Donald B Kohn

List of Publications by Year
in descending order

296 papers	20,260 citations	12330 69 h-index	13771 129 g-index
305 all docs	305 docs citations	305 times ranked	16717 citing authors

#	Article	IF	Citations
1	Gene therapy comes of age. Science, 2018, 359, .	12.6	936
2	Human hematopoietic stem/progenitor cells modified by zinc-finger nucleases targeted to CCR5 control HIV-1 in vivo. Nature Biotechnology, 2010, 28, 839-847.	17.5	618
3	Engraftment of geneâ€“modified umbilical cord blood cells in neonates with adenosine deaminase deficiency. Nature Medicine, 1995, 1, 1017-1023.	30.7	616
4	Transplantation Outcomes for Severe Combined Immunodeficiency, 2000â€“2009. New England Journal of Medicine, 2014, 371, 434-446.	27.0	594
5	Newborn Screening for Severe Combined Immunodeficiency in 11 Screening Programs in the United States. JAMA - Journal of the American Medical Association, 2014, 312, 729.	7.4	586
6	Lack of expression from a retroviral vector after transduction of murine hematopoietic stem cells is associated with methylation in vivo.. Proceedings of the National Academy of Sciences of the United States of America, 1994, 91, 2567-2571.	7.1	414
7	Analyzing CRISPR genome-editing experiments with CRISPResso. Nature Biotechnology, 2016, 34, 695-697.	17.5	410
8	Stable transduction of quiescent CD34⁺CD38^{â^'}human hematopoietic cells by HIV-1-based lentiviral vectors. Proceedings of the National Academy of Sciences of the United States of America, 1999, 96, 2988-2993.	7.1	395
9	Selection-free genome editing of the sickle mutation in human adult hematopoietic stem/progenitor cells. Science Translational Medicine, 2016, 8, 360ra134.	12.4	386
10	A Modified Î³-Retrovirus Vector for X-Linked Severe Combined Immunodeficiency. New England Journal of Medicine, 2014, 371, 1407-1417.	27.0	358
11	Occurrence of leukaemia following gene therapy of X-linked SCID. Nature Reviews Cancer, 2003, 3, 477-488.	28.4	323
12	T lymphocytes with a normal ADA gene accumulate after transplantation of transduced autologous umbilical cord blood CD34+ cells in ADA-deficient SCID neonates. Nature Medicine, 1998, 4, 775-780.	30.7	321
13	A Single CRISPR-Cas9 Deletion Strategy that Targets the Majority of DMD Patients Restores Dystrophin Function in hiPSC-Derived Muscle Cells. Cell Stem Cell, 2016, 18, 533-540.	11.1	307
14	Immune response to green fluorescent protein: implications for gene therapy. Gene Therapy, 1999, 6, 1305-1312.	4.5	306
15	Establishing diagnostic criteria for severe combined immunodeficiency disease (SCID), leaky SCID, and Omenn syndrome: The Primary Immune Deficiency Treatment Consortium experience. Journal of Allergy and Clinical Immunology, 2014, 133, 1092-1098.	2.9	301
16	Correction of the sickle cell disease mutation in human hematopoietic stem/progenitor cells. Blood, 2015, 125, 2597-2604.	1.4	292
17	A Clinical Trial of Retroviral-Mediated Transfer of arev-Responsive Element Decoy Gene Into CD34+Cells From the Bone Marrow of Human Immunodeficiency Virus-1â€“Infected Children. Blood, 1999, 94, 368-371.	1.4	258
18	Redirecting Specificity of T-Cell Populations For CD19 Using the Sleeping Beauty System. Cancer Research, 2008, 68, 2961-2971.	0.9	232

#	Article	IF	Citations
19	Gene therapy for adenosine deaminaseâ€“deficient severe combined immune deficiency: clinical comparison of retroviral vectors and treatment plans. Blood, 2012, 120, 3635-3646.	1.4	222
20	Genetic therapies against HIV. Nature Biotechnology, 2007, 25, 1444-1454.	17.5	214
21	Immune reconstitution and survival of 100 SCID patients postâ€“hematopoietic cell transplant: a PIDTC natural history study. Blood, 2017, 130, 2718-2727.	1.4	212
22	Adoptive Transfer of MART-1 T-Cell Receptor Transgenic Lymphocytes and Dendritic Cell Vaccination in Patients with Metastatic Melanoma. Clinical Cancer Research, 2014, 20, 2457-2465.	7.0	204
23	Transduction of pluripotent human hematopoietic stem cells demonstrated by clonal analysis after engraftment in immune-deficient mice.. Proceedings of the National Academy of Sciences of the United States of America, 1996, 93, 2414-2419.	7.1	190
24	Newborn screening for severe combined immunodeficiency and T-cell lymphopenia in California: Results of the first 2 years. Journal of Allergy and Clinical Immunology, 2013, 132, 140-150.e7.	2.9	189
25	Hematopoietic Stem Cell Gene Therapy: Progress and Lessons Learned. Cell Stem Cell, 2017, 21, 574-590.	11.1	181
26	Transient Gene Expression by Nonintegrating Lentiviral Vectors. Molecular Therapy, 2006, 13, 1121-1132.	8.2	175
27	Lentiviral gene therapy for X-linked chronic granulomatous disease. Nature Medicine, 2020, 26, 200-206.	30.7	175
28	Generation of mature T cells from human hematopoietic stem and progenitor cells in artificial thymic organoids. Nature Methods, 2017, 14, 521-530.	19.0	165
29	FLT3 Ligand Preserves the Ability of Human CD34+ Progenitors to Sustain Long-Term Hematopoiesis in Immune-Deficient Mice After Ex Vivo Retroviral-Mediated Transduction. Blood, 1997, 89, 446-456.	1.4	157
30	CRISPR/Cas9-Mediated Correction of the Sickle Mutation in Human CD34+ cells. Molecular Therapy, 2016, 24, 1561-1569.	8.2	157
31	Critical Factors Influencing Stable Transduction of Human CD34+ Cells with HIV-1-Derived Lentiviral Vectors. Molecular Therapy, 2000, 2, 71-80.	8.2	154
32	Long-term efficacy of enzyme replacement therapy for Adenosine deaminase (ADA)-deficient Severe Combined Immunodeficiency (SCID). Clinical Immunology, 2005, 117, 133-143.	3.2	154
33	Dynamic tracking of human hematopoietic stem cell engraftment using in vivo bioluminescence imaging. Blood, 2003, 102, 3478-3482.	1.4	149
34	Newborn Screening for Severe Combined Immunodeficiency and T-cell Lymphopenia in California, 2010â€“2017. Pediatrics, 2019, 143, .	2.1	148
35	American society of gene therapy (ASGT) ad hoc subcommittee on retroviral-mediated gene transfer to hematopoietic stem cells. Molecular Therapy, 2003, 8, 180-187.	8.2	147
36	Improved Expression in Hematopoietic and Lymphoid Cells in Mice After Transplantation of Bone Marrow Transduced With a Modified Retroviral Vector. Blood, 1999, 94, 3349-3357.	1.4	146

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37	Retroviral Transfer of the Glucocerebrosidase Gene into CD34⁺Cells from Patients with Gaucher Disease:In VivoDetection of Transduced Cells without Myeloablation. Human Gene Therapy, 1998, 9, 2629-2640.	2.7	144
38	Prostate cancer originating in basal cells progresses to adenocarcinoma propagated by luminal-like cells. Proceedings of the National Academy of Sciences of the United States of America, 2013, 110, 20111-20116.	7.1	144
39	Marrow-Derived Cells as Vehicles for Delivery of Gene Therapy to Pulmonary Epithelium. American Journal of Respiratory Cell and Molecular Biology, 2002, 27, 645-651.	2.9	138
40	Clonality analysis after retroviral-mediated gene transfer to CD34+ cells from the cord blood of ADA-deficient SCID neonates. Nature Medicine, 2003, 9, 463-468.	30.7	134
41	Comparison of the Effects of Growth Factors on Retroviral Vector-Mediated Gene Transfer and the Proliferative Status of Human Hematopoietic Progenitor Cells. Human Gene Therapy, 1990, 1, 257-268.	2.7	131
42	Inhibition of HIV-1 in human T-lymphocytes by retrovirally transduced anti-tat and rev hammerhead ribozymes. Gene, 1994, 149, 33-39.	2.2	129
43	SCID genotype and 6-month posttransplant CD4 count predict survival and immune recovery. Blood, 2018, 132, 1737-1749.	1.4	128
44	Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. New England Journal of Medicine, 2021, 384, 2002-2013.	27.0	122
45	Site-Specific Gene Editing of Human Hematopoietic Stem Cells for X-Linked Hyper-IgM Syndrome. Cell Reports, 2018, 23, 2606-2616.	6.4	119
46	Inhibition of Human Immunodeficiency Virus-1 (HIV-1) Replication After Transduction of Granulocyte Colony-Stimulating Factorâ€“Mobilized CD34+ Cells From HIV-1â€“Infected Donors Using Retroviral Vectors Containing Antiâ€“HIV-1 Genes. Blood, 1997, 89, 2259-2267.	1.4	116
47	Neonatal Gene Therapy of MPS I Mice by Intravenous Injection of a Lentiviral Vector. Molecular Therapy, 2005, 11, 776-789.	8.2	114
48	Retroviral Transfer of the Glucocerebrosidase Gene into CD34+ Cells from Patients with Gaucher Disease: In Vivo Detection of Transduced Cells without Myeloablation. Human Gene Therapy, 1998, 9, 2629-2640.	2.7	112
49	Improving cellular therapy for primary immune deficiency diseases: Recognition, diagnosis, and management. Journal of Allergy and Clinical Immunology, 2009, 124, 1152-1160.e12.	2.9	110
50	Consensus approach for the management of severe combined immune deficiency caused by adenosine deaminase deficiency. Journal of Allergy and Clinical Immunology, 2019, 143, 852-863.	2.9	104
51	Factors Influencing the Titer and Infectivity of Lentiviral Vectors. Human Gene Therapy, 2004, 15, 976-988.	2.7	102
52	Lentiviral vectors for efficient delivery of CD80 and granulocyte-macrophageâ€“ colony-stimulating factor in human acute lymphoblastic leukemia and acute myeloid leukemia cells to induce antileukemic immune responses. Blood, 2000, 96, 1317-1326.	1.4	100
53	The Natural History of Children with Severe Combined Immunodeficiency: Baseline Features of the First Fifty Patients of the Primary Immune Deficiency Treatment Consortium Prospective Study 6901. Journal of Clinical Immunology, 2013, 33, 1156-1164.	3.8	100
54	Antitumor activity from antigen-specific CD8 T cells generated in vivo from genetically engineered human hematopoietic stem cells. Proceedings of the National Academy of Sciences of the United States of America, 2011, 108, E1408-16.	7.1	97

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55	Î²-globin gene transfer to human bone marrow for sickle cell disease. Journal of Clinical Investigation, 2013, 123, 3317-3330.	8.2	92
56	Infection of Human Marrow Stroma by Human Immunodeficiency Virus-1 (HIV-1) Is Both Required and Sufficient for HIV-1â€"Induced Hematopoietic Suppression In Vitro: Demonstration by Gene Modification of Primary Human Stroma. Blood, 1997, 90, 1787-1798.	1.4	91
57	Gene Therapy Fulfilling Its Promise. New England Journal of Medicine, 2009, 360, 518-521.	27.0	88
58	Excellent outcomes following hematopoietic cell transplantation for Wiskott-Aldrich syndrome: a PIDTC report. Blood, 2020, 135, 2094-2105.	1.4	87
59	Editing the Sickle Cell Disease Mutation in Human Hematopoietic Stem Cells: Comparison of Endonucleases and Homologous Donor Templates. Molecular Therapy, 2019, 27, 1389-1406.	8.2	83
60	An In Vitro Model of Human Red Blood Cell Production From Hematopoietic Progenitor Cells. Blood, 1998, 91, 2664-2671.	1.4	82
61	Advances in lentiviral vector design for gene-modification of hematopoietic stem cells. Current Opinion in Biotechnology, 2002, 13, 429-436.	6.6	81
62	Adenosine Deaminase (ADA)-Deficient Severe Combined Immune Deficiency (SCID): Molecular Pathogenesis and Clinical Manifestations. Journal of Clinical Immunology, 2017, 37, 626-637.	3.8	78
63	Selective survival of peripheral blood lymphocytes in children with HIV-1 following delivery of an anti-HIV gene to bone marrow CD34+ cells. Molecular Therapy, 2005, 12, 77-86.	8.2	77
64	Preclinical Demonstration of Lentiviral Vector-mediated Correction of Immunological and Metabolic Abnormalities in Models of Adenosine Deaminase Deficiency. Molecular Therapy, 2014, 22, 607-622.	8.2	77
65	Gene therapy of RAG-2â^'/â^' mice: sustained correction of the immunodeficiency. Blood, 2002, 100, 3942-3949.	1.4	76
66	Treatment of the mouse model of mucopolysaccharidosis I with retrovirally transduced bone marrow. Molecular Genetics and Metabolism, 2003, 79, 233-244.	1.1	76
67	Integrated Self-Inactivating Lentiviral Vectors Produce Full-Length Genomic Transcripts Competent for Encapsidation and Integration. Journal of Virology, 2004, 78, 8421-8436.	3.4	76
68	Antiviral drug screen identifies DNA-damage response inhibitor as potent blocker of SARS-CoV-2 replication. Cell Reports, 2021, 35, 108940.	6.4	76
69	Hematopoietic Stem Cell Gene Therapy for the Multisystemic Lysosomal Storage Disorder Cystinosis. Molecular Therapy, 2013, 21, 433-444.	8.2	74
70	The enhanced green fluorescent protein (eGFP) is minimally immunogenic in C57BL/6 mice. Gene Therapy, 2001, 8, 1813-1814.	4.5	71
71	Allogeneic hematopoietic cell transplantation for primary immune deficiency diseases: Current status and critical needs. Journal of Allergy and Clinical Immunology, 2008, 122, 1087-1096.	2.9	70
72	In Vivo Biosafety Model to Assess the Risk of Adverse Events From Retroviral and Lentiviral Vectors. Molecular Therapy, 2008, 16, 1308-1315.	8.2	70

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73	Improving Gene Editing Outcomes in Human Hematopoietic Stem and Progenitor Cells by Temporal Control of DNA Repair. Stem Cells, 2019, 37, 284-294.	3.2	70
74	Clinical efficacy of gene-modified stem cells in adenosine deaminaseâ€“deficient immunodeficiency. Journal of Clinical Investigation, 2017, 127, 1689-1699.	8.2	70
75	Rhesus Monkey Model for Fetal Gene Transfer: Studies with Retroviral- Based Vector Systems. Molecular Therapy, 2001, 3, 128-138.	8.2	69
76	Intrapulmonary and intramyocardial gene transfer in rhesus monkeys (Macaca mulatta): Safety and efficiency of HIV-1-derived lentiviral vectors for fetal gene delivery. Molecular Therapy, 2005, 12, 87-98.	8.2	66
77	The current status of gene therapy using hematopoietic stem cells. Current Opinion in Pediatrics, 1995, 7, 56-63.	2.0	65
78	The woodchuck hepatitis virus post-transcriptional regulatory element reduces readthrough transcription from retroviral vectors. Gene Therapy, 2007, 14, 1298-1304.	4.5	65
79	Hematopoietic stem cells for cancer immunotherapy. Immunological Reviews, 2014, 257, 237-249.	6.0	65
80	Primary Immune Deficiency Treatment Consortium (PIDTC) report. Journal of Allergy and Clinical Immunology, 2014, 133, 335-347.e11.	2.9	65
81	Artificial thymic organoids represent a reliable tool to study T-cell differentiation in patients with severe T-cell lymphopenia. Blood Advances, 2020, 4, 2611-2616.	5.2	65
82	Human Gene Marker/Therapy Clinical Protocols (Complete Updated Listings). Human Gene Therapy, 2000, 11, 919-979.	2.7	64
83	Update on gene therapy for immunodeficiencies. Clinical Immunology, 2010, 135, 247-254.	3.2	64
84	The genetic landscape of severe combined immunodeficiency in the United States and Canada in the current era (2010-2018). Journal of Allergy and Clinical Immunology, 2019, 143, 405-407.	2.9	64
85	Integrase-defective Lentiviral Vectors as a Delivery Platform for Targeted Modification of Adenosine Deaminase Locus. Molecular Therapy, 2013, 21, 1705-1717.	8.2	63
86	Anti-human CD117 antibody-mediated bone marrow niche clearance in nonhuman primates and humanized NSG mice. Blood, 2019, 133, 2104-2108.	1.4	63
87	Lentiviral Vector Gene Transfer into Fetal Rhesus Monkeys (Macaca mulatta): Lung-Targeting Approaches. Molecular Therapy, 2001, 4, 614-621.	8.2	62
88	Highly efficient large-scale lentiviral vector concentration by tandem tangential flow filtration. Journal of Virological Methods, 2011, 177, 1-9.	2.1	60
89	Gene Therapy Using Hematopoietic Stem Cells: Sisyphus Approaches the Crest. Human Gene Therapy, 2000, 11, 1259-1267.	2.7	58
90	Novel Pol II Fusion Promoter Directs Human Immunodeficiency Virus Type 1-Inducible Coexpression of a Short Hairpin RNA and Protein. Journal of Virology, 2006, 80, 1863-1873.	3.4	56

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91	In Vivo Transduction by Intravenous Injection of a Lentiviral Vector Expressing Human ADA into Neonatal ADA Gene Knockout Mice: A Novel Form of Enzyme Replacement Therapy for ADA Deficiency. Molecular Therapy, 2006, 13, 1110-1120.	8.2	56
92	The Effects of Campath 1H upon Graft-Versus-Host Disease, Infection, Relapse, and Immune Reconstitution in Recipients of Pediatric Unrelated Transplants. Biology of Blood and Marrow Transplantation, 2007, 13, 584-593.	2.0	56
93	Progressive Declines in Neurocognitive Function Among Survivors of Hematopoietic Stem Cell Transplantation for Pediatric Hematologic Malignancies. Journal of Pediatric Hematology/Oncology, 2008, 30, 411-418.	0.6	56
94	Stable gene transfer to human CD34+ hematopoietic cells using the Sleeping Beauty transposon. Experimental Hematology, 2006, 34, 1333-1343.	0.4	55
95	Manipulation of OCT4 Levels in Human Embryonic Stem Cells Results in Induction of Differential Cell Types. Experimental Biology and Medicine, 2007, 232, 1368-1380.	2.4	55
96	Gene Therapy for the Treatment of Recurrent Pediatric Malignant Astrocytomas with In Vivo Tumor Transduction with the Herpes Simplex Thymidine Kinase Gene/Ganciclovir System. Childrens Hospital, Los Angeles, California. Human Gene Therapy, 1994, 5, 863-890.	2.7	54
97	Retroviral Mediated Transfer of the cDNA for Human Glucocerebrosidase into Hematopoietic Stem Cells of Patients with Gaucher Disease. A Phase I Study. National Institutes of Health, Bethesda, Maryland. Human Gene Therapy, 1996, 7, 231-253.	2.7	54
98	T lymphocyte ontogeny in adenosine deaminase-deficient severe combined immune deficiency after treatment with polyethylene glycol-modified adenosine deaminase.. Journal of Clinical Investigation, 1993, 92, 596-602.	8.2	54
99	Fetal Gene Transfer Using Lentiviral Vectors and the Potential for Germ Cell Transduction in Rhesus Monkeys (Macaca mulatta). Human Gene Therapy, 2005, 16, 417-425.	2.7	53
100	Gene Therapy for the Treatment of Primary Immune Deficiencies. Current Allergy and Asthma Reports, 2016, 16, 39.	5.3	52
101	Differentiation of RPE cells from integration-free iPS cells and their cell biological characterization. Stem Cell Research and Therapy, 2017, 8, 217.	5.5	52
102	Gene therapy for genetic haematological disorders and immunodeficiencies. Journal of Internal Medicine, 2001, 249, 379-390.	6.0	50
103	Retrovirally transduced bone marrow has a therapeutic effect on brain in the mouse model of mucopolysaccharidosis IIIB. Molecular Genetics and Metabolism, 2004, 82, 286-295.	1.1	50
104	Long-term in vivo monitoring of mouse and human hematopoietic stem cell engraftment with a human positron emission tomography reporter gene. Proceedings of the National Academy of Sciences of the United States of America, 2013, 110, 1857-1862.	7.1	50
105	Allelic Exclusion and Peripheral Reconstitution by TCR Transgenic T Cells Arising From Transduced Human Hematopoietic Stem/Progenitor Cells. Molecular Therapy, 2013, 21, 1044-1054.	8.2	49
106	Modification of Hematopoietic Stem/Progenitor Cells with CD19-Specific Chimeric Antigen Receptors as a Novel Approach for Cancer Immunotherapy. Human Gene Therapy, 2013, 24, 824-839.	2.7	49
107	Development of Hematopoietic Stem Cell-Engineered Invariant Natural Killer T Cell Therapy for Cancer. Cell Stem Cell, 2019, 25, 542-557.e9.	11.1	48
108	Domain-swapped T cell receptors improve the safety of TCR gene therapy. ELife, 2016, 5, .	6.0	48

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109	Toward Gene Therapy for Gaucher Disease. Human Gene Therapy, 1991, 2, 101-105.	2.7	47
110	Stable Transgene Expression in Primitive Human CD34⁺Hematopoietic Stem/Progenitor Cells, Using theSleeping BeautyTransposon System. Human Gene Therapy, 2009, 20, 1607-1626.	2.7	46
111	Comparison of Gene Transfer Efficiencies and Gene Expression Levels Achieved with Equine Infectious Anemia Virus- and Human Immunodeficiency Virus Type 1-Derived Lentivirus Vectors. Journal of Virology, 2002, 76, 1510-1515.	3.4	45
112	Myeloid dysplasia and bone marrow hypocellularity in adenosine deaminase-deficient severe combined immune deficiency. Blood, 2011, 118, 2688-2694.	1.4	45
113	Reactivating Fetal Hemoglobin Expression in Human Adult Erythroblasts Through BCL11A Knockdown Using Targeted Endonucleases. Molecular Therapy - Nucleic Acids, 2016, 5, e351.	5.1	45
114	Lentiviral Gene Therapy in HSCs Restores Lineage-Specific Foxp3 Expression and Suppresses Autoimmunity in a Mouse Model of IPEX Syndrome. Cell Stem Cell, 2019, 24, 309-317.e7.	11.1	45
115	HIV-1-derived lentiviral vectors and fetal route of administration on transgene biodistribution and expression in rhesus monkeys. Gene Therapy, 2005, 12, 821-830.	4.5	44
116	Direct FGF-2 Gene Transfer via Recombinant Adeno-Associated Virus Vectors Stimulates Cell Proliferation, Collagen Production, and the Repair of Experimental Lesions in the Human ACL. American Journal of Sports Medicine, 2013, 41, 194-202.	4.2	44
117	Lentivirus Mediated Correction of Artemis-Deficient Severe Combined Immunodeficiency. Human Gene Therapy, 2017, 28, 112-124.	2.7	44
118	Gene Therapy for Genetic Diseases. Cancer Investigation, 1989, 7, 179-192.	1.3	43
119	Morphological Analysis and Lentiviral Transduction of Fetal Monkey Bone Marrow-Derived Mesenchymal Stem Cells. Molecular Therapy, 2004, 9, 112-123.	8.2	43
120	CD4+CD25â^' T Cells Transduced to Express MHC Class I-Restricted Epitope-Specific TCR Synthesize Th1 Cytokines and Exhibit MHC Class I-Restricted Cytolytic Effector Function in a Human Melanoma Model. Journal of Immunology, 2008, 181, 1063-1070.	0.8	43
121	From Skin Biopsy to Neurons Through a Pluripotent Intermediate Under Good Manufacturing Practice Protocols. Stem Cells Translational Medicine, 2012, 1, 36-43.	3.3	43
122	Generation and characterization of transgene-free human induced pluripotent stem cells and conversion to putative clinical-grade status. Stem Cell Research and Therapy, 2013, 4, 87.	5.5	43
123	How We Manage Adenosine Deaminase-Deficient Severe Combined Immune Deficiency (ADA SCID). Journal of Clinical Immunology, 2017, 37, 351-356.	3.8	43
124	Gene therapy/bone marrow transplantation in ADA-deficient mice: roles of enzyme-replacement therapy and cytoreduction. Blood, 2012, 120, 3677-3687.	1.4	42
125	Lentivirus Vectors Incorporating the Immunoglobulin Heavy Chain Enhancer and Matrix Attachment Regions Provide Position-Independent Expression in B Lymphocytes. Journal of Virology, 2003, 77, 7341-7351.	3.4	40
126	Rescue of splicing-mediated intron loss maximizes expression in lentiviral vectors containing the human ubiquitin C promoter. Nucleic Acids Research, 2015, 43, 682-690.	14.5	40

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127	Myoblast Gene Therapy in Canine Mucopolysaccharidosis I: Abrogation by an Immune Response toÎ±-l-Iduronidase. Human Gene Therapy, 1996, 7, 1595-1603.	2.7	39
128	Combination of CD80 and Granulocyte-Macrophage Colony-Stimulating Factor Coexpression by a Leukemia Cell Vaccine: Preclinical Studies in a Murine Model Recapitulating Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia. Human Gene Therapy, 1999, 10, 2109-2122.	2.7	39
129	Lentiviral Vector Transduction of a Dominant-negative Rev Gene Into Human CD34+ Hematopoietic Progenitor Cells Potently Inhibits Human Immunodeficiency Virus-1 Replication. Molecular Therapy, 2007, 15, 76-85.	8.2	39
130	Development of allogeneic HSC-engineered iNKT cells for off-the-shelf cancer immunotherapy. Cell Reports Medicine, 2021, 2, 100449.	6.5	39
131	New frontiers in the therapy of primary immunodeficiency: From gene addition to gene editing. Journal of Allergy and Clinical Immunology, 2017, 139, 726-732.	2.9	38
132	PGE2 and Poloxamer Synperonic F108 Enhance Transduction of Human HSPCs with a Î²-Globin Lentiviral Vector. Molecular Therapy - Methods and Clinical Development, 2019, 13, 390-398.	4.1	38
133	Preclinical correction of human Fanconi anemia complementation group A bone marrow cells using a safety-modified lentiviral vector. Gene Therapy, 2010, 17, 1244-1252.	4.5	37
134	Propagating Humanized BLT Mice for the Study of Human Immunology and Immunotherapy. Stem Cells and Development, 2016, 25, 1863-1873.	2.1	37
135	B-cell differentiation and IL-21 response in IL2RG/JAK3 SCID patients after hematopoietic stem cell transplantation. Blood, 2018, 131, 2967-2977.	1.4	37
136	The Moloney Murine Leukemia Virus Repressor Binding Site Represses Expression in Murine and Human Hematopoietic Stem Cells. Journal of Virology, 2003, 77, 9439-9450.	3.4	36
137	Prolonged pancytopenia in a gene therapy patient with ADA-deficient SCID and trisomy 8 mosaicism: a case report. Blood, 2007, 109, 503-506.	1.4	36
138	A CD19/Fc fusion protein for detection of anti-CD19 chimeric antigen receptors. Journal of Translational Medicine, 2013, 11, 23.	4.4	36
139	Ethical and regulatory aspects of genome editing. Blood, 2016, 127, 2553-2560.	1.4	36
140	Infections in Infants with SCID: Isolation, Infection Screening, and Prophylaxis in PIDTC Centers. Journal of Clinical Immunology, 2021, 41, 38-50.	3.8	36
141	Constitutive HOXA5 Expression Inhibits Erythropoiesis and Increases Myelopoiesis From Human Hematopoietic Progenitors. Blood, 1999, 94, 519-528.	1.4	36
142	Reconstitution of T cell receptor signaling in ZAP-70-deficient cells by retroviral transduction of the ZAP-70 gene.. Journal of Experimental Medicine, 1996, 184, 2031-2036.	8.5	35
143	Successful Hematopoietic Stem Cell Transplantation for Niemann-Pick Disease Type B. Pediatrics, 2005, 116, 1022-1025.	2.1	35
144	Gene Therapies for Primary Immune Deficiencies. Frontiers in Immunology, 2021, 12, 648951.	4.8	35

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145	Gene Delivery to Human B-Precursor Acute Lymphoblastic Leukemia Cells. Blood, 1998, 92, 3537-3545.	1.4	34
146	In UteroInjection ofÎ±-L-Iduronidase-Carrying Retrovirus in Canine Mucopolysaccharidosis Type I: Infection of Multiple Tissues and Neonatal Gene Expression. Human Gene Therapy, 2002, 13, 1809-1820.	2.7	34
147	Improving Gene Therapy Efficiency through the Enrichment of Human Hematopoietic Stem Cells. Molecular Therapy, 2017, 25, 2163-2175.	8.2	34
148	High-Resolution Analysis of Cytosine Methylation in the 5â€² Long Terminal Repeat of Retroviral Vectors. Human Gene Therapy, 1998, 9, 2321-2330.	2.7	33
149	Primary Immune Deficiency Treatment Consortium (PIDTC) update. Journal of Allergy and Clinical Immunology, 2016, 138, 375-385.	2.9	33
150	Gene Therapy for Sickle Cell Disease:A Lentiviral Vector Comparison Study. Human Gene Therapy, 2018, 29, 1153-1166.	2.7	33
151	Gene Therapy for Adenosine Deaminase Deficiency. Annual Review of Medicine, 2000, 51, 33-47.	12.2	32
152	Lentiviral vectors ready for prime-time. Nature Biotechnology, 2007, 25, 65-66.	17.5	32
153	Potentially therapeutic levels of anti-sickling globin gene expression following lentivirus-mediated gene transfer in sickle cell disease bone marrow CD34+ cells. Experimental Hematology, 2015, 43, 346-351.	0.4	32
154	Scaffold attachment regionâ€“containing retrovirus vectors improve long-term proviral expression after transplantation of GFP-modified CD34+ baboon repopulating cells. Blood, 2003, 102, 3117-3119.	1.4	31
155	Effective Suicide Gene Therapy for Leukemia in a Model of Insertional Oncogenesis in Mice. Molecular Therapy, 2007, 15, 183-192.	8.2	31
156	Retrovirus-mediated transfer of the human Î±- L-iduronidase cDNA into human hematopoietic progenitor cells leads to correction in trans of Hurler fibroblasts. Gene Therapy, 1997, 4, 1150-1159.	4.5	30
157	HSV-sr39TK Positron Emission Tomography and Suicide Gene Elimination of Human Hematopoietic Stem Cells and Their Progeny in Humanized Mice. Cancer Research, 2014, 74, 5173-5183.	0.9	30
158	Clinical and genetic heterogeneity in Omenn syndrome and severe combined immune deficiency. Pediatric Transplantation, 2009, 13, 244-250.	1.0	29
159	Specific and Stable Gene Transfer to Human Embryonic Stem Cells Using Pseudotyped Lentiviral Vectors. Stem Cells and Development, 2006, 15, 109-117.	2.1	28
160	Gene therapy for childhood immunological diseases. Bone Marrow Transplantation, 2008, 41, 199-205.	2.4	28
161	Foamy Virus Vectors Expressing Anti-HIV Transgenes Efficiently Block HIV-1 Replication. Molecular Therapy, 2008, 16, 46-51.	8.2	28
162	T cell dynamics and response of the microbiota after gene therapy to treat X-linked severe combined immunodeficiency. Genome Medicine, 2018, 10, 70.	8.2	28

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