

Fulvio Mavilio

List of Publications by Year in descending order

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189
papers

18,056
citations

16411

64
h-index

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190
docs citations

190
times ranked

13989
citing authors

#	ARTICLE	IF	CITATIONS
1	Muscle Regeneration by Bone Marrow-Derived Myogenic Progenitors. <i>Science</i> , 1998, 279, 1528-1530.	6.0	2,541
2	HSV-TK Gene Transfer into Donor Lymphocytes for Control of Allogeneic Graft-Versus-Leukemia. <i>Science</i> , 1997, 276, 1719-1724.	6.0	1,146
3	Gene Therapy in Peripheral Blood Lymphocytes and Bone Marrow for ADA- Immunodeficient Patients. <i>Science</i> , 1995, 270, 470-475.	6.0	775
4	Correction of junctional epidermolysis bullosa by transplantation of genetically modified epidermal stem cells. <i>Nature Medicine</i> , 2006, 12, 1397-1402.	15.2	593
5	Sequential activation of HOX2 homeobox genes by retinoic acid in human embryonal carcinoma cells. <i>Nature</i> , 1990, 346, 763-766.	13.7	527
6	IL-7 and IL-15 instruct the generation of human memory stem T cells from naive precursors. <i>Blood</i> , 2013, 121, 573-584.	0.6	455
7	Muscle-derived hematopoietic stem cells are hematopoietic in origin. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2002, 99, 1341-1346.	3.3	431
8	Outcomes Following Gene Therapy in Patients With Severe Wiskott-Aldrich Syndrome. <i>JAMA - Journal of the American Medical Association</i> , 2015, 313, 1550.	3.8	327
9	Differential regulation by retinoic acid of the homeobox genes of the four HOX loci in human embryonal carcinoma cells. <i>Mechanisms of Development</i> , 1991, 33, 215-227.	1.7	289
10	Nuclear architecture dictates HIV-1 integration site selection. <i>Nature</i> , 2015, 521, 227-231.	13.7	277
11	Human embryonic hemopoiesis. Kinetics of progenitors and precursors underlying the yolk sac---liver transition.. <i>Journal of Clinical Investigation</i> , 1986, 78, 51-60.	3.9	265
12	The future of gene therapy. <i>Nature</i> , 2004, 427, 779-781.	13.7	262
13	Cytokine-dependent granulocytic differentiation. Regulation of proliferative and differentiative responses in a murine progenitor cell line. <i>Journal of Immunology</i> , 1987, 138, 3829-35.	0.4	262
14	Hot spots of retroviral integration in human CD34+ hematopoietic cells. <i>Blood</i> , 2007, 110, 1770-1778.	0.6	248
15	Peripheral blood lymphocytes as target cells of retroviral vector- mediated gene transfer. <i>Blood</i> , 1994, 83, 1988-1997.	0.6	234
16	Multilineage hematopoietic reconstitution without clonal selection in ADA-SCID patients treated with stem cell gene therapy. <i>Journal of Clinical Investigation</i> , 2007, 117, 2233-2240.	3.9	231
17	Haemoglobin switching in human embryos: asynchrony of $\hat{\Gamma}\hat{\eta}$ $\hat{\alpha}\hat{\tau}'$ $\hat{\Gamma}\hat{\pm}$ and $\hat{\Gamma}\mu$ $\hat{\alpha}\hat{\tau}'$ $\hat{\Gamma}\hat{\beta}$ -globin switches in primitive and definitive erythropoietic lineage. <i>Nature</i> , 1985, 313, 235-238.	13.7	218
18	HMG1 interacts with HOX proteins and enhances their DNA binding and transcriptional activation.. <i>EMBO Journal</i> , 1996, 15, 4981-4991.	3.5	216

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19	The subcellular localization of PBX1 and EXD proteins depends on nuclear import and export signals and is modulated by association with PREP1 and HTH. <i>Genes and Development</i> , 1999, 13, 946-953.	2.7	214
20	Differential and stage-related expression in embryonic tissues of a new human homoeobox gene. <i>Nature</i> , 1986, 324, 664-668.	13.7	208
21	The novel homeoprotein Prep1 modulates Pbx-Hox protein cooperativity. <i>EMBO Journal</i> , 1998, 17, 1434-1445.	3.5	193
22	HOX gene activation by retinoic acid. <i>Trends in Genetics</i> , 1991, 7, 329-334.	2.9	189
23	C/EBP β regulates cell cycle and self-renewal of human limbal stem cells. <i>Journal of Cell Biology</i> , 2007, 177, 1037-1049.	2.3	181
24	Long-term microdystrophin gene therapy is effective in a canine model of Duchenne muscular dystrophy. <i>Nature Communications</i> , 2017, 8, 16105.	5.8	175
25	Comprehensive genomic access to vector integration in clinical gene therapy. <i>Nature Medicine</i> , 2009, 15, 1431-1436.	15.2	173
26	Retroviral vector integration deregulates gene expression but has no consequence on the biology and function of transplanted T cells. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2006, 103, 1457-1462.	3.3	172
27	Safety of retroviral gene marking with a truncated NGF receptor. <i>Nature Medicine</i> , 2003, 9, 367-369.	15.2	169
28	Clonal analysis of stably transduced human epidermal stem cells in culture.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1996, 93, 10371-10376.	3.3	164
29	Prep1, a novel functional partner of Pbx proteins. <i>EMBO Journal</i> , 1998, 17, 1423-1433.	3.5	159
30	Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated editing of the human β -globin locus. <i>Blood</i> , 2018, 131, 1960-1973.	0.6	156
31	High-definition mapping of retroviral integration sites identifies active regulatory elements in human multipotent hematopoietic progenitors. <i>Blood</i> , 2010, 116, 5507-5517.	0.6	150
32	Retinoic acid induces stage-specific antero-posterior transformation of rostral central nervous system. <i>Mechanisms of Development</i> , 1995, 51, 83-98.	1.7	143
33	Gene Therapy Prolongs Survival and Restores Function in Murine and Canine Models of Myotubular Myopathy. <i>Science Translational Medicine</i> , 2014, 6, 220ra10.	5.8	141
34	Nup153 and Nup98 bind the HIV-1 core and contribute to the early steps of HIV-1 replication. <i>Virology</i> , 2013, 440, 8-18.	1.1	139
35	Transfer of the HSV-tk Gene into Donor Peripheral Blood Lymphocytes for In Vivo Modulation of Donor Anti-Tumor Immunity after Allogeneic Bone Marrow Transplantation. The San Raffaele Hospital, Milan, Italy. <i>Human Gene Therapy</i> , 1995, 6, 813-819.	1.4	137
36	Failure to correct murine muscular dystrophy. <i>Nature</i> , 2001, 411, 1014-1015.	13.7	137

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37	Activation of four homeobox gene clusters in human embryonal carcinoma cells induced to differentiate by retinoic acid. <i>Differentiation</i> , 1988, 37, 73-79.	1.0	136
38	Lentiviral vector integration in the human genome induces alternative splicing and generates aberrant transcripts. <i>Journal of Clinical Investigation</i> , 2012, 122, 1653-1666.	3.9	134
39	An in vivo model of somatic cell gene therapy for human severe combined immunodeficiency. <i>Science</i> , 1991, 251, 1363-1366.	6.0	132
40	Myogenic stem cells for the therapy of primary myopathies: wishful thinking or therapeutic perspective?. <i>Journal of Clinical Investigation</i> , 2000, 105, 1669-1674.	3.9	131
41	Two human homeobox genes, c1 and c8: structure analysis and expression in embryonic development.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1987, 84, 4914-4918.	3.3	127
42	High efficiency myogenic conversion of human fibroblasts by adenoviral vector-mediated MyoD gene transfer. An alternative strategy for ex vivo gene therapy of primary myopathies.. <i>Journal of Clinical Investigation</i> , 1998, 101, 2119-2128.	3.9	127
43	Molecular mechanisms of human hemoglobin switching: selective undermethylation and expression of globin genes in embryonic, fetal, and adult erythroblasts.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1983, 80, 6907-6911.	3.3	126
44	Specificity of HOX protein function depends on DNA-protein and protein-protein interactions, both mediated by the homeo domain.. <i>Genes and Development</i> , 1994, 8, 732-744.	2.7	120
45	High-level erythroid-specific gene expression in primary human and murine hematopoietic cells with self-inactivating lentiviral vectors. <i>Blood</i> , 2001, 98, 2664-2672.	0.6	106
46	Epithelial stem cells in corneal regeneration and epidermal gene therapy. <i>Journal of Pathology</i> , 2009, 217, 217-228.	2.1	106
47	IL4 gene delivery to the CNS recruits regulatory T cells and induces clinical recovery in mouse models of multiple sclerosis. <i>Gene Therapy</i> , 2008, 15, 504-515.	2.3	101
48	Functional dissection of a transcriptionally active, target-specific Hox-Pbx complex. <i>EMBO Journal</i> , 1997, 16, 3644-3654.	3.5	100
49	Optimization of CRISPR/Cas9 Delivery to Human Hematopoietic Stem and Progenitor Cells for Therapeutic Genomic Rearrangements. <i>Molecular Therapy</i> , 2019, 27, 137-150.	3.7	97
50	HMG1 interacts with HOX proteins and enhances their DNA binding and transcriptional activation. <i>EMBO Journal</i> , 1996, 15, 4981-91.	3.5	96
51	A human homoeo box gene specifically expressed in spinal cord during embryonic development. <i>Nature</i> , 1986, 320, 763-765.	13.7	95
52	Mechanisms of Retroviral Integration and Mutagenesis. <i>Human Gene Therapy</i> , 2013, 24, 119-131.	1.4	94
53	Site-Specific Integration of Functional Transgenes into the Human Genome by Adeno/AAV Hybrid Vectors. <i>Molecular Therapy</i> , 2004, 10, 660-670.	3.7	92
54	Editing a β -globin repressor binding site restores fetal hemoglobin synthesis and corrects the sickle cell disease phenotype. <i>Science Advances</i> , 2020, 6, .	4.7	91

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55	Transcription Factor Binding Sites Are Genetic Determinants of Retroviral Integration in the Human Genome. PLoS ONE, 2009, 4, e4571.	1.1	87
56	Gene therapy for Wiskott-Aldrich syndrome in a severely affected adult. Blood, 2017, 130, 1327-1335.	0.6	83
57	Correction of β -thalassemia major by gene transfer in haematopoietic progenitors of pediatric patients. EMBO Molecular Medicine, 2010, 2, 315-328.	3.3	82
58	Systemic AAV8-Mediated Gene Therapy Drives Whole-Body Correction of Myotubular Myopathy in Dogs. Molecular Therapy, 2017, 25, 839-854.	3.7	81
59	Transcriptional Enhancers Induce Insertional Gene Deregulation Independently From the Vector Type and Design. Molecular Therapy, 2009, 17, 851-856.	3.7	79
60	The upstream region of the human homeobox gene HOX3D is a target for regulation by retinoic acid and HOX homeoproteins.. EMBO Journal, 1992, 11, 265-277.	3.5	77
61	Regulation of vertebrate homeobox-containing genes by morphogens. FEBS Journal, 1993, 212, 273-288.	0.2	76
62	In vitro and transgenic analysis of a human HOXD4 retinoid-responsive enhancer. Development (Cambridge), 1996, 122, 1895-1907.	1.2	74
63	The thyroid transcription factor-1 gene is a candidate target for regulation by Hox proteins.. EMBO Journal, 1994, 13, 3339-3347.	3.5	66
64	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
65	Expression of cellular oncogenes in primary cells from human acute leukemias.. Proceedings of the National Academy of Sciences of the United States of America, 1986, 83, 4394-4398.	3.3	64
66	A Retroviral Vector Containing a Muscle-Specific Enhancer Drives Gene Expression Only in Differentiated Muscle Fibers. Human Gene Therapy, 1995, 6, 733-742.	1.4	64
67	Long-term Engraftment of Single Genetically Modified Human Epidermal Holoclones Enables Safety Pre-assessment of Cutaneous Gene Therapy. Molecular Therapy, 2007, 15, 1670-1676.	3.7	64
68	Regulation of the human HOXD4 gene by retinoids. Mechanisms of Development, 1993, 44, 139-154.	1.7	62
69	Retroviral Vector-Mediated Gene Transfer into Human Primary Myogenic Cells Leads to Expression in Muscle Fibers <i>In Vivo</i> . Human Gene Therapy, 1993, 4, 713-723.	1.4	61
70	The Recruitment of SOX/OCT Complexes and the Differential Activity of HOXA1 and HOXB1 Modulate the Hoxb1 Auto-regulatory Enhancer Function. Journal of Biological Chemistry, 2001, 276, 20506-20515.	1.6	61
71	Correction of Laminin-5 Deficiency in Human Epidermal Stem Cells by Transcriptionally Targeted Lentiviral Vectors. Molecular Therapy, 2008, 16, 1977-1985.	3.7	60
72	Inhibition of retinoic acid-induced activation of 3' human HOXB genes by antisense oligonucleotides affects sequential activation of genes located upstream in the four HOX clusters.. Proceedings of the National Academy of Sciences of the United States of America, 1994, 91, 5335-5339.	3.3	58

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73	Transcriptional Targeting of Retroviral Vectors to the Erythroblastic Progeny of Transduced Hematopoietic Stem Cells. <i>Blood</i> , 1999, 93, 3276-3285.	0.6	58
74	Toward Epidermal Stem Cell-Mediated <i>ex Vivo</i> Gene Therapy of Junctional Epidermolysis Bullosa. <i>Human Gene Therapy</i> , 2000, 11, 2283-2287.	1.4	58
75	Interactions between Retroviruses and the Host Cell Genome. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 8, 31-41.	1.8	57
76	Dynamic Transcriptional and Epigenetic Regulation of Human Epidermal Keratinocyte Differentiation. <i>Stem Cell Reports</i> , 2016, 6, 618-632.	2.3	55
77	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
78	Efficient Non-viral Gene Delivery into Human Hematopoietic Stem Cells by Minicircle Sleeping Beauty Transposon Vectors. <i>Molecular Therapy</i> , 2018, 26, 1137-1153.	3.7	53
79	<i>Ex Vivo</i> COL7A1 Correction for Recessive Dystrophic Epidermolysis Bullosa Using CRISPR/Cas9 and Homology-Directed Repair. <i>Molecular Therapy - Nucleic Acids</i> , 2018, 12, 554-567.	2.3	53
80	Transcriptional Targeting of Lentiviral Vectors by Long Terminal Repeat Enhancer Replacement. <i>Journal of Virology</i> , 2002, 76, 3996-4007.	1.5	52
81	Myogenic conversion of mammalian fibroblasts induced by differentiating muscle cells. <i>Journal of Cell Science</i> , 1995, 108, 2733-2739.	1.2	50
82	Risk assessment in skin gene therapy: viral cellular fusion transcripts generated by proviral transcriptional read-through in keratinocytes transduced with self-inactivating lentiviral vectors. <i>Gene Therapy</i> , 2011, 18, 674-681.	2.3	49
83	Gene Therapy Approaches to Hemoglobinopathies. <i>Hematology/Oncology Clinics of North America</i> , 2017, 31, 835-852.	0.9	49
84	Reversible Immortalization of Human Myogenic Cells by Site-Specific Excision of a Retrovirally Transferred Oncogene. <i>Human Gene Therapy</i> , 1999, 10, 1607-1617.	1.4	48
85	Gene therapy of inherited skin adhesion disorders: a critical overview. <i>British Journal of Dermatology</i> , 2009, 161, 19-24.	1.4	48
86	Insertion Sites in Engrafted Cells Cluster Within a Limited Repertoire of Genomic Areas After Gammaretroviral Vector Gene Therapy. <i>Molecular Therapy</i> , 2011, 19, 2031-2039.	3.7	48
87	Preclinical Corrective Gene Transfer in Xeroderma Pigmentosum Human Skin Stem Cells. <i>Molecular Therapy</i> , 2012, 20, 798-807.	3.7	44
88	Correction of the Exon 2 Duplication in DMD Myoblasts by a Single CRISPR/Cas9 System. <i>Molecular Therapy - Nucleic Acids</i> , 2017, 7, 11-19.	2.3	44
89	Myogenic stem cells from the bone marrow: a therapeutic alternative for muscular dystrophy?. <i>Neuromuscular Disorders</i> , 2002, 12, S7-S10.	0.3	43
90	Transfer of the ADA gene into human ADA-deficient T lymphocytes reconstitutes specific immune functions. <i>Blood</i> , 1992, 80, 1120-1124.	0.6	41

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91	Definition of the Transcriptional Activation Domains of Three Human HOX Proteins Depends on the DNA-Binding Context. <i>Molecular and Cellular Biology</i> , 1998, 18, 6201-6212.	1.1	41
92	Efficacy and biodistribution analysis of intracerebroventricular administration of an optimized scAAV9-SMN1 vector in a mouse model of spinal muscular atrophy. <i>Molecular Therapy - Methods and Clinical Development</i> , 2016, 3, 16060.	1.8	41
93	Correction of β^2 -thalassemia by CRISPR/Cas9 editing of the β -globin locus in human hematopoietic stem cells. <i>Blood Advances</i> , 2021, 5, 1137-1153.	2.5	41
94	Hemoglobin Legnano (β^{2141} (HC3) Arg α^1 Leu β^2): A New Abnormal Human Hemoglobin with High Oxygen Affinity. <i>Hemoglobin</i> , 1978, 2, 249-259.	0.4	40
95	A single epidermal stem cell strategy for safe <i>ex vivo</i> gene therapy. <i>EMBO Molecular Medicine</i> , 2015, 7, 380-393.	3.3	40
96	Correction of Murine SCID-X1 by Lentiviral Gene Therapy Using a Codon-optimized IL2RG Gene and Minimal Pretransplant Conditioning. <i>Molecular Therapy</i> , 2011, 19, 1867-1877.	3.7	39
97	High-Definition Mapping of Retroviral Integration Sites Defines the Fate of Allogeneic T Cells After Donor Lymphocyte Infusion. <i>PLoS ONE</i> , 2010, 5, e15688.	1.1	39
98	Preclinical Development of a Lentiviral Vector for Gene Therapy of X-Linked Severe Combined Immunodeficiency. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 9, 257-269.	1.8	38
99	RD2-MolPack-Chim3, a Packaging Cell Line for Stable Production of Lentiviral Vectors for Anti-HIV Gene Therapy. <i>Human Gene Therapy Methods</i> , 2013, 24, 228-240.	2.1	34
100	Genotoxic Signature in Cord Blood Cells of Newborns Exposed In Utero to a Zidovudine-Based Antiretroviral Combination. <i>Journal of Infectious Diseases</i> , 2013, 208, 235-243.	1.9	34
101	Gene Therapy for Hemoglobinopathies. <i>Human Gene Therapy</i> , 2018, 29, 1106-1113.	1.4	34
102	Gene Therapy for Sickle Cell Disease: A Lentiviral Vector Comparison Study. <i>Human Gene Therapy</i> , 2018, 29, 1153-1166.	1.4	33
103	The upstream region of the human homeobox gene HOX3D is a target for regulation by retinoic acid and HOX homeoproteins. <i>EMBO Journal</i> , 1992, 11, 265-77.	3.5	33
104	Genomic Analysis of Sleeping Beauty Transposon Integration in Human Somatic Cells. <i>PLoS ONE</i> , 2014, 9, e112712.	1.1	32
105	Heterocellular hereditary persistence of fetal hemoglobin (HPFH). Molecular mechanisms of abnormal γ -gene expression in association with β thalassemia and linkage relationship with the γ -globin gene cluster. <i>Human Genetics</i> , 1984, 66, 151-156.	1.8	31
106	Gene therapy in combination with tissue engineering to treat epidermolysis bullosa. <i>Expert Opinion on Biological Therapy</i> , 2006, 6, 367-378.	1.4	31
107	French gene therapy group reports on the adverse event in a clinical trial of gene therapy for X-linked severe combined immune deficiency (X-SCID). <i>Journal of Gene Medicine</i> , 2003, 5, 82-84.	1.4	30
108	Gene therapy approaches for epidermolysis bullosa. <i>Clinics in Dermatology</i> , 2005, 23, 430-436.	0.8	30

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109	Role of CD34 Antigen in Myeloid Differentiation of Human Hematopoietic Progenitor Cells. <i>Stem Cells</i> , 2008, 26, 950-959.	1.4	30
110	Translocation of c-myc into the immunoglobulin heavy-chain locus in human acute B-cell leukemia. A molecular analysis.. <i>EMBO Journal</i> , 1986, 5, 905-911.	3.5	29
111	Transduction of Human Hematopoietic Stem Cells by Lentiviral Vectors Pseudotyped with the RD114-TR Chimeric Envelope Glycoprotein. <i>Human Gene Therapy</i> , 2007, 18, 811-820.	1.4	27
112	Designing Lentiviral Vectors for Gene Therapy of Genetic Diseases. <i>Viruses</i> , 2021, 13, 1526.	1.5	27
113	Translocation and rearrangement of c-myc into immunoglobulin alpha heavy chain locus in primary cells from acute lymphocytic leukemia.. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 1984, 81, 5514-5518.	3.3	26
114	Stem cell plasticity: time for a reappraisal?. <i>Haematologica</i> , 2005, 90, 360-81.	1.7	25
115	Hemoglobin Gavello - Î± ₂ Î² ₂ (CD6) Asp â† Gly a new Hemoglobin Variant from Polesine (Italy). <i>Hemoglobin</i> , 1977, 1, 771-779.	0.4	24
116	A new human hemoglobin variant: Hb Bari (Î± ₂ 45 (CD3) His â† Gln Î² ₂). <i>Biochimica Et Biophysica Acta (BBA) - Protein Structure</i> , 1980, 622, 315-319.	1.7	24
117	The thyroid transcription factor-1 gene is a candidate target for regulation by Hox proteins. <i>EMBO Journal</i> , 1994, 13, 3339-47.	3.5	24
118	Selective expression of fos proto-oncogene in human acute myelomonocytic and monocytic leukemias: a molecular marker of terminal differentiation. <i>Blood</i> , 1987, 69, 160-164.	0.6	23
119	The GATA1-HS2 Enhancer Allows Persistent and Position-Independent Expression of a Î²-globin Transgene. <i>PLoS ONE</i> , 2011, 6, e27955.	1.1	23
120	Genome-Wide Analysis of Alpharetroviral Integration in Human Hematopoietic Stem/Progenitor Cells. <i>Genes</i> , 2014, 5, 415-429.	1.0	23
121	Haemoglobin Lepore Trait: Haematological and Structural Studies on the Italian Population. <i>British Journal of Haematology</i> , 1979, 42, 557-565.	1.2	21
122	Evaluation of tolerance to lentiviral LV-RPE65 gene therapy vector after subretinal delivery in non-human primates. <i>Translational Research</i> , 2017, 188, 40-57.e4.	2.2	21
123	Multiple Integrated Non-clinical Studies Predict the Safety of Lentivirus-Mediated Gene Therapy for Î²-Thalassemia. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 11, 9-28.	1.8	21
124	T Lymphocytes Transduced with a Lentiviral Vector Expressing F12-vif Are Protected from HIV-1 Infection in an APOBEC3G-Independent Manner. <i>Molecular Therapy</i> , 2005, 12, 697-706.	3.7	20
125	Self-inactivating MLV vectors have a reduced genotoxic profile in human epidermal keratinocytes. <i>Gene Therapy</i> , 2013, 20, 949-957.	2.3	20
126	An Optimized Lentiviral Vector Efficiently Corrects the Human Sickle Cell Disease Phenotype. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 10, 268-280.	1.8	20

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127	Hemoglobin bologna (β^2 61 (E5) Lys \rightarrow Met) An abnormal human hemoglobin with low oxygen affinity. <i>Biochimica Et Biophysica Acta (BBA) - Protein Structure</i> , 1981, 668, 209-215.	1.7	19
128	The delta beta crossover region in Lepore boston hemoglobinopathy is restricted to a 59 base pairs region around the 5' splice junction of the large globin gene intervening sequence. <i>Blood</i> , 1983, 62, 230-233.	0.6	18
129	Absence of an intrathecal immune reaction to a helper-dependent adenoviral vector delivered into the cerebrospinal fluid of non-human primates. <i>Gene Therapy</i> , 2008, 15, 233-238.	2.3	18
130	Transcriptional, epigenetic and retroviral signatures identify regulatory regions involved in hematopoietic lineage commitment. <i>Scientific Reports</i> , 2016, 6, 24724.	1.6	18
131	Biosafety Studies of a Clinically Applicable Lentiviral Vector for the Gene Therapy of Artemis-SCID. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 15, 232-245.	1.8	18
132	Estimated Comparative Integration Hotspots Identify Different Behaviors of Retroviral Gene Transfer Vectors. <i>PLoS Computational Biology</i> , 2011, 7, e1002292.	1.5	17
133	Muscle-directed gene therapy corrects Pompe disease and uncovers species-specific GAA immunogenicity. <i>EMBO Molecular Medicine</i> , 2022, 14, e13968.	3.3	17
134	Transcriptional targeting of retroviral vectors to the erythroblastic progeny of transduced hematopoietic stem cells. <i>Blood</i> , 1999, 93, 3276-85.	0.6	17
135	Posttranscriptional control of human homeobox gene expression in induced NTERA-2 embryonal carcinoma cells. <i>Molecular Reproduction and Development</i> , 1989, 1, 107-115.	1.0	16
136	Characterization of Two Neuroblastoma Cell Lines Expressing Recombinant Nerve Growth Factor Receptors. <i>Journal of Neurochemistry</i> , 1991, 56, 67-74.	2.1	16
137	Pre-clinical Development of a Lentiviral Vector Expressing the Anti-sickling β^2 AS3 Globin for Gene Therapy for Sickle Cell Disease. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 11, 167-179.	1.8	16
138	Deletion of the LTR Enhancer/Promoter Has No Impact on the Integration Profile of MLV Vectors in Human Hematopoietic Progenitors. <i>PLoS ONE</i> , 2013, 8, e55721.	1.1	16
139	A new abnormal human hemoglobin: Hb prato (β^2 31 (B12) Arg \rightarrow Ser β^2). <i>Biochimica Et Biophysica Acta (BBA) - Protein Structure</i> , 1979, 578, 534-540.	1.7	15
140	Translocation of c-myc into the immunoglobulin heavy-chain locus in human acute B-cell leukemia. A molecular analysis. <i>EMBO Journal</i> , 1986, 5, 905-11.	3.5	15
141	Perspectives on Best Practices for Gene Therapy Programs. <i>Human Gene Therapy</i> , 2015, 26, 127-133.	1.4	14
142	Competitive Engraftment of Hematopoietic Stem Cells Genetically Modified with a Truncated Erythropoietin Receptor. <i>Human Gene Therapy</i> , 2005, 16, 594-608.	1.4	13
143	Gene therapies need new development models. <i>Nature</i> , 2012, 490, 7-7.	13.7	13
144	A nonproducer, interfering human immunodeficiency virus (HIV) type 1 provirus can be transduced through a murine leukemia virus-based retroviral vector: recovery of an anti-HIV mouse/human pseudotype retrovirus. <i>Journal of Virology</i> , 1995, 69, 6618-6626.	1.5	13

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145	Translating the Genomics Revolution: The Need for an International Gene Therapy Consortium for Monogenic Diseases. <i>Molecular Therapy</i> , 2013, 21, 266-268.	3.7	12
146	Î² Thalassaemia Associated with Increased HB F Production. Evidence for the Existence of a Heterocellular Hereditary Persistence of Fetal Hemoglobin (Hpfh) Determinant Linked to Î² Thalassaemia in a Southern Italian Population. <i>Hemoglobin</i> , 1981, 5, 1-17.	0.4	11
147	Towards a Gene Therapy Clinical Trial for Epidermolysis Bullosa. <i>Reviews on Recent Clinical Trials</i> , 2006, 1, 155-162.	0.4	11
148	Developing gene and cell therapies for rare diseases: an opportunity for synergy between academia and industry. <i>Gene Therapy</i> , 2017, 24, 590-592.	2.3	11
149	Anti-HIV Viral Interference Induced by Retroviral Vectors Expressing a Nonproducer HIV-1 Variant. <i>Acta Haematologica</i> , 1996, 95, 199-203.	0.7	10
150	Hb O Indonesia (Î±2116(Gh4) Glu â†’ Lys Î²2): in Association with Î²-Thalassaemia. <i>Hemoglobin</i> , 1978, 2, 59-63.	0.4	9
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