Luigi M Naldini

List of Publications by Year
in descending order

312 papers	50,469 citations	2091 103 h-index	1764 218 g-index
320 all docs	320 docs citations	320 times ranked	45330 citing authors

#	Article	IF	Citations
1	In Vivo Gene Delivery and Stable Transduction of Nondividing Cells by a Lentiviral Vector. Science, 1996, 272, 263-267.	6.0	4,589
2	A Third-Generation Lentivirus Vector with a Conditional Packaging System. Journal of Virology, 1998, 72, 8463-8471.	1.5	2,931
3	Multiply attenuated lentiviral vector achieves efficient gene delivery in vivo. Nature Biotechnology, 1997, 15, 871-875.	9.4	1,826
4	Self-Inactivating Lentivirus Vector for Safe and Efficient In Vivo Gene Delivery. Journal of Virology, 1998, 72, 9873-9880.	1.5	1,676
5	Efficient transfer, integration, and sustained long-term expression of the transgene in adult rat brains injected with a lentiviral vector.. Proceedings of the National Academy of Sciences of the United States of America, 1996, 93, 11382-11388.	3.3	1,420
6	Hepatocyte growth factor is a potent angiogenic factor which stimulates endothelial cell motility and growth.. Journal of Cell Biology, 1992, 119, 629-641.	2.3	1,282
7	Tie2 identifies a hematopoietic lineage of proangiogenic monocytes required for tumor vessel formation and a mesenchymal population of pericyte progenitors. Cancer Cell, 2005, 8, 211-226.	7.7	1,212
8	Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics. Nature Medicine, 2001, 7, 33-40.	15.2	1,205
9	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. Science, 2013, 341, 1233158.	6.0	998
10	Gene therapy returns to centre stage. Nature, 2015, 526, 351-360.	13.7	943
11	Lentiviral Hematopoietic Stem Cell Gene Therapy in Patients with Wiskott-Aldrich Syndrome. Science, 2013, 341, 1233151.	6.0	900
12	Gene transfer by lentiviral vectors is limited by nuclear translocation and rescued by HIV-1 pol sequences. Nature Genetics, 2000, 25, 217-222.	9.4	887
13	Gene editing in human stem cells using zinc finger nucleases and integrase-defective lentiviral vector delivery. Nature Biotechnology, 2007, 25, 1298-1306.	9.4	797
14	Hematopoietic stem cell gene transfer in a tumor-prone mouse model uncovers low genotoxicity of lentiviral vector integration. Nature Biotechnology, 2006, 24, 687-696.	9.4	648
15	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.	7.7	543
16	Targeting exogenous genes to tumor angiogenesis by transplantation of genetically modified hematopoietic stem cells. Nature Medicine, 2003, 9, 789-795.	15.2	539
17	Endogenous microRNA can be broadly exploited to regulate transgene expression according to tissue, lineage and differentiation state. Nature Biotechnology, 2007, 25, 1457-1467.	9.4	539
18	FcRÎ³ Activation Regulates Inflammation-Associated Squamous Carcinogenesis. Cancer Cell, 2010, 17, 121-134.	7.7	537

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19	Targeted genome editing in human repopulating haematopoietic stem cells. Nature, 2014, 510, 235-240.	13.7	517
20	Cleavage of the Plasma Membrane Na+/Ca2+ Exchanger in Excitotoxicity. Cell, 2005, 120, 275-285.	13.5	511
21	An unbiased genome-wide analysis of zinc-finger nuclease specificity. Nature Biotechnology, 2011, 29, 816-823.	9.4	488
22	The genotoxic potential of retroviral vectors is strongly modulated by vector design and integration site selection in a mouse model of HSC gene therapy. Journal of Clinical Investigation, 2009, 119, 964-975.	3.9	488
23	Endogenous microRNA regulation suppresses transgene expression in hematopoietic lineages and enables stable gene transfer. Nature Medicine, 2006, 12, 585-591.	15.2	460
24	Identification of proangiogenic TIE2-expressing monocytes (TEMs) in human peripheral blood and cancer. Blood, 2007, 109, 5276-5285.	0.6	451
25	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.	0.6	437
26	Forebrain ependymal cells are Notch-dependent and generate neuroblasts and astrocytes after stroke. Nature Neuroscience, 2009, 12, 259-267.	7.1	415
27	Editing T cell specificity towards leukemia by zinc finger nucleases and lentiviral gene transfer. Nature Medicine, 2012, 18, 807-815.	15.2	398
28	Stable transduction of quiescent CD34+CD38- human hematopoietic cells by HIV-1-based lentiviral vectors. Proceedings of the National Academy of Sciences of the United States of America, 1999, 96, 2988-2993.	3.3	395
29	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.	6.3	393
30	Inheritable Silencing of Endogenous Genes by Hit-and-Run Targeted Epigenetic Editing. Cell, 2016, 167, 219-232.e14.	13.5	363
31	Exploiting and antagonizing microRNA regulation for therapeutic and experimental applications. Nature Reviews Genetics, 2009, 10, 578-585.	7.7	362
32	Cardiomyocytes induce endothelial cells to trans-differentiate into cardiac muscle: Implications for myocardium regeneration. Proceedings of the National Academy of Sciences of the United States of America, 2001, 98, 10733-10738.	3.3	357
33	Ex vivo gene transfer and correction for cell-based therapies. Nature Reviews Genetics, 2011, 12, 301-315.	7.7	340
34	Lentiviral vectors: excellent tools for experimental gene transfer and promising candidates for gene therapy. Journal of Gene Medicine, 2000, 2, 308-316.	1.4	318
35	Adopt a moratorium on heritable genome editing. Nature, 2019, 567, 165-168.	13.7	314
36	A distinguishing gene signature shared by tumor-infiltrating Tie2-expressing monocytes, blood â€œresidentâ€•monocytes, and embryonic macrophages suggests common functions and developmental relationships. Blood, 2009, 114, 901-914.	0.6	306

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37	CD44v6-targeted T cells mediate potent antitumor effects against acute myeloid leukemia and multiple myeloma. Blood, 2013, 122, 3461-3472.	0.6	306
38	Cell-substratum interaction of cultured avian osteoclasts is mediated by specific adhesion structures.. Journal of Cell Biology, 1984, 99, 1696-1705.	2.3	303
39	Stable knockdown of microRNA in vivo by lentiviral vectors. Nature Methods, 2009, 6, 63-66.	9.0	301
40	Site-specific integration and tailoring of cassette design for sustainable gene transfer. Nature Methods, 2011, 8, 861-869.	9.0	300
41	Coordinate dual-gene transgenesis by lentiviral vectors carrying synthetic bidirectional promoters. Nature Biotechnology, 2005, 23, 108-116.	9.4	293
42	Targeting the tumor and its microenvironment by a dual-function decoy Met receptor. Cancer Cell, 2004, 6, 61-73.	7.7	282
43	Efficient lentiviral transduction of liver requires cell cycling in vivo. Nature Genetics, 2000, 24, 49-52.	9.4	278
44	Lentiviruses as gene transfer agents for delivery to non-dividing cells. Current Opinion in Biotechnology, 1998, 9, 457-463.	3.3	269
45	Tumor-Targeted Interferon-Î± Delivery by Tie2-Expressing Monocytes Inhibits Tumor Growth and Metastasis. Cancer Cell, 2008, 14, 299-311.	7.7	267
46	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. Journal of Clinical Investigation, 2004, 113, 1118-1129.	3.9	256
47	Tie2-expressing monocytes: regulation of tumor angiogenesis and therapeutic implications. Trends in Immunology, 2007, 28, 519-524.	2.9	255
48	A microRNA-regulated lentiviral vector mediates stable correction of hemophilia B mice. Blood, 2007, 110, 4144-4152.	0.6	246
49	The MET oncogene drives a genetic programme linking cancer to haemostasis. Nature, 2005, 434, 396-400.	13.7	245
50	Lentiviral vectors containing the human immunodeficiency virus type-1 central polypurine tract can efficiently transduce nondividing hepatocytes and antigen-presenting cells in vivo. Blood, 2002, 100, 813-822.	0.6	240
51	Tie2-Expressing Monocytes and Tumor Angiogenesis: Regulation by Hypoxia and Angiopoietin-2. Cancer Research, 2007, 67, 8429-8432.	0.4	240
52	Precise Gene Editing Preserves Hematopoietic Stem Cell Function following Transient p53-Mediated DNA Damage Response. Cell Stem Cell, 2019, 24, 551-565.e8.	5.2	237
53	Biological Activation of pro-HGF (Hepatocyte Growth Factor) by Urokinase Is Controlled by a Stoichiometric Reaction. Journal of Biological Chemistry, 1995, 270, 603-611.	1.6	232
54	Efficient Gene Delivery and Targeted Expression to HepatocytesIn Vivoby Improved Lentiviral Vectors. Human Gene Therapy, 2002, 13, 243-260.	1.4	230

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55	A functional domain in the heavy chain of scatter factor/hepatocyte growth factor binds the c-Met receptor and induces cell dissociation but not mitogenesis.. Proceedings of the National Academy of Sciences of the United States of America, 1992, 89, 11574-11578.	3.3	219
56	miR-126 Regulates Distinct Self-Renewal Outcomes in Normal and Malignant Hematopoietic Stem Cells. Cancer Cell, 2016, 29, 214-228.	7.7	216
57	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.	3.3	210
58	Targeting lentiviral vector expression to hepatocytes limits transgene-specific immune response and establishes long-term expression of human antihemophilic factor IX in mice. Blood, 2004, 103, 3700-3709.	0.6	206
59	Generation of Potent and Stable Human CD4+ T Regulatory Cells by Activation-independent Expression of FOXP3. Molecular Therapy, 2008, 16, 194-202.	3.7	206
60	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.	3.9	204
61	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.	0.6	201
62	In vivo gene therapy of metachromatic leukodystrophy by lentiviral vectors: correction of neuropathology and protection against learning impairments in affected mice. Nature Medicine, 2001, 7, 310-316.	15.2	198
63	Attenuation of miR-126 Activity Expands HSC InÂVivo without Exhaustion. Cell Stem Cell, 2012, 11, 799-811.	5.2	197
64	Gene therapy of metachromatic leukodystrophy reverses neurological damage and deficits in mice. Journal of Clinical Investigation, 2006, 116, 3070-3082.	3.9	197
65	miR-511-3p Modulates Genetic Programs of Tumor-Associated Macrophages. Cell Reports, 2012, 1, 141-154.	2.9	193
66	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent ÃŸ-thalassemia. Nature Medicine, 2019, 25, 234-241.	15.2	188
67	InÂVivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases. Cell Stem Cell, 2016, 19, 107-119.	5.2	187
68	ERK1 and ERK2 mitogen-activated protein kinases affect Ras-dependent cell signaling differentially. Journal of Biology, 2006, 5, 14.	2.7	185
69	Transduction of Human CD34+CD38- Bone Marrow and Cord Blood-Derived SCID-Repopulating Cells with Third-Generation Lentiviral Vectors. Molecular Therapy, 2000, 1, 566-573.	3.7	180
70	Identification of Hematopoietic Stem Cellâ€“Specific miRNAs Enables Gene Therapy of Globoid Cell Leukodystrophy. Science Translational Medicine, 2010, 2, 58ra84.	5.8	180
71	[26] Generation of HIV-1 derived lentiviral vectors. Methods in Enzymology, 2002, 346, 454-465.	0.4	178
72	Preclinical modeling highlights the therapeutic potential of hematopoietic stem cell gene editing for correction of SCID-X1. Science Translational Medicine, 2017, 9, .	5.8	176

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73	Comprehensive genomic access to vector integration in clinical gene therapy. Nature Medicine, 2009, 15, 1431-1436.	15.2	173
74	In vivo administration of lentiviral vectors triggers a type I interferon response that restricts hepatocyte gene transfer and promotes vector clearance. Blood, 2007, 109, 2797-2805.	0.6	168
75	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.	3.3	168
76	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.	2.2	166
77	Large-Scale Manufacture and Characterization of a Lentiviral Vector Produced for ClinicalEx VivoGene Therapy Application. Human Gene Therapy, 2011, 22, 343-356.	1.4	165
78	Promoter trapping reveals significant differences in integration site selection between MLV and HIV vectors in primary hematopoietic cells. Blood, 2005, 105, 2307-2315.	0.6	164
79	Robust in vivo gene transfer into adult mammalian neural stem cells by lentiviral vectors. Proceedings of the National Academy of Sciences of the United States of America, 2004, 101, 14835-14840.	3.3	163
80	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.	0.6	159
81	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.	2.1	159
82	A New-Generation Stable Inducible Packaging Cell Line for Lentiviral Vectors. Human Gene Therapy, 2001, 12, 981-997.	1.4	149
83	Robust and Efficient Regulation of Transgene Expression in Vivo by Improved Tetracycline-Dependent Lentiviral Vectors. Molecular Therapy, 2002, 5, 252-261.	3.7	145
84	Human T lymphocytes transduced by lentiviral vectors in the absence of TCR activation maintain an intact immune competence. Blood, 2003, 102, 497-505.	0.6	142
85	Uncovering and Dissecting the Genotoxicity of Self-inactivating Lentiviral Vectors In Vivo. Molecular Therapy, 2014, 22, 774-785.	3.7	142
86	Genomic instability in induced stem cells. Cell Death and Differentiation, 2011, 18, 745-753.	5.0	138
87	Stability of Lentiviral Vector-Mediated Transgene Expression in the Brain in the Presence of Systemic Antivector Immune Responses. Human Gene Therapy, 2005, 16, 741-751.	1.4	137
88	Elusive Identities and Overlapping Phenotypes of Proangiogenic Myeloid Cells in Tumors. American Journal of Pathology, 2010, 176, 1564-1576.	1.9	137
89	Dynamics and genomic landscape of CD8+ T cells undergoing hepatic priming. Nature, 2019, 574, 200-205.	13.7	135
90	ISSCR Guidelines for Stem Cell Research and Clinical Translation: The 2021 update. Stem Cell Reports, 2021, 16, 1398-1408.	2.3	134

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91	Hepatocyte Growth Factor Is a Regulator of Monocyte-Macrophage Function. Journal of Immunology, 2001, 166, 1241-1247.	0.4	129
92	CD4 ⁺ T Cells from IPEX Patients Convert into Functional and Stable Regulatory T Cells by FOXP3 Gene Transfer. Science Translational Medicine, 2013, 5, 215ra174.	5.8	129
93	In vivo delivery of a microRNA-regulated transgene induces antigen-specific regulatory T cells and promotes immunologic tolerance. Blood, 2009, 114, 5152-5161.	0.6	128
94	miR-142-3p Prevents Macrophage Differentiation during Cancer-Induced Myelopoiesis. Immunity, 2013, 38, 1236-1249.	6.6	127
95	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.	2.3	124
96	MET Overexpression Turns Human Primary Osteoblasts into Osteosarcomas. Cancer Research, 2006, 66, 4750-4757.	0.4	123
97	Hepatocyteâ€targeted expression by integraseâ€defective lentiviral vectors induces antigenâ€specific tolerance in mice with low genotoxic risk. Hepatology, 2011, 53, 1696-1707.	3.6	123
98	Liver-directed lentiviral gene therapy in a dog model of hemophilia B. Science Translational Medicine, 2015, 7, 277ra28.	5.8	118
99	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. Journal of Clinical Investigation, 2004, 113, 1118-1129.	3.9	117
100	Efficient gene editing of human long-term hematopoietic stem cells validated by clonal tracking. Nature Biotechnology, 2020, 38, 1298-1308.	9.4	116
101	In VivoTargeting of Tumor Endothelial Cells by Systemic Delivery of Lentiviral Vectors. Human Gene Therapy, 2003, 14, 1193-1206.	1.4	114
102	CRISPR germline engineeringâ€"the community speaks. Nature Biotechnology, 2015, 33, 478-486.	9.4	110
103	â€˜Advancedâ€™ generation lentiviruses as efficient vectors for cardiomyocyte gene transduction in vitro and in vivo. Gene Therapy, 2003, 10, 630-636.	2.3	109
104	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.	6.3	109
105	Interaction of Human Immunodeficiency Virus-Derived Vectors with Wild-Type Virus in Transduced Cells. Journal of Virology, 1999, 73, 7087-7092.	1.5	108
106	Lentiviral Vector-Mediated Gene Transfer in T Cells from Wiskottâ€"Aldrich Syndrome Patients Leads to Functional Correction. Molecular Therapy, 2004, 10, 903-915.	3.7	106
107	Pseudotyped human lentiviral vector-mediated gene transfer to airway epithelia in vivo. Gene Therapy, 2000, 7, 568-574.	2.3	105
108	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.	1.1	105

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109	Whole transcriptome characterization of aberrant splicing events induced by lentiviral vector integrations. Journal of Clinical Investigation, 2012, 122, 1667-1676.	3.9	104
110	A role for miR-155 in enabling tumor-infiltrating innate immune cells to mount effective antitumor responses in mice. Blood, 2013, 122, 243-252.	0.6	102
111	Lentiviral vectors, two decades later. Science, 2016, 353, 1101-1102.	6.0	96
112	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
113	Loss of transcriptional control over endogenous retroelements during reprogramming to pluripotency. Genome Research, 2014, 24, 1251-1259.	2.4	94
114	HIV-based vectors. Preparation and use. Methods in Molecular Medicine, 2002, 69, 259-74.	0.8	89
115	Lentiviral vectors. Advances in Virus Research, 2000, 55, 599-609.	0.9	88
116	Angiopoietin-2 TIEs Up Macrophages in Tumor Angiogenesis. Clinical Cancer Research, 2011, 17, 5226-5232.	3.2	88
117	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.	3.7	86
118	Lentiviral vectorâ€“based insertional mutagenesis identifies genes associated with liver cancer. Nature Methods, 2013, 10, 155-161.	9.0	86
119	Genetic Engineering of Hematopoiesis for Targeted IFN-Î± Delivery Inhibits Breast Cancer Progression. Science Translational Medicine, 2014, 6, 217ra3.	5.8	86
120	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.	5.2	86
121	Genetic engineering of hematopoiesis: current stage of clinical translation and future perspectives. EMBO Molecular Medicine, 2019, 11, .	3.3	86
122	An uncleavable form of proâ€“scatter factor suppresses tumor growth and dissemination in mice. Journal of Clinical Investigation, 2004, 114, 1418-1432.	3.9	85
123	Lentiviral gene transfer and ex vivo expansion of human primitive stem cells capable of primary, secondary, and tertiary multilineage repopulation in NOD/SCID mice. Blood, 2002, 100, 4391-4400.	0.6	84
124	Hyperfunctional coagulation factor IX improves the efficacy of gene therapy in hemophilic mice. Blood, 2012, 120, 4517-4520.	0.6	84
125	Regulated and Multiple miRNA and siRNA Delivery Into Primary Cells by a Lentiviral Platform. Molecular Therapy, 2009, 17, 1039-1052.	3.7	83
126	TIE2â€expressing monocytes/macrophages regulate revascularization of the ischemic limb. EMBO Molecular Medicine, 2013, 5, 858-869.	3.3	83

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127	Efficacy of Gene Therapy for Wiskott-Aldrich Syndrome Using a WAS Promoter/cDNA-Containing Lentiviral Vector and Nonlethal Irradiation. Human Gene Therapy, 2006, 17, 303-313.	1.4	82
128	[29] Transduction of a gene expression cassette using advanced generation lentiviral vectors. Methods in Enzymology, 2002, 346, 514-529.	0.4	78
129	Evidence for Long-term Efficacy and Safety of Gene Therapy for Wiskottâ€“Aldrich Syndrome in Preclinical Models. Molecular Therapy, 2009, 17, 1073-1082.	3.7	77
130	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.	1.4	77
131	Ex vivo gene therapy with lentiviral vectors rescues adenosine deaminase (ADA)â€“deficient mice and corrects their immune and metabolic defects. Blood, 2006, 108, 2979-2988.	0.6	76
132	Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. New England Journal of Medicine, 2021, 385, 1929-1940.	13.9	75
133	Proteasome activity restricts lentiviral gene transfer into hematopoietic stem cells and is down-regulated by cytokines that enhance transduction. Blood, 2006, 107, 4257-4265.	0.6	73
134	Gene Therapy for a Mucopolysaccharidosis Type I Murine Model with Lentiviral-IDUA Vector. Human Gene Therapy, 2005, 16, 81-90.	1.4	72
135	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.	3.7	72
136	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.	3.7	72
137	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.	0.6	71
138	SUMF1 enhances sulfatase activities in vivo in five sulfatase deficiencies. Biochemical Journal, 2007, 403, 305-312.	1.7	69
139	Targeted gene therapy and cell reprogramming in <scp>F</scp>anconi anemia. EMBO Molecular Medicine, 2014, 6, 835-848.	3.3	66
140	Integration of retroviral vectors induces minor changes in the transcriptional activity of T cells from ADA-SCID patients treated with gene therapy. Blood, 2009, 114, 3546-3556.	0.6	65
141	Phagocytosis-shielded lentiviral vectors improve liver gene therapy in nonhuman primates. Science Translational Medicine, 2019, 11, .	5.8	65
142	Role of haematopoietic cells and endothelial progenitors in tumour angiogenesis. Biochimica Et Biophysica Acta: Reviews on Cancer, 2006, 1766, 159-166.	3.3	63
143	Neural Stem Cell Gene Therapy Ameliorates Pathology and Function in a Mouse Model of Globoid Cell Leukodystrophy. Stem Cells, 2011, 29, 1559-1571.	1.4	62
144	Efficient Tet-Dependent Expression of Human Factor IX in Vivo by a New Self-Regulating Lentiviral Vector. Molecular Therapy, 2005, 11, 763-775.	3.7	61

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145	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. Nature Communications, 2022, 13, 1315.	5.8	61
146	Dual-regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. Molecular Therapy, 2014, 22, 1472-1483.	3.7	59
147	Development of lentiviral vectors for antiangiogenic gene delivery. Cancer Gene Therapy, 2001, 8, 879-889.	2.2	58
148	Intracellular distribution of nerve growth factor in rat pheochromocytoma PC12 cells: evidence for a perinuclear and intranuclear location.. Proceedings of the National Academy of Sciences of the United States of America, 1980, 77, 1656-1660.	3.3	57
149	Lentivirus-mediated gene transfer into hematopoietic repopulating cells in baboons. Gene Therapy, 2002, 9, 1464-1471.	2.3	57
150	miRNA-126 Orchestrates an Oncogenic Program in B Cell Precursor Acute Lymphoblastic Leukemia. Cancer Cell, 2016, 29, 905-921.	7.7	57
151	A Human Immunodeficiency Virus Type 1polGene-Derived Sequence (cPPT/CTS) Increases the Efficiency of Transduction of Human Nondividing Monocytes and T Lymphocytes by Lentiviral Vectors. Human Gene Therapy, 2002, 13, 1793-1807.	1.4	56
152	Treatment of the mouse model of mucopolysaccharidosis type IIIB with lentiviral-NAGLU vector. Biochemical Journal, 2005, 388, 639-646.	1.7	56
153	A Comeback for Gene Therapy. Science, 2009, 326, 805-806.	6.0	56
154	Therapeutic benefit of lentiviral-mediated neonatal intracerebral gene therapy in a mouse model of globoid cell leukodystrophy. Human Molecular Genetics, 2014, 23, 3250-3268.	1.4	56
155	Lentiviral vectors escape innate sensing but trigger p53 in human hematopoietic stem and progenitor cells. EMBO Molecular Medicine, 2017, 9, 1198-1211.	3.3	56
156	Reprogramming T Lymphocytes for Melanoma Adoptive Immunotherapy by T-Cell Receptor Gene Transfer with Lentiviral Vectors. Cancer Research, 2009, 69, 9385-9394.	0.4	55
157	Post-natal cardiomyocytes can generate iPS cells with an enhanced capacity toward cardiomyogenic re-differentation. Cell Death and Differentiation, 2012, 19, 1162-1174.	5.0	55
158	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.	3.3	55
159	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.	5.8	55
160	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.	1.5	54
161	Therapeutic gene editing in <scp>CD</scp> 34 ⁺ hematopoietic progenitors from Fanconi anemia patients. EMBO Molecular Medicine, 2017, 9, 1574-1588.	3.3	54
162	Targeted Gene Addition in Human Epithelial Stem Cells by Zinc-finger Nuclease-mediated Homologous Recombination. Molecular Therapy, 2013, 21, 1695-1704.	3.7	53

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163	Transcriptional Targeting of Lentiviral Vectors by Long Terminal Repeat Enhancer Replacement. Journal of Virology, 2002, 76, 3996-4007.	1.5	52
164	Axons mediate the distribution of arylsulfatase a within the mouse hippocampus upon gene delivery. Molecular Therapy, 2005, 12, 669-679.	3.7	52
165	Lentiviral gene transfer ameliorates disease progression in Long-Evans cinnamon rats: An animal model for Wilson disease. Scandinavian Journal of Gastroenterology, 2006, 41, 974-982.	0.6	51
166	Lentiviral Vector Gene Transfer Is Limited by the Proteasome at Postentry Steps in Various Types of Stem Cells. Stem Cells, 2008, 26, 2142-2152.	1.4	51
167	Lentiviral Vector Integration Profiles Differ in Rodent Postmitotic Tissues. Molecular Therapy, 2011, 19, 703-710.	3.7	51
168	The galactocerebrosidase enzyme contributes to the maintenance of a functional hematopoietic stem cell niche. Blood, 2010, 116, 1857-1866.	0.6	50
169	Cyclosporin A and Rapamycin Relieve Distinct Lentiviral Restriction Blocks in Hematopoietic Stem and Progenitor Cells. Molecular Therapy, 2015, 23, 352-362.	3.7	50
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