Adrian J Thrasher

List of Publications by Year
in descending order

466 papers	37,080 citations	2101 100 h-index	4432 172 g-index
478 all docs	478 docs citations	478 times ranked	29952 citing authors

#	Article	IF	Citations
1	Effect of Gene Therapy on Visual Function in Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2231-2239.	27.0	1,793
2	Correction of X-linked chronic granulomatous disease by gene therapy, augmented by insertional activation of MDS1-EVI1, PRDM16 or SETBP1. Nature Medicine, 2006, 12, 401-409.	30.7	1,129
3	Sustained Correction of X-Linked Severe Combined Immunodeficiency by ex Vivo Gene Therapy. New England Journal of Medicine, 2002, 346, 1185-1193.	27.0	1,075
4	Insertional mutagenesis combined with acquired somatic mutations causes leukemogenesis following gene therapy of SCID-X1 patients. Journal of Clinical Investigation, 2008, 118, 3143-3150.	8.2	1,069
5	Genomic instability and myelodysplasia with monosomy 7 consequent to EVI1 activation after gene therapy for chronic granulomatous disease. Nature Medicine, 2010, 16, 198-204.	30.7	727
6	Molecular remission of infant B-ALL after infusion of universal TALEN gene-edited CAR T cells. Science Translational Medicine, 2017, 9, .	12.4	707
7	Gene therapy of X-linked severe combined immunodeficiency by use of a pseudotyped gammaretroviral vector. Lancet, The, 2004, 364, 2181-2187.	13.7	636
8	A robust model for read count data in exome sequencing experiments and implications for copy number variant calling. Bioinformatics, 2012, 28, 2747-2754.	4.1	534
9	The cytoplasm of living cells behaves as a poroelastic material. Nature Materials, 2013, 12, 253-261.	27.5	527
10	High-Level Transduction and Gene Expression in Hematopoietic Repopulating Cells Using a Human Imunodeficiency Virus Type 1-Based Lentiviral Vector Containing an Internal Spleen Focus Forming Virus Promoter. Human Gene Therapy, 2002, 13, 803-813.	2.7	457
11	Effective gene therapy with nonintegrating lentiviral vectors. Nature Medicine, 2006, 12, 348-353.	30.7	416
12	Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. New England Journal of Medicine, 2017, 377, 1630-1638.	27.0	412
13	The European Society for Immunodeficiencies (ESID) Registry Working Definitions for the ClinicalÂDiagnosis of Inborn Errors of Immunity. Journal of Allergy and Clinical Immunology: in Practice, 2019, 7, 1763-1770.	3.8	381
14	Clinical Features and Outcome of Patients With IRAK-4 and MyD88 Deficiency. Medicine (United States), 2010, 89, 403-425.	1.0	366
15	A Modified Î³-Retrovirus Vector for X-Linked Severe Combined Immunodeficiency. New England Journal of Medicine, 2014, 371, 1407-1417.	27.0	358
16	WASP: a key immunological multitasker. Nature Reviews Immunology, 2010, 10, 182-192.	22.7	354
17	The Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2006, 117, 725-738.	2.9	350
18	Whole-genome sequencing of patients with rare diseases in a national health system. Nature, 2020, 583, 96-102.	27.8	338

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19	Insertional Transformation of Hematopoietic Cells by Self-inactivating Lentiviral and Gammaretroviral Vectors. Molecular Therapy, 2009, 17, 1919-1928.	8.2	337
20	Outcomes Following Gene Therapy in Patients With Severe Wiskott-Aldrich Syndrome. JAMA - Journal of the American Medical Association, 2015, 313, 1550.	7.4	327
21	Configuration of human dendritic cell cytoskeleton by Rho GTPases, the WAS protein, and differentiation. Blood, 2001, 98, 1142-1149.	1.4	300
22	Long-term outcome and lineage-specific chimerism in 194 patients with Wiskott-Aldrich syndrome treated by hematopoietic cell transplantation in the period 1980-2009: an international collaborative study. Blood, 2011, 118, 1675-1684.	1.4	296
23	Restoration of photoreceptor ultrastructure and function in retinal degeneration slow mice by gene therapy. Nature Genetics, 2000, 25, 306-310.	21.4	295
24	Essential Role of the NADPH Oxidase Subunit p47^{phox}in Endothelial Cell Superoxide Production in Response to Phorbol Ester and Tumor Necrosis Factor-Î±. Circulation Research, 2002, 90, 143-150.	4.5	295
25	The future of gene therapy. Nature, 2004, 427, 779-781.	27.8	262
26	Hematopoietic Stem Cell Gene Therapy for Adenosine Deaminaseâ€“Deficient Severe Combined Immunodeficiency Leads to Long-Term Immunological Recovery and Metabolic Correction. Science Translational Medicine, 2011, 3, 97ra80.	12.4	257
27	Relevance of biallelic versus monoallelic TNFRSF13B mutations in distinguishing disease-causing from risk-increasing TNFRSF13B variants in antibody deficiency syndromes. Blood, 2009, 113, 1967-1976.	1.4	254
28	Hot spots of retroviral integration in human CD34+ hematopoietic cells. Blood, 2007, 110, 1770-1778.	1.4	248
29	Chemotaxis of macrophages is abolished in the Wiskottâ€Aldrich syndrome. British Journal of Haematology, 1998, 101, 659-665.	2.5	225
30	Oncogenesis Following Delivery of a Nonprimate Lentiviral Gene Therapy Vector to Fetal and Neonatal Mice. Molecular Therapy, 2005, 12, 763-771.	8.2	224
31	Vector integration is nonrandom and clustered and influences the fate of lymphopoiesis in SCID-X1 gene therapy. Journal of Clinical Investigation, 2007, 117, 2225-2232.	8.2	221
32	Cellular Control of Cortical Actin Nucleation. Current Biology, 2014, 24, 1628-1635.	3.9	219
33	Long-term outcome following hematopoietic stem-cell transplantation in Wiskott-Aldrich syndrome: collaborative study of the European Society for Immunodeficiencies and European Group for Blood and Marrow Transplantation. Blood, 2008, 111, 439-445.	1.4	216
34	Gene transfer into the mouse retina mediated by an adeno-associated viral vector. Human Molecular Genetics, 1996, 5, 591-594.	2.9	209
35	Long-Term Persistence of a Polyclonal T Cell Repertoire After Gene Therapy for X-Linked Severe Combined Immunodeficiency. Science Translational Medicine, 2011, 3, 97ra79.	12.4	208
36	Efficient and Selective AAV2-Mediated Gene Transfer Directed to Human Vascular Endothelial Cells. Molecular Therapy, 2001, 4, 174-181.	8.2	204

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37	Two novel activating mutations in the Wiskott-Aldrich syndrome protein result in congenital neutropenia. Blood, 2006, 108, 2182-2189.	1.4	200
38	Successful Reconstitution of Immunity in ADA-SCID by Stem Cell Gene Therapy Following Cessation of PEG-ADA and Use of Mild Preconditioning. Molecular Therapy, 2006, 14, 505-513.	8.2	200
39	Enhanced human cell engraftment in mice deficient in RAG2 and the common cytokine receptor Î³ chain. British Journal of Haematology, 1998, 103, 335-342.	2.5	199
40	Wasp in immune-system organization and function. Nature Reviews Immunology, 2002, 2, 635-646.	22.7	192
41	Reconstitution of neutrophil NADPH oxidase activity in the cell-free system by four components: p67-phox, p47-phox, p21rac1, and cytochrome b-245. Journal of Biological Chemistry, 1992, 267, 16767-70.	3.4	191
42	In vivo gene transfer to the mouse eye using an HIV-based lentiviral vector; efficient long-term transduction of corneal endothelium and retinal pigment epithelium. Gene Therapy, 2001, 8, 1665-1668.	4.5	186
43	Loss-of-function nuclear factor Î²B subunit 1 (NFKB1) variants are the most common monogenic cause of common variable immunodeficiency in Europeans. Journal of Allergy and Clinical Immunology, 2018, 142, 1285-1296.	2.9	185
44	Gammaretrovirus-mediated correction of SCID-X1 is associated with skewed vector integration site distribution in vivo. Journal of Clinical Investigation, 2007, 117, 2241-2249.	8.2	185
45	Lancet Commission: Stem cells and regenerative medicine. Lancet, The, 2018, 391, 883-910.	13.7	184
46	Lipid-Mediated Enhancement of Transfection by a Nonviral Integrin-Targeting Vector. Human Gene Therapy, 1998, 9, 575-585.	2.7	183
47	Deficiency in the Wiskott-Aldrich protein induces premature proplatelet formation and platelet production in the bone marrow compartment. Blood, 2006, 108, 134-140.	1.4	183
48	Wiskott-Aldrich syndrome protein is necessary for efficient IgG-mediated phagocytosis. Blood, 2000, 95, 2943-2946.	1.4	180
49	X-linked thrombocytopenia (XLT) due to WAS mutations: clinical characteristics, long-term outcome, and treatment options. Blood, 2010, 115, 3231-3238.	1.4	178
50	Neonatal dendritic cells are intrinsically biased against Th-1 immune responses. Clinical and Experimental Immunology, 2002, 128, 118-123.	2.6	177
51	Elimination of human leukemia cells in NOD/SCID mice by WT1-TCR geneâ€“transduced human T cells. Blood, 2005, 106, 3062-3067.	1.4	176
52	Lentiviral gene therapy for X-linked chronic granulomatous disease. Nature Medicine, 2020, 26, 200-206.	30.7	175
53	Automated manufacturing of chimeric antigen receptor T cells for adoptive immunotherapy using CliniMACS Prodigy. Cytotherapy, 2016, 18, 1002-1011.	0.7	174
54	Comprehensive genomic access to vector integration in clinical gene therapy. Nature Medicine, 2009, 15, 1431-1436.	30.7	173

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55	Stable Gene Transfer to Muscle Using Non-integrating Lentiviral Vectors. Molecular Therapy, 2007, 15, 1947-1954.	8.2	165
56	Update on the hyper immunoglobulin M syndromes. British Journal of Haematology, 2010, 149, 167-180.	2.5	164
57	Codon optimization of human factor VIII cDNAs leads to high-level expression. Blood, 2011, 117, 798-807.	1.4	163
58	Immunotherapy of HCC metastases with autologous T cell receptor redirected T cells, targeting HBsAg in a liver transplant patient. Journal of Hepatology, 2015, 62, 486-491.	3.7	160
59	Unregulated actin polymerization by WASp causes defects of mitosis and cytokinesis in X-linked neutropenia. Journal of Experimental Medicine, 2007, 204, 2213-2224.	8.5	158
60	Autoimmune lymphoproliferative syndrome: molecular basis of disease and clinical phenotype. British Journal of Haematology, 2006, 133, 124-140.	2.5	157
61	Lentiviral vectors containing an enhancer-less ubiquitously acting chromatin opening element (UCOE) provide highly reproducible and stable transgene expression in hematopoietic cells. Blood, 2007, 110, 1448-1457.	1.4	157
62	Loss of the interleukin-6 receptor causes immunodeficiency, atopy, and abnormal inflammatory responses. Journal of Experimental Medicine, 2019, 216, 1986-1998.	8.5	153
63	Gene therapy using haematopoietic stem and progenitor cells. Nature Reviews Genetics, 2021, 22, 216-234.	16.3	151
64	Self-inactivating Gammaretroviral Vectors for Gene Therapy of X-linked Severe Combined Immunodeficiency. Molecular Therapy, 2008, 16, 590-598.	8.2	150
65	Inhibition of retinal neovascularisation by gene transfer of soluble VEGF receptor sFlt-1. Gene Therapy, 2002, 9, 320-326.	4.5	149
66	Whole-genome sequencing of a sporadic primary immunodeficiency cohort. Nature, 2020, 583, 90-95.	27.8	148
67	Gene Therapy of Chronic Granulomatous Disease: The Engraftment Dilemma. Molecular Therapy, 2011, 19, 28-35.	8.2	147
68	Valproic Acid Confers Functional Pluripotency to Human Amniotic Fluid Stem Cells in a Transgene-free Approach. Molecular Therapy, 2012, 20, 1953-1967.	8.2	145
69	X-SCID transgene leukaemogenicity. Nature, 2006, 443, E5-E6.	27.8	144
70	Biochemical Correction of X-CGD by a Novel Chimeric Promoter Regulating High Levels of Transgene Expression in Myeloid Cells. Molecular Therapy, 2011, 19, 122-132.	8.2	141
71	Diminished production of anti-inflammatory mediators during neutrophil apoptosis and macrophage phagocytosis in chronic granulomatous disease (CGD). Journal of Leukocyte Biology, 2003, 73, 591-599.	3.3	137
72	Maturation of DC is associated with changes in motile characteristics and adherence. Cytoskeleton, 2004, 57, 118-132.	4.4	137

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73	Gene therapy for PIDs: Progress, pitfalls and prospects. Gene, 2013, 525, 174-181.	2.2	137
74	Human Î³Î´ T Cells: A Lymphoid Lineage Cell Capable of Professional Phagocytosis. Journal of Immunology, 2009, 183, 5622-5629.	0.8	136
75	The leukocyte podosome. European Journal of Cell Biology, 2006, 85, 151-157.	3.6	135
76	Mechanisms of WASp-mediated hematologic and immunologic disease. Blood, 2004, 104, 3454-3462.	1.4	134
77	Permanent phenotypic correction of hemophilia B in immunocompetent mice by prenatal gene therapy. Blood, 2004, 104, 2714-2721.	1.4	132
78	Wiskott-Aldrich syndrome protein deficiency leads to reduced B-cell adhesion, migration, and homing, and a delayed humoral immune response. Blood, 2005, 105, 1144-1152.	1.4	130
79	AAV-Mediated gene transfer slows photoreceptor loss in the RCS rat model of retinitis pigmentosa. Molecular Therapy, 2003, 8, 188-195.	8.2	128
80	Failure of SCID-X1 gene therapy in older patients. Blood, 2005, 105, 4255-4257.	1.4	128
81	Intraocular gene delivery of ciliary neurotrophic factor results in significant loss of retinal function in normal mice and in the Prph2Rd2/Rd2 model of retinal degeneration. Gene Therapy, 2003, 10, 523-527.	4.5	127
82	Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. New England Journal of Medicine, 2021, 384, 2002-2013.	27.0	122
83	Long-term preservation of retinal function in the RCS rat model of retinitis pigmentosa following lentivirus-mediated gene therapy. Gene Therapy, 2005, 12, 694-701.	4.5	119
84	Adeno-Associated Virus Gene Transfer to Mouse Retina. Human Gene Therapy, 1998, 9, 81-86.	2.7	118
85	Cutting Edge: The Wiskott-Aldrich Syndrome Protein Is Required for Efficient Phagocytosis of Apoptotic Cells. Journal of Immunology, 2001, 166, 4831-4834.	0.8	118
86	Gene replacement therapy in the retinal degeneration slow (rds)mouse: the effect on retinal degeneration following partial transduction of the retina. Human Molecular Genetics, 2001, 10, 2353-2361.	2.9	117
87	Autoinflammatory periodic fever, immunodeficiency, and thrombocytopenia (PFIT) caused by mutation in actin-regulatory gene WDR1 . Journal of Experimental Medicine, 2017, 214, 59-71.	8.5	117
88	A Ubiquitous Chromatin Opening Element (UCOE) Confers Resistance to DNA Methylationâ€“mediated Silencing of Lentiviral Vectors. Molecular Therapy, 2010, 18, 1640-1649.	8.2	116
89	Inhibition of calpain stabilises podosomes and impairs dendritic cell motility. Journal of Cell Science, 2006, 119, 2375-2385.	2.0	115
90	WIP Regulates the Stability and Localization of WASP to Podosomes in Migrating Dendritic Cells. Current Biology, 2006, 16, 2337-2344.	3.9	114

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91	Polarized expression of bone morphogenetic protein-4 in the human aorta-gonad-mesonephros region. Blood, 2000, 96, 1591-1593.	1.4	113
92	Chronic granulomatous disease. Clinical and Experimental Immunology, 2000, 122, 1-9.	2.6	111
93	WASp deficiency in mice results in failure to form osteoclast sealing zones and defects in bone resorption. Blood, 2004, 103, 3552-3561.	1.4	111
94	Phenotypic Characterization of EIF2AK4 Mutation Carriers in a Large Cohort of Patients Diagnosed Clinically With Pulmonary Arterial Hypertension. Circulation, 2017, 136, 2022-2033.	1.6	111
95	Impaired dendritic-cell homing in vivo in the absence of Wiskott-Aldrich syndrome protein. Blood, 2005, 105, 1590-1597.	1.4	110
96	Intrinsic dendritic cell abnormalities in Wiskott-Aldrich syndrome. European Journal of Immunology, 1998, 28, 3259-3267.	2.9	109
97	Gene therapy: progress and predictions. Proceedings of the Royal Society B: Biological Sciences, 2015, 282, 20143003.	2.6	108
98	Thymus transplantation for complete DiGeorge syndrome: European experience. Journal of Allergy and Clinical Immunology, 2017, 140, 1660-1670.e16.	2.9	108
99	Progress and Prospects: Gene Therapy Clinical Trials (Part 1). Gene Therapy, 2007, 14, 1439-1447.	4.5	106
100	Actin cytoskeletal defects in immunodeficiency. Immunological Reviews, 2013, 256, 282-299.	6.0	106
101	SAP mediates specific cytotoxic T-cell functions in X-linked lymphoproliferative disease. Blood, 2004, 103, 3821-3827.	1.4	104
102	The Tight Junction Associated Signalling Proteins ZO-1 and ZONAB Regulate Retinal Pigment Epithelium Homeostasis in Mice. PLoS ONE, 2010, 5, e15730.	2.5	104
103	Lentiviral vectors targeting WASp expression to hematopoietic cells, efficiently transduce and correct cells from WAS patients. Gene Therapy, 2007, 14, 415-428.	4.5	102
104	Immune responses limit adenovirally mediated gene expression in the adult mouse eye. Gene Therapy, 1998, 5, 1038-1046.	4.5	101
105	B cellâ€“intrinsic deficiency of the Wiskott-Aldrich syndrome protein (WASp) causes severe abnormalities of the peripheral B-cell compartment in mice. Blood, 2012, 119, 2819-2828.	1.4	99
106	Restoration of podosomes and chemotaxis in Wiskottâ€“Aldrich syndrome macrophages following induced expression of WASp. International Journal of Biochemistry and Cell Biology, 2002, 34, 806-815.	2.8	97
107	Treating Immunodeficiency through HSC Gene Therapy. Trends in Molecular Medicine, 2016, 22, 317-327.	6.7	96
108	Cognitive and behavioral abnormalities in children after hematopoietic stem cell transplantation for severe congenital immunodeficiencies. Blood, 2008, 112, 3907-3913.	1.4	94

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109	Betibeglogene Autotemcel Gene Therapy for Nonâ€“Î² ⁰ /Î² ⁰ Genotype Î²-Thalassemia. New England Journal of Medicine, 2022, 386, 415-427.	27.0	91
110	Wiskottâ€“Aldrich Syndrome: Immunodeficiency resulting from defective cell migration and impaired immunostimulatory activation. Immunobiology, 2009, 214, 778-790.	1.9	90
111	LRBA gene deletion in a patient presenting with autoimmunity without hypogammaglobulinemia. Journal of Allergy and Clinical Immunology, 2012, 130, 1428-1432.	2.9	90
112	Wiskott-Aldrich syndrome protein deficiency in B cells results in impaired peripheral homeostasis. Blood, 2008, 112, 4158-4169.	1.4	89
113	Efficient gene delivery to the adult and fetal CNS using pseudotyped non-integrating lentiviral vectors. Gene Therapy, 2009, 16, 509-520.	4.5	89
114	The Wiskott-Aldrich Syndrome: The Actin Cytoskeleton and Immune Cell Function. Disease Markers, 2010, 29, 157-175.	1.3	87
115	Megakaryocytes assemble podosomes that degrade matrix and protrude through basement membrane. Blood, 2013, 121, 2542-2552.	1.4	87
116	Targeted gene correction of human hematopoietic stem cells for the treatment of Wiskott -ÂAldrich Syndrome. Nature Communications, 2020, 11, 4034.	12.8	87
117	In vivo myocardial gene transfer: Optimization, evaluation and direct comparison of gene transfer vectors. Basic Research in Cardiology, 2001, 96, 227-236.	5.9	86
118	Long-term reversal of chronic anemia using a hypoxia-regulated erythropoietin gene therapy. Blood, 2002, 100, 2406-2413.	1.4	86
119	Wiskott-Aldrich syndrome protein and the cytoskeletal dynamics of dendritic cells. Journal of Pathology, 2004, 204, 460-469.	4.5	86
120	Impaired T-cell priming in vivo resulting from dysfunction of WASp-deficient dendritic cells. Blood, 2007, 110, 4278-4284.	1.4	86
121	Use of Nonintegrating Lentiviral Vectors for Gene Therapy. Human Gene Therapy, 2007, 18, 483-489.	2.7	86
122	Platelet actin nodules are podosome-like structures dependent on Wiskottâ€“Aldrich syndrome protein and ARP2/3 complex. Nature Communications, 2015, 6, 7254.	12.8	86
123	Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. Journal of Allergy and Clinical Immunology: in Practice, 2018, 6, 1418-1420.e10.	3.8	85
124	Chorioretinal lesions in patients and carriers of chronic granulomatous disease. Journal of Pediatrics, 1999, 134, 780-783.	1.8	84
125	Repeated courses of rituximab for autoimmune cytopenias may precipitate profound hypogammaglobulinaemia requiring replacement intravenous immunoglobulin. British Journal of Haematology, 2009, 146, 120-122.	2.5	83
126	Gene therapy for Wiskott-Aldrich syndrome in a severely affected adult. Blood, 2017, 130, 1327-1335.	1.4	83

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127	Nonintegrating Lentivector Vaccines Stimulate Prolonged T-Cell and Antibody Responses and Are Effective in Tumor Therapy. Journal of Virology, 2009, 83, 3094-3103.	3.4	82
128	Brief Report: Self-Organizing Neuroepithelium from Human Pluripotent Stem Cells Facilitates Derivation of Photoreceptors. Stem Cells, 2013, 31, 408-414.	3.2	82
129	Normal development of human fetal hematopoiesis between eight and seventeen weeksâ€™ gestation. American Journal of Obstetrics and Gynecology, 2000, 183, 1029-1034.	1.3	79
130	Correction of murine Rag1 deficiency by self-inactivating lentiviral vector-mediated gene transfer. Leukemia, 2011, 25, 1471-1483.	7.2	78
131	Characterization of the clinical and immunologic phenotype and management of 157 individuals with 56 distinct heterozygous NFKB1 mutations. Journal of Allergy and Clinical Immunology, 2020, 146, 901-911.	2.9	78
132	Preclinical Demonstration of Lentiviral Vector-mediated Correction of Immunological and Metabolic Abnormalities in Models of Adenosine Deaminase Deficiency. Molecular Therapy, 2014, 22, 607-622.	8.2	77
133	Translational Mini-Review Series on Immunodeficiency:â€¨Molecular defects in common variable immunodeficiency. Clinical and Experimental Immunology, 2007, 149, 401-409.	2.6	75
134	First Clinical Application of Talen Engineered Universal CAR19 T Cells in B-ALL. Blood, 2015, 126, 2046-2046.	1.4	75
135	Polyphenol E Enhances the Antitumor Immune Response in Neuroblastoma by Inactivating Myeloid Suppressor Cells. Clinical Cancer Research, 2013, 19, 1116-1125.	7.0	74
136	Construction of stable packaging cell lines for clinical lentiviral vector production. Scientific Reports, 2015, 5, 9021.	3.3	74
137	Hyperinflammation in patients with chronic granulomatous disease leads to impairment of hematopoietic stem cell functions. Journal of Allergy and Clinical Immunology, 2016, 138, 219-228.e9.	2.9	74
138	Cytoskeletal remodeling mediated by WASp in dendritic cells is necessary for normal immune synapse formation and T-cell priming. Blood, 2011, 118, 2492-2501.	1.4	73
139	Wiskottâ€Aldrich syndrome protein: Emerging mechanisms in immunity. European Journal of Immunology, 2017, 47, 1857-1866.	2.9	72
140	Ectopic retroviral expression of LMO2, but not IL2RÎ³, blocks human T-cell development from CD34+ cells: implications for leukemogenesis in gene therapy. Leukemia, 2007, 21, 754-763.	7.2	71
141	Sleeping Beauty Transposition From Nonintegrating Lentivirus. Molecular Therapy, 2009, 17, 1197-1204.	8.2	71
142	Accumulation of the inhibitory receptor EphA4 may prevent regeneration of corticospinal tract axons following lesion. European Journal of Neuroscience, 2006, 23, 1721-1730.	2.6	70
143	The embryonic origins of human haematopoiesis. British Journal of Haematology, 2001, 112, 838-850.	2.5	69
144	High Efficiency Gene Transfer to Human Hematopoietic SCID-Repopulating Cells Under Serum-Free Conditions. Blood, 1998, 92, 3163-3171.	1.4	68

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145	Validation of a mutated PRE sequence allowing high and sustained transgene expression while abrogating WHV-X protein synthesis: application to the gene therapy of WAS. Gene Therapy, 2009, 16, 605-619.	4.5	68
146	Myogenic cell proliferation and generation of a reversible tumorigenic phenotype are triggered by preirradiation of the recipient site. Journal of Cell Biology, 2002, 157, 693-702.	5.2	67
147	Activating WASP mutations associated with X-linked neutropenia result in enhanced actin polymerization, altered cytoskeletal responses, and genomic instability in lymphocytes. Journal of Experimental Medicine, 2010, 207, 1145-1152.	8.5	67
148	Functional Gap Junctions Accumulate at the Immunological Synapse and Contribute to T Cell Activation. Journal of Immunology, 2011, 187, 3121-3132.	0.8	67
149	Altered BCR and TLR signals promote enhanced positive selection of autoreactive transitional B cells in Wiskott-Aldrich syndrome. Journal of Experimental Medicine, 2015, 212, 1663-1677.	8.5	67
150	Diagnosis of X-linked lymphoproliferative disease by analysis of SLAM-associated protein expression. European Journal of Immunology, 2000, 30, 1691-1697.	2.9	66
151	Immunodeficiency and severe susceptibility to bacterial infection associated with a loss-of-function homozygous mutation of MKL1. Blood, 2015, 126, 1527-1535.	1.4	66
152	Evolving Gene Therapy in Primary Immunodeficiency. Molecular Therapy, 2017, 25, 1132-1141.	8.2	66
153	Ex-vivo Gene Therapy Restores LEKTI Activity and Corrects the Architecture of Netherton Syndrome-derived Skin Grafts. Molecular Therapy, 2011, 19, 408-416.	8.2	65
154	Primary Immune Deficiency Treatment Consortium (PIDTC) report. Journal of Allergy and Clinical Immunology, 2014, 133, 335-347.e11.	2.9	65
155	High-Throughput In Vitro, Ex Vivo, and In Vivo Screen of Adeno-Associated Virus Vectors Based on Physical and Functional Transduction. Human Gene Therapy, 2020, 31, 575-589.	2.7	65
156	Wiskott-Aldrich syndrome protein is necessary for efficient IgG-mediated phagocytosis. Blood, 2000, 95, 2943-6.	1.4	65
157	Long-term safety and efficacy of lentiviral hematopoietic stem/progenitor cell gene therapy for Wiskottâ€“Aldrich syndrome. Nature Medicine, 2022, 28, 71-80.	30.7	64
158	Concise Review: MicroRNAs as Modulators of Stem Cells and Angiogenesis. Stem Cells, 2014, 32, 1059-1066.	3.2	63
159	Progress and prospects: gene therapy for inherited immunodeficiencies. Gene Therapy, 2009, 16, 1285-1291.	4.5	62
160	Human Mid-Trimester Amniotic Fluid Stem Cells Cultured Under Embryonic Stem Cell Conditions with Valproic Acid Acquire Pluripotent Characteristics. Stem Cells and Development, 2013, 22, 444-458.	2.1	62
161	Lack of T-cell responses following autologous tumour lysate pulsed dendritic cell vaccination, in patients with relapsed osteosarcoma. Clinical and Translational Oncology, 2012, 14, 271-279.	2.4	60
162	X-linked lymphoproliferative disease: clinical, diagnostic and molecular perspective. British Journal of Haematology, 2002, 119, 585-595.	2.5	59

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163	New insights into the biology of Wiskott-Aldrich syndrome (WAS). Hematology American Society of Hematology Education Program, 2009, 2009, 132-138.	2.5	59
164	Current and emerging treatment options for Wiskottâ€“Aldrich syndrome. Expert Review of Clinical Immunology, 2015, 11, 1015-1032.	3.0	59
165	EROS/CYBC1 mutations: Decreased NADPH oxidase function and chronic granulomatous disease. Journal of Allergy and Clinical Immunology, 2019, 143, 782-785.e1.	2.9	59
166	Clonal expansion of T memory stem cells determines early anti-leukemic responses and long-term CAR T cell persistence in patients. Nature Cancer, 2021, 2, 629-642.	13.2	59
167	Correction of Murine Rag2 Severe Combined Immunodeficiency by Lentiviral Gene Therapy Using a Codon-optimized RAG2 Therapeutic Transgene. Molecular Therapy, 2012, 20, 1968-1980.	8.2	57
168	Gene therapy matures in the clinic. Nature Biotechnology, 2012, 30, 588-593.	17.5	57
169	Stable rAAV-mediated transduction of rod and cone photoreceptors in the canine retina. Gene Therapy, 2003, 10, 1336-1344.	4.5	56
170	Gene Therapy for Primary Immunodeficiencies. Human Gene Therapy, 2012, 23, 668-675.	2.7	56
171	Recombination-activating gene 1 (Rag1)â€“deficient mice with severe combined immunodeficiency treated with lentiviral gene therapy demonstrate autoimmune Omenn-like syndrome. Journal of Allergy and Clinical Immunology, 2014, 133, 1116-1123.	2.9	56
172	Safety and early efficacy outcomes for lentiviral fibroblast gene therapy in recessive dystrophic epidermolysis bullosa. JCI Insight, 2019, 4, .	5.0	56
173	Impaired bone marrow homing of cytokine-activated CD34+ cells in the NOD/SCID model. Blood, 2004, 103, 2079-2087.	1.4	55
174	Lentiviral vectors transcriptionally targeted to hematopoietic cells by WASP gene proximal promoter sequences. Gene Therapy, 2005, 12, 715-723.	4.5	55
175	Retrovirus and Lentivirus Vector Design and Methods of Cell Conditioning. Methods in Enzymology, 2012, 507, 29-57.	1.0	55
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