

Luigi M Naldini

List of Publications by Year in descending order

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Version: 2024-02-01

312
papers

50,469
citations

2091

103
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1764

218
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320
docs citations

320
times ranked

45330
citing authors

#	ARTICLE	IF	CITATIONS
1	Lentiviral haematopoietic stem-cell gene therapy for early-onset metachromatic leukodystrophy: long-term results from a non-randomised, open-label, phase 1/2 trial and expanded access. <i>Lancet</i> , The, 2022, 399, 372-383.	6.3	109
2	The EHA Research Roadmap: Hematopoietic Stem Cell Gene Therapy. <i>HemaSphere</i> , 2022, 6, e671.	1.2	8
3	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. <i>Nature Communications</i> , 2022, 13, 1315.	5.8	61
4	Liver-directed lentiviral gene therapy corrects hemophilia A mice and achieves normal-range factor VIII activity in non-human primates. <i>Nature Communications</i> , 2022, 13, 2454.	5.8	11
5	Mobilization-based chemotherapy-free engraftment of gene-edited human hematopoietic stem cells. <i>Cell</i> , 2022, 185, 2248-2264.e21.	13.5	26
6	Targeted inducible delivery of immunoactivating cytokines reprograms glioblastoma microenvironment and inhibits growth in mouse models. <i>Science Translational Medicine</i> , 2022, 14, .	5.8	32
7	WFH State-of-the-Art paper 2020: In vivo lentiviral vector gene therapy for haemophilia. <i>Haemophilia</i> , 2021, 27, 122-125.	1.0	21
8	Hematopoietic Tumors in a Mouse Model of X-linked Chronic Granulomatous Disease after Lentiviral Vector-Mediated Gene Therapy. <i>Molecular Therapy</i> , 2021, 29, 86-102.	3.7	17
9	Conditioning Regimens in Long-Term Pre-Clinical Studies to Support Development of <i>Ex Vivo</i> Gene Therapy: Review of Nonproliferative and Proliferative Changes. <i>Human Gene Therapy</i> , 2021, 32, 66-76.	1.4	10
10	Modeling, optimization, and comparable efficacy of T cell and hematopoietic stem cell gene editing for treating hyper-IgM syndrome. <i>EMBO Molecular Medicine</i> , 2021, 13, e13545.	3.3	36
11	BAR-Seq clonal tracking of gene-edited cells. <i>Nature Protocols</i> , 2021, 16, 2991-3025.	5.5	11
12	Retrieval of vector integration sites from cell-free DNA. <i>Nature Medicine</i> , 2021, 27, 1458-1470.	15.2	26
13	Therapeutic liver repopulation by transient acetaminophen selection of gene-modified hepatocytes. <i>Science Translational Medicine</i> , 2021, 13, .	5.8	16
14	ISSCR Guidelines for Stem Cell Research and Clinical Translation: The 2021 update. <i>Stem Cell Reports</i> , 2021, 16, 1398-1408.	2.3	134
15	Myeloid cell-based delivery of IFN γ reprograms the leukemia microenvironment and induces anti-tumoral immune responses. <i>EMBO Molecular Medicine</i> , 2021, 13, e13598.	3.3	13
16	Lentiviral correction of enzymatic activity restrains macrophage inflammation in adenosine deaminase 2 deficiency. <i>Blood Advances</i> , 2021, 5, 3174-3187.	2.5	18
17	Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1. <i>Blood</i> , 2021, 138, 3978-3978.	0.6	0
18	Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. <i>New England Journal of Medicine</i> , 2021, 385, 1929-1940.	13.9	75

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19	Assessing Stealth and Sensed Base Editing in Human Hematopoietic Stem/Progenitor Cells. <i>Blood</i> , 2021, 138, 3976-3976.	0.6	0
20	Lentiviral-Mediated Gene Therapy for the Treatment of Adenosine Deaminase 2 Deficiency. <i>Blood</i> , 2021, 138, 2937-2937.	0.6	0
21	MNK2 governs the macrophage antiinflammatory phenotype. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2020, 117, 27556-27565.	3.3	24
22	Laboratory-Scale Lentiviral Vector Production and Purification for Enhanced Ex Vivo and In Vivo Genetic Engineering. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 19, 411-425.	1.8	21
23	Efficient gene editing of human long-term hematopoietic stem cells validated by clonal tracking. <i>Nature Biotechnology</i> , 2020, 38, 1298-1308.	9.4	116
24	In Vivo Selection for Gene-Corrected HSPCs Advances Gene Therapy for a Rare Stem Cell Disease. <i>Cell Stem Cell</i> , 2019, 25, 592-593.	5.2	6
25	Adopt a moratorium on heritable genome editing. <i>Nature</i> , 2019, 567, 165-168.	13.7	314
26	Dynamics and genomic landscape of CD8+ T cells undergoing hepatic priming. <i>Nature</i> , 2019, 574, 200-205.	13.7	135
27	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent β -thalassemia. <i>Nature Medicine</i> , 2019, 25, 234-241.	15.2	188
28	Genetic engineering of hematopoiesis: current stage of clinical translation and future perspectives. <i>EMBO Molecular Medicine</i> , 2019, 11, .	3.3	86
29	Gene Modification and Three-Dimensional Scaffolds as Novel Tools to Allow the Use of Postnatal Thymic Epithelial Cells for Thymus Regeneration Approaches. <i>Stem Cells Translational Medicine</i> , 2019, 8, 1107-1122.	1.6	31
30	Phagocytosis-shielded lentiviral vectors improve liver gene therapy in nonhuman primates. <i>Science Translational Medicine</i> , 2019, 11, .	5.8	65
31	Targeting a Pre-existing Anti-transgene T Cell Response for Effective Gene Therapy of MPS-I in the Mouse Model of the Disease. <i>Molecular Therapy</i> , 2019, 27, 1215-1227.	3.7	17
32	Assessing the Impact of Cyclosporin A on Lentiviral Transduction and Preservation of Human Hematopoietic Stem Cells in Clinically Relevant Ex Vivo Gene Therapy Settings. <i>Human Gene Therapy</i> , 2019, 30, 1133-1146.	1.4	8
33	Precise Gene Editing Preserves Hematopoietic Stem Cell Function following Transient p53-Mediated DNA Damage Response. <i>Cell Stem Cell</i> , 2019, 24, 551-565.e8.	5.2	237
34	Lentiviral haemopoietic stem/progenitor cell gene therapy for treatment of Wiskott-Aldrich syndrome: interim results of a non-randomised, open-label, phase 1/2 clinical study. <i>Lancet Haematology</i> , 2019, 6, e239-e253.	2.2	166
35	Modulation of immune responses in lentiviral vector-mediated gene transfer. <i>Cellular Immunology</i> , 2019, 342, 103802.	1.4	49
36	Extensive Metabolic Correction of Hurler Disease By Hematopoietic Stem Cell-Based Gene Therapy: Preliminary Results from a Phase I/II Trial. <i>Blood</i> , 2019, 134, 607-607.	0.6	5

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37	TEM-MM-101: A Phase I/IIa Dose Escalation Study Evaluating the Safety and Activity of Autologous CD34+ Enriched Hematopoietic Progenitor Cells Genetically Modified for Human Interferon- γ 2 in Multiple Myeloma Patients with Early Relapse after Intensive Front Line Therapy. <i>Blood</i> , 2019, 134, 2064-2064.	0.6	0
38	Use of Defibrotide to help prevent post-transplant endothelial injury in a genetically predisposed infant with metachromatic leukodystrophy undergoing hematopoietic stem cell gene therapy. <i>Bone Marrow Transplantation</i> , 2018, 53, 913-917.	1.3	10
39	Reversible immortalisation enables genetic correction of human muscle progenitors and engineering of next-generation human artificial chromosomes for Duchenne muscular dystrophy. <i>EMBO Molecular Medicine</i> , 2018, 10, 254-275.	3.3	30
40	Cyclosporine H Overcomes Innate Immune Restrictions to Improve Lentiviral Transduction and Gene Editing In Human Hematopoietic Stem Cells. <i>Cell Stem Cell</i> , 2018, 23, 820-832.e9.	5.2	86
41	Multiple Integrated Non-clinical Studies Predict the Safety of Lentivirus-Mediated Gene Therapy for β -Thalassemia. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 11, 9-28.	1.8	21
42	Interferon gene therapy reprograms the leukemia microenvironment inducing protective immunity to multiple tumor antigens. <i>Nature Communications</i> , 2018, 9, 2896.	5.8	39
43	Generation of Memory Stem T Cells Specific for Tumor Antigens and Resistant to Inhibitory Signals By Genome Editing. <i>Blood</i> , 2018, 132, 2202-2202.	0.6	0
44	Gene therapy for ADA-SCID, the first marketing approval of an ex vivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. <i>EMBO Molecular Medicine</i> , 2017, 9, 737-740.	3.3	210
45	Efficient Ex Vivo Engineering and Expansion of Highly Purified Human Hematopoietic Stem and Progenitor Cell Populations for Gene Therapy. <i>Stem Cell Reports</i> , 2017, 8, 977-990.	2.3	124
46	Preclinical modeling highlights the therapeutic potential of hematopoietic stem cell gene editing for correction of SCID-X1. <i>Science Translational Medicine</i> , 2017, 9, .	5.8	176
47	Genome editing for scalable production of alloantigen-free lentiviral vectors for in vivo gene therapy. <i>EMBO Molecular Medicine</i> , 2017, 9, 1558-1573.	3.3	41
48	Therapeutic gene editing in CD ³⁴ hematopoietic progenitors from Fanconi anemia patients. <i>EMBO Molecular Medicine</i> , 2017, 9, 1574-1588.	3.3	54
49	NY-ESO-1 TCR single edited stem and central memory T cells to treat multiple myeloma without graft-versus-host disease. <i>Blood</i> , 2017, 130, 606-618.	0.6	71
50	Lentiviral vectors escape innate sensing but trigger p53 in human hematopoietic stem and progenitor cells. <i>EMBO Molecular Medicine</i> , 2017, 9, 1198-1211.	3.3	56
51	IFN- γ gene/cell therapy curbs colorectal cancer colonization of the liver by acting on the hepatic microenvironment. <i>EMBO Molecular Medicine</i> , 2016, 8, 155-170.	3.3	29
52	Angiopoietin 2 expression in the cornea and its control of corneal neovascularisation. <i>British Journal of Ophthalmology</i> , 2016, 100, 1005-1010.	2.1	7
53	Pervasive supply of therapeutic lysosomal enzymes in the CNS of normal and Krabbe-affected non-human primates by intracerebral lentiviral gene therapy. <i>EMBO Molecular Medicine</i> , 2016, 8, 489-510.	3.3	50
54	42. Correction of SCID-X1 by Targeted Genome Editing of Hematopoietic Stem/Progenitor Cells (HSPC) in the Mouse Model. <i>Molecular Therapy</i> , 2016, 24, S18-S19.	3.7	1

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55	130. Purification of Large Scale mRNA Encoding ZFN Nucleases by dHPLC Technology. <i>Molecular Therapy</i> , 2016, 24, S53-S54.	3.7	2
56	512. The Cytokine Release Syndrome Crucially Contributes to the Anti-Leukemic Effects of CD44v6 CAR-T Cells. <i>Molecular Therapy</i> , 2016, 24, S204.	3.7	0
57	Leukocytes recruited by tumor-derived HMGB1 sustain peritoneal carcinomatosis. <i>Oncolmmunology</i> , 2016, 5, e1122860.	2.1	20
58	Lentiviral vectors, two decades later. <i>Science</i> , 2016, 353, 1101-1102.	6.0	96
59	Debate on Germline Gene Editing. <i>Human Gene Therapy Methods</i> , 2016, 27, 135-142.	2.1	8
60	The Renaissance of Gene and Cell Therapy: Florence 2016. <i>Human Gene Therapy</i> , 2016, 27, 727-728.	1.4	0
61	Inheritable Silencing of Endogenous Genes by Hit-and-Run Targeted Epigenetic Editing. <i>Cell</i> , 2016, 167, 219-232.e14.	13.5	363
62	Safer conditioning for blood stem cell transplants. <i>Nature Biotechnology</i> , 2016, 34, 721-723.	9.4	14
63	Preclinical Testing of the Safety and Tolerability of Lentiviral Vectorâ€‘Mediated Above-Normal Alpha-L-Iduronidase Expression in Murine and Human Hematopoietic Cells Using Toxicology and Biodistribution Good Laboratory Practice Studies. <i>Human Gene Therapy</i> , 2016, 27, 813-829.	1.4	40
64	InÂVivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases. <i>Cell Stem Cell</i> , 2016, 19, 107-119.	5.2	187
65	miRNA-126 Orchestrates an Oncogenic Program in B Cell Precursor Acute Lymphoblastic Leukemia. <i>Cancer Cell</i> , 2016, 29, 905-921.	7.7	57
66	Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. <i>Lancet, The</i> , 2016, 388, 476-487.	6.3	393
67	miR-126 Regulates Distinct Self-Renewal Outcomes in Normal and Malignant Hematopoietic Stem Cells. <i>Cancer Cell</i> , 2016, 29, 214-228.	7.7	216
68	Incremental Innovation of Ex Vivo Hematopoietic Stem Cell Engineering to Expand Clinical Gene Therapy Applications. <i>Blood</i> , 2016, 128, 4707-4707.	0.6	0
69	27. Aberrant Expression of the Stem Cell microRNA-126 Induces B Cell Malignancy. <i>Molecular Therapy</i> , 2015, 23, S12.	3.7	0
70	281. Engineering Hematopoiesis for Tumor-Targeted Interferon-alpha Delivery Inhibits Multiple Myeloma and B Cell Malignancies. <i>Molecular Therapy</i> , 2015, 23, S112.	3.7	0
71	288. Dual-Regulated Lentiviral Vector for Gene Therapy of X-Linked Chronic Granulomatous Disease. <i>Molecular Therapy</i> , 2015, 23, S115-S116.	3.7	0
72	209. TCR Gene Editing in a Single Step of T Cell Activation To Redirect T Cell Specificity and Prevent GvHD. <i>Molecular Therapy</i> , 2015, 23, S82-S83.	3.7	0

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73	690. Permanent Epigenetic Silencing of Human Genes With Artificial Transcriptional Repressors. <i>Molecular Therapy</i> , 2015, 23, S275.	3.7	0
74	Design of a regulated lentiviral vector for hematopoietic stem cell gene therapy of globoid cell leukodystrophy. <i>Molecular Therapy - Methods and Clinical Development</i> , 2015, 2, 15038.	1.8	29
75	Shedding of clinical-grade lentiviral vectors is not detected in a gene therapy setting. <i>Gene Therapy</i> , 2015, 22, 496-502.	2.3	12
76	Insulin B chain 9 α 23 gene transfer to hepatocytes protects from type 1 diabetes by inducing Ag-specific FoxP3 ⁺ T _{regs} . <i>Science Translational Medicine</i> , 2015, 7, 289ra81.	5.8	55
77	The Impact of Amino Acid Variability on Alloreactivity Defines a Functional Distance Predictive of Permissive HLA-DPB1 Mismatches in Hematopoietic Stem Cell Transplantation. <i>Biology of Blood and Marrow Transplantation</i> , 2015, 21, 233-241.	2.0	95
78	MicroRNA-223 dose levels fine tune proliferation and differentiation in human cord blood progenitors and acute myeloid leukemia. <i>Experimental Hematology</i> , 2015, 43, 858-868.e7.	0.2	28
79	Fighting Rare Diseases: The Model of the Telethon Research Institutes in Italy. <i>Human Gene Therapy</i> , 2015, 26, 183-185.	1.4	2
80	B-cell reconstitution after lentiviral vector-mediated gene therapy in patients with Wiskott-Aldrich syndrome. <i>Journal of Allergy and Clinical Immunology</i> , 2015, 136, 692-702.e2.	1.5	41
81	Liver-directed lentiviral gene therapy in a dog model of hemophilia B. <i>Science Translational Medicine</i> , 2015, 7, 277ra28.	5.8	118
82	CRISPR germline engineering—the community speaks. <i>Nature Biotechnology</i> , 2015, 33, 478-486.	9.4	110
83	Cellular Innate Immunity and Restriction of Viral Infection: Implications for Lentiviral Gene Therapy in Human Hematopoietic Cells. <i>Human Gene Therapy</i> , 2015, 26, 201-209.	1.4	30
84	Gene therapy returns to centre stage. <i>Nature</i> , 2015, 526, 351-360.	13.7	943
85	Targeted Gene Correction in Osteopetrotic-Induced Pluripotent Stem Cells for the Generation of Functional Osteoclasts. <i>Stem Cell Reports</i> , 2015, 5, 558-568.	2.3	21
86	Cyclosporin A and Rapamycin Relieve Distinct Lentiviral Restriction Blocks in Hematopoietic Stem and Progenitor Cells. <i>Molecular Therapy</i> , 2015, 23, 352-362.	3.7	50
87	Safety and Clinical Benefit of Lentiviral Hematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome. <i>Blood</i> , 2015, 126, 259-259.	0.6	7
88	Engineered tumor-infiltrating macrophages as gene delivery vehicles for interferon- γ activates immunity and inhibits breast cancer progression. <i>Oncolmmunology</i> , 2014, 3, e28696.	2.1	16
89	Dual-regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. <i>Molecular Therapy</i> , 2014, 22, 1472-1483.	3.7	59
90	Targeted gene therapy and cell reprogramming in α -anemia. <i>EMBO Molecular Medicine</i> , 2014, 6, 835-848.	3.3	66

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91	Therapeutic benefit of lentiviral-mediated neonatal intracerebral gene therapy in a mouse model of globoid cell leukodystrophy. <i>Human Molecular Genetics</i> , 2014, 23, 3250-3268.	1.4	56
92	Targeted genome editing in human repopulating haematopoietic stem cells. <i>Nature</i> , 2014, 510, 235-240.	13.7	517
93	Loss of transcriptional control over endogenous retroelements during reprogramming to pluripotency. <i>Genome Research</i> , 2014, 24, 1251-1259.	2.4	94
94	Genetic Engineering of Hematopoiesis for Targeted IFN- γ Delivery Inhibits Breast Cancer Progression. <i>Science Translational Medicine</i> , 2014, 6, 217ra3.	5.8	86
95	Lentiviral Vector-based Insertional Mutagenesis Identifies Genes Involved in the Resistance to Targeted Anticancer Therapies. <i>Molecular Therapy</i> , 2014, 22, 2056-2068.	3.7	16
96	Genome Editing: A Tool For Research and Therapy: Targeted genome editing hits the clinic. <i>Nature Medicine</i> , 2014, 20, 1101-1103.	15.2	22
97	Uncovering and Dissecting the Genotoxicity of Self-inactivating Lentiviral Vectors In Vivo. <i>Molecular Therapy</i> , 2014, 22, 774-785.	3.7	142
98	Charting a Clear Path: The ASGCT Standardized Pathways Conference. <i>Molecular Therapy</i> , 2014, 22, 1235-1238.	3.7	10
99	Comprehensive Clonal Mapping of Hematopoiesis in Vivo in Humans By Retroviral Vector Insertional Barcoding. <i>Blood</i> , 2014, 124, 5-5.	0.6	2
100	NY-ESO-1 Single Edited T Cells to Treat Multiple Myeloma without Inducing GvHD. <i>Blood</i> , 2014, 124, 308-308.	0.6	0
101	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. <i>Science</i> , 2013, 341, 1233158.	6.0	998
102	Lentiviral Hematopoietic Stem Cell Gene Therapy in Patients with Wiskott-Aldrich Syndrome. <i>Science</i> , 2013, 341, 1233151.	6.0	900
103	Liver gene therapy by lentiviral vectors reverses anti-factor α pre-existing immunity in haemophilic mice. <i>EMBO Molecular Medicine</i> , 2013, 5, 1684-1697.	3.3	55
104	miR-142-3p Prevents Macrophage Differentiation during Cancer-Induced Myelopoiesis. <i>Immunity</i> , 2013, 38, 1236-1249.	6.6	127
105	Immune responses in liver-directed lentiviral gene therapy. <i>Translational Research</i> , 2013, 161, 230-240.	2.2	21
106	Lentiviral vector-based insertional mutagenesis identifies genes associated with liver cancer. <i>Nature Methods</i> , 2013, 10, 155-161.	9.0	86
107	Targeted Gene Addition in Human Epithelial Stem Cells by Zinc-finger Nuclease-mediated Homologous Recombination. <i>Molecular Therapy</i> , 2013, 21, 1695-1704.	3.7	53
108	A Double-Switch Vector System Positively Regulates Transgene Expression by Endogenous microRNA Expression (miR-ON Vector). <i>Molecular Therapy</i> , 2013, 21, 934-946.	3.7	31

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109	CD4 ⁺ T Cells from IPEX Patients Convert into Functional and Stable Regulatory T Cells by FOXP3 Gene Transfer. <i>Science Translational Medicine</i> , 2013, 5, 215ra174.	5.8	129
110	TIE2-expressing monocytes/macrophages regulate revascularization of the ischemic limb. <i>EMBO Molecular Medicine</i> , 2013, 5, 858-869.	3.3	83
111	Preclinical Safety and Efficacy of Human CD34+ Cells Transduced With Lentiviral Vector for the Treatment of Wiskott-Aldrich Syndrome. <i>Molecular Therapy</i> , 2013, 21, 175-184.	3.7	72
112	A role for miR-155 in enabling tumor-infiltrating innate immune cells to mount effective antitumor responses in mice. <i>Blood</i> , 2013, 122, 243-252.	0.6	102
113	CD44v6-targeted T cells mediate potent antitumor effects against acute myeloid leukemia and multiple myeloma. <i>Blood</i> , 2013, 122, 3461-3472.	0.6	306
114	Dynamic Activity of miR-125b and miR-93 during Murine Neural Stem Cell Differentiation In Vitro and in the Subventricular Zone Neurogenic Niche. <i>PLoS ONE</i> , 2013, 8, e67411.	1.1	30
115	Off-Tumor Target Expression Levels Do Not Predict CAR-T Cell Killing: A Foundation For The Safety Of CD44v6-Targeted T Cells. <i>Blood</i> , 2013, 122, 142-142.	0.6	2
116	Mir-126 Governs Human Leukemia Stem Cell Quiescence and Chemotherapy Resistance. <i>Blood</i> , 2013, 122, 1647-1647.	0.6	1
117	CD44v6 Is Required For In Vivo Tumorigenesis Of Human AML and MM Cells: Role Of Microenvironmental Signals and Therapeutic Implications. <i>Blood</i> , 2013, 122, 605-605.	0.6	6
118	TCR Gene Editing Achieved In a Single Round Of T Cell Activation Is Sufficient To Redirect T Cell Specificity and Prevent GvHD. <i>Blood</i> , 2013, 122, 2898-2898.	0.6	0
119	Potent In Vivo Anti-Tumor Activity Of Extracellular Vesicles Isolated From Genetically Engineered Primary Mesenchymal Stromal Cells Expressing The Trans-Membrane TNF-Related Apoptosis-Inducing Ligand (TRAIL). <i>Blood</i> , 2013, 122, 1658-1658.	0.6	7
120	A Mechanistic Role For Mir-126, a Hematopoietic Stem Cell Microrna, In Acute Leukemias. <i>Blood</i> , 2013, 122, 886-886.	0.6	1
121	Brain conditioning is instrumental for successful microglia reconstitution following hematopoietic stem cell transplantation. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2012, 109, 15018-15023.	3.3	168
122	Post-natal cardiomyocytes can generate iPS cells with an enhanced capacity toward cardiomyogenic re-differentiation. <i>Cell Death and Differentiation</i> , 2012, 19, 1162-1174.	5.0	55
123	Exploiting microRNA regulation for genetic engineering. <i>Tissue Antigens</i> , 2012, 80, 393-403.	1.0	30
124	miR-511-3p Modulates Genetic Programs of Tumor-Associated Macrophages. <i>Cell Reports</i> , 2012, 1, 141-154.	2.9	193
125	Hyperfunctional coagulation factor IX improves the efficacy of gene therapy in hemophilic mice. <i>Blood</i> , 2012, 120, 4517-4520.	0.6	84
126	Attenuation of miR-126 Activity Expands HSC In Vivo without Exhaustion. <i>Cell Stem Cell</i> , 2012, 11, 799-811.	5.2	197

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127	A foundation for universal T-cell based immunotherapy: T cells engineered to express a CD19-specific chimeric-antigen-receptor and eliminate expression of endogenous TCR. <i>Blood</i> , 2012, 119, 5697-5705.	0.6	437
128	Editing T cell specificity towards leukemia by zinc finger nucleases and lentiviral gene transfer. <i>Nature Medicine</i> , 2012, 18, 807-815.	15.2	398
129	The first reported generation of several induced pluripotent stem cell lines from homozygous and heterozygous Huntington's disease patients demonstrates mutation related enhanced lysosomal activity. <i>Neurobiology of Disease</i> , 2012, 46, 41-51.	2.1	159
130	Whole transcriptome characterization of aberrant splicing events induced by lentiviral vector integrations. <i>Journal of Clinical Investigation</i> , 2012, 122, 1667-1676.	3.9	104
131	Co-Expression of a Suicide Gene in CAR-Redirected T Cells Enables the Safe Targeting of CD44v6 for Leukemia and Myeloma Eradication. <i>Blood</i> , 2012, 120, 949-949.	0.6	3
132	Hematopoietic Stem Cell Expansion, without Exhaustion or Transformation, by Stable MicroRNA Antagonism in Vivo. <i>Blood</i> , 2012, 120, 30-30.	0.6	0
133	HIV-1-Derived Lentiviral Vectors Directly Activate Plasmacytoid Dendritic Cells, Which in Turn Induce the Maturation of Myeloid Dendritic Cells. <i>Human Gene Therapy</i> , 2011, 22, 177-188.	1.4	40
134	Minicircle DNA-based Gene Therapy Coupled With Immune Modulation Permits Long-term Expression of β -L-Iduronidase in Mice With Mucopolysaccharidosis Type I. <i>Molecular Therapy</i> , 2011, 19, 450-460.	3.7	86
135	Forkhead box protein 3 (FOXP3) mutations lead to increased TH17 cell numbers and regulatory T-cell instability. <i>Journal of Allergy and Clinical Immunology</i> , 2011, 128, 1376-1379.e1.	1.5	54
136	Large-Scale Manufacture and Characterization of a Lentiviral Vector Produced for Clinical Ex Vivo Gene Therapy Application. <i>Human Gene Therapy</i> , 2011, 22, 343-356.	1.4	165
137	Site-specific integration and tailoring of cassette design for sustainable gene transfer. <i>Nature Methods</i> , 2011, 8, 861-869.	9.0	300
138	An unbiased genome-wide analysis of zinc-finger nuclease specificity. <i>Nature Biotechnology</i> , 2011, 29, 816-823.	9.4	488
139	Lentiviral-mediated gene therapy leads to improvement of B-cell functionality in a murine model of Wiskott-Aldrich syndrome. <i>Journal of Allergy and Clinical Immunology</i> , 2011, 127, 1376-1384.e5.	1.5	34
140	Manipulating Immune Tolerance with Micro-RNA Regulated Gene Therapy. <i>Frontiers in Microbiology</i> , 2011, 2, 221.	1.5	16
141	Lentiviral vector common integration sites in preclinical models and a clinical trial reflect a benign integration bias and not oncogenic selection. <i>Blood</i> , 2011, 117, 5332-5339.	0.6	201
142	TIE2-expressing macrophages limit the therapeutic efficacy of the vascular-disrupting agent combretastatin A4 phosphate in mice. <i>Journal of Clinical Investigation</i> , 2011, 121, 1969-1973.	3.9	204
143	Ex vivo gene transfer and correction for cell-based therapies. <i>Nature Reviews Genetics</i> , 2011, 12, 301-315.	7.7	340
144	Genomic instability in induced stem cells. <i>Cell Death and Differentiation</i> , 2011, 18, 745-753.	5.0	138

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145	Targeting the ANG2/TIE2 Axis Inhibits Tumor Growth and Metastasis by Impairing Angiogenesis and Disabling Rebounds of Proangiogenic Myeloid Cells. <i>Cancer Cell</i> , 2011, 19, 512-526.	7.7	543
146	Neural Stem Cell Gene Therapy Ameliorates Pathology and Function in a Mouse Model of Globoid Cell Leukodystrophy. <i>Stem Cells</i> , 2011, 29, 1559-1571.	1.4	62
147	A microRNA-Based System for Selecting and Maintaining the Pluripotent State in Human Induced Pluripotent Stem Cells. <i>Stem Cells</i> , 2011, 29, 1684-1695.	1.4	29
148	Hepatocyte-targeted expression by integrase-defective lentiviral vectors induces antigen-specific tolerance in mice with low genotoxic risk. <i>Hepatology</i> , 2011, 53, 1696-1707.	3.6	123
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