Luigi M Naldini

List of Publications by Year
in descending order

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312 papers	50,469 citations	1799 103 h-index	1536 218 g-index
320 all docs	320 docs citations	320 times ranked	41234 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. Lancet, The, 2022, 399, 372-383.	13.7	109
2	The EHA Research Roadmap: Hematopoietic Stem Cell Gene Therapy. HemaSphere, 2022, 6, e671.	2.7	8
3	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. Nature Communications, 2022, 13, 1315.	12.8	61
4	Liver-directed lentiviral gene therapy corrects hemophilia A mice and achieves normal-range factor VIII activity in non-human primates. Nature Communications, 2022, 13, 2454.	12.8	11
5	Mobilization-based chemotherapy-free engraftment of gene-edited human hematopoietic stem cells. Cell, 2022, 185, 2248-2264.e21.	28.9	26
6	Targeted inducible delivery of immunoactivating cytokines reprograms glioblastoma microenvironment and inhibits growth in mouse models. Science Translational Medicine, 2022, 14, .	12.4	32
7	WFH Stateâ€ofâ€theâ€art paper 2020: In vivo lentiviral vector gene therapy for haemophilia. Haemophilia, 2021, 27, 122-125.	2.1	21
8	Hematopoietic Tumors in a Mouse Model of X-linked Chronic Granulomatous Disease after Lentiviral Vector-Mediated Gene Therapy. Molecular Therapy, 2021, 29, 86-102.	8.2	17
9	Conditioning Regimens in Long-Term Pre-Clinical Studies to Support Development of Ex Vivo Gene Therapy: Review of Nonproliferative and Proliferative Changes. Human Gene Therapy, 2021, 32, 66-76.	2.7	10
10	Modeling, optimization, and comparable efficacy of T cell and hematopoietic stem cell gene editing for treating hyperâ€IgM syndrome. EMBO Molecular Medicine, 2021, 13, e13545.	6.9	36
11	BAR-Seq clonal tracking of gene-edited cells. Nature Protocols, 2021, 16, 2991-3025.	12.0	11
12	Retrieval of vector integration sites from cell-free DNA. Nature Medicine, 2021, 27, 1458-1470.	30.7	26
13	Therapeutic liver repopulation by transient acetaminophen selection of gene-modified hepatocytes. Science Translational Medicine, 2021, 13, .	12.4	16
14	ISSCR Guidelines for Stem Cell Research and Clinical Translation: The 2021 update. Stem Cell Reports, 2021, 16, 1398-1408.	4.8	134
15	Myeloid cellâ€based delivery of IFNâ€Î³ reprograms the leukemia microenvironment and induces antiâ€tumoral immune responses. EMBO Molecular Medicine, 2021, 13, e13598.	6.9	13
16	Lentiviral correction of enzymatic activity restrains macrophage inflammation in adenosine deaminase 2 deficiency. Blood Advances, 2021, 5, 3174-3187.	5.2	18
17	Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1. Blood, 2021, 138, 3978-3978.	1.4	0
18	Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. New England Journal of Medicine, 2021, 385, 1929-1940.	27.0	75

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19	Assessing Stealth and Sensed Base Editing in Human Hematopoietic Stem/Progenitor Cells. Blood, 2021, 138, 3976-3976.	1.4	0
20	Lentiviral-Mediated Gene Therapy for the Treatment of Adenosine Deaminase 2 Deficiency. Blood, 2021, 138, 2937-2937.	1.4	0
21	MNK2 governs the macrophage antiinflammatory phenotype. Proceedings of the National Academy of Sciences of the United States of America, 2020, 117, 27556-27565.	7.1	24
22	Laboratory-Scale Lentiviral Vector Production and Purification for Enhanced ExÂVivo and InÂVivo Genetic Engineering. Molecular Therapy - Methods and Clinical Development, 2020, 19, 411-425.	4.1	21
23	Efficient gene editing of human long-term hematopoietic stem cells validated by clonal tracking. Nature Biotechnology, 2020, 38, 1298-1308.	17.5	116
24	InÂVivo Selection for Gene-Corrected HSPCs Advances Gene Therapy for a Rare Stem Cell Disease. Cell Stem Cell, 2019, 25, 592-593.	11.1	6
25	Adopt a moratorium on heritable genome editing. Nature, 2019, 567, 165-168.	27.8	314
26	Dynamics and genomic landscape of CD8+ T cells undergoing hepatic priming. Nature, 2019, 574, 200-205.	27.8	135
27	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent ÃŸ-thalassemia. Nature Medicine, 2019, 25, 234-241.	30.7	188
28	Genetic engineering of hematopoiesis: current stage of clinical translation and future perspectives. EMBO Molecular Medicine, 2019, 11, .	6.9	86
29	Gene Modification and Three-Dimensional Scaffolds as Novel Tools to Allow the Use of Postnatal Thymic Epithelial Cells for Thymus Regeneration Approaches. Stem Cells Translational Medicine, 2019, 8, 1107-1122.	3.3	31
30	Phagocytosis-shielded lentiviral vectors improve liver gene therapy in nonhuman primates. Science Translational Medicine, 2019, 11, .	12.4	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. Molecular Therapy, 2019, 27, 1215-1227.	8.2	17
32	Assessing the Impact of Cyclosporin A on Lentiviral Transduction and Preservation of Human Hematopoietic Stem Cells in Clinically RelevantEx VivoGene Therapy Settings. Human Gene Therapy, 2019, 30, 1133-1146.	2.7	8
33	Precise Gene Editing Preserves Hematopoietic Stem Cell Function following Transient p53-Mediated DNA Damage Response. Cell Stem Cell, 2019, 24, 551-565.e8.	11.1	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. Lancet Haematology,the, 2019, 6, e239-e253.	4.6	166
35	Modulation of immune responses in lentiviral vector-mediated gene transfer. Cellular Immunology, 2019, 342, 103802.	3.0	49
36	Extensive Metabolic Correction of Hurler Disease By Hematopoietic Stem Cell-Based Gene Therapy: Preliminary Results from a Phase I/II Trial. Blood, 2019, 134, 607-607.	1.4	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-Î±2 in Multiple Myeloma Patients with Early Relapse after Intensive Front Line Therapy. Blood, 2019, 134, 2064-2064.	1.4	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. Bone Marrow Transplantation, 2018, 53, 913-917.	2.4	10
39	Reversible immortalisation enables genetic correction of human muscle progenitors and engineering of nextâ€generation human artificial chromosomes for Duchenne muscular dystrophy. EMBO Molecular Medicine, 2018, 10, 254-275.	6.9	30
40	Cyclosporine H Overcomes Innate Immune Restrictions to Improve Lentiviral Transduction and Gene Editing In Human Hematopoietic Stem Cells. Cell Stem Cell, 2018, 23, 820-832.e9.	11.1	86
41	Multiple Integrated Non-clinical Studies Predict the Safety of Lentivirus-Mediated Gene Therapy for Î²-Thalassemia. Molecular Therapy - Methods and Clinical Development, 2018, 11, 9-28.	4.1	21
42	Interferon gene therapy reprograms the leukemia microenvironment inducing protective immunity to multiple tumor antigens. Nature Communications, 2018, 9, 2896.	12.8	39
43	Generation of Memory Stem T Cells Specific for Tumor Antigens and Resistant to Inhibitory Signals By Genome Editing. Blood, 2018, 132, 2202-2202.	1.4	0
44	Gene therapy for ADAâ€SCID, the first marketing approval of an exÂvivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. EMBO Molecular Medicine, 2017, 9, 737-740.	6.9	210
45	Efficient ExÂVivo Engineering and Expansion of Highly Purified Human Hematopoietic Stem and Progenitor Cell Populations for Gene Therapy. Stem Cell Reports, 2017, 8, 977-990.	4.8	124
46	Preclinical modeling highlights the therapeutic potential of hematopoietic stem cell gene editing for correction of SCID-X1. Science Translational Medicine, 2017, 9, .	12.4	176
47	Genome editing for scalable production of alloantigenâ€free lentiviral vectors for inÂvivo geneÂtherapy. EMBO Molecular Medicine, 2017, 9, 1558-1573.	6.9	41
48	Therapeutic gene editing in <scp>CD</scp> 34 ⁺ hematopoietic progenitors from Fanconi anemia patients. EMBO Molecular Medicine, 2017, 9, 1574-1588.	6.9	54
49	NY-ESO-1 TCR single edited stem and central memory T cells to treat multiple myeloma without graft-versus-host disease. Blood, 2017, 130, 606-618.	1.4	71
50	Lentiviral vectors escape innate sensing but trigger p53 in human hematopoietic stem and progenitor cells. EMBO Molecular Medicine, 2017, 9, 1198-1211.	6.9	56
51	<scp>IFN</scp>Î± gene/cell therapy curbs colorectal cancer colonization of the liver by acting on the hepatic microenvironment. EMBO Molecular Medicine, 2016, 8, 155-170.	6.9	29
52	Angiopoietin 2 expression in the cornea and its control of corneal neovascularisation. British Journal of Ophthalmology, 2016, 100, 1005-1010.	3.9	7
53	Pervasive supply of therapeutic lysosomal enzymes in the <scp>CNS</scp> of normal and Krabbeâ€affected nonâ€human primates by intracerebral lentiviral gene therapy. EMBO Molecular Medicine, 2016, 8, 489-510.	6.9	50
54	42. Correction of SCID-X1 by Targeted Genome Editing of Hematopoietic Stem/Progenitor Cells (HSPC) in the Mouse Model. Molecular Therapy, 2016, 24, S18-S19.	8.2	1

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55	130. Purification of Large Scale mRNA Encoding ZFN Nucleases by dHPLC Technology. Molecular Therapy, 2016, 24, S53-S54.	8.2	2
56	512. The Cytokine Release Syndrome Crucially Contributes to the Anti-Leukemic Effects of CD44v6 CAR-T Cells. Molecular Therapy, 2016, 24, S204.	8.2	0
57	Leukocytes recruited by tumor-derived HMGB1 sustain peritoneal carcinomatosis. OncoImmunology, 2016, 5, e1122860.	4.6	20
58	Lentiviral vectors, two decades later. Science, 2016, 353, 1101-1102.	12.6	96
59	Debate on Germline Gene Editing. Human Gene Therapy Methods, 2016, 27, 135-142.	2.1	8
60	The Renaissance of Gene and Cell Therapy: Florence 2016. Human Gene Therapy, 2016, 27, 727-728.	2.7	0
61	Inheritable Silencing of Endogenous Genes by Hit-and-Run Targeted Epigenetic Editing. Cell, 2016, 167, 219-232.e14.	28.9	363
62	Safer conditioning for blood stem cell transplants. Nature Biotechnology, 2016, 34, 721-723.	17.5	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. Human Gene Therapy, 2016, 27, 813-829.	2.7	40
64	InÂVivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases. Cell Stem Cell, 2016, 19, 107-119.	11.1	187
65	miRNA-126 Orchestrates an Oncogenic Program in B Cell Precursor Acute Lymphoblastic Leukemia. Cancer Cell, 2016, 29, 905-921.	16.8	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. Lancet, The, 2016, 388, 476-487.	13.7	393
67	miR-126 Regulates Distinct Self-Renewal Outcomes in Normal and Malignant Hematopoietic Stem Cells. Cancer Cell, 2016, 29, 214-228.	16.8	216
68	Incremental Innovation of Ex Vivo Hematopoietic Stem Cell Engineering to Expand Clinical Gene Therapy Applications. Blood, 2016, 128, 4707-4707.	1.4	0
69	27. Aberrant Expression of the Stem Cell microRNA-126 Induces B Cell Malignancy. Molecular Therapy, 2015, 23, S12.	8.2	0
70	281. Engineering Hematopoiesis for Tumor-Targeted Interferon-alpha Delivery Inhibits Multuple Myeloma and B Cell Malignancies. Molecular Therapy, 2015, 23, S112.	8.2	0
71	288. Dual-Regulated Lentiviral Vector for Gene Therapy of X-Linked Chronic Granulomatous Disease. Molecular Therapy, 2015, 23, S115-S116.	8.2	0
72	209. TCR Gene Editing in a Single Step of T Cell Activation To Redirect T Cell Specificity and Prevent GvHD. Molecular Therapy, 2015, 23, S82-S83.	8.2	0

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73	690. Permanent Epigenetic Silencing of Human Genes With Artificial Transcriptional Repressors. Molecular Therapy, 2015, 23, S275.	8.2	0
74	Design of a regulated lentiviral vector for hematopoietic stem cell gene therapy of globoid cell leukodystrophy. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15038.	4.1	29
75	Shedding of clinical-grade lentiviral vectors is not detected in a gene therapy setting. Gene Therapy, 2015, 22, 496-502.	4.5	12
76	Insulin B chain 9â€“23 gene transfer to hepatocytes protects from type 1 diabetes by inducing Ag-specific FoxP3 ⁺ T _{regs}. Science Translational Medicine, 2015, 7, 289ra81.	12.4	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. Biology of Blood and Marrow Transplantation, 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. Experimental Hematology, 2015, 43, 858-868.e7.	0.4	28
79	Fighting Rare Diseases: The Model of the Telethon Research Institutes in Italy. Human Gene Therapy, 2015, 26, 183-185.	2.7	2
80	B-cell reconstitution after lentiviral vectorâ€“mediated gene therapy in patients with Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2015, 136, 692-702.e2.	2.9	41
81	Liver-directed lentiviral gene therapy in a dog model of hemophilia B. Science Translational Medicine, 2015, 7, 277ra28.	12.4	118
82	CRISPR germline engineeringâ€”the community speaks. Nature Biotechnology, 2015, 33, 478-486.	17.5	110
83	Cellular Innate Immunity and Restriction of Viral Infection: Implications for Lentiviral Gene Therapy in Human Hematopoietic Cells. Human Gene Therapy, 2015, 26, 201-209.	2.7	30
84	Gene therapy returns to centre stage. Nature, 2015, 526, 351-360.	27.8	943
85	Targeted Gene Correction in Osteopetrotic-Induced Pluripotent Stem Cells for the Generation of Functional Osteoclasts. Stem Cell Reports, 2015, 5, 558-568.	4.8	21
86	Cyclosporin A and Rapamycin Relieve Distinct Lentiviral Restriction Blocks in Hematopoietic Stem and Progenitor Cells. Molecular Therapy, 2015, 23, 352-362.	8.2	50
87	Safety and Clinical Benefit of Lentiviral Hematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome. Blood, 2015, 126, 259-259.	1.4	7
88	Engineered tumor-infiltrating macrophages as gene delivery vehicles for interferon-Î± activates immunity and inhibits breast cancer progression. OncoImmunology, 2014, 3, e28696.	4.6	16
89	Dual-regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. Molecular Therapy, 2014, 22, 1472-1483.	8.2	59
90	Targeted gene therapy and cell reprogramming in <scp>F</scp>anconi anemia. EMBO Molecular Medicine, 2014, 6, 835-848.	6.9	66

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91	Therapeutic benefit of lentiviral-mediated neonatal intracerebral gene therapy in a mouse model of globoid cell leukodystrophy. Human Molecular Genetics, 2014, 23, 3250-3268.	2.9	56
92	Targeted genome editing in human repopulating haematopoietic stem cells. Nature, 2014, 510, 235-240.	27.8	517
93	Loss of transcriptional control over endogenous retroelements during reprogramming to pluripotency. Genome Research, 2014, 24, 1251-1259.	5.5	94
94	Genetic Engineering of Hematopoiesis for Targeted IFN-Î± Delivery Inhibits Breast Cancer Progression. Science Translational Medicine, 2014, 6, 217ra3.	12.4	86
95	Lentiviral Vector-based Insertional Mutagenesis Identifies Genes Involved in the Resistance to Targeted Anticancer Therapies. Molecular Therapy, 2014, 22, 2056-2068.	8.2	16
96	Genome Editing: A Tool For Research and Therapy: Targeted genome editing hits the clinic. Nature Medicine, 2014, 20, 1101-1103.	30.7	22
97	Uncovering and Dissecting the Genotoxicity of Self-inactivating Lentiviral Vectors In Vivo. Molecular Therapy, 2014, 22, 774-785.	8.2	142
98	Charting a Clear Path: The ASGCT Standardized Pathways Conference. Molecular Therapy, 2014, 22, 1235-1238.	8.2	10
99	Comprehensive Clonal Mapping of Hematopoiesis in Vivo in Humans By Retroviral Vector Insertional Barcoding. Blood, 2014, 124, 5-5.	1.4	2
100	NY-ESO-1 Single Edited T Cells to Treat Multiple Myeloma without Inducing GvHD. Blood, 2014, 124, 308-308.	1.4	0
101	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. Science, 2013, 341, 1233158.	12.6	998
102	Lentiviral Hematopoietic Stem Cell Gene Therapy in Patients with Wiskott-Aldrich Syndrome. Science, 2013, 341, 1233151.	12.6	900
103	Liver gene therapy by lentiviral vectors reverses antiâ€factor <scp>IX</scp> preâ€existing immunity in haemophilic mice. EMBO Molecular Medicine, 2013, 5, 1684-1697.	6.9	55
104	miR-142-3p Prevents Macrophage Differentiation during Cancer-Induced Myelopoiesis. Immunity, 2013, 38, 1236-1249.	14.3	127
105	Immune responses in liver-directed lentiviral gene therapy. Translational Research, 2013, 161, 230-240.	5.0	21
106	Lentiviral vectorâ€“based insertional mutagenesis identifies genes associated with liver cancer. Nature Methods, 2013, 10, 155-161.	19.0	86
107	Targeted Gene Addition in Human Epithelial Stem Cells by Zinc-finger Nuclease-mediated Homologous Recombination. Molecular Therapy, 2013, 21, 1695-1704.	8.2	53
108	A Double-Switch Vector System Positively Regulates Transgene Expression by Endogenous microRNA Expression (miR-ON Vector). Molecular Therapy, 2013, 21, 934-946.	8.2	31

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109	CD4 ⁺ T Cells from IPEX Patients Convert into Functional and Stable Regulatory T Cells by FOXP3 Gene Transfer. Science Translational Medicine, 2013, 5, 215ra174.	12.4	129
110	TIE2â€expressing monocytes/macrophages regulate revascularization of the ischemic limb. EMBO Molecular Medicine, 2013, 5, 858-869.	6.9	83
111	Preclinical Safety and Efficacy of Human CD34+ Cells Transduced With Lentiviral Vector for the Treatment of Wiskott-Aldrich Syndrome. Molecular Therapy, 2013, 21, 175-184.	8.2	72
112	A role for miR-155 in enabling tumor-infiltrating innate immune cells to mount effective antitumor responses in mice. Blood, 2013, 122, 243-252.	1.4	102
113	CD44v6-targeted T cells mediate potent antitumor effects against acute myeloid leukemia and multiple myeloma. Blood, 2013, 122, 3461-3472.	1.4	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. PLoS ONE, 2013, 8, e67411.	2.5	30
115	Off-Tumor Target Expression Levels Do Not Predict CAR-T Cell Killing: A Foundation For The Safety Of CD44v6-Targeted T Cells. Blood, 2013, 122, 142-142.	1.4	2
116	Mir-126 Governs Human Leukemia Stem Cell Quiescence and Chemotherapy Resistance. Blood, 2013, 122, 1647-1647.	1.4	1
117	CD44v6 Is Required For In Vivo Tumorigenesis Of Human AML and MM Cells: Role Of Microenvironmental Signals and Therapeutic Implications. Blood, 2013, 122, 605-605.	1.4	6
118	TCR Gene Editing Achieved In a Single Round Of T Cell Activation Is Sufficient To Redirect T Cell Specificity and Prevent GvHD. Blood, 2013, 122, 2898-2898.	1.4	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). Blood, 2013, 122, 1658-1658.	1.4	7
120	A Mechanistic Role For Mir-126, a Hematopoietic Stem Cell Microrna, In Acute Leukemias. Blood, 2013, 122, 886-886.	1.4	1
121	Brain conditioning is instrumental for successful microglia reconstitution following hematopoietic stem cell transplantation. Proceedings of the National Academy of Sciences of the United States of America, 2012, 109, 15018-15023.	7.1	168
122	Post-natal cardiomyocytes can generate iPS cells with an enhanced capacity toward cardiomyogenic re-differentation. Cell Death and Differentiation, 2012, 19, 1162-1174.	11.2	55
123	Exploiting <scp>microRNA</scp> regulation for genetic engineering. Tissue Antigens, 2012, 80, 393-403.	1.0	30
124	miR-511-3p Modulates Genetic Programs of Tumor-Associated Macrophages. Cell Reports, 2012, 1, 141-154.	6.4	193
125	Hyperfunctional coagulation factor IX improves the efficacy of gene therapy in hemophilic mice. Blood, 2012, 120, 4517-4520.	1.4	84
126	Attenuation of miR-126 Activity Expands HSC InÂVivo without Exhaustion. Cell Stem Cell, 2012, 11, 799-811.	11.1	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. Blood, 2012, 119, 5697-5705.	1.4	437
128	Editing T cell specificity towards leukemia by zinc finger nucleases and lentiviral gene transfer. Nature Medicine, 2012, 18, 807-815.	30.7	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. Neurobiology of Disease, 2012, 46, 41-51.	4.4	159
130	Whole transcriptome characterization of aberrant splicing events induced by lentiviral vector integrations. Journal of Clinical Investigation, 2012, 122, 1667-1676.	8.2	104
131	Co-Expression of a Suicide Gene in CAR-Redirected T Cells Enables the Safe Targeting of CD44v6 for Leukemia and Myeloma Eradication. Blood, 2012, 120, 949-949.	1.4	3
132	Hematopoietic Stem Cell Expansion, without Exhaustion or Transformation, by Stable Microrna Antagonism in Vivo. Blood, 2012, 120, 30-30.	1.4	0
133	HIV-1-Derived Lentiviral Vectors Directly Activate Plasmacytoid Dendritic Cells, Which in Turn Induce the Maturation of Myeloid Dendritic Cells. Human Gene Therapy, 2011, 22, 177-188.	2.7	40
134	Minicircle DNA-based Gene Therapy Coupled With Immune Modulation Permits Long-term Expression of Î±-L-Iduronidase in Mice With Mucopolysaccharidosis Type I. Molecular Therapy, 2011, 19, 450-460.	8.2	86
135	Forkhead box protein 3 (FOXP3) mutations lead to increased TH17 cell numbers and regulatory T-cell instability. Journal of Allergy and Clinical Immunology, 2011, 128, 1376-1379.e1.	2.9	54
136	Large-Scale Manufacture and Characterization of a Lentiviral Vector Produced for ClinicalEx VivoGene Therapy Application. Human Gene Therapy, 2011, 22, 343-356.	2.7	165
137	Site-specific integration and tailoring of cassette design for sustainable gene transfer. Nature Methods, 2011, 8, 861-869.	19.0	300
138	An unbiased genome-wide analysis of zinc-finger nuclease specificity. Nature Biotechnology, 2011, 29, 816-823.	17.5	488
139	Lentiviral-mediated gene therapy leads to improvement of B-cell functionality in a murine model of Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2011, 127, 1376-1384.e5.	2.9	34
140	Manipulating Immune Tolerance with Micro-RNA Regulated Gene Therapy. Frontiers in Microbiology, 2011, 2, 221.	3.5	16
141	Lentiviral vector common integration sites in preclinical models and a clinical trial reflect a benign integration bias and not oncogenic selection. Blood, 2011, 117, 5332-5339.	1.4	201
142	TIE2-expressing macrophages limit the therapeutic efficacy of the vascular-disrupting agent combretastatin A4 phosphate in mice. Journal of Clinical Investigation, 2011, 121, 1969-1973.	8.2	204
143	Ex vivo gene transfer and correction for cell-based therapies. Nature Reviews Genetics, 2011, 12, 301-315.	16.3	340
144	Genomic instability in induced stem cells. Cell Death and Differentiation, 2011, 18, 745-753.	11.2	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. Cancer Cell, 2011, 19, 512-526.	16.8	543
146	Neural Stem Cell Gene Therapy Ameliorates Pathology and Function in a Mouse Model of Globoid Cell Leukodystrophy. Stem Cells, 2011, 29, 1559-1571.	3.2	62
147	A microRNA-Based System for Selecting and Maintaining the Pluripotent State in Human Induced Pluripotent Stem Cells. Stem Cells, 2011, 29, 1684-1695.	3.2	29
148	Hepatocyteâ€targeted expression by integraseâ€defective lentiviral vectors induces antigenâ€specific tolerance in mice with low genotoxic risk. Hepatology, 2011, 53, 1696-1707.	7.3	123
149	Stem Cell Gene Therapy for Fanconi Anemia: Report from the 1st International Fanconi Anemia Gene Therapy Working Group Meeting. Molecular Therapy, 2011, 19, 1193-1198.	8.2	45
150	Lentiviral Vector Integration Profiles Differ in Rodent Postmitotic Tissues. Molecular Therapy, 2011, 19, 703-710.	8.2	51
151	Systemic and Targeted Delivery of Semaphorin 3A Inhibits Tumor Angiogenesis and Progression in Mouse Tumor Models. Arteriosclerosis, Thrombosis, and Vascular Biology, 2011, 31, 741-749.	2.4	105
152	Angiopoietin-2 TIEs Up Macrophages in Tumor Angiogenesis. Clinical Cancer Research, 2011, 17, 5226-5232.	7.0	88
153	Effects of phosphorylation and neuronal activity on the control of synapse formation by synapsin I. Journal of Cell Science, 2011, 124, 3643-3653.	2.0	32
154	A MicroRNA-regulated and GP64-pseudotyped Lentiviral Vector Mediates Stable Expression of FVIII in a Murine Model of Hemophilia A. Molecular Therapy, 2011, 19, 723-730.	8.2	72
155	TCR Gene Editing Results in Effective Immunotherapy of Leukemia without the Development of GvHD. Blood, 2011, 118, 667-667.	1.4	1
156	Dual Transgenesis of T Cells with a Novel CD44v6-Specific Chimeric Antigen Receptor and a Suicide Gene for Safe and Effective Targeting of Chemoresistance in Hematopoietic Tumors. Blood, 2011, 118, 3125-3125.	1.4	1
157	The galactocerebrosidase enzyme contributes to the maintenance of a functional hematopoietic stem cell niche. Blood, 2010, 116, 1857-1866.	1.4	50
158	Gene therapy augments the efficacy of hematopoietic cell transplantation and fully corrects mucopolysaccharidosis type I phenotype in the mouse model. Blood, 2010, 116, 5130-5139.	1.4	159
159	FcRÎ³ Activation Regulates Inflammation-Associated Squamous Carcinogenesis. Cancer Cell, 2010, 17, 121-134.	16.8	537
160	Antagonizing metastasis. Nature Biotechnology, 2010, 28, 331-332.	17.5	4
161	Identification of Hematopoietic Stem Cellâ€“Specific miRNAs Enables Gene Therapy of Globoid Cell Leukodystrophy. Science Translational Medicine, 2010, 2, 58ra84.	12.4	180
162	Widespread enzymatic correction of CNS tissues by a single intracerebral injection of therapeutic lentiviral vector in leukodystrophy mouse models. Human Molecular Genetics, 2010, 19, 2208-2227.	2.9	77

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163	Tracking differentiating neural progenitors in pluripotent cultures using microRNA-regulated lentiviral vectors. Proceedings of the National Academy of Sciences of the United States of America, 2010, 107, 11602-11607.	7.1	42
164	Elusive Identities and Overlapping Phenotypes of Proangiogenic Myeloid Cells in Tumors. American Journal of Pathology, 2010, 176, 1564-1576.	3.8	137
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