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	Granulocyte Transfusions in Patients with Chronic Granulomatous Disease Undergoing Hematopoietic Cell Transplantation or Gene Therapy. Journal of Clinical Immunology, 2022, 42, 1026-1035.	3.8	3
2	High-level correction of the sickle mutation is amplified inÂvivo during erythroid differentiation. IScience, 2022, 25, 104374.	4.1	22
3	Outcomes following treatment for ADA-deficient severe combined immunodeficiency: a report from the PIDTC. Blood, 2022, 140, 685-705.	1.4	26
4	Infections in Infants with SCID: Isolation, Infection Screening, and Prophylaxis in PIDTC Centers. Journal of Clinical Immunology, 2021, 41, 38-50.	3.8	36
5	Î²-Globin Lentiviral Vectors Have Reduced Titers due to Incomplete Vector RNA Genomes and Lowered Virion Production. Stem Cell Reports, 2021, 16, 198-211.	4.8	15
6	Gene Therapies for Primary Immune Deficiencies. Frontiers in Immunology, 2021, 12, 648951.	4.8	35
7	Gene delivery using AAV8 inÂvivo for disease stabilization in a bimodal gene therapy approach for the treatment of ADA-deficient SCID. Molecular Therapy - Methods and Clinical Development, 2021, 20, 765-778.	4.1	1
8	Optimizing Integration and Expression of Transgenic Bruton's Tyrosine Kinase for CRISPR-Cas9-Mediated Gene Editing of X-Linked Agammaglobulinemia. CRISPR Journal, 2021, 4, 191-206.	2.9	17
9	Antiviral drug screen identifies DNA-damage response inhibitor as potent blocker of SARS-CoV-2 replication. Cell Reports, 2021, 35, 108940.	6.4	76
10	Regional gene therapy for bone healing using a <scp>3D</scp> printed scaffold in a rat femoral defect model. Journal of Biomedical Materials Research - Part A, 2021, 109, 2346-2356.	4.0	6
11	Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. New England Journal of Medicine, 2021, 384, 2002-2013.	27.0	122
12	Long-term outcomes after gene therapy for adenosine deaminase severe combined immune deficiency. Blood, 2021, 138, 1304-1316.	1.4	28
13	Normal IgH Repertoire Diversity in an Infant with ADA Deficiency After Gene Therapy. Journal of Clinical Immunology, 2021, 41, 1597-1606.	3.8	0
14	Regional Gene Therapy with Transduced Human Cells: The Influence of â€œCell Doseâ€•on Bone Repair. Tissue Engineering - Part A, 2021, 27, 1422-1433.	3.1	8
15	Evidence generation and reproducibility in cell and gene therapy research: A call to action. Molecular Therapy - Methods and Clinical Development, 2021, 22, 11-14.	4.1	13
16	Safe and EffectiveIn VivoTargeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development. Human Gene Therapy, 2021, 32, 31-42.	2.7	15
17	Improved lentiviral vector titers from a multi-gene knockout packaging line. Molecular Therapy - Oncolytics, 2021, 23, 582-592.	4.4	8
18	Improved SARS-CoV-2 Spike Glycoproteins for Pseudotyping Lentiviral Vectors. Frontiers in Virology, 2021, 1, .	1.4	1

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19	Development of allogeneic HSC-engineered iNKT cells for off-the-shelf cancer immunotherapy. Cell Reports Medicine, 2021, 2, 100449.	6.5	39
20	Improved Titer and Gene Transfer by Lentiviral Vectors Using Novel, Small Î²-Globin Locus Control Region Elements. Molecular Therapy, 2020, 28, 328-340.	8.2	27
21	Dosing and Re-Administration of Lentiviral Vector for InÂVivo Gene Therapy in Rhesus Monkeys and ADA-Deficient Mice. Molecular Therapy - Methods and Clinical Development, 2020, 16, 78-93.	4.1	10
22	Creating New Î²-Globin-Expressing Lentiviral Vectors by High-Resolution Mapping of Locus Control Region Enhancer Sequences. Molecular Therapy - Methods and Clinical Development, 2020, 17, 999-1013.	4.1	9
23	AT1R Activating Autoantibodies in Hematopoietic Stem Cell Transplantation. Biology of Blood and Marrow Transplantation, 2020, 26, 2061-2067.	2.0	5
24	Busulfan Pharmacokinetics in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Gene Therapy. Biology of Blood and Marrow Transplantation, 2020, 26, 1819-1827.	2.0	8
25	Human CLEC9A antibodies deliver NY-ESO-1 antigen to CD141⁺ dendritic cells to activate naÃ¯ve and memory NY-ESO-1-specific CD8⁺ T cells. , 2020, 8, e000691.		28
26	Overview of the current status of gene therapy for primary immune deficiencies (PIDs). Journal of Allergy and Clinical Immunology, 2020, 146, 229-233.	2.9	8
27	Supramolecular nanosubstrateâ€“mediated delivery system enables CRISPR-Cas9 knockin of hemoglobin beta gene for hemoglobinopathies. Science Advances, 2020, 6, .	10.3	25
28	Adenosine Deaminase (ADA)â€“Deficient Severe Combined Immune Deficiency (SCID) in the US Immunodeficiency Network (USIDNet) Registry. Journal of Clinical Immunology, 2020, 40, 1124-1131.	3.8	19
29	Global and Local Manipulation of DNA Repair Mechanisms to Alter Site-Specific Gene Editing Outcomes in Hematopoietic Stem Cells. Frontiers in Genome Editing, 2020, 2, 601541.	5.2	8
30	Excellent outcomes following hematopoietic cell transplantation for Wiskott-Aldrich syndrome: a PIDTC report. Blood, 2020, 135, 2094-2105.	1.4	87
31	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
32	Gene therapy for primary immune deficiencies. , 2020, , 1215-1228.		0
33	Lentiviral gene therapy for X-linked chronic granulomatous disease. Nature Medicine, 2020, 26, 200-206.	30.7	175
34	A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Results from Phase 1. Blood, 2020, 136, 15-15.	1.4	8
35	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
36	Newborn Screening for Severe Combined Immunodeficiency and T-cell Lymphopenia in California, 2010â€“2017. Pediatrics, 2019, 143, .	2.1	148

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37	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
38	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
39	Chemistry, manufacturing and controls for gene modified hematopoietic stem cells. Cytotherapy, 2019, 21, 358-366.	0.7	5
40	Gene therapy for blood diseases. Current Opinion in Biotechnology, 2019, 60, 39-45.	6.6	27
41	IND-Enabling Studies for a Clinical Trial to Genetically Program a Persistent Cancer-Targeted Immune System. Clinical Cancer Research, 2019, 25, 1000-1011.	7.0	9
42	Anti-human CD117 antibody-mediated bone marrow niche clearance in nonhuman primates and humanized NSG mice. Blood, 2019, 133, 2104-2108.	1.4	63
43	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
44	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
45	Gene Therapy for Primary Immune Deficiency Diseases. , 2019, , 1155-1164.e1.		0
46	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
47	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
48	Gene therapy comes of age. Science, 2018, 359, .	12.6	936
49	Characterization of Gene Alterations following Editing of the Î²-Globin Gene Locus in Hematopoietic Stem/Progenitor Cells. Molecular Therapy, 2018, 26, 468-479.	8.2	26
50	Pre-clinical Development of a Lentiviral Vector Expressing the Anti-sickling Î²AS3 Globin for Gene Therapy for Sickle Cell Disease. Molecular Therapy - Methods and Clinical Development, 2018, 11, 167-179.	4.1	16
51	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
52	Superior lentiviral vectors designed for BSL-0 environment abolish vector mobilization. Gene Therapy, 2018, 25, 454-472.	4.5	8
53	Gene Therapy for Sickle Cell Disease:A Lentiviral Vector Comparison Study. Human Gene Therapy, 2018, 29, 1153-1166.	2.7	33
54	SCID genotype and 6-month posttransplant CD4 count predict survival and immune recovery. Blood, 2018, 132, 1737-1749.	1.4	128

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55	Site-Specific Gene Editing of Human Hematopoietic Stem Cells for X-Linked Hyper-IgM Syndrome. Cell Reports, 2018, 23, 2606-2616.	6.4	119
56	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
57	How We Manage Adenosine Deaminase-Deficient Severe Combined Immune Deficiency (ADA SCID). Journal of Clinical Immunology, 2017, 37, 351-356.	3.8	43
58	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
59	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
60	Cytoreductive conditioning intensity predicts clonal diversity in ADA-SCID retroviral gene therapy patients. Blood, 2017, 129, 2624-2635.	1.4	27
61	Hematopoietic Stem Cell Gene Therapy: Progress and Lessons Learned. Cell Stem Cell, 2017, 21, 574-590.	11.1	181
62	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
63	Gene therapy: WAS (not) just for kids. Blood, 2017, 130, 1278-1279.	1.4	2
64	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
65	Historical Perspective on the Current Renaissance for Hematopoietic Stem Cell Gene Therapy. Hematology/Oncology Clinics of North America, 2017, 31, 721-735.	2.2	23
66	Preclinical studies for a phase 1 clinical trial of autologous hematopoietic stem cell gene therapy for sickle cell disease. Cytotherapy, 2017, 19, 1096-1112.	0.7	14
67	Gene Therapy. Hematology/Oncology Clinics of North America, 2017, 31, xiii-xiv.	2.2	0
68	Improving Gene Therapy Efficiency through the Enrichment of Human Hematopoietic Stem Cells. Molecular Therapy, 2017, 25, 2163-2175.	8.2	34
69	Lentivirus Mediated Correction of Artemis-Deficient Severe Combined Immunodeficiency. Human Gene Therapy, 2017, 28, 112-124.	2.7	44
70	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
71	Clinical efficacy of gene-modified stem cells in adenosine deaminaseâ€“deficient immunodeficiency. Journal of Clinical Investigation, 2017, 127, 1689-1699.	8.2	70
72	Analyzing CRISPR genome-editing experiments with CRISPResso. Nature Biotechnology, 2016, 34, 695-697.	17.5	410

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73	Genetic Tagging During Human Mesoderm Differentiation Reveals Tripotent Lateral Plate Mesodermal Progenitors. Stem Cells, 2016, 34, 1239-1250.	3.2	10
74	Gene Therapy for the Treatment of Primary Immune Deficiencies. Current Allergy and Asthma Reports, 2016, 16, 39.	5.3	52
75	Primary Immune Deficiency Treatment Consortium (PIDTC) update. Journal of Allergy and Clinical Immunology, 2016, 138, 375-385.	2.9	33
76	Ethical and regulatory aspects of genome editing. Blood, 2016, 127, 2553-2560.	1.4	36
77	Delivery of Genome Editing Reagents to Hematopoietic Stem/Progenitor Cells. Current Protocols in Stem Cell Biology, 2016, 36, 5B.4.1-5B.4.10.	3.0	8
78	Propagating Humanized BLT Mice for the Study of Human Immunology and Immunotherapy. Stem Cells and Development, 2016, 25, 1863-1873.	2.1	37
79	CRISPR/Cas9-Mediated Correction of the Sickle Mutation in Human CD34+ cells. Molecular Therapy, 2016, 24, 1561-1569.	8.2	157
80	Selection-free genome editing of the sickle mutation in human adult hematopoietic stem/progenitor cells. Science Translational Medicine, 2016, 8, 360ra134.	12.4	386
81	Reactivating Fetal Hemoglobin Expression in Human Adult Erythroblasts Through BCL11A Knockdown Using Targeted Endonucleases. Molecular Therapy - Nucleic Acids, 2016, 5, e351.	5.1	45
82	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
83	Hematopoietic Stem Cell Therapy. , 2016, , 152-159.e3.		0
84	Domain-swapped T cell receptors improve the safety of TCR gene therapy. ELife, 2016, 5, .	6.0	48
85	Preservation of Gene Edited Hematopoietic Stem Cells By Transient Overexpression of BCL-2 mRNA. Blood, 2016, 128, 3636-3636.	1.4	0
86	Gene therapy outpaces haplo for SCID-X1. Blood, 2015, 125, 3521-3522.	1.4	8
87	C-8. Immunological and Metabolic Correction After Lentiviral Vector Gene Therapy for ADA Deficiency. Molecular Therapy, 2015, 23, S102-S103.	8.2	8
88	Impulse oscillometry identifies peripheral airway dysfunction in children with adenosine deaminase deficiency. Orphanet Journal of Rare Diseases, 2015, 10, 159.	2.7	10
89	Unrelated donor hematopoietic stem cell transplantation for the treatment of nonâ€malignant genetic diseases: An alemtuzumab based regimen is associated with cure of clinical disease; earlier clearance of alemtuzumab may be associated with graft rejection. American Journal of Hematology, 2015, 90, 1021-1026.	4.1	9
90	The human ankyrin 1 promoter insulator sustains gene expression in a Î²-globin lentiviral vector in hematopoietic stem cells. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15012.	4.1	17

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91	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
92	Correction of the sickle cell disease mutation in human hematopoietic stem/progenitor cells. Blood, 2015, 125, 2597-2604.	1.4	292
93	A Reduced-Toxicity Regimen Is Associated with Durable Engraftment and Clinical Cure of Nonmalignant Genetic Diseases among Children Undergoing Blood and Marrow Transplantation with an HLA-Matched Related Donor. Biology of Blood and Marrow Transplantation, 2015, 21, 440-444.	2.0	10
94	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
95	Enrichment of Human Hematopoietic Stem/Progenitor Cells Facilitates Transduction for Stem Cell Gene Therapy. Stem Cells, 2015, 33, 1532-1542.	3.2	26
96	Putative Immunogenicity Expression Profiling Using Human Pluripotent Stem Cells and Derivatives. Stem Cells Translational Medicine, 2015, 4, 136-145.	3.3	5
97	Gene Therapy for Primary Immune Deficiencies. , 2014, , 1043-1058.		0
98	Human Lymphoid Development in the Absence of Common Î³-Chain Receptor Signaling. Journal of Immunology, 2014, 192, 5050-5058.	0.8	15
99	Effects of Vector Backbone and Pseudotype on Lentiviral Vector-mediated Gene Transfer: Studies in Infant ADA-Deficient Mice and Rhesus Monkeys. Molecular Therapy, 2014, 22, 1803-1816.	8.2	6
100	Dissecting the Mechanism of Histone Deacetylase Inhibitors to Enhance the Activity of Zinc Finger Nucleases Delivered by Integrase-Defective Lentiviral Vectors. Human Gene Therapy, 2014, 25, 599-608.	2.7	15
101	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
102	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
103	Eliminating SCID row: new approaches to SCID. Hematology American Society of Hematology Education Program, 2014, 2014, 475-480.	2.5	5
104	Hematopoietic stem cells for cancer immunotherapy. Immunological Reviews, 2014, 257, 237-249.	6.0	65
105	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
106	HIV eradicationâ€"from Berlin to Boston. Nature Biotechnology, 2014, 32, 315-316.	17.5	14
107	Erythropoiesis from Human Embryonic Stem Cells Through Erythropoietin-Independent AKT Signaling. Stem Cells, 2014, 32, 1503-1514.	3.2	9
108	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

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109	Primary Immune Deficiency Treatment Consortium (PIDTC) report. Journal of Allergy and Clinical Immunology, 2014, 133, 335-347.e11.	2.9	65
110	Gene Therapy: Charting a Future Courseâ€"Summary of a National Institutes of Health Workshop, April 12, 2013. Human Gene Therapy, 2014, 25, 488-497.	2.7	12
111	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
112	Transplantation Outcomes for Severe Combined Immunodeficiency, 2000â€"2009. New England Journal of Medicine, 2014, 371, 434-446.	27.0	594
113	A Modified Î³-Retrovirus Vector for X-Linked Severe Combined Immunodeficiency. New England Journal of Medicine, 2014, 371, 1407-1417.	27.0	358
114	Envelope, please. And the award goes toâ€¦. Blood, 2014, 124, 1203-1204.	1.4	3
115	A CD19/Fc fusion protein for detection of anti-CD19 chimeric antigen receptors. Journal of Translational Medicine, 2013, 11, 23.	4.4	36
116	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
117	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
118	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
119	Gene Therapy Through Autologous Transplantation of Gene-Modified Hematopoietic Stem Cells. Biology of Blood and Marrow Transplantation, 2013, 19, S64-S69.	2.0	23
120	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
121	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
122	Hematopoietic Stem Cell Gene Therapy for the Multisystemic Lysosomal Storage Disorder Cystinosis. Molecular Therapy, 2013, 21, 433-444.	8.2	74
123	Integrase-defective Lentiviral Vectors as a Delivery Platform for Targeted Modification of Adenosine Deaminase Locus. Molecular Therapy, 2013, 21, 1705-1717.	8.2	63
124	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
125	Î²-globin gene transfer to human bone marrow for sickle cell disease. Journal of Clinical Investigation, 2013, 123, 3317-3330.	8.2	92
126	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

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127	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
128	Zinc Finger Nucleases Targeting The Î²-Globin Locus Drive Efficient Correction Of The Sickle Mutation In CD34+ Cells. Blood, 2013, 122, 2904-2904.	1.4	1
129	A Pre-Clinical Model Of Hematopoietic Stem Cell Based Immunotherapy For Cancer Utilizing The NY-ESO-1 T-Cell Receptor and sr39TK PET Reporter / Suicide Gene. Blood, 2013, 122, 2020-2020.	1.4	0
130	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
131	Nonmyeloablative Conditioning Regimen to Increase Engraftment of Gene-modified Hematopoietic Stem Cells in Young Rhesus Monkeys. Molecular Therapy, 2012, 20, 1033-1045.	8.2	22
132	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
133	Gene therapy/bone marrow transplantation in ADA-deficient mice: roles of enzyme-replacement therapy and cytoreduction. Blood, 2012, 120, 3677-3687.	1.4	42
134	Novel Pathways to Erythropoiesis Induced by Dimerization of Intracellular C-Mpl in Human Hematopoietic Progenitors. Stem Cells, 2012, 30, 697-708.	3.2	8
135	Guidance for Developing Phase II Cell Therapy Trial Proposals for Consideration by the Blood and Marrow Transplant Clinical Trials Network. Biology of Blood and Marrow Transplantation, 2011, 17, 192-196.	2.0	1
136	Myeloid dysplasia and bone marrow hypocellularity in adenosine deaminase-deficient severe combined immune deficiency. Blood, 2011, 118, 2688-2694.	1.4	45
137	Highly efficient large-scale lentiviral vector concentration by tandem tangential flow filtration. Journal of Virological Methods, 2011, 177, 1-9.	2.1	60
138	A Tale of Two SCIDs. Science Translational Medicine, 2011, 3, 97ps36.	12.4	19
139	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
140	Somatic Gene Therapy for X-Linked Severe Combined Immunodeficiency Using a Self-Inactivating Modified Gammaretroviral Vector Results in An Improved Preclinical Safety Profile and Early Clinical Efficacy in a Human Patient. Blood, 2011, 118, 164-164.	1.4	3
141	Laser Tweezers Raman Spectroscopy As a Novel Red Blood Cell Functional Assay for Sickle Cell Disease. Blood, 2011, 118, 4847-4847.	1.4	2
142	Preclinical Studies for Sickle Cell Disease Gene Therapy Using Bone Marrow CD34+ Cells Modified with a Î²AS3-Globin Lentiviral Vector. Blood, 2011, 118, 3119-3119.	1.4	13
143	Gene Transfer to Hematopoietic Stem/Progenitor Cells As a Novel Approach for Immunotherapy Against B-Lineage Malignancies: In Vivo Xenograft Model,. Blood, 2011, 118, 4168-4168.	1.4	0
144	Update on gene therapy for immunodeficiencies. Clinical Immunology, 2010, 135, 247-254.	3.2	64

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145	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
146	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
147	Regulated Expansion of Human Pancreatic Î²-Cells. Molecular Therapy, 2010, 18, 1389-1396.	8.2	4
148	Neurocognitive Function of Patients with Severe Combined Immunodeficiency. Immunology and Allergy Clinics of North America, 2010, 30, 143-151.	1.9	2
149	Hematopoietic Stem Cell Transplantation and Gene Therapy for Primary Immune Deficiency Diseases. , 2010, , 223-231.		0
150	CD19 Fc-Fusion Protein for Detection of Cells Expressing Anti-CD19 Chimeric Antigen Receptors.. Blood, 2010, 116, 3756-3756.	1.4	0
151	Preloading Potential of Retroviral Vectors Is Packaging Cell Clone Dependent and Centrifugation onto CH-296 Ensures Highest Transduction Efficiency. Human Gene Therapy, 2009, 20, 337-349.	2.7	4
152	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
153	Long-Term Neurocognitive Function of Pediatric Patients with Severe Combined Immune Deficiency (SCID): Pre- and Post-Hematopoietic Stem Cell Transplant (HSCT). Journal of Clinical Immunology, 2009, 29, 231-237.	3.8	20
154	Preâ€•and postâ€•natal treatment of hemophagocytic lymphohistiocytosis. Pediatric Blood and Cancer, 2009, 52, 139-142.	1.5	8
155	Lentiviral vectors with amplified Î² cell-specific gene expression. Gene Therapy, 2009, 16, 998-1008.	4.5	7
156	Clinical and genetic heterogeneity in Omenn syndrome and severe combined immune deficiency. Pediatric Transplantation, 2009, 13, 244-250.	1.0	29
157	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
158	Gene Therapy Fulfilling Its Promise. New England Journal of Medicine, 2009, 360, 518-521.	27.0	88
159	Gene therapy for childhood immunological diseases. Bone Marrow Transplantation, 2008, 41, 199-205.	2.4	28
160	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
161	Neonatal bone marrow transplantation of ADA-deficient SCID mice results in immunologic reconstitution despite low levels of engraftment and an absence of selective donor T lymphoid expansion. Blood, 2008, 111, 5745-5754.	1.4	24
162	Human Hematopoietic Cell Culture, Transduction, and Analyses. Current Protocols in Human Genetics, 2008, 56, Unit 13.7.	3.5	1

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163	Foamy Virus Vectors Expressing Anti-HIV Transgenes Efficiently Block HIV-1 Replication. Molecular Therapy, 2008, 16, 46-51.	8.2	28
164	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
165	Redirecting Specificity of T-Cell Populations For CD19 Using the Sleeping Beauty System. Cancer Research, 2008, 68, 2961-2971.	0.9	232
166	Tissue-specific restriction of cyclophilin A-independent HIV-1- and SIV-derived lentiviral vectors. Gene Therapy, 2008, 15, 1079-1089.	4.5	13
167	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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