

# James M Wilson

## List of Publications by Year in descending order

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Version: 2024-02-01

623  
papers

62,064  
citations

435

131  
h-index

1424

221  
g-index

642  
all docs

642  
docs citations

642  
times ranked

30960  
citing authors

#	ARTICLE	IF	CITATIONS
1	Determining the Minimally Effective Dose of a Clinical Candidate Adeno-Associated Virus Vector in a Mouse Model of Hemophilia A. <i>Human Gene Therapy</i> , 2022, 33, 421-431.	2.7	2
2	Prednisolone reduces the interferon response to AAV in cynomolgus macaques and may increase liver gene expression. <i>Molecular Therapy - Methods and Clinical Development</i> , 2022, 24, 292-305.	4.1	10
3	Efficacy and Safety of a Krabbe Disease Gene Therapy. <i>Human Gene Therapy</i> , 2022, 33, 499-517.	2.7	24
4	Durable immunogenicity, adaptation to emerging variants, and low-dose efficacy of an AAV-based COVID-19 vaccine platform in macaques. <i>Molecular Therapy</i> , 2022, 30, 2952-2967.	8.2	2
5	Helper lipid structure influences protein adsorption and delivery of lipid nanoparticles to spleen and liver. <i>Biomaterials Science</i> , 2021, 9, 1449-1463.	5.4	84
6	Increasing the Specificity of AAV-Based Gene Editing through Self-Targeting and Short-Promoter Strategies. <i>Molecular Therapy</i> , 2021, 29, 1047-1056.	8.2	11
7	CRISPR/Cas9 directed to the Ube3a antisense transcript improves Angelman syndrome phenotype in mice. <i>Journal of Clinical Investigation</i> , 2021, 131, .	8.2	31
8	Scalable mRNA and siRNA Lipid Nanoparticle Production Using a Parallelized Microfluidic Device. <i>Nano Letters</i> , 2021, 21, 5671-5680.	9.1	120
9	Long-term stable reduction of low-density lipoprotein in nonhuman primates following in vivo genome editing of PCSK9. <i>Molecular Therapy</i> , 2021, 29, 2019-2029.	8.2	42
10	Intranasal gene therapy to prevent infection by SARS-CoV-2 variants. <i>PLoS Pathogens</i> , 2021, 17, e1009544.	4.7	36
11	Isolating Natural Adeno-Associated Viruses from Primate Tissues with a High-Fidelity Polymerase. <i>Human Gene Therapy</i> , 2021, 32, 1439-1449.	2.7	3
12	Adeno-Associated Virus Vector-Mediated Expression of Antirespiratory Syncytial Virus Antibody Prevents Infection in Mouse Airways. <i>Human Gene Therapy</i> , 2021, , .	2.7	4
13	Muscle-directed AAV gene therapy rescues the maple syrup urine disease phenotype in a mouse model. <i>Molecular Genetics and Metabolism</i> , 2021, 134, 139-146.	1.1	6
14	Developing a second-generation clinical candidate AAV vector for gene therapy of familial hypercholesterolemia. <i>Molecular Therapy - Methods and Clinical Development</i> , 2021, 22, 1-10.	4.1	14
15	Context-Specific Function of the Engineered Peptide Domain of PHP.B. <i>Journal of Virology</i> , 2021, 95, e0116421.	3.4	13
16	An AAV-based, room-temperature-stable, single-dose COVID-19 vaccine provides durable immunogenicity and protection in non-human primates. <i>Cell Host and Microbe</i> , 2021, 29, 1437-1453.e8.	11.0	53
17	A Single Injection of an Optimized Adeno-Associated Viral Vector into Cerebrospinal Fluid Corrects Neurological Disease in a Murine Model of GM1 Gangliosidosis. <i>Human Gene Therapy</i> , 2020, 31, 1169-1177.	2.7	22
18	Translational Feasibility of Lumbar Puncture for Intrathecal AAV Administration. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 969-974.	4.1	26

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19	MicroRNA-mediated inhibition of transgene expression reduces dorsal root ganglion toxicity by AAV vectors in primates. <i>Science Translational Medicine</i> , 2020, 12, .	12.4	96
20	Moving Forward after Two Deaths in a Gene Therapy Trial of Myotubular Myopathy. <i>Genetic Engineering and Biotechnology News</i> , 2020, 40, 14, 16.	0.1	1
21	Adeno-Associated Virus-Induced Dorsal Root Ganglion Pathology. <i>Human Gene Therapy</i> , 2020, 31, 808-818.	2.7	129
22	Adeno-associated virus serotype 1-based gene therapy for FTD caused by <i>GRN</i> mutations. <i>Annals of Clinical and Translational Neurology</i> , 2020, 7, 1843-1853.	3.7	26
23	Sensitive Determination of Infectious Titer of Recombinant Adeno-Associated Viruses (rAAVs) Using TCID <sub>50</sub> End-Point Dilution and Quantitative Polymerase Chain Reaction (qPCR). <i>Cold Spring Harbor Protocols</i> , 2020, 2020, pdb.prot095653.	0.3	5
24	ITR-Seq, a next-generation sequencing assay, identifies genome-wide DNA editing sites in vivo following adeno-associated viral vector-mediated genome editing. <i>BMC Genomics</i> , 2020, 21, 239.	2.8	35
25	Moving Forward After Two Deaths in a Gene Therapy Trial of Myotubular Myopathy. <i>Human Gene Therapy</i> , 2020, 31, 695-696.	2.7	145
26	Isolating Human Monoclonal Antibodies Against Adeno-Associated Virus From Donors With Pre-existing Immunity. <i>Frontiers in Immunology</i> , 2020, 11, 1135.	4.8	7
27	Adenovirus-Antibody Complexes Contributed to Lethal Systemic Inflammation in a Gene Therapy Trial. <i>Molecular Therapy</i> , 2020, 28, 784-793.	8.2	35
28	A mutation-independent CRISPR-Cas9-mediated gene targeting approach to treat a murine model of ornithine transcarbamylase deficiency. <i>Science Advances</i> , 2020, 6, eaax5701.	10.3	44
29	Modified Adenovirus Prime-Protein Boost Clade C HIV Vaccine Strategy Results in Reduced Viral DNA in Blood and Tissues Following Tier 2 SHIV Challenge. <i>Frontiers in Immunology</i> , 2020, 11, 626464.	4.8	4
30	Cycling at the Frontiers of Gene Therapy. <i>Human Gene Therapy Clinical Development</i> , 2019, 30, 47-49.	3.1	1
31	Ionizable lipid nanoparticles encapsulating barcoded mRNA for accelerated in vivo delivery screening. <i>Journal of Controlled Release</i> , 2019, 316, 404-417.	9.9	111
32	TLR9 signaling mediates adaptive immunity following systemic AAV gene therapy. <i>Cellular Immunology</i> , 2019, 346, 103997.	3.0	33
33	A Birds-Eye View: An Interview with Nick Leschly. <i>Human Gene Therapy Clinical Development</i> , 2019, 30, 5-6.	3.1	0
34	Breakthrough to Bedside: Bringing Gene Therapy to Neuromuscular Diseases: An Interview with Dr. Jerry R. Mendell. <i>Human Gene Therapy Clinical Development</i> , 2019, 30, 93-96.	3.1	1
35	A Gene Therapy Approach to Improve Copper Metabolism and Prevent Liver Damage in a Mouse Model of Wilson Disease. <i>Human Gene Therapy Clinical Development</i> , 2019, 30, 29-39.	3.1	14
36	Safe and Sustained Expression of Human Iduronidase After Intrathecal Administration of Adeno-Associated Virus Serotype 9 in Infant Rhesus Monkeys. <i>Human Gene Therapy</i> , 2019, 30, 957-966.	2.7	60

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37	The GPI-Linked Protein LY6A Drives AAV-PHP.B Transport across the Blood-Brain Barrier. <i>Molecular Therapy</i> , 2019, 27, 912-921.	8.2	158
38	CRISPR/Cas9-mediated in vivo gene targeting corrects hemostasis in newborn and adult factor IX <sup>−/−</sup> knockout mice. <i>Blood</i> , 2019, 133, 2745-2752.	1.4	57
39	Adeno-associated virus-mediated expression of human butyrylcholinesterase to treat organophosphate poisoning. <i>PLoS ONE</i> , 2019, 14, e0225188.	2.5	5
40	Susceptibility to SIV Infection After Adenoviral Vaccination in a Low Dose Rhesus Macaque Challenge Model. <i>Pathogens and Immunity</i> , 2019, 4, 1.	3.1	3
41	Interview with Jean Bennett, MD, PhD. <i>Human Gene Therapy Clinical Development</i> , 2018, 29, 7-9.	3.1	3
42	Assessment of Humoral, Innate, and T-Cell Immune Responses to Adeno-Associated Virus Vectors. <i>Human Gene Therapy Methods</i> , 2018, 29, 86-95.	2.1	46
43	AAV8 Gene Therapy Rescues the Newborn Phenotype of a Mouse Model of Crigler <sup>−/−</sup> Najjar. <i>Human Gene Therapy</i> , 2018, 29, 763-770.	2.7	19
44	The Neurotropic Properties of AAV-PHP.B Are Limited to C57BL/6J Mice. <i>Molecular Therapy</i> , 2018, 26, 664-668.	8.2	300
45	Severe Toxicity in Nonhuman Primates and Piglets Following High-Dose Intravenous Administration of an Adeno-Associated Virus Vector Expressing Human SMN. <i>Human Gene Therapy</i> , 2018, 29, 285-298.	2.7	543
46	AAV8-antiVEGFFab Ocular Gene Transfer for Neovascular Age-Related Macular Degeneration. <i>Molecular Therapy</i> , 2018, 26, 542-549.	8.2	36
47	Lancet Commission: Stem cells and regenerative medicine. <i>Lancet, The</i> , 2018, 391, 883-910.	13.7	184
48	Evaluation of Intrathecal Routes of Administration for Adeno-Associated Viral Vectors in Large Animals. <i>Human Gene Therapy</i> , 2018, 29, 15-24.	2.7	92
49	Combination Adenovirus and Protein Vaccines Prevent Infection or Reduce Viral Burden after Heterologous Clade C Simian-Human Immunodeficiency Virus Mucosal Challenge. <i>Journal of Virology</i> , 2018, 92, .	3.4	24
50	Tachi Yamada: An Academic, Drug Developer and Humanist. <i>Human Gene Therapy Clinical Development</i> , 2018, 29, 176-178.	3.1	0
51	AAV8 Gene Therapy for Crigler-Najjar Syndrome in Macaques Elicited Transgene T Cell Responses That Are Resident to the Liver. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 11, 191-201.	4.1	14
52	Gene Therapy Entering the Land of Oz. <i>Human Gene Therapy Clinical Development</i> , 2018, 29, 171-171.	3.1	0
53	University Flunk-Out to Genomics Pioneer: An Interview with George Church, PhD. <i>Human Gene Therapy Clinical Development</i> , 2018, 29, 118-120.	3.1	0
54	The RAC Retires After a Job Well Done. <i>Human Gene Therapy Clinical Development</i> , 2018, 29, 115-117.	3.1	0

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55	Universal protection against influenza infection by a multidomain antibody to influenza hemagglutinin. <i>Science</i> , 2018, 362, 598-602.	12.6	170
56	Deamidation of Amino Acids on the Surface of Adeno-Associated Virus Capsids Leads to Charge Heterogeneity and Altered Vector Function. <i>Molecular Therapy</i> , 2018, 26, 2848-2862.	8.2	68
57	Adeno-associated viral gene therapy corrects a mouse model of argininosuccinic aciduria. <i>Molecular Genetics and Metabolism</i> , 2018, 125, 241-250.	1.1	16
58	Accurate and Rapid Sequence Analysis of Adeno-Associated Virus Plasmids by Illumina Next-Generation Sequencing. <i>Human Gene Therapy Methods</i> , 2018, 29, 201-211.	2.1	6
59	Intrathecal Viral Vector Delivery of Trastuzumab Prevents or Inhibits Tumor Growth of Human HER2-Positive Xenografts in Mice. <i>Cancer Research</i> , 2018, 78, 6171-6182.	0.9	15
60	Preparation of Nonhuman Primate Eyes for Histological Evaluation After Retinal Gene Transfer. <i>Human Gene Therapy Methods</i> , 2018, 29, 115-123.	2.1	0
61	Standardized Method for Intra-Cisterna Magna Delivery Under Fluoroscopic Guidance in Nonhuman Primates. <i>Human Gene Therapy Methods</i> , 2018, 29, 212-219.	2.1	17
62	Toxicology Study of Intra-Cisterna Magna Adeno-Associated Virus 9 Expressing Human Alpha-L-Iduronidase in Rhesus Macaques. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 10, 79-88.	4.1	79
63	Meganuclease targeting of PCSK9 in macaque liver leads to stable reduction in serum cholesterol. <i>Nature Biotechnology</i> , 2018, 36, 717-725.	17.5	95
64	Preclinical development of a platform for enzyme therapy in the CNS of MPS I and MPS II patients based on intrathecal AAV delivery. <i>Molecular Genetics and Metabolism</i> , 2018, 123, S64.	1.1	0
65	Toxicology Study of Intra-Cisterna Magna Adeno-Associated Virus 9 Expressing Iduronate-2-Sulfatase in Rhesus Macaques. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 10, 68-78.	4.1	60
66	Mapping an Adeno-associated Virus 9-Specific Neutralizing Epitope To Develop Next-Generation Gene Delivery Vectors. <i>Journal of Virology</i> , 2018, 92, .	3.4	33
67	Determining the Minimally Effective Dose of a Clinical Candidate AAV Vector in a Mouse Model of Crigler-Najjar Syndrome. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 10, 237-244.	4.1	10
68	Optimized Adeno-Associated Viral-Mediated Human Factor VIII Gene Therapy in Cynomolgus Macaques. <i>Human Gene Therapy</i> , 2018, 29, 1364-1375.	2.7	18
69	Characterization of Adeno-Associated Viral Vector-Mediated Human Factor VIII Gene Therapy in Hemophilia A Mice. <i>Human Gene Therapy</i> , 2017, 28, 392-402.	2.7	29
70	Class I-restricted T-cell responses to a polymorphic peptide in a gene therapy clinical trial for $\alpha$ -1-antitrypsin deficiency. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2017, 114, 1655-1659.	7.1	52
71	AAV gene therapy corrects OTC deficiency and prevents liver fibrosis in aged OTC-knock out heterozygous mice. <i>Molecular Genetics and Metabolism</i> , 2017, 120, 299-305.	1.1	39
72	Jurassic Park, Gene Therapy, and Neuroscience: An Interview with Feng Zhang, PhD. <i>Human Gene Therapy Clinical Development</i> , 2017, 28, 4-6.	3.1	0

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73	5 Year Expression and Neutrophil Defect Repair after Gene Therapy in Alpha-1 Antitrypsin Deficiency. Molecular Therapy, 2017, 25, 1387-1394.	8.2	84
74	Alternative Start Sites Downstream of Non-Sense Mutations Drive Antigen Presentation and Tolerance Induction to C-Terminal Epitopes. Journal of Immunology, 2017, 198, 4581-4587.	0.8	1
75	The Past, Present, and Future of Gene Therapy from Nobel Laureate David Baltimore. Human Gene Therapy Clinical Development, 2017, 28, 65-67.	3.1	1
76	Non-Clinical Study Examining AAV8.TBG.hLDLR Vector-Associated Toxicity in Chow-Fed Wild-Type and LDLR <sup>+/Δ</sup> Rhesus Macaques. Human Gene Therapy Clinical Development, 2017, 28, 39-50.	3.1	46
77	Regulatory and Exhausted T Cell Responses to AAV Capsid. Human Gene Therapy, 2017, 28, 338-349.	2.7	35
78	Nonclinical Pharmacology/Toxicology Study of AAV8.TBG.mLDLR and AAV8.TBG.hLDLR in a Mouse Model of Homozygous Familial Hypercholesterolemia. Human Gene Therapy Clinical Development, 2017, 28, 28-38.	3.1	33
79	Wnt10b and Dkk-1 gene therapy differentially influenced trabecular bone architecture, soft tissue integrity, and osteophytosis in a skeletally mature rat model of osteoarthritis. Connective Tissue Research, 2017, 58, 542-552.	2.3	11
80	Challenges in the gene therapy commercial ecosystem. Nature Biotechnology, 2017, 35, 813-815.	17.5	6
81	The Story of RNA Interference as a New Therapeutic Paradigm from Nobel Laureate Craig Mello. Human Gene Therapy Clinical Development, 2017, 28, 121-125.	3.1	0
82	Abnormal polyamine metabolism is unique to the neuropathic forms of MPS: potential for biomarker development and insight into pathogenesis. Human Molecular Genetics, 2017, 26, 3837-3849.	2.9	5
83	2017 Was the Year We Have Been Waiting For. Human Gene Therapy Clinical Development, 2017, 28, 165-166.	3.1	2
84	Carl June Speaks of His Pioneering Efforts That Led to the First Food and Drug Administration-Approved Gene Therapy Product. Human Gene Therapy Clinical Development, 2017, 28, 175-177.	3.1	1
85	The Gene Therapy Resource Program: A Decade of Dedication to Translational Research by the National Heart, Lung, and Blood Institute. Human Gene Therapy Clinical Development, 2017, 28, 178-186.	3.1	2
86	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
87	Impact of intravenous infusion time on AAV8 vector pharmacokinetics, safety, and liver transduction in cynomolgus macaques. Molecular Therapy - Methods and Clinical Development, 2016, 3, 16079.	4.1	14
88	695. An AAV8 Mutant with Better Transduction in Murine Muscle and Nasal Airway Than AAV8. Molecular Therapy, 2016, 24, S275.	8.2	0
89	699. Effective AAV9 Vector Delivery to Nasal Mucosa for Protection Against Airborne Challenge with Influenza A and B. Molecular Therapy, 2016, 24, S276.	8.2	0
90	24. Sustained Expression with Partial Correction of Neutrophil Defects 5 Years After Intramuscular rAAV1 Gene Therapy for Alpha-1 Antitrypsin Deficiency. Molecular Therapy, 2016, 24, S11-S12.	8.2	3

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91	60. Engineering AAV Vector for the Delivery of Human BuChE to Protect Against Exposure to Organophosphates. Molecular Therapy, 2016, 24, S26.	8.2	0
92	82. Mapping the Humoral Immune Response to AAV by Molecular Docking and Cryo-Electron Microscopy for the Design of Next-Generation AAV Vectors. Molecular Therapy, 2016, 24, S36.	8.2	0
93	189. Therapeutic Gene Transfer as a Treatment Option for Age-Related Macular Degeneration. Molecular Therapy, 2016, 24, S74.	8.2	0
94	227. A Dose-Escalating Preclinical Study to Determine the Efficacy, MED, and Safety of a Clinical Candidate Vector in a Mouse Model of Hemophilia B. Molecular Therapy, 2016, 24, S89.	8.2	0
95	346. AAV9 Delivery into Cerebrospinal Fluid Corrects CNS Disease in a Murine Model of Mucopolysaccharidosis Type II. Molecular Therapy, 2016, 24, S138-S139.	8.2	0
96	481. CRISPR/Cas9-Mediated In Vivo Genome Editing to Correct the OTC spfash Mutation in Newborn Mice. Molecular Therapy, 2016, 24, S190-S191.	8.2	0
97	696. TLR9 Signaling Mediates Transgene Antibody Formation. Molecular Therapy, 2016, 24, S275.	8.2	0
98	760. Optimized AAV-Mediated Human Factor VIII Gene Therapy in Hemophilia A Mice and Cynomolgus Macaques. Molecular Therapy, 2016, 24, S300.	8.2	0
99	Adeno-Associated Virus Serotype 9-Expressed ZMapp in Mice Confers Protection Against Systemic and Airway-Acquired Ebola Virus Infection. Journal of Infectious Diseases, 2016, 214, 1975-1979.	4.0	14
100	Delivery of an Adeno-Associated Virus Vector into Cerebrospinal Fluid Attenuates Central Nervous System Disease in Mucopolysaccharidosis Type II Mice. Human Gene Therapy, 2016, 27, 906-915.	2.7	36
101	Neonatal tolerance induction enables accurate evaluation of gene therapy for MPS I in a canine model. Molecular Genetics and Metabolism, 2016, 119, 124-130.	1.1	34
102	Recollections from a Pioneer Who Provided the Foundation for the Success of Gene Therapy in Treating Severe Combined Immune Deficiencies. Human Gene Therapy Clinical Development, 2016, 27, 53-56.	3.1	1
103	Intramuscular administration of AAV overcomes pre-existing neutralizing antibodies in rhesus macaques. Vaccine, 2016, 34, 6323-6329.	3.8	36
104	AAV natural infection induces broad cross-neutralizing antibody responses to multiple AAV serotypes in chimpanzees. Human Gene Therapy Clinical Development, 2016, , .	3.1	1
105	AAV Natural Infection Induces Broad Cross-Neutralizing Antibody Responses to Multiple AAV Serotypes in Chimpanzees. Human Gene Therapy Clinical Development, 2016, 27, 79-82.	3.1	58
106	Stable liver-specific expression of human IDOL in humanized mice raises plasma cholesterol. Cardiovascular Research, 2016, 110, 23-29.	3.8	12
107	Evaluation of AAV-mediated Gene Therapy for Central Nervous System Disease in Canine Mucopolysaccharidosis VII. Molecular Therapy, 2016, 24, 206-216.	8.2	70
108	Interview with Inder Verma, PhD. Human Gene Therapy Clinical Development, 2016, 27, 5-8.	3.1	1



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109	Neutralizing Antibodies Against Adeno-Associated Viral Capsids in Patients with <i>mut</i> Methylmalonic Acidemia. Human Gene Therapy, 2016, 27, 345-353.	2.7	30
110	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
111	Crispr/Cas9-Mediated In Vivo Gene Targeting Corrects Haemostasis in Newborn and Adult FIX-KO Mice. Blood, 2016, 128, 1174-1174.	1.4	9
112	Strategies for Selection of AAV Vectors for Administration to Liver: Studies in Nonhuman Primates. Blood, 2016, 128, 2316-2316.	1.4	1
113	A randomised, double-blind, placebo-controlled trial of repeated nebulisation of non-viral cystic fibrosis transmembrane conductance regulator (CFTR) gene therapy in patients with cystic fibrosis. Efficacy and Mechanism Evaluation, 2016, 3, 1-210.	0.7	22
114	90. Identification of an Adeno-Associated Virus Binding Epitope for AVB Sepharose Affinity Resin. Molecular Therapy, 2015, 23, S38-S39.	8.2	2
115	174. Liver Fibrosis in Aged OTC-KO Heterozygotes and Successful Correction by AAV8-Mediated Gene Therapy. Molecular Therapy, 2015, 23, S69.	8.2	0
116	525. CD8+ T Cell Tolerance To Epitopes Downstream of Non-Sense Mutations. Molecular Therapy, 2015, 23, S210-S211.	8.2	0
117	646. Isolation and Evaluation of Novel Anti-AAV2 and AAV3B Antibody Clones from a Human Donor. Molecular Therapy, 2015, 23, S257.	8.2	0
118	Identification of an adeno-associated virus binding epitope for AVB sepharose affinity resin. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15040.	4.1	31
119	There and Back Again: Mitchell Finer on the Journey of Biotech from Start-Up to Success. Human Gene Therapy Clinical Development, 2015, 26, 140-143.	3.1	2
120	A Journey in the Development of Gene Therapy for Inherited Disorders of the Bone Marrow. Human Gene Therapy Clinical Development, 2015, 26, 203-207.	3.1	0
121	Perspectives on Best Practices for Gene Therapy Programs. Human Gene Therapy, 2015, 26, 127-133.	2.7	14
122	The Next Chapter. Human Gene Therapy, 2015, 26, 331-331.	2.7	1
123	A Call to Arms for Improved Vector Analytics!. Human Gene Therapy Methods, 2015, 26, 1-2.	2.1	6
124	$\beta$ -Defensin 1 Plays a Role in Acute Mucosal Defense against <i>Candida albicans</i> . Journal of Immunology, 2015, 194, 1788-1795.	0.8	76
125	Repeated nebulisation of non-viral CFTR gene therapy in patients with cystic fibrosis: a randomised, double-blind, placebo-controlled, phase 2b trial. Lancet Respiratory Medicine, 2015, 3, 684-691.	10.7	344
126	Development and rescue of human familial hypercholesterolaemia in a xenograft mouse model. Nature Communications, 2015, 6, 7339.	12.8	51



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127	Neonatal Systemic AAV Induces Tolerance to CNS Gene Therapy in MPS I Dogs and Nonhuman Primates. <i>Molecular Therapy</i> , 2015, 23, 1298-1307.	8.2	72
128	Preexisting Neutralizing Antibodies to Adeno-Associated Virus Capsids in Large Animals Other Than Monkeys May Confound <i>In Vivo</i> Gene Therapy Studies. <i>Human Gene Therapy Methods</i> , 2015, 26, 103-105.	2.1	52
129	Motor Neuron Transduction After Intracisternal Delivery of AAV9 in a Cynomolgus Macaque. <i>Human Gene Therapy Methods</i> , 2015, 26, 43-44.	2.1	6
130	Human immune system mice immunized with <i>Plasmodium falciparum</i> circumsporozoite protein induce protective human humoral immunity against malaria. <i>Journal of Immunological Methods</i> , 2015, 427, 42-50.	1.4	30
131	Comparative Study of Liver Gene Transfer With AAV Vectors Based on Natural and Engineered AAV Capsids. <i>Molecular Therapy</i> , 2015, 23, 1877-1887.	8.2	94
132	Humoral and Cell-Mediated Immune Response, and Growth Factor Synthesis After Direct Intraarticular Injection of rAAV2-IGF-I and rAAV5-IGF-I in the Equine Middle Carpal Joint. <i>Human Gene Therapy</i> , 2015, 26, 161-171.	2.7	15
133	Structure of neurotropic adeno-associated virus AAVrh.8. <i>Journal of Structural Biology</i> , 2015, 192, 21-36.	2.8	47
134	Human Gene Therapy Clinical Development: Where the Academy and Industry Meet. <i>Human Gene Therapy Clinical Development</i> , 2015, 26, 139-139.	3.1	0
135	Widespread gene transfer in the central nervous system of cynomolgus macaques following delivery of AAV9 into the cisterna magna. <i>Molecular Therapy - Methods and Clinical Development</i> , 2014, 1, 14051.	4.1	84
136	Intramuscular Injection of AAV8 in Mice and Macaques Is Associated with Substantial Hepatic Targeting and Transgene Expression. <i>PLoS ONE</i> , 2014, 9, e112268.	2.5	47
137	AAV Vectors Expressing LDLR Gain-of-Function Variants Demonstrate Increased Efficacy in Mouse Models of Familial Hypercholesterolemia. <i>Circulation Research</i> , 2014, 115, 591-599.	4.5	44
138	P204 Immune Responses To Single And Repeated Administration Of Pgm169/g167a: The Uk Cf Gene Therapy Consortium Clinical Trials. <i>Thorax</i> , 2014, 69, A166-A166.	5.6	0
139	AAV8 Induces Tolerance in Murine Muscle as a Result of Poor APC Transduction, T Cell Exhaustion, and Minimal MHC I Upregulation on Target Cells. <i>Molecular Therapy</i> , 2014, 22, 28-41.	8.2	50
140	<i>In Vivo</i> Evaluation of Adeno-Associated Virus Gene Transfer in Airways of Mice with Acute or Chronic Respiratory Infection. <i>Human Gene Therapy</i> , 2014, 25, 966-976.	2.7	10
141	Genetic Diseases, Immunology, Viruses, and Gene Therapy. <i>Human Gene Therapy</i> , 2014, 25, 257-261.	2.7	8
142	Formation of Newly Synthesized Adeno-Associated Virus Capsids in the Cell Nucleus. <i>Human Gene Therapy Methods</i> , 2014, 25, 179-180.	2.1	0
143	AAV8 capsid variable regions at the two-fold symmetry axis contribute to high liver transduction by mediating nuclear entry and capsid uncoating. <i>Virology</i> , 2014, 454-455, 227-236.	2.4	14
144	Monocular and binocular low-contrast visual acuity and optical coherence tomography in pediatric multiple sclerosis. <i>Multiple Sclerosis and Related Disorders</i> , 2014, 3, 326-334.	2.0	41

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145	Absolute Determination of Single-Stranded and Self-Complementary Adeno-Associated Viral Vector Genome Titers by Droplet Digital PCR. <i>Human Gene Therapy Methods</i> , 2014, 25, 115-125.	2.1	132
146	The First Journal on Human Gene Therapy Celebrates its 25th Anniversary. <i>Human Gene Therapy</i> , 2014, 25, 1-2.	2.7	5
147	Liver-directed gene therapy corrects cardiovascular lesions in feline mucopolysaccharidosis type I. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2014, 111, 14894-14899.	7.1	42
148	Intrathecal Gene Therapy Corrects CNS Pathology in a Feline Model of Mucopolysaccharidosis I. <i>Molecular Therapy</i> , 2014, 22, 2018-2027.	8.2	89
149	Adeno-Associated Virus 9-Mediated Airway Expression of Antibody Protects Old and Immunodeficient Mice against Influenza Virus. <i>Vaccine Journal</i> , 2014, 21, 1528-1533.	3.1	31
150	Increased Mucosal CD4 <sup>+</sup> T Cell Activation in Rhesus Macaques following Vaccination with an Adenoviral Vector. <i>Journal of Virology</i> , 2014, 88, 8468-8478.	3.4	29
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