular Therapy*, **2017**, 25, 1187-1198	11.7	8
29	Selecting the Best AAV Capsid for Human Studies. *Molecular Therapy*, **2015**, 23, 1800-1	11.7	7
28	Extracellular matrix component cotransplantation prolongs survival of heterotopically transplanted human hepatocytes in mice. *Transplantation Proceedings*, **2004**, 36, 2469-70	1.1	7
27	RNA structure probing reveals the structural basis of Dicer binding and cleavage. *Nature Communications*, **2021**, 12, 3397	17.4	7
26	Increased precursor microRNA-21 following status epilepticus can compete with mature microRNA-21 to alter translation. *Experimental Neurology*, **2016**, 286, 137-146	5.7	7
25	Donor-derived, liver-specific protein expression after bone marrow transplantation. *Transplantation*, **2004**, 78, 530-6	1.8	6
24	Characterization of vector-based delivery of neurogenin-3 in murine diabetes. *Human Gene Therapy* , **2014**, 25, 651-61	4.8	4
23	Evaluating the Genomic Parameters Governing rAAV-Mediated Homologous Recombination. *Molecular Therapy*, **2021**, 29, 1028-1046	11.7	3
22	Viral Vectors Take On HIV Infection. *New England Journal of Medicine*, **2015**, 373, 770-2	59.2	2
21	Translational Data from AAV-Mediated Gene Therapy of Hemophilia B in Dogs. *Human Gene Therapy Clinical Development*, **2014**, 150127063140004	3.2	2
20	Adeno-Associated Viral Vector Mediated Gene Transfer for Hemophilia B. *Blood*, **2011**, 118, 5-5	2.2	2
19	Escherichia coli Sepsis and Prolonged Hypophosphatemia Following Exertional Heat Stroke. *Pediatrics*, **1990**, 86, 307-309	7.4	2
18	Improved Genome Editing through Inhibition of FANCM and Members of the BTR Dissolvase Complex. *Molecular Therapy*, **2021**, 29, 1016-1027	11.7	2
17	AAV vectors engineered to target insulin receptor greatly enhance intramuscular gene delivery. *Molecular Therapy - Methods and Clinical Development*, **2020**, 19, 496-506	6.4	1

16	Gene transfer for hemophilia and viral infection. *International Journal of Hematology*, **2002**, 76, 147-147	2.3	1
15	Stable Factor IX Activity Following AAV-Mediated Gene Transfer in Patients with Severe Hemophilia B. *Blood*, **2012**, 120, 752-752	2.2	1
14	The 3′ tsRNAs are aminoacylated: Implications for their biogenesis. *PLoS Genetics*, **2021**, 17, e1009675	6	1
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