## List of Publications by Year in descending order

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#	Article	IF	CITATIONS
1	Novel adeno-associated viruses from rhesus monkeys as vectors for human gene therapy. Proceedings of the National Academy of Sciences of the United States of America, 2002, 99, 11854-11859.	7.1	1,398
2	Treatment of Leber Congenital Amaurosis Due to <i>RPE65</i> Mutations by Ocular Subretinal Injection of Adeno-Associated Virus Gene Vector: Short-Term Results of a Phase I Trial. Human Gene Therapy, 2008, 19, 979-990.	2.7	880
3	A dual AAV system enables the Cas9-mediated correction of a metabolic liver disease in newborn mice. Nature Biotechnology, 2016, 34, 334-338.	17.5	476
4	Chronic suppression of heart-failure progression by a pseudophosphorylated mutant of phospholamban via in vivo cardiac rAAV gene delivery. Nature Medicine, 2002, 8, 864-871.	30.7	344
5	Sustained transgene expression despite T lymphocyte responses in a clinical trial of rAAV1-AAT gene therapy. Proceedings of the National Academy of Sciences of the United States of America, 2009, 106, 16363-16368.	7.1	295
6	Adeno-Associated Virus Antibody Profiles in Newborns, Children, and Adolescents. Vaccine Journal, 2011, 18, 1586-1588.	3.1	269
7	Hybrid Vectors Based on Adeno-Associated Virus Serotypes 2 and 5 for Muscle-Directed Gene Transfer. Journal of Virology, 2001, 75, 6199-6203.	3.4	203
8	Heparin binding directs activation of T cells against adeno-associated virus serotype 2 capsid. Nature Medicine, 2006, 12, 967-971.	30.7	193
9	Total correction of hemophilia A mice with canine FVIII using an AAV 8 serotype. Blood, 2004, 103, 1253-1260.	1.4	188
10	Dosage Thresholds for AAV2 and AAV8 Photoreceptor Gene Therapy in Monkey. Science Translational Medicine, 2011, 3, 88ra54.	12.4	179
11	Sustained Expression of Therapeutic Level of Factor IX in Hemophilia B Dogs by AAV-Mediated Gene Therapy in Liver. Molecular Therapy, 2000, 1, 154-158.	8.2	171
12	Impact of Pre-Existing Immunity on Gene Transfer to Nonhuman Primate Liver with Adeno-Associated Virus 8 Vectors. Human Gene Therapy, 2011, 22, 1389-1401.	2.7	170
13	Sustained correction of disease in naive and AAV2-pretreated hemophilia B dogs: AAV2/8-mediated, liver-directed gene therapy. Blood, 2005, 105, 3079-3086.	1.4	162
14	Biology of AAV Serotype Vectors in Liver-Directed Gene Transfer to Nonhuman Primates. Molecular Therapy, 2006, 13, 77-87.	8.2	161
15	The Pleiotropic Effects of Natural AAV Infections on Liver-directed Gene Transfer in Macaques. Molecular Therapy, 2010, 18, 126-134.	8.2	123
16	Scalable mRNA and siRNA Lipid Nanoparticle Production Using a Parallelized Microfluidic Device. Nano Letters, 2021, 21, 5671-5680.	9.1	120
17	Ionizable lipid nanoparticles encapsulating barcoded mRNA for accelerated in vivo delivery screening. Journal of Controlled Release, 2019, 316, 404-417.	9.9	111
18	Systematic Evaluation of AAV Vectors for Liver directed Gene Transfer in Murine Models. Molecular Therapy, 2010, 18, 118-125.	8.2	110

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19	Gene transfer into skeletal muscle using novel AAV serotypes. Journal of Gene Medicine, 2005, 7, 442-451.	2.8	108
20	Cross-Presentation of Adeno-Associated Virus Serotype 2 Capsids Activates Cytotoxic T Cells But Does Not Render Hepatocytes Effective Cytolytic Targets. Human Gene Therapy, 2007, 18, 185-194.	2.7	105
21	Noninvasive gene transfer to the lung for systemic delivery of therapeutic proteins. Journal of Clinical Investigation, 2002, 110, 499-504.	8.2	104
22	No Evidence for Tumorigenesis of AAV Vectors in a Large-Scale Study in Mice. Molecular Therapy, 2005, 12, 299-306.	8.2	103
23	Meganuclease targeting of PCSK9 in macaque liver leads to stable reduction in serum cholesterol. Nature Biotechnology, 2018, 36, 717-725.	17.5	95
24	Comparative Study of Liver Gene Transfer With AAV Vectors Based on Natural and Engineered AAV Capsids. Molecular Therapy, 2015, 23, 1877-1887.	8.2	94
25	Hepatic Gene Transfer in Neonatal Mice by Adeno-Associated Virus Serotype 8 Vector. Human Gene Therapy, 2012, 23, 533-539.	2.7	90
26	Adeno-Associated Virus-Mediated Gene Transfer to Nonhuman Primate Liver Can Elicit Destructive Transgene-Specific T Cell Responses. Human Gene Therapy, 2009, 20, 930-942.	2.7	88
27	AAV8-mediated Hepatic Gene Transfer in Infant Rhesus Monkeys (Macaca mulatta). Molecular Therapy, 2011, 19, 2012-2020.	8.2	85
28	Helper lipid structure influences protein adsorption and delivery of lipid nanoparticles to spleen and liver. Biomaterials Science, 2021, 9, 1449-1463.	5.4	84
29	Inhibition of protein phosphatase 1 by inhibitorâ€2 gene delivery ameliorates heart failure progression in genetic cardiomyopathy. FASEB Journal, 2006, 20, 1197-1199.	0.5	77
30	Noninvasive gene transfer to the lung for systemic delivery of therapeutic proteins. Journal of Clinical Investigation, 2002, 110, 499-504.	8.2	76
31	Host Immune Responses to Chronic Adenovirus Infections in Human and Nonhuman Primates. Journal of Virology, 2009, 83, 2623-2631.	3.4	61
32	Inverse zonation of hepatocyte transduction with AAV vectors between mice and non-human primates. Molecular Genetics and Metabolism, 2011, 104, 395-403.	1.1	58
33	CRISPR/Cas9-mediated in vivo gene targeting corrects hemostasis in newborn and adult factor IX–knockout mice. Blood, 2019, 133, 2745-2752.	1.4	57
34	Development and rescue of human familial hypercholesterolaemia in a xenograft mouse model. Nature Communications, 2015, 6, 7339.	12.8	51
35	AAV8 Induces Tolerance in Murine Muscle as a Result of Poor APC Transduction, T Cell Exhaustion, and Minimal MHCI Upregulation on Target Cells. Molecular Therapy, 2014, 22, 28-41.	8.2	50
36	Preclinical evaluation of a clinical candidate AAV8 vector for ornithine transcarbamylase (OTC) deficiency reveals functional enzyme from each persisting vector genome. Molecular Genetics and Metabolism, 2012, 105, 203-211.	1.1	46

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37	A mutation-independent CRISPR-Cas9–mediated gene targeting approach to treat a murine model of ornithine transcarbamylase deficiency. Science Advances, 2020, 6, eaax5701.	10.3	44
38	Long-term stable reduction of low-density lipoprotein in nonhuman primates following inÂvivo genome editing of PCSK9. Molecular Therapy, 2021, 29, 2019-2029.	8.2	42
39	AAV gene therapy corrects OTC deficiency and prevents liver fibrosis in aged OTC-knock out heterozygous mice. Molecular Genetics and Metabolism, 2017, 120, 299-305.	1.1	39
40	Adenovirus-mediated overexpression of follistatin enlarges intact liver of adult rats. Hepatology, 2003, 38, 1107-1115.	7.3	38
41	Evaluation of Adeno-Associated Viral Vectors for Liver-Directed Gene Transfer in Dogs. Human Gene Therapy, 2011, 22, 985-997.	2.7	35
42	ITR-Seq, a next-generation sequencing assay, identifies genome-wide DNA editing sites in vivo following adeno-associated viral vector-mediated genome editing. BMC Genomics, 2020, 21, 239.	2.8	35
43	Characterization of Adeno-Associated Viral Vector-Mediated Human Factor VIII Gene Therapy in Hemophilia A Mice. Human Gene Therapy, 2017, 28, 392-402.	2.7	29
44	Mapping the Structural Determinants Responsible for Enhanced T Cell Activation to the Immunogenic Adeno-Associated Virus Capsid from Isolate Rhesus 32.33. Journal of Virology, 2013, 87, 9473-9485.	3.4	24
45	Cell polarity and the mechanism of asymmetric cell division. BioEssays, 1994, 16, 925-931.	2.5	23
46	Novel AAV-based genetic vaccines encoding truncated dengue virus envelope proteins elicit humoral immune responses in mice. Microbes and Infection, 2012, 14, 1000-1007.	1.9	21
47	Advances and challenges in adeno-associated viral inner-ear gene therapy for sensorineural hearing loss. Molecular Therapy - Methods and Clinical Development, 2021, 21, 209-236.	4.1	20
48	Optimized Adeno-Associated Viral-Mediated Human Factor VIII Gene Therapy in Cynomolgus Macaques. Human Gene Therapy, 2018, 29, 1364-1375.	2.7	18
49	Adeno-associated Viral Vectors for Correction of Inborn Errors of Metabolism: Progressing Towards Clinical Application. Current Pharmaceutical Design, 2011, 17, 2500-2515.	1.9	17
50	Effects of Self-Complementarity, Codon Optimization, Transgene, and Dose on Liver Transduction with AAV8. Human Gene Therapy Methods, 2016, 27, 228-237.	2.1	15
51	Developing a second-generation clinical candidate AAV vector for gene therapy of familial hypercholesterolemia. Molecular Therapy - Methods and Clinical Development, 2021, 22, 1-10.	4.1	14
52	Effect of Adenovirus-Mediated Overexpression of Follistatin and Extracellular Domain of Activin Receptor Type II on Gonadotropin Secretion in Vitro and in Vivo. Endocrinology, 2002, 143, 964-969.	2.8	13
53	Prednisolone reduces the interferon response to AAV in cynomolgus macaques and may increase liver gene expression. Molecular Therapy - Methods and Clinical Development, 2022, 24, 292-305.	4.1	10
54	Crispr/Cas9-Mediated In Vivo Gene Targeting Corrects Haemostasis in Newborn and Adult FIX-KO Mice. Blood, 2016, 128, 1174-1174.	1.4	9

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55	Activation of the mec-3 promoter in two classes of stereotyped lineages in Caenorhabditis elegans. Mechanisms of Development, 1996, 56, 165-181.	1.7	6
56	Promoter sequences for the establishment of mec-3 expression in the nematode Caenorhabditis elegans. Mechanisms of Development, 1996, 56, 183-196.	1.7	6
57	Adeno-associated virus gene therapy prevents hepatocellular adenoma in murine model of glycogen storage disease type Ia. Hepatology, 2012, 56, 1593-1595.	7.3	2
58	Strategies for Selection of AAV Vectors for Administration to Liver: Studies in Nonhuman Primates. Blood, 2016, 128, 2316-2316.	1.4	1