## **Axel Schambach**

List of Publications by Year in descending order

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#	Article	IF	CITATIONS
1	Successful gene therapy of Diamond-Blackfan anemia in a mouse model and human CD34 <sup>+</sup> cord blood hematopoietic stem cells using a clinically applicable lentiviral vector. Haematologica, 2022, 107, 446-456.	3.5	13
2	Improved alpharetrovirus-based Gag.MS2 particles for efficient and transient delivery of CRISPR-Cas9 into target cells. Molecular Therapy - Nucleic Acids, 2022, 27, 810-823.	5.1	8
3	Ex Vivo Generation of CAR Macrophages from Hematopoietic Stem and Progenitor Cells for Use in Cancer Therapy. Cells, 2022, 11, 994.	4.1	18
4	Selection and Validation of siRNAs Preventing Uptake and Replication of SARS-CoV-2. Frontiers in Bioengineering and Biotechnology, 2022, 10, 801870.	4.1	13
5	GMP-Compliant Manufacturing of TRUCKs: CAR T Cells targeting GD2 and Releasing Inducible IL-18. Frontiers in Immunology, 2022, 13, 839783.	4.8	20
6	Cell transcriptomic atlas of the non-human primate Macaca fascicularis. Nature, 2022, 604, 723-731.	27.8	81
7	Meteorin-like promotes heart repair through endothelial KIT receptor tyrosine kinase. Science, 2022, 376, 1343-1347.	12.6	34
8	Knockout-Induced Pluripotent Stem Cells for Disease and Therapy Modeling of IL-10-Associated Primary Immunodeficiencies. Human Gene Therapy, 2021, 32, 77-95.	2.7	9
9	The hemogenic endothelium: a critical source for the generation of PSC-derived hematopoietic stem and progenitor cells. Cellular and Molecular Life Sciences, 2021, 78, 4143-4160.	5.4	25
10	Genetic Correction of IL-10RB Deficiency Reconstitutes Anti-Inflammatory Regulation in iPSC-Derived Macrophages. Journal of Personalized Medicine, 2021, 11, 221.	2.5	5
11	Rescue from Pseudomonas aeruginosa Airway Infection via Stem Cell Transplantation. Molecular Therapy, 2021, 29, 1324-1334.	8.2	6
12	Conditionally immortalised leukaemia initiating cells co-expressing Hoxa9/Meis1 demonstrate microenvironmental adaptation properties ex vivo while maintaining myelomonocytic memory. Scientific Reports, 2021, 11, 5294.	3.3	0
13	Retroviral gene therapy in Germany with a view on previous experience and future perspectives. Gene Therapy, 2021, 28, 494-512.	4.5	15
14	Gene therapy for infantile malignant osteopetrosis: review of pre-clinical research and proof-of-concept for phenotypic reversal. Molecular Therapy - Methods and Clinical Development, 2021, 20, 389-397.	4.1	5
15	Correction of pathology in mice displaying Gaucher disease type 1 by a clinically-applicable lentiviral vector. Molecular Therapy - Methods and Clinical Development, 2021, 20, 312-323.	4.1	13
16	A combination of cyclophosphamide and interleukin-2 allows CD4+ T cells converted to Tregs to control <i>scurfy</i> syndrome. Blood, 2021, 137, 2326-2336.	1.4	9
17	Multiple Genes Surrounding <i>Bcl-x<sub>L</sub></i> , a Common Retroviral Insertion Site, Can Influence Hematopoiesis Individually or in Concert. Human Gene Therapy, 2021, 32, 458-472.	2.7	4
18	DNA methylation changes during long-term in vitro cell culture are caused by epigenetic drift. Communications Biology, 2021, 4, 598.	4.4	27

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19	An Improved Lentiviral Fluorescent Genetic Barcoding Approach Distinguishes Hematopoietic Stem Cell Properties in Multiplexed <i>In Vivo</i> Experiments. Human Gene Therapy, 2021, 32, 1280-1294.	2.7	5
20	Low Energy Electron Irradiation Is a Potent Alternative to Gamma Irradiation for the Inactivation of (CAR-)NK-92 Cells in ATMP Manufacturing. Frontiers in Immunology, 2021, 12, 684052.	4.8	11
21	Efficient Genetic Safety Switches for Future Application of iPSC-Derived Cell Transplants. Journal of Personalized Medicine, 2021, 11, 565.	2.5	11
22	Predicting genotoxicity of viral vectors for stem cell gene therapy using gene expression-based machine learning. Molecular Therapy, 2021, 29, 3383-3397.	8.2	25
23	Improved Activity against Acute Myeloid Leukemia with Chimeric Antigen Receptor (CAR)-NK-92 Cells Designed to Target CD123. Viruses, 2021, 13, 1365.	3.3	16
24	Targeted cytokine delivery: cell therapy to remodel the pre-metastatic niche. Signal Transduction and Targeted Therapy, 2021, 6, 282.	17.1	0
25	Pulmonary transplantation of alpha-1 antitrypsin (AAT)-transgenic macrophages provides a source of functional human AAT in vivo. Gene Therapy, 2021, 28, 477-493.	4.5	5
26	Therapeutic HNF4A mRNA attenuates liver fibrosis in a preclinical model. Journal of Hepatology, 2021, 75, 1420-1433.	3.7	70
27	Reprogramming enriches for somatic cell clones with small-scale mutations in cancer-associated genes. Molecular Therapy, 2021, 29, 2535-2553.	8.2	9
28	Impaired immune response mediated by prostaglandin E2 promotes severe COVID-19 disease. PLoS ONE, 2021, 16, e0255335.	2.5	48
29	A Multiplex CRISPR-Screen Identifies PLA2G4A as Prognostic Marker and Druggable Target for HOXA9 and MEIS1 Dependent AML. International Journal of Molecular Sciences, 2021, 22, 9411.	4.1	11
30	A first step toward in vivo gene editing in patients. Nature Medicine, 2021, 27, 1515-1517.	30.7	5
31	NK Cell-Mediated Eradication of Ovarian Cancer Cells with a Novel Chimeric Antigen Receptor Directed against CD44. Biomedicines, 2021, 9, 1339.	3.2	18
32	Two cases of TÂcell lymphoma following Piggybac-mediated CAR TÂcell therapy. Molecular Therapy, 2021, 29, 2631-2633.	8.2	10
33	Preclinical Optimization and Safety Studies of a New Lentiviral Gene Therapy for p47 <sup>phox</sup> -Deficient Chronic Granulomatous Disease. Human Gene Therapy, 2021, 32, 949-958.	2.7	4
34	Generation of hiPSC-derived low threshold mechanoreceptors containing axonal termini resembling bulbous sensory nerve endings and expressing Piezo1 and Piezo2. Stem Cell Research, 2021, 56, 102535.	0.7	4
35	Gene Therapy "Made in Germanyâ€: A Historical Perspective, Analysis of the Status Quo, and Recommendations for Action by the German Society for Gene Therapy. Human Gene Therapy, 2021, 32, 987-996.	2.7	3
36	Generation of an NFκB-Driven Alpharetroviral "All-in-One―Vector Construct as a Potent Tool for CAR NK Cell Therapy. Frontiers in Immunology, 2021, 12, 751138.	4.8	11

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37	Effective hematopoietic stem cell-based gene therapy in a murine model of hereditary pulmonary alveolar proteinosis. Haematologica, 2020, 105, 1147-1157.	3.5	7
38	RAD50 regulates mitotic progression independent of DNA repair functions. FASEB Journal, 2020, 34, 2812-2820.	0.5	9
39	Conditional Immortalization of Lymphoid Progenitors via Tetracycline-Regulated <i>LMO2</i> Expression. Human Gene Therapy, 2020, 31, 183-198.	2.7	3
40	ExÂVivo/InÂvivo Gene Editing in Hepatocytes Using "All-in-One―CRISPR-Adeno-Associated Virus Vectors with a Self-Linearizing Repair Template. IScience, 2020, 23, 100764.	4.1	33
41	Inducible Forward Programming of Human Pluripotent Stem Cells to Hemato-endothelial Progenitor Cells with Hematopoietic Progenitor Potential. Stem Cell Reports, 2020, 14, 122-137.	4.8	27
42	Differential Transgene Silencing of Myeloid-Specific Promoters in the <i>AAVS1</i> Safe Harbor Locus of Induced Pluripotent Stem Cell-Derived Myeloid Cells. Human Gene Therapy, 2020, 31, 199-210.	2.7	31
43	Competitive sgRNA Screen Identifies p38 MAPK as a Druggable Target to Improve HSPC Engraftment. Cells, 2020, 9, 2194.	4.1	3
44	EVI1 phosphorylation at S436 regulates interactions with CtBP1 and DNMT3A and promotes self-renewal. Cell Death and Disease, 2020, 11, 878.	6.3	4
45	Use of Cell and Genome Modification Technologies to Generate Improved "Off-the-Shelf―CAR T and CAR NK Cells. Frontiers in Immunology, 2020, 11, 1965.	4.8	85
46	β-Catenin safeguards the ground state of mousepluripotency by strengthening the robustness of the transcriptional apparatus. Science Advances, 2020, 6, eaba1593.	10.3	10
47	Hematopoietic stem-cell senescence and myocardial repair - Coronary artery disease genotype/phenotype analysis of post-MI myocardial regeneration response induced by CABG/CD133+ bone marrow hematopoietic stem cell treatment in RCT PERFECT Phase 3. EBioMedicine, 2020, 57, 102862.	6.1	22
48	CAR-T cells and TRUCKs that recognize an EBNA-3C-derived epitope presented on HLA-B*35 control Epstein-Barr virus-associated lymphoproliferation. , 2020, 8, e000736.		27
49	Cooperating, congenital neutropenia–associated Csf3r and Runx1 mutations activate pro-inflammatory signaling and inhibit myeloid differentiation of mouse HSPCs. Annals of Hematology, 2020, 99, 2329-2338.	1.8	5
50	Synthetic Notch-Receptor-Mediated Transmission of a Transient Signal into Permanent Information via CRISPR/Cas9-Based Genome Editing. Cells, 2020, 9, 1929.	4.1	3
51	Preclinical Evaluation of a Novel Lentiviral Vector Driving Lineage-Specific BCL11A Knockdown for Sickle Cell Gene Therapy. Molecular Therapy - Methods and Clinical Development, 2020, 17, 589-600.	4.1	39
52	Effective drug treatment identified by in vivo screening in a transplantable patient-derived xenograft model of chronic myelomonocytic leukemia. Leukemia, 2020, 34, 2951-2963.	7.2	13
53	Design and Characterization of an "All-in-One―Lentiviral Vector System Combining Constitutive Anti-GD2 CAR Expression and Inducible Cytokines. Cancers, 2020, 12, 375.	3.7	68
54	Lentiviral gene therapy and vitamin B3 treatment enable granulocytic differentiation of G6PC3-deficient induced pluripotent stem cells. Gene Therapy, 2020, 27, 297-306.	4.5	8

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55	Challenges and advances in translating gene therapy for hearing disorders. Expert Review of Precision Medicine and Drug Development, 2020, 5, 23-34.	0.7	4
56	Successful Preclinical Development of Gene Therapy for Recombinase-Activating Gene-1-Deficient SCID. Molecular Therapy - Methods and Clinical Development, 2020, 17, 666-682.	4.1	37
57	Targeted Integration of Inducible Caspase-9 in Human iPSCs Allows Efficient in vitro Clearance of iPSCs and iPSC-Macrophages. International Journal of Molecular Sciences, 2020, 21, 2481.	4.1	12
58	CXCR4/MIF axis amplifies tumor growth and epithelial-mesenchymal interaction in non-small cell lung cancer. Cellular Signalling, 2020, 73, 109672.	3.6	28
59	Gene therapy as a possible option to treat hereditary hearing loss. Medizinische Genetik, 2020, 32, 149-159.	0.2	2
60	Cytokine Selection of MSC Clones with Different Functionality. Stem Cell Reports, 2019, 13, 262-273.	4.8	22
61	Enhancing Lentiviral and Alpharetroviral Transduction of Human Hematopoietic Stem Cells for Clinical Application. Molecular Therapy - Methods and Clinical Development, 2019, 14, 134-147.	4.1	37
62	Biosafety Studies of a Clinically Applicable Lentiviral Vector for the Gene Therapy of Artemis-SCID. Molecular Therapy - Methods and Clinical Development, 2019, 15, 232-245.	4.1	18
63	Umbilical cord as a long-term source of activatable mesenchymal stromal cells for immunomodulation. Stem Cell Research and Therapy, 2019, 10, 285.	5.5	25
64	Targeted Repair of p47-CGD in iPSCs by CRISPR/Cas9: Functional Correction without Cleavage in the Highly Homologous Pseudogenes. Stem Cell Reports, 2019, 13, 590-598.	4.8	20
65	Hematopoietic Stem Cell-Targeted Neonatal Gene Therapy with a Clinically Applicable Lentiviral Vector Corrects Osteopetrosis in <i>oc/oc</i> Mice. Human Gene Therapy, 2019, 30, 1395-1404.	2.7	17
66	Development of Automated Separation, Expansion, and Quality Control Protocols for Clinical-Scale Manufacturing of Primary Human NK Cells and Alpharetroviral Chimeric Antigen Receptor Engineering. Human Gene Therapy Methods, 2019, 30, 102-120.	2.1	43
67	Characterization of a Novel Third-Generation Anti-CD24-CAR against Ovarian Cancer. International Journal of Molecular Sciences, 2019, 20, 660.	4.1	70
68	Preclinical Assessment of Suitable Natural Killer Cell Sources for Chimeric Antigen Receptor Natural Killer–Based "Off-the-Shelf―Acute Myeloid Leukemia Immunotherapies. Human Gene Therapy, 2019, 30, 381-401.	2.7	36
69	High Cytotoxic Efficiency of Lentivirally and Alpharetrovirally Engineered CD19-Specific Chimeric Antigen Receptor Natural Killer Cells Against Acute Lymphoblastic Leukemia. Frontiers in Immunology, 2019, 10, 3123.	4.8	67
70	Chimeric antigen receptor–induced BCL11B suppression propagates NK-like cell development. Journal of Clinical Investigation, 2019, 129, 5108-5122.	8.2	16
71	Non-Clinical Efficacy and Safety Studies on G1XCGD, a Lentiviral Vector for <i>Ex Vivo</i> Gene Therapy of X-Linked Chronic Granulomatous Disease. Human Gene Therapy Clinical Development, 2018, 29, 69-79.	3.1	31
72	Preclinical Development of a Lentiviral Vector for Gene Therapy of X-Linked Severe Combined Immunodeficiency. Molecular Therapy - Methods and Clinical Development, 2018, 9, 257-269.	4.1	38

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73	T-cell gene therapy for perforin deficiency correctsÂcytotoxicity defects and prevents hemophagocytic lymphohistiocytosis manifestations. Journal of Allergy and Clinical Immunology, 2018, 142, 904-913.e3.	2.9	44
74	Impaired IFNÎ <sup>3</sup> -Signaling and Mycobacterial Clearance in IFNÎ <sup>3</sup> R1-Deficient Human iPSC-Derived Macrophages. Stem Cell Reports, 2018, 10, 7-16.	4.8	25
75	Pooled Generation of Lentiviral Tetracycline-Regulated microRNA Embedded Short Hairpin RNA Libraries. Human Gene Therapy Methods, 2018, 29, 16-29.	2.1	3
76	Refined sgRNA efficacy prediction improves large- and small-scale CRISPR–Cas9 applications. Nucleic Acids Research, 2018, 46, 1375-1385.	14.5	213
77	Targeting NSG Mice Engrafting Cells with a Clinically Applicable Lentiviral Vector Corrects Osteoclasts in Infantile Malignant Osteopetrosis. Human Gene Therapy, 2018, 29, 938-949.	2.7	12
78	Analyzing the Genotoxicity of Retroviral Vectors in Hematopoietic Cell Gene Therapy. Molecular Therapy - Methods and Clinical Development, 2018, 8, 21-30.	4.1	34
79	Pre-clinical Development of a Lentiviral Vector Expressing the Anti-sickling βAS3 Globin for Gene Therapy for Sickle Cell Disease. Molecular Therapy - Methods and Clinical Development, 2018, 11, 167-179.	4.1	16
80	Bioreactor-based mass production of human iPSC-derived macrophages enables immunotherapies against bacterial airway infections. Nature Communications, 2018, 9, 5088.	12.8	105
81	Engineering CAR-T Cells for Improved Function Against Solid Tumors. Frontiers in Immunology, 2018, 9, 2493.	4.8	67
82	Transient Retrovirus-Based CRISPR/Cas9 All-in-One Particles for Efficient, Targeted Gene Knockout. Molecular Therapy - Nucleic Acids, 2018, 13, 256-274.	5.1	34
83	Human Teratoma-Derived Hematopoiesis Is a Highly Polyclonal Process Supported by Human Umbilical Vein Endothelial Cells. Stem Cell Reports, 2018, 11, 1051-1060.	4.8	10
84	CRISPR/Cas9 Immunoengineering of Hoxb8-Immortalized Progenitor Cells for Revealing CCR7-Mediated Dendritic Cell Signaling and Migration Mechanisms in vivo. Frontiers in Immunology, 2018, 9, 1949.	4.8	21
85	Chimeric Antigen Receptor T Cells: Extending Translation from Liquid to Solid Tumors. Human Gene Therapy, 2018, 29, 1083-1097.	2.7	11
86	EVI1 carboxy-terminal phosphorylation is ATM-mediated and sustains transcriptional modulation and self-renewal via enhanced CtBP1 association. Nucleic Acids Research, 2018, 46, 7662-7674.	14.5	11
87	Fluorescent genetic barcoding for cellular multiplex analyses. Experimental Hematology, 2018, 67, 10-17.	0.4	5
88	A patient-specific induced pluripotent stem cell model for West syndrome caused by ST3GAL3 deficiency. European Journal of Human Genetics, 2018, 26, 1773-1783.	2.8	15
89	Effect of <i>TP53</i> contact and conformational mutations on cell survival and erythropoiesis of human hematopoietic stem cells in a long term culture model. Oncotarget, 2018, 9, 29869-29876.	1.8	1
90	Inhibition of miRNA-212/132 improves the reprogramming of fibroblasts into induced pluripotent stem cells by de-repressing important epigenetic remodelling factors. Stem Cell Research, 2017, 20, 70-75.	0.7	20

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91	Rapid and efficient generation of oligodendrocytes from human induced pluripotent stem cells using transcription factors. Proceedings of the National Academy of Sciences of the United States of America, 2017, 114, E2243-E2252.	7.1	189
92	CSF-1–induced Src signaling can instruct monocytic lineage choice. Blood, 2017, 129, 1691-1701.	1.4	21
93	Forced expression of human macrophage colonyâ€stimulating factor in CD34 <sup>+</sup> cells promotes monocyte differentiation in vitro and in vivo but blunts osteoclastogenesis in vitro. European Journal of Haematology, 2017, 98, 517-526.	2.2	6
94	A Lentiviral Fluorescent Genetic Barcoding System for Flow Cytometry-Based Multiplex Tracking. Molecular Therapy, 2017, 25, 606-620.	8.2	16
95	Lentiviral Vectors with Cellular Promoters Correct Anemia and Lethal Bone Marrow Failure in a Mouse Model for Diamond-Blackfan Anemia. Molecular Therapy, 2017, 25, 1805-1814.	8.2	19
96	An optimized lentiviral vector system for conditional RNAi and efficient cloning of microRNA embedded short hairpin RNA libraries. Biomaterials, 2017, 139, 102-115.	11.4	24
97	Eliminating HIV-1 Packaging Sequences from Lentiviral Vector Proviruses Enhances Safety and Expedites Gene Transfer for Gene Therapy. Molecular Therapy, 2017, 25, 1790-1804.	8.2	32
98	Lentivector Iterations and Pre-Clinical Scale-Up/Toxicity Testing: Targeting Mobilized CD34 + Cells for Correction of Fabry Disease. Molecular Therapy - Methods and Clinical Development, 2017, 5, 241-258.	4.1	36
99	Scavenger receptor class B member 1 ( SCARB1 ) variants modulate hepatitis C virus replication cycle and viral load. Journal of Hepatology, 2017, 67, 237-245.	3.7	26
100	An RNA-targeted therapy for dystrophic epidermolysis bullosa. Nucleic Acids Research, 2017, 45, 10259-10269.	14.5	21
101	The CpG-sites of the CBX3 ubiquitous chromatin opening element are critical structural determinants for the anti-silencing function. Scientific Reports, 2017, 7, 7919.	3.3	8
102	Optimization of Human NK Cell Manufacturing: Fully Automated Separation, Improved <i>Ex Vivo</i> Expansion Using IL-21 with Autologous Feeder Cells, and Generation of Anti-CD123-CAR-Expressing Effector Cells. Human Gene Therapy, 2017, 28, 897-913.	2.7	106
103	Improved Killing of Ovarian Cancer Stem Cells by Combining a Novel Chimeric Antigen Receptor–Based Immunotherapy and Chemotherapy. Human Gene Therapy, 2017, 28, 886-896.	2.7	65
104	Integrating Vectors for Gene Therapy and Clonal Tracking of Engineered Hematopoiesis. Hematology/Oncology Clinics of North America, 2017, 31, 737-752.	2.2	16
105	The non-coding RNA landscape of human hematopoiesis and leukemia. Nature Communications, 2017, 8, 218.	12.8	131
106	Function and Safety of Lentivirus-Mediated Gene Transfer for <i>CSF2RA</i> -Deficiency. Human Gene Therapy Methods, 2017, 28, 318-329.	2.1	16
107	Multimodal Lentiviral Vectors for Pharmacologically Controlled Switching Between Constitutive Single Gene Expression and Tetracycline-Regulated Multiple Gene Collaboration. Human Gene Therapy Methods, 2017, 28, 191-204.	2.1	3
108	Uncoupling the Oncogenic Engine. Cancer Research, 2017, 77, 6060-6064.	0.9	3

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109	Potent and reversible lentiviral vector restriction in murine induced pluripotent stem cells. Retrovirology, 2017, 14, 34.	2.0	11
110	Lentivirus Mediated Correction of Artemis-Deficient Severe Combined Immunodeficiency. Human Gene Therapy, 2017, 28, 112-124.	2.7	44
111	Gene correction of HAX1 reversed Kostmann disease phenotype in patient-specific induced pluripotent stem cells. Blood Advances, 2017, 1, 903-914.	5.2	18
112	Comparison of Tetracycline-regulated Promoters in Lentiviral-based Vectors in Murine Transplantation Studies. Current Gene Therapy, 2016, 16, 242-248.	2.0	4
113	Generation of HLA-Universal iPSC-Derived Megakaryocytes and Platelets for Survival Under Refractoriness Conditions. Molecular Medicine, 2016, 22, 274-285.	4.4	74
114	Letter to the Editor: Production of Mature Healthy Hematopoietic Cells from Induced Pluripotent Stem Cells Derived from an AML Diagnostic Sample Containing the t(8;21) Translocation. Stem Cells, 2016, 34, 797-799.	3.2	6
115	Viral and Synthetic RNA Vector Technologies and Applications. Molecular Therapy, 2016, 24, 1513-1527.	8.2	62
116	Preclinical validation: LV/IL-12 transduction of patient leukemia cells for immunotherapy of AML. Molecular Therapy - Methods and Clinical Development, 2016, 3, 16074.	4.1	17
117	Improved bi-allelic modification of a transcriptionally silent locus in patient-derived iPSC by Cas9 nickase. Scientific Reports, 2016, 6, 38198.	3.3	29
118	GABP is necessary for stem/progenitor cell maintenance and myeloid differentiation in human hematopoiesis and chronic myeloid leukemia. Stem Cell Research, 2016, 16, 677-681.	0.7	6
119	Direct Reprogramming of Hepatic Myofibroblasts into Hepatocytes InÂVivo Attenuates Liver Fibrosis. Cell Stem Cell, 2016, 18, 797-808.	11.1	181
120	Safe and Efficient Gene Therapy for Pyruvate Kinase Deficiency. Molecular Therapy, 2016, 24, 1187-1198.	8.2	55
121	Generation of Genetically Engineered Precursor T-Cells From Human Umbilical Cord Blood Using an Optimized Alpharetroviral Vector Platform. Molecular Therapy, 2016, 24, 1216-1226.	8.2	20
122	Alpharetroviral self-inactivating vectors produced by a superinfection-resistant stable packaging cell line allow genetic modification of primary human T lymphocytes. Biomaterials, 2016, 97, 97-109.	11.4	13
123	Murine iPSC-Derived Macrophages as a Tool for Disease Modeling of Hereditary Pulmonary Alveolar Proteinosis due to Csf2rb Deficiency. Stem Cell Reports, 2016, 7, 292-305.	4.8	23
124	Massive Clonal Selection and Transiently Contributing Clones During Expansion of Mesenchymal Stem Cell Cultures Revealed by Lentiviral RGB-Barcode Technology. Stem Cells Translational Medicine, 2016, 5, 591-601.	3.3	66
125	GTPase domain driven dimerization of SEPT7 is dispensable for the critical role of septins in fibroblast cytokinesis. Scientific Reports, 2016, 6, 20007.	3.3	27
126	Development of Inducible Molecular Switches Based on All-in-One Lentiviral Vectors Equipped with Drug Controlled FLP Recombinase. Methods in Molecular Biology, 2016, 1448, 23-39.	0.9	0

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127	Transduction of Murine Hematopoietic Stem Cells with Tetracycline-regulated Lentiviral Vectors. Methods in Molecular Biology, 2016, 1448, 65-76.	0.9	1
128	Establishing a murine xenograft-model for long-term analysis of factors inducing chromosomal instability in myelodysplastic syndrome: Pitfalls and successes. Cancer Genetics, 2016, 209, 258-266.	0.4	2
129	Glycomic Characterization of Induced Pluripotent Stem Cells Derived from a Patient Suffering from Phosphomannomutase 2 Congenital Disorder of Glycosylation (PMM2-CDG). Molecular and Cellular Proteomics, 2016, 15, 1435-1452.	3.8	51
130	Long noncoding RNA <i>Chast</i> promotes cardiac remodeling. Science Translational Medicine, 2016, 8, 326ra22.	12.4	321
131	Gene Insertion Into Genomic Safe Harbors for Human Gene Therapy. Molecular Therapy, 2016, 24, 678-684.	8.2	175
132	Directed evolution of a recombinase that excises the provirus of most HIV-1 primary isolates with high specificity. Nature Biotechnology, 2016, 34, 401-409.	17.5	108
133	Efficient generation of gene-modified human natural killer cells via alpharetroviral vectors. Journal of Molecular Medicine, 2016, 94, 83-93.	3.9	65
134	Retroviral Vectors for Cancer Gene Therapy. Recent Results in Cancer Research, 2016, 209, 17-35.	1.8	24
135	Generation of Functional Regulatory T Cells By FOXP3 Gene Transfer into CD4 T Cells from Scurfy Mice and IPEX Patients. Blood, 2016, 128, 2526-2526.	1.4	2
136	New Molecular Surrogate Assay for Genotoxicity Assessment of Gene Therapy Vectors (SAGA). Blood, 2016, 128, 4710-4710.	1.4	4
137	Preclinical Development of Gene Therapy for X-Linked Severe Combined Immunodeficiency (SCID-X1). Blood, 2016, 128, 4705-4705.	1.4	1
138	Deciphering the impact of parameters influencing transgene expression kinetics after repeated cell transduction with integrationâ€deficient retroviral vectors. Cytometry Part A: the Journal of the International Society for Analytical Cytology, 2015, 87, 405-418.	1.5	4
139	Chemoprotection of murine hematopoietic cells by combined gene transfer of cytidine deaminase (CDD) and multidrug resistance 1 gene (MDR1). Journal of Experimental and Clinical Cancer Research, 2015, 34, 148.	8.6	5
140	Novel Self-Inactivating Vectors for Reconstitution of Wiskott-Aldrich Syndrome. Current Gene Therapy, 2015, 15, 245-254.	2.0	4
141	Limited niche availability suppresses murine intrathymic dendritic-cell development from noncommitted progenitors. Blood, 2015, 125, 457-464.	1.4	13
142	Lentiviral Gene Therapy Using Cellular Promoters Cures Type 1 Gaucher Disease in Mice. Molecular Therapy, 2015, 23, 835-844.	8.2	55
143	Large-Scale Hematopoietic Differentiation of Human Induced Pluripotent Stem Cells Provides Granulocytes or Macrophages for Cell Replacement Therapies. Stem Cell Reports, 2015, 4, 282-296.	4.8	173
144	A minimal ubiquitous chromatin opening element (UCOE) effectively prevents silencing of juxtaposed heterologous promoters by epigenetic remodeling in multipotent and pluripotent stem cells. Nucleic Acids Research, 2015, 43, 1577-1592.	14.5	70

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145	Inherited DOCK2 Deficiency in Patients with Early-Onset Invasive Infections. New England Journal of Medicine, 2015, 372, 2409-2422.	27.0	169
146	The heteromeric transcription factor GABP activates the ITGAM/CD11b promoter and induces myeloid differentiation. Biochimica Et Biophysica Acta - Gene Regulatory Mechanisms, 2015, 1849, 1145-1154.	1.9	12
147	Reticular dysgenesis–associated AK2 protects hematopoietic stem and progenitor cell development from oxidative stress. Journal of Experimental Medicine, 2015, 212, 1185-1202.	8.5	49
148	Advantages and applications of CAR-expressing natural killer cells. Frontiers in Pharmacology, 2015, 6, 21.	3.5	204
149	Lentiviral vector system for coordinated constitutive and drug controlled tetracycline-regulated gene co-expression. Biomaterials, 2015, 63, 189-201.	11.4	8
150	Expression of the ETS transcription factor GABPα is positively correlated toÂthe BCR-ABL1/ABL1 ratio in CML patients and affects imatinib sensitivity inÂvitro. Experimental Hematology, 2015, 43, 880-890.	0.4	5
151	Rescue of DNA-PK Signaling and T-Cell Differentiation by Targeted Genome Editing in a prkdc Deficient iPSC Disease Model. PLoS Genetics, 2015, 11, e1005239.	3.5	17
152	Responsiveness of Developing T Cells to IL-7 Signals Is Sustained by miR-17â^¼92. Journal of Immunology, 2015, 195, 4832-4840.	0.8	24
153	Lentiviral Protein Transfer Vectors Are an Efficient Vaccine Platform and Induce a Strong Antigen-Specific Cytotoxic T Cell Response. Journal of Virology, 2015, 89, 9044-9060.	3.4	25
154	Retrovirus-based vectors for transient and permanent cell modification. Current Opinion in Pharmacology, 2015, 24, 135-146.	3.5	7
155	TALEN-mediated functional correction of X-linked chronic granulomatous disease in patient-derived induced pluripotent stem cells. Biomaterials, 2015, 69, 191-200.	11.4	76
156	Perforin Gene Transfer Into Hematopoietic Stem Cells Improves Immune Dysregulation in Murine Models of Perforin Deficiency. Molecular Therapy, 2015, 23, 737-745.	8.2	41
157	Comparison of Different Cytokine Conditions Reveals Resveratrol as a New Molecule for Ex Vivo Cultivation of Cord Blood-Derived Hematopoietic Stem Cells. Stem Cells Translational Medicine, 2015, 4, 1064-1072.	3.3	23
158	Innovative Hematopoietic Gene-Therapy Concepts for Hereditary Pulmonary Alveolar Proteinosis Utilizing Hematopoietic Stem Cell Derived Macrophages. Blood, 2015, 126, 4417-4417.	1.4	0
159	Dynamic Telomere Shortening and Chromosomal Instability in Irradiated CD34+ Cells Transduced with TP53 Hotspot Mutations R175H, R248W and R249S. Blood, 2015, 126, 4832-4832.	1.4	0
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