

Luigi M Naldini

List of Publications by Year in descending order

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312
papers

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citations

1799

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all docs

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docs citations

320
times ranked

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#	ARTICLE	IF	CITATIONS
1	In Vivo Gene Delivery and Stable Transduction of Nondividing Cells by a Lentiviral Vector. <i>Science</i> , 1996, 272, 263-267.	12.6	4,589
2	A Third-Generation Lentivirus Vector with a Conditional Packaging System. <i>Journal of Virology</i> , 1998, 72, 8463-8471.	3.4	2,931
3	Multiply attenuated lentiviral vector achieves efficient gene delivery in vivo. <i>Nature Biotechnology</i> , 1997, 15, 871-875.	17.5	1,826
4	Self-Inactivating Lentivirus Vector for Safe and Efficient In Vivo Gene Delivery. <i>Journal of Virology</i> , 1998, 72, 9873-9880.	3.4	1,676
5	Efficient transfer, integration, and sustained long-term expression of the transgene in adult rat brains injected with a lentiviral vector.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1996, 93, 11382-11388.	7.1	1,420
6	Hepatocyte growth factor is a potent angiogenic factor which stimulates endothelial cell motility and growth.. <i>Journal of Cell Biology</i> , 1992, 119, 629-641.	5.2	1,282
7	Tie2 identifies a hematopoietic lineage of proangiogenic monocytes required for tumor vessel formation and a mesenchymal population of pericyte progenitors. <i>Cancer Cell</i> , 2005, 8, 211-226.	16.8	1,212
8	Viral vectors for gene therapy: the art of turning infectious agents into vehicles of therapeutics. <i>Nature Medicine</i> , 2001, 7, 33-40.	30.7	1,205
9	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. <i>Science</i> , 2013, 341, 1233-1235.	12.6	998
10	Gene therapy returns to centre stage. <i>Nature</i> , 2015, 526, 351-360.	27.8	943
11	Lentiviral Hematopoietic Stem Cell Gene Therapy in Patients with Wiskott-Aldrich Syndrome. <i>Science</i> , 2013, 341, 1233-1235.	12.6	900
12	Gene transfer by lentiviral vectors is limited by nuclear translocation and rescued by HIV-1 pol sequences. <i>Nature Genetics</i> , 2000, 25, 217-222.	21.4	887
13	Gene editing in human stem cells using zinc finger nucleases and integrase-defective lentiviral vector delivery. <i>Nature Biotechnology</i> , 2007, 25, 1298-1306.	17.5	797
14	Hematopoietic stem cell gene transfer in a tumor-prone mouse model uncovers low genotoxicity of lentiviral vector integration. <i>Nature Biotechnology</i> , 2006, 24, 687-696.	17.5	648
15	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.	16.8	543
16	Targeting exogenous genes to tumor angiogenesis by transplantation of genetically modified hematopoietic stem cells. <i>Nature Medicine</i> , 2003, 9, 789-795.	30.7	539
17	Endogenous microRNA can be broadly exploited to regulate transgene expression according to tissue, lineage and differentiation state. <i>Nature Biotechnology</i> , 2007, 25, 1457-1467.	17.5	539
18	FcR γ Activation Regulates Inflammation-Associated Squamous Carcinogenesis. <i>Cancer Cell</i> , 2010, 17, 121-134.	16.8	537

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19	Targeted genome editing in human repopulating haematopoietic stem cells. <i>Nature</i> , 2014, 510, 235-240.	27.8	517
20	Cleavage of the Plasma Membrane Na ⁺ /Ca ²⁺ Exchanger in Excitotoxicity. <i>Cell</i> , 2005, 120, 275-285.	28.9	511
21	An unbiased genome-wide analysis of zinc-finger nuclease specificity. <i>Nature Biotechnology</i> , 2011, 29, 816-823.	17.5	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. <i>Journal of Clinical Investigation</i> , 2009, 119, 964-975.	8.2	488
23	Endogenous microRNA regulation suppresses transgene expression in hematopoietic lineages and enables stable gene transfer. <i>Nature Medicine</i> , 2006, 12, 585-591.	30.7	460
24	Identification of proangiogenic TIE2-expressing monocytes (TEMs) in human peripheral blood and cancer. <i>Blood</i> , 2007, 109, 5276-5285.	1.4	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. <i>Blood</i> , 2012, 119, 5697-5705.	1.4	437
26	Forebrain ependymal cells are Notch-dependent and generate neuroblasts and astrocytes after stroke. <i>Nature Neuroscience</i> , 2009, 12, 259-267.	14.8	415
27	Editing T cell specificity towards leukemia by zinc finger nucleases and lentiviral gene transfer. <i>Nature Medicine</i> , 2012, 18, 807-815.	30.7	398
28	Stable transduction of quiescent CD34 ⁺ CD38 ^{âˆ’} human hematopoietic cells by HIV-1-based lentiviral vectors. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1999, 96, 2988-2993.	7.1	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. <i>Lancet, The</i> , 2016, 388, 476-487.	13.7	393
30	Inheritable Silencing of Endogenous Genes by Hit-and-Run Targeted Epigenetic Editing. <i>Cell</i> , 2016, 167, 219-232.e14.	28.9	363
31	Exploiting and antagonizing microRNA regulation for therapeutic and experimental applications. <i>Nature Reviews Genetics</i> , 2009, 10, 578-585.	16.3	362
32	Cardiomyocytes induce endothelial cells to trans-differentiate into cardiac muscle: Implications for myocardium regeneration. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2001, 98, 10733-10738.	7.1	357
33	Ex vivo gene transfer and correction for cell-based therapies. <i>Nature Reviews Genetics</i> , 2011, 12, 301-315.	16.3	340
34	Lentiviral vectors: excellent tools for experimental gene transfer and promising candidates for gene therapy. <i>Journal of Gene Medicine</i> , 2000, 2, 308-316.	2.8	318
35	Adopt a moratorium on heritable genome editing. <i>Nature</i> , 2019, 567, 165-168.	27.8	314
36	A distinguishing gene signature shared by tumor-infiltrating Tie2-expressing monocytes, blood resident monocytes, and embryonic macrophages suggests common functions and developmental relationships. <i>Blood</i> , 2009, 114, 901-914.	1.4	306

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37	CD44v6-targeted T cells mediate potent antitumor effects against acute myeloid leukemia and multiple myeloma. <i>Blood</i> , 2013, 122, 3461-3472.	1.4	306
38	Cell-substratum interaction of cultured avian osteoclasts is mediated by specific adhesion structures.. <i>Journal of Cell Biology</i> , 1984, 99, 1696-1705.	5.2	303
39	Stable knockdown of microRNA in vivo by lentiviral vectors. <i>Nature Methods</i> , 2009, 6, 63-66.	19.0	301
40	Site-specific integration and tailoring of cassette design for sustainable gene transfer. <i>Nature Methods</i> , 2011, 8, 861-869.	19.0	300
41	Coordinate dual-gene transgenesis by lentiviral vectors carrying synthetic bidirectional promoters. <i>Nature Biotechnology</i> , 2005, 23, 108-116.	17.5	293
42	Targeting the tumor and its microenvironment by a dual-function decoy Met receptor. <i>Cancer Cell</i> , 2004, 6, 61-73.	16.8	282
43	Efficient lentiviral transduction of liver requires cell cycling in vivo. <i>Nature Genetics</i> , 2000, 24, 49-52.	21.4	278
44	Lentiviruses as gene transfer agents for delivery to non-dividing cells. <i>Current Opinion in Biotechnology</i> , 1998, 9, 457-463.	6.6	269
45	Tumor-Targeted Interferon- β Delivery by Tie2-Expressing Monocytes Inhibits Tumor Growth and Metastasis. <i>Cancer Cell</i> , 2008, 14, 299-311.	16.8	267
46	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. <i>Journal of Clinical Investigation</i> , 2004, 113, 1118-1129.	8.2	256
47	Tie2-expressing monocytes: regulation of tumor angiogenesis and therapeutic implications. <i>Trends in Immunology</i> , 2007, 28, 519-524.	6.8	255
48	A microRNA-regulated lentiviral vector mediates stable correction of hemophilia B mice. <i>Blood</i> , 2007, 110, 4144-4152.	1.4	246
49	The MET oncogene drives a genetic programme linking cancer to haemostasis. <i>Nature</i> , 2005, 434, 396-400.	27.8	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. <i>Blood</i> , 2002, 100, 813-822.	1.4	240
51	Tie2-Expressing Monocytes and Tumor Angiogenesis: Regulation by Hypoxia and Angiopoietin-2. <i>Cancer Research</i> , 2007, 67, 8429-8432.	0.9	240
52	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.	11.1	237
53	Biological Activation of pro-HGF (Hepatocyte Growth Factor) by Urokinase Is Controlled by a Stoichiometric Reaction. <i>Journal of Biological Chemistry</i> , 1995, 270, 603-611.	3.4	232
54	Efficient Gene Delivery and Targeted Expression to Hepatocytes In Vivo by Improved Lentiviral Vectors. <i>Human Gene Therapy</i> , 2002, 13, 243-260.	2.7	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.	7.1	219
56	miR-126 Regulates Distinct Self-Renewal Outcomes in Normal and Malignant Hematopoietic Stem Cells. Cancer Cell, 2016, 29, 214-228.	16.8	216
57	Gene therapy for ADAâ€¦SCID, the first marketing approval of an <i>exÂ´vivo</i> 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
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.	1.4	206
59	Generation of Potent and Stable Human CD4+ T Regulatory Cells by Activation-independent Expression of FOXP3. Molecular Therapy, 2008, 16, 194-202.	8.2	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.	8.2	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.	1.4	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.	30.7	198
63	Attenuation of miR-126 Activity Expands HSC InÂ´vivo without Exhaustion. Cell Stem Cell, 2012, 11, 799-811.	11.1	197
64	Gene therapy of metachromatic leukodystrophy reverses neurological damage and deficits in mice. Journal of Clinical Investigation, 2006, 116, 3070-3082.	8.2	197
65	miR-511-3p Modulates Genetic Programs of Tumor-Associated Macrophages. Cell Reports, 2012, 1, 141-154.	6.4	193
66	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent Å-thalassemia. Nature Medicine, 2019, 25, 234-241.	30.7	188
67	InÂ´vivo 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
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.	8.2	180
70	Identification of Hematopoietic Stem Cellâ€¦Specific miRNAs Enables Gene Therapy of Globoid Cell Leukodystrophy. Science Translational Medicine, 2010, 2, 58ra84.	12.4	180
71	[26] Generation of HIV-1 derived lentiviral vectors. Methods in Enzymology, 2002, 346, 454-465.	1.0	178
72	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

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73	Comprehensive genomic access to vector integration in clinical gene therapy. <i>Nature Medicine</i> , 2009, 15, 1431-1436.	30.7	173
74	In vivo administration of lentiviral vectors triggers a type I interferon response that restricts hepatocyte gene transfer and promotes vector clearance. <i>Blood</i> , 2007, 109, 2797-2805.	1.4	168
75	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.	7.1	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. <i>Lancet Haematology</i> , 2019, 6, e239-e253.	4.6	166
77	Large-Scale Manufacture and Characterization of a Lentiviral Vector Produced for Clinical <i>In Vivo</i> Gene Therapy Application. <i>Human Gene Therapy</i> , 2011, 22, 343-356.	2.7	165
78	Promoter trapping reveals significant differences in integration site selection between MLV and HIV vectors in primary hematopoietic cells. <i>Blood</i> , 2005, 105, 2307-2315.	1.4	164
79	Robust in vivo gene transfer into adult mammalian neural stem cells by lentiviral vectors. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2004, 101, 14835-14840.	7.1	163
80	Gene therapy augments the efficacy of hematopoietic cell transplantation and fully corrects mucopolysaccharidosis type I phenotype in the mouse model. <i>Blood</i> , 2010, 116, 5130-5139.	1.4	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. <i>Neurobiology of Disease</i> , 2012, 46, 41-51.	4.4	159
82	A New-Generation Stable Inducible Packaging Cell Line for Lentiviral Vectors. <i>Human Gene Therapy</i> , 2001, 12, 981-997.	2.7	149
83	Robust and Efficient Regulation of Transgene Expression in Vivo by Improved Tetracycline-Dependent Lentiviral Vectors. <i>Molecular Therapy</i> , 2002, 5, 252-261.	8.2	145
84	Human T lymphocytes transduced by lentiviral vectors in the absence of TCR activation maintain an intact immune competence. <i>Blood</i> , 2003, 102, 497-505.	1.4	142
85	Uncovering and Dissecting the Genotoxicity of Self-inactivating Lentiviral Vectors In Vivo. <i>Molecular Therapy</i> , 2014, 22, 774-785.	8.2	142
86	Genomic instability in induced stem cells. <i>Cell Death and Differentiation</i> , 2011, 18, 745-753.	11.2	138
87	Stability of Lentiviral Vector-Mediated Transgene Expression in the Brain in the Presence of Systemic Antivector Immune Responses. <i>Human Gene Therapy</i> , 2005, 16, 741-751.	2.7	137
88	Elusive Identities and Overlapping Phenotypes of Proangiogenic Myeloid Cells in Tumors. <i>American Journal of Pathology</i> , 2010, 176, 1564-1576.	3.8	137
89	Dynamics and genomic landscape of CD8+ T cells undergoing hepatic priming. <i>Nature</i> , 2019, 574, 200-205.	27.8	135
90	ISSCR Guidelines for Stem Cell Research and Clinical Translation: The 2021 update. <i>Stem Cell Reports</i> , 2021, 16, 1398-1408.	4.8	134

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91	Hepatocyte Growth Factor Is a Regulator of Monocyte-Macrophage Function. <i>Journal of Immunology</i> , 2001, 166, 1241-1247.	0.8	129
92	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.	12.4	129
93	In vivo delivery of a microRNA-regulated transgene induces antigen-specific regulatory T cells and promotes immunologic tolerance. <i>Blood</i> , 2009, 114, 5152-5161.	1.4	128
94	miR-142-3p Prevents Macrophage Differentiation during Cancer-Induced Myelopoiesis. <i>Immunity</i> , 2013, 38, 1236-1249.	14.3	127
95	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.	4.8	124
96	MET Overexpression Turns Human Primary Osteoblasts into Osteosarcomas. <i>Cancer Research</i> , 2006, 66, 4750-4757.	0.9	123
97	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.	7.3	123
98	Liver-directed lentiviral gene therapy in a dog model of hemophilia B. <i>Science Translational Medicine</i> , 2015, 7, 277ra28.	12.4	118
99	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. <i>Journal of Clinical Investigation</i> , 2004, 113, 1118-1129.	8.2	117
100	Efficient gene editing of human long-term hematopoietic stem cells validated by clonal tracking. <i>Nature Biotechnology</i> , 2020, 38, 1298-1308.	17.5	116
101	In Vivo Targeting of Tumor Endothelial Cells by Systemic Delivery of Lentiviral Vectors. <i>Human Gene Therapy</i> , 2003, 14, 1193-1206.	2.7	114
102	CRISPR germline engineering—the community speaks. <i>Nature Biotechnology</i> , 2015, 33, 478-486.	17.5	110
103	Advanced™ generation lentiviruses as efficient vectors for cardiomyocyte gene transduction in vitro and in vivo. <i>Gene Therapy</i> , 2003, 10, 630-636.	4.5	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. <i>Lancet</i> , The, 2022, 399, 372-383.	13.7	109
105	Interaction of Human Immunodeficiency Virus-Derived Vectors with Wild-Type Virus in Transduced Cells. <i>Journal of Virology</i> , 1999, 73, 7087-7092.	3.4	108
106	Lentiviral Vector-Mediated Gene Transfer in T Cells from Wiskott-Aldrich Syndrome Patients Leads to Functional Correction. <i>Molecular Therapy</i> , 2004, 10, 903-915.	8.2	106
107	Pseudotyped human lentiviral vector-mediated gene transfer to airway epithelia in vivo. <i>Gene Therapy</i> , 2000, 7, 568-574.	4.5	105
108	Systemic and Targeted Delivery of Semaphorin 3A Inhibits Tumor Angiogenesis and Progression in Mouse Tumor Models. <i>Arteriosclerosis, Thrombosis, and Vascular Biology</i> , 2011, 31, 741-749.	2.4	105

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109	Whole transcriptome characterization of aberrant splicing events induced by lentiviral vector integrations. <i>Journal of Clinical Investigation</i> , 2012, 122, 1667-1676.	8.2	104
110	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.	1.4	102
111	Lentiviral vectors, two decades later. <i>Science</i> , 2016, 353, 1101-1102.	12.6	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. <i>Biology of Blood and Marrow Transplantation</i> , 2015, 21, 233-241.	2.0	95
113	Loss of transcriptional control over endogenous retroelements during reprogramming to pluripotency. <i>Genome Research</i> , 2014, 24, 1251-1259.	5.5	94
114	HIV-based vectors. Preparation and use. <i>Methods in Molecular Medicine</i> , 2002, 69, 259-74.	0.8	89
115	Lentiviral vectors. <i>Advances in Virus Research</i> , 2000, 55, 599-609.	2.1	88
116	Angiopoietin-2 TIEs Up Macrophages in Tumor Angiogenesis. <i>Clinical Cancer Research</i> , 2011, 17, 5226-5232.	7.0	88
117	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.	8.2	86
118	Lentiviral vector-based insertional mutagenesis identifies genes associated with liver cancer. <i>Nature Methods</i> , 2013, 10, 155-161.	19.0	86
119	Genetic Engineering of Hematopoiesis for Targeted IFN- γ Delivery Inhibits Breast Cancer Progression. <i>Science Translational Medicine</i> , 2014, 6, 217ra3.	12.4	86
120	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.	11.1	86
121	Genetic engineering of hematopoiesis: current stage of clinical translation and future perspectives. <i>EMBO Molecular Medicine</i> , 2019, 11, .	6.9	86
122	An uncleavable form of pro- α 1(I) scatter factor suppresses tumor growth and dissemination in mice. <i>Journal of Clinical Investigation</i> , 2004, 114, 1418-1432.	8.2	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. <i>Blood</i> , 2002, 100, 4391-4400.	1.4	84
124	Hyperfunctional coagulation factor IX improves the efficacy of gene therapy in hemophilic mice. <i>Blood</i> , 2012, 120, 4517-4520.	1.4	84
125	Regulated and Multiple miRNA and siRNA Delivery Into Primary Cells by a Lentiviral Platform. <i>Molecular Therapy</i> , 2009, 17, 1039-1052.	8.2	83
126	TIE2-expressing monocytes/macrophages regulate revascularization of the ischemic limb. <i>EMBO Molecular Medicine</i> , 2013, 5, 858-869.	6.9	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. <i>Human Gene Therapy</i> , 2006, 17, 303-313.	2.7	82
128	[29] Transduction of a gene expression cassette using advanced generation lentiviral vectors. <i>Methods in Enzymology</i> , 2002, 346, 514-529.	1.0	78
129	Evidence for Long-term Efficacy and Safety of Gene Therapy for Wiskott-Aldrich Syndrome in Preclinical Models. <i>Molecular Therapy</i> , 2009, 17, 1073-1082.	8.2	77
130	Widespread enzymatic correction of CNS tissues by a single intracerebral injection of therapeutic lentiviral vector in leukodystrophy mouse models. <i>Human Molecular Genetics</i> , 2010, 19, 2208-2227.	2.9	77
131	Ex vivo gene therapy with lentiviral vectors rescues adenosine deaminase (ADA) deficient mice and corrects their immune and metabolic defects. <i>Blood</i> , 2006, 108, 2979-2988.	1.4	76
132	Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. <i>New England Journal of Medicine</i> , 2021, 385, 1929-1940.	27.0	75
133	Proteasome activity restricts lentiviral gene transfer into hematopoietic stem cells and is down-regulated by cytokines that enhance transduction. <i>Blood</i> , 2006, 107, 4257-4265.	1.4	73
134	Gene Therapy for a Mucopolysaccharidosis Type I Murine Model with Lentiviral-IDUA Vector. <i>Human Gene Therapy</i> , 2005, 16, 81-90.	2.7	72
135	A MicroRNA-regulated and GP64-pseudotyped Lentiviral Vector Mediates Stable Expression of FVIII in a Murine Model of Hemophilia A. <i>Molecular Therapy</i> , 2011, 19, 723-730.	8.2	72
136	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.	8.2	72
137	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.	1.4	71
138	SUMF1 enhances sulfatase activities in vivo in five sulfatase deficiencies. <i>Biochemical Journal</i> , 2007, 403, 305-312.	3.7	69
139	Targeted gene therapy and cell reprogramming in α -thalassaemia. <i>EMBO Molecular Medicine</i> , 2014, 6, 835-848.	6.9	66
140	Integration of retroviral vectors induces minor changes in the transcriptional activity of T cells from ADA-SCID patients treated with gene therapy. <i>Blood</i> , 2009, 114, 3546-3556.	1.4	65
141	Phagocytosis-shielded lentiviral vectors improve liver gene therapy in nonhuman primates. <i>Science Translational Medicine</i> , 2019, 11, .	12.4	65
142	Role of haematopoietic cells and endothelial progenitors in tumour angiogenesis. <i>Biochimica Et Biophysica Acta: Reviews on Cancer</i> , 2006, 1766, 159-166.	7.4	63
143	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.	3.2	62
144	Efficient Tet-Dependent Expression of Human Factor IX in Vivo by a New Self-Regulating Lentiviral Vector. <i>Molecular Therapy</i> , 2005, 11, 763-775.	8.2	61

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145	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.	12.8	61
146	Dual-regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. <i>Molecular Therapy</i> , 2014, 22, 1472-1483.	8.2	59
147	Development of lentiviral vectors for antiangiogenic gene delivery. <i>Cancer Gene Therapy</i> , 2001, 8, 879-889.	4.6	58
148	Intracellular distribution of nerve growth factor in rat pheochromocytoma PC12 cells: evidence for a perinuclear and intranuclear location.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1980, 77, 1656-1660.	7.1	57
149	Lentivirus-mediated gene transfer into hematopoietic repopulating cells in baboons. <i>Gene Therapy</i> , 2002, 9, 1464-1471.	4.5	57
150	miRNA-126 Orchestrates an Oncogenic Program in B Cell Precursor Acute Lymphoblastic Leukemia. <i>Cancer Cell</i> , 2016, 29, 905-921.	16.8	57
151	A Human Immunodeficiency Virus Type 1 polGene-Derived Sequence (cPPT/CTS) Increases the Efficiency of Transduction of Human Nondividing Monocytes and T Lymphocytes by Lentiviral Vectors. <i>Human Gene Therapy</i> , 2002, 13, 1793-1807.	2.7	56
152	Treatment of the mouse model of mucopolysaccharidosis type IIIB with lentiviral-NAGLU vector. <i>Biochemical Journal</i> , 2005, 388, 639-646.	3.7	56
153	A Comeback for Gene Therapy. <i>Science</i> , 2009, 326, 805-806.	12.6	56
154	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.	2.9	56
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311	Assessing Stealth and Sensed Base Editing in Human Hematopoietic Stem/Progenitor Cells. Blood, 2021, 138, 3976-3976.	1.4	0
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