Alessandro Aiuti

List of Publications by Year
in descending order

227	22,051	12330	9861
papers	citations	69 h-index	141 g-index

233	233	233	22576
all docs	docs citations	times ranked	citing authors

#	Article	IF	Citations
1	Vaccine breakthrough hypoxemic COVID-19 pneumonia in patients with auto-Abs neutralizing type I IFNs. Science Immunology, 2023, 8, .	11.9	35
2	A Case of Two Adult Brothers with Wiskott-Aldrich Syndrome, One Treated with Gene Therapy and One with HLA-Identical Hematopoietic Stem Cell Transplantation. Journal of Clinical Immunology, 2022, 42, 421-425.	3.8	7
3	Human genetic and immunological determinants of critical COVID-19 pneumonia. Nature, 2022, 603, 587-598.	27.8	216
4	Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT Inborn ErrorsÂWorking Party analysis. Blood, 2022, 139, 2066-2079.	1.4	33
5	Lentiviral haematopoietic stem-cell gene therapy for early-onset metachromatic leukodystrophy: long-term results from a non-randomised, open-label, phase 1/2 trial and expanded access. Lancet, The, 2022, 399, 372-383.	13.7	109
6	Third cranial nerve palsy in an 88-year-old man after SARS-CoV-2 mRNA vaccination: change of injection site and type of vaccine resulted in an uneventful second dose with humoral immune response. BMJ Case Reports, 2022, 15, e246485.	0.5	10
7	The EHA Research Roadmap: Hematopoietic Stem Cell Gene Therapy. HemaSphere, 2022, 6, e671.	2.7	8
8	<scp>Wiskottâ€“Aldrich</scp> syndrome: Oral findings and microbiota in children and review of the literature. Clinical and Experimental Dental Research, 2022, 8, 28-36.	1.9	4
9	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. Nature Communications, 2022, 13, 1315.	12.8	61
10	Follicular helper T cell signature of replicative exhaustion, apoptosis, and senescence in common variable immunodeficiency. European Journal of Immunology, 2022, 52, 1171-1189.	2.9	9
11	The risk of COVID-19 death is much greater and age dependent with type I IFN autoantibodies. Proceedings of the National Academy of Sciences of the United States of America, 2022, 119, e2200413119.	7.1	110
12	Respiratory viral infections in otherwise healthy humans with inherited IRF7 deficiency. Journal of Experimental Medicine, 2022, 219, .	8.5	21
13	Recessive inborn errors of type I IFN immunity in children with COVID-19 pneumonia. Journal of Experimental Medicine, 2022, 219, .	8.5	59
14	ExÂVivo and InÂVivo Gene Therapy for Mucopolysaccharidoses: State of the Art. Hematology/Oncology Clinics of North America, 2022, 36, 865-878.	2.2	5
15	Hematopoietic Tumors in a Mouse Model of X-linked Chronic Granulomatous Disease after Lentiviral Vector-Mediated Gene Therapy. Molecular Therapy, 2021, 29, 86-102.	8.2	17
16	Coronavirus disease 2019 in patients with inborn errors of immunity: An international study. Journal of Allergy and Clinical Immunology, 2021, 147, 520-531.	2.9	278
17	Gene therapy using haematopoietic stem and progenitor cells. Nature Reviews Genetics, 2021, 22, 216-234.	16.3	151
18	Toward Reference Intervals of ARSA Activity in the Cerebrospinal Fluid: Implication for the Clinical Practice of Metachromatic Leukodystrophy. journal of applied laboratory medicine, The, 2021, 6, 354-366.	1.3	6

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19	Emapalumab treatment in an ADA-SCID patient with refractory hemophagocytic lymphohistiocytosis-related graft failure and disseminated bacillus Calmette-GuÃ©rin infection. Haematologica, 2021, 106, 641-646.	3.5	17
20	Immunosuppressive therapy in childhoodâ€onset arrhythmogenic inflammatory cardiomyopathy. PACE - Pacing and Clinical Electrophysiology, 2021, 44, 552-556.	1.2	11
21	Update on Clinical ExÂVivo Hematopoietic Stem Cell Gene Therapy for Inherited Monogenic Diseases. Molecular Therapy, 2021, 29, 489-504.	8.2	46
22	Clinical outcome, incidence, and SARS-CoV-2 infection-fatality rates in Italian patients with inborn errors of immunity. Journal of Allergy and Clinical Immunology: in Practice, 2021, 9, 2904-2906.e2.	3.8	56
23	SARS-CoV-2â€"related MIS-C: A key to the viral and genetic causes of Kawasaki disease?. Journal of Experimental Medicine, 2021, 218, .	8.5	100
24	Metachromatic leukodystrophy: A singleâ€center longitudinal study of 45 patients. Journal of Inherited Metabolic Disease, 2021, 44, 1151-1164.	3.6	27
25	Retrieval of vector integration sites from cell-free DNA. Nature Medicine, 2021, 27, 1458-1470.	30.7	26
26	Harnessing Type I IFN Immunity Against SARS-CoV-2 with Early Administration of IFN-Î². Journal of Clinical Immunology, 2021, 41, 1425-1442.	3.8	39
27	Oncogene-induced senescence in hematopoietic progenitors features myeloid restricted hematopoiesis, chronic inflammation and histiocytosis. Nature Communications, 2021, 12, 4559.	12.8	17
28	From Your Nose to Your Toes: A Review of Severe Acute Respiratory Syndrome Coronavirus 2 Pandemicâ€'Associated Pernio. Journal of Investigative Dermatology, 2021, 141, 2791-2796.	0.7	21
29	Lentiviral correction of enzymatic activity restrains macrophage inflammation in adenosine deaminase 2 deficiency. Blood Advances, 2021, 5, 3174-3187.	5.2	18
30	Autoantibodies neutralizing type I IFNs are present in ~4% of uninfected individuals over 70 years old and account for ~20% of COVID-19 deaths. Science Immunology, 2021, 6, .	11.9	357
31	X-linked recessive TLR7 deficiency in ~1% of men under 60 years old with life-threatening COVID-19. Science Immunology, 2021, 6, .	11.9	267
32	Peripheral blood stem and progenitor cell collection in pediatric candidates for exÂvivo gene therapy: a 10-year series. Molecular Therapy - Methods and Clinical Development, 2021, 22, 76-83.	4.1	8
33	Evidence of treatment benefits in patients with MPSI-Hurler in long-term follow up using a new MRI scoring system. Journal of Pediatrics, 2021, , .	1.8	1
34	Potentialities of gene therapy in pediatric endocrinology. Hormone Research in Paediatrics, 2021, , .	1.8	2
35	Lentiviral-Mediated Gene Therapy for the Treatment of Adenosine Deaminase 2 Deficiency. Blood, 2021, 138, 2937-2937.	1.4	0
36	Health-Related Quality of Life and Emotional Difficulties in Chronic Granulomatous Disease: Data on Adult and Pediatric Patients from Italian Network for Primary Immunodeficiency (IPINet). Journal of Clinical Immunology, 2020, 40, 289-298.	3.8	11

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37	NFKB2 regulates human Tfh and Tfr pool formation and germinal center potential. Clinical Immunology, 2020, 210, 108309.	3.2	14
38	Mild SARS-CoV-2 Infection After Gene Therapy in a Child With Wiskott-Aldrich Syndrome: A Case Report. Frontiers in Immunology, 2020, 11, 603428.	4.8	8
39	Autoantibodies against type I IFNs in patients with life-threatening COVID-19. Science, 2020, 370, .	12.6	1,983
40	Consensus of the Italian Primary Immunodeficiency Network on transition management from pediatric to adult care in patients affected with childhood-onset inborn errors of immunity. Journal of Allergy and Clinical Immunology, 2020, 146, 967-983.	2.9	12
41	A Global Effort to Define the Human Genetics of Protective Immunity to SARS-CoV-2 Infection. Cell, 2020, 181, 1194-1199.	28.9	185
42	Expanded circulating hematopoietic stem/progenitor cells as novel cell source for the treatment of TCIRG1 osteopetrosis. Haematologica, 2020, 106, 74-86.	3.5	20
43	Gene therapy for Wiskott-Aldrich syndrome: History, new vectors, future directions. Journal of Allergy and Clinical Immunology, 2020, 146, 262-265.	2.9	31
44	Leukocyte and Dried Blood Spot Arylsulfatase A Assay by Tandem Mass Spectrometry. Analytical Chemistry, 2020, 92, 6341-6348.	6.5	17
45	New perspectives in gene therapy for inherited disorders. Pediatric Allergy and Immunology, 2020, 31, 5-7.	2.6	8
46	Urogenital Abnormalities in Adenosine Deaminase Deficiency. Journal of Clinical Immunology, 2020, 40, 610-618.	3.8	7
47	Hematopoietic Stem Cells Are Endowed with Erythroid Signature in Beta-Thalassemia. Blood, 2020, 136, 31-31.	1.4	0
48	A novel disorder involving dyshematopoiesis, inflammation, and HLH due to aberrant CDC42 function. Journal of Experimental Medicine, 2019, 216, 2778-2799.	8.5	132
49	Clinical, Immunological, and Molecular Features of Typical and Atypical Severe Combined Immunodeficiency: Report of the Italian Primary Immunodeficiency Network. Frontiers in Immunology, 2019, 10, 1908.	4.8	41
50	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent ÃŸ-thalassemia. Nature Medicine, 2019, 25, 234-241.	30.7	188
51	Advances in stem cell research and therapeutic development. Nature Cell Biology, 2019, 21, 801-811.	10.3	158
52	Lentiviral gene therapy corrects platelet phenotype and function in patients with Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2019, 144, 825-838.	2.9	50
53	Bone marrow harvesting from paediatric patients undergoing haematopoietic stem cell gene therapy. Bone Marrow Transplantation, 2019, 54, 1995-2003.	2.4	9
54	Targeting a Pre-existing Anti-transgene T Cell Response for Effective Gene Therapy of MPS-I in the Mouse Model of the Disease. Molecular Therapy, 2019, 27, 1215-1227.	8.2	17

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55	Targeted NGS Platforms for Genetic Screening and Gene Discovery in Primary Immunodeficiencies. Frontiers in Immunology, 2019, 10, 316.	4.8	42
56	A combined immunodeficiency with severe infections, inflammation, and allergy caused by ARPC1B deficiency. Journal of Allergy and Clinical Immunology, 2019, 143, 2296-2299.	2.9	87
57	Lentiviral haemopoietic stem/progenitor cell gene therapy for treatment of Wiskott-Aldrich syndrome: interim results of a non-randomised, open-label, phase 1/2 clinical study. Lancet Haematology,the, 2019, 6, e239-e253.	4.6	166
58	ALPS-Like Phenotype Caused by ADA2 Deficiency Rescued by Allogeneic Hematopoietic Stem Cell Transplantation. Frontiers in Immunology, 2019, 9, 2767.	4.8	42
59	In vivo dynamics of human hematopoietic stem cells: novel concepts and future directions. Blood Advances, 2019, 3, 1916-1924.	5.2	34
60	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. Frontiers in Pediatrics, 2019, 7, 443.	1.9	66
61	Penalized inference of the hematopoietic cell differentiation network via high-dimensional clonal tracking. Applied Network Science, 2019, 4, .	1.5	6
62	Consensus approach for the management of severe combined immune deficiency caused by adenosine deaminase deficiency. Journal of Allergy and Clinical Immunology, 2019, 143, 852-863.	2.9	104
63	Bone marrow stromal cells from Î²-thalassemia patients have impaired hematopoietic supportive capacity. Journal of Clinical Investigation, 2019, 129, 1566-1580.	8.2	46
64	Extensive Metabolic Correction of Hurler Disease By Hematopoietic Stem Cell-Based Gene Therapy: Preliminary Results from a Phase I/II Trial. Blood, 2019, 134, 607-607.	1.4	5
65	Biological Properties of HSC: Scientific Basis for HSCT. , 2019, , 49-56.		0
66	Autonomous role of Wiskott-Aldrich syndrome platelet deficiency in inducing autoimmunity and inflammation. Journal of Allergy and Clinical Immunology, 2018, 142, 1272-1284.	2.9	28
67	Impaired X-CGD T cell compartment is gp91phox-NADPH oxidase independent. Clinical Immunology, 2018, 193, 52-59.	3.2	15
68	Neutrophils drive type I interferon production and autoantibodies in patients with Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2018, 142, 1605-1617.e4.	2.9	21
69	Use of Defibrotide to help prevent post-transplant endothelial injury in a genetically predisposed infant with metachromatic leukodystrophy undergoing hematopoietic stem cell gene therapy. Bone Marrow Transplantation, 2018, 53, 913-917.	2.4	10
70	Long-term follow-up of IPEX syndrome patients after different therapeutic strategies: An international multicenter retrospective study. Journal of Allergy and Clinical Immunology, 2018, 141, 1036-1049.e5.	2.9	233
71	Gene Therapy for Adenosine Deaminase Deficiency: A Comprehensive Evaluation of Short- and Medium-Term Safety. Molecular Therapy, 2018, 26, 917-931.	8.2	50
72	Gene therapy in rare diseases: the benefits and challenges of developing a patient-centric registry for Strimvelis in ADA-SCID. Orphanet Journal of Rare Diseases, 2018, 13, 49.	2.7	34

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73	Gene therapy for mucopolysaccharidoses: in vivo and ex vivo approaches. Italian Journal of Pediatrics, 2018, 44, 130.	2.6	38
74	T-cell defects in patients with ARPC1B germline mutations account for combined immunodeficiency. Blood, 2018, 132, 2362-2374.	1.4	99
75	Dynamics of genetically engineered hematopoietic stem and progenitor cells after autologous transplantation in humans. Nature Medicine, 2018, 24, 1683-1690.	30.7	90
76	Hematopoietic stem cell gene therapy for the cure of blood diseases: primary immunodeficiencies. Rendiconti Lincei, 2018, 29, 755-764.	2.2	10
77	Gene Therapy for Primary Immunodeficiencies. , 2018, , 413-431.		1
78	<scp>JAK</scp>3 mutations in Italian patients affected by <scp>SCID</scp>: New molecular aspects of a longâ€known gene. Molecular Genetics & Genomic Medicine, 2018, 6, 713-721.	1.2	25
79	First Occurrence of Plasmablastic Lymphoma in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Disease Patient and Review of the Literature. Frontiers in Immunology, 2018, 9, 113.	4.8	25
80	Disease Evolution and Response to Rapamycin in Activated Phosphoinositide 3-Kinase Î´ Syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase Î´ Syndrome Registry. Frontiers in Immunology, 2018, 9, 543.	4.8	137
81	Successful Treatment With Ledipasvir/Sofosbuvir in an Infant With Severe Combined Immunodeficiency Caused by Adenosine Deaminase Deficiency With HCV Allowed Gene Therapy with Strimvelis. Hepatology, 2018, 68, 2434-2437.	7.3	16
82	The case of an APDS patient: Defects in maturation and function and decreased in vitro anti-mycobacterial activity in the myeloid compartment. Clinical Immunology, 2017, 178, 20-28.	3.2	31
83	Good Laboratory Practice Preclinical Safety Studies for GSK2696273 (MLV Vector-Based Ex Vivo) Tj ETQq1 1 0.784314 rgBT /O Human Gene Therapy Clinical Development, 2017, 28, 17-27.	3.1	12
84	Gene therapy for ADAâ€SCID, the first marketing approval of an exÂvivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. EMBO Molecular Medicine, 2017, 9, 737-740.	6.9	210
85	Efficient ExÂVivo Engineering and Expansion of Highly Purified Human Hematopoietic Stem and Progenitor Cell Populations for Gene Therapy. Stem Cell Reports, 2017, 8, 977-990.	4.8	124
86	A map of human circular RNAs in clinically relevant tissues. Journal of Molecular Medicine, 2017, 95, 1179-1189.	3.9	286
87	Twenty-Five Years of Gene Therapy for ADA-SCID: FromBubble Babiesto an Approved Drug. Human Gene Therapy, 2017, 28, 972-981.	2.7	87
88	Severe Toxoplasma gondii infection in a member of a NFKB2-deficient family with T and B cell dysfunction. Clinical Immunology, 2017, 183, 273-277.	3.2	32
89	Biological and functional characterization of bone marrow-derived mesenchymal stromal cells from patients affected by primary immunodeficiency. Scientific Reports, 2017, 7, 8153.	3.3	17
90	Neonatal umbilical cord blood transplantation halts skeletal disease progression in the murine model of MPS-I. Scientific Reports, 2017, 7, 9473.	3.3	9

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91	Multiparametric Whole Blood Dissection: A oneâ€shot comprehensive picture of the human hematopoietic system. Cytometry Part A: the Journal of the International Society for Analytical Cytology, 2017, 91, 952-965.	1.5	18
92	Long-Term Outcome of Adenosine Deaminase-Deficient Patientsâ€"a Single-Center Experience. Journal of Clinical Immunology, 2017, 37, 582-591.	3.8	26
93	Gene therapy for lysosomal storage disorders: recent advances for metachromatic leukodystrophy and mucopolysaccaridosis I. Journal of Inherited Metabolic Disease, 2017, 40, 543-554.	3.6	67
94	Large Deletion of MAGT1 Gene in a Patient with Classic Kaposi Sarcoma, CD4 Lymphopenia, and EBV Infection. Journal of Clinical Immunology, 2017, 37, 32-35.	3.8	38
95	A prospective study on the natural history of patients with profound combined immunodeficiency: An interim analysis. Journal of Allergy and Clinical Immunology, 2017, 139, 1302-1310.e4.	2.9	71
96	Primary immunodeficiency diseases: Genomic approaches delineate heterogeneous Mendelian disorders. Journal of Allergy and Clinical Immunology, 2017, 139, 232-245.	2.9	261
97	Alterations in the brain adenosine metabolism cause behavioral and neurological impairment in ADA-deficient mice and patients. Scientific Reports, 2017, 7, 40136.	3.3	38
98	Immunotherapy of acute leukemia by chimeric antigen receptor-modified lymphocytes using an improved Sleeping Beauty transposon platform. Oncotarget, 2016, 7, 51581-51597.	1.8	43
99	Pioglitazone as a novel therapeutic approach in chronic granulomatous disease. Journal of Allergy and Clinical Immunology, 2016, 137, 1913-1915.e2.	2.9	23
100	Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. Blood, 2016, 128, 45-54.	1.4	173
101	Lentiviral Vector Gene Therapy Protects XCGD Mice From Acute Staphylococcus aureus Pneumonia and Inflammatory Response. Molecular Therapy, 2016, 24, 1873-1880.	8.2	14
102	The Role of Conditioning in Hematopoietic Stem-Cell Gene Therapy. Human Gene Therapy, 2016, 27, 741-748.	2.7	40
103	Safer conditioning for blood stem cell transplants. Nature Biotechnology, 2016, 34, 721-723.	17.5	14
104	Bone marrowâ€"derived CD34 â^' fraction: A rich source of mesenchymal stromal cells for clinical application. Cytotherapy, 2016, 18, 1560-1563.	0.7	11
105	InÂVivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases. Cell Stem Cell, 2016, 19, 107-119.	11.1	187
106	AQP8 transports NOX2-generated H2O2 across the plasma membrane to promote signaling in B cells. Journal of Leukocyte Biology, 2016, 100, 1071-1079.	3.3	69
107	A novel genomic inversion in Wiskott-Aldrichâ€"associated autoinflammation. Journal of Allergy and Clinical Immunology, 2016, 138, 619-622.e7.	2.9	15
108	Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. Lancet, The, 2016, 388, 476-487.	13.7	393

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109	Combined immunodeficiency due to JAK3 mutation in a child presenting with skin granuloma. Journal of Allergy and Clinical Immunology, 2016, 137, 948-951.e5.	2.9	17
110	Incremental Innovation of Ex Vivo Hematopoietic Stem Cell Engineering to Expand Clinical Gene Therapy Applications. Blood, 2016, 128, 4707-4707.	1.4	0
111	Wiskott-Aldrich Syndrome: A Retrospective Study on 575 Patients Analyzing the Impact of Splenectomy, Stem Cell Transplantation, or No Definitive Treatment on Frequency of Disease-Related Complications and Physician-Perceived Quality of Life. Blood, 2016, 128, 366-366.	1.4	2
112	Autoimmunity and regulatory T cells in 22q11.2 deletion syndrome patients. Pediatric Allergy and Immunology, 2015, 26, 591-594.	2.6	29
113	Tracking genetically engineered lymphocytes long-term reveals the dynamics of T cell immunological memory. Science Translational Medicine, 2015, 7, 317ra198.	12.4	102
114	Defective B-cell proliferation and maintenance of long-term memory in patients with chronic granulomatous disease. Journal of Allergy and Clinical Immunology, 2015, 135, 753-761.e2.	2.9	49
115	B-cell reconstitution after lentiviral vectorâ€“mediated gene therapy in patients with Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2015, 136, 692-702.e2.	2.9	41
116	Longitudinal Evaluation of Immune Reconstitution and B-cell Function After Hematopoietic Cell Transplantation for Primary Immunodeficiency. Journal of Clinical Immunology, 2015, 35, 373-383.	3.8	15
117	Clinical Applications of Gene Therapy for Primary Immunodeficiencies. Human Gene Therapy, 2015, 26, 210-219.	2.7	78
118	In vivo tracking of T cells in humans unveils decade-long survival and activity of genetically modified T memory stem cells. Science Translational Medicine, 2015, 7, 273ra13.	12.4	160
119	Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. Journal of Clinical Investigation, 2015, 125, 3941-3951.	8.2	43
120	Dual-regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. Molecular Therapy, 2014, 22, 1472-1483.	8.2	59
121	Etiology, clinical outcome, and laboratory features in children with neutropenia: Analysis of 104 cases. Pediatric Allergy and Immunology, 2014, 25, 283-289.	2.6	21
122	Progress in gene therapy for primary immunodeficiencies using lentiviral vectors. Current Opinion in Allergy and Clinical Immunology, 2014, 14, 527-534.	2.3	24
123	B-cell development and functions and therapeutic options in adenosine deaminaseâ€“deficient patients. Journal of Allergy and Clinical Immunology, 2014, 133, 799-806.e10.	2.9	30
124	Lentiviral vectors for the treatment of primary immunodeficiencies. Journal of Inherited Metabolic Disease, 2014, 37, 525-533.	3.6	18
125	Clinical Features and Follow-Up in Patients with 22q11.2 Deletion Syndrome. Journal of Pediatrics, 2014, 164, 1475-1480.e2.	1.8	119
126	Wiskottâ€“Aldrich Syndrome protein deficiency perturbs the homeostasis of B-cell compartment in humans. Journal of Autoimmunity, 2014, 50, 42-50.	6.5	72

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127	Chronic Granulomatous Disease Presenting With Salmonella Brain Abscesses. Pediatric Infectious Disease Journal, 2014, 33, 525-528.	2.0	7
128	Gene Therapy for Wiskott-Aldrich Syndrome. Current Gene Therapy, 2014, 14, 413-421.	2.0	18
129	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. Science, 2013, 341, 1233158.	12.6	998
130	Lentiviral Hematopoietic Stem Cell Gene Therapy in Patients with Wiskott-Aldrich Syndrome. Science, 2013, 341, 1233151.	12.6	900
131	Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity. Clinical Immunology, 2013, 146, 248-261.	3.2	186
132	The Committee for Advanced Therapies' of the European Medicines Agency Reflection Paper on Management of Clinical Risks Deriving from Insertional Mutagenesis. Human Gene Therapy Clinical Development, 2013, 24, 47-54.	3.1	30
133	Preclinical Safety and Efficacy of Human CD34+ Cells Transduced With Lentiviral Vector for the Treatment of Wiskott-Aldrich Syndrome. Molecular Therapy, 2013, 21, 175-184.	8.2	72
134	Wiskott-Aldrich syndrome proteinâ€“mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. Journal of Experimental Medicine, 2013, 210, 355-374.	8.5	49
135	Serratia marcescens Osteomyelitis in a Newborn With Chronic Granulomatous Disease. Pediatric Infectious Disease Journal, 2013, 32, 926.	2.0	17
136	Wiskott-Aldrich syndrome proteinâ€“mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. Journal of Cell Biology, 2013, 200, i6-i6.	5.2	0
137	Autoimmune Dysregulation and Purine Metabolism in Adenosine Deaminase Deficiency. Frontiers in Immunology, 2012, 3, 265.	4.8	102
138	T-cell suicide gene therapy prompts thymic renewal in adults after hematopoietic stem cell transplantation. Blood, 2012, 120, 1820-1830.	1.4	47
139	HIV-1 envelope-dependent restriction of CXCR4-using viruses in child but not adult untransformed CD4+ T-lymphocyte lines. Blood, 2012, 119, 2013-2023.	1.4	6
140	Alterations in the adenosine metabolism and CD39/CD73 adenosinergic machinery cause loss of Treg cell function and autoimmunity in ADA-deficient SCID. Blood, 2012, 119, 1428-1439.	1.4	107
141	Outcome of hematopoietic stem cell transplantation for adenosine deaminaseâ€“deficient severe combined immunodeficiency. Blood, 2012, 120, 3615-3624.	1.4	151
142	Retroviral Integrations in Gene Therapy Trials. Molecular Therapy, 2012, 20, 709-716.	8.2	108
143	Gene therapy for primary immunodeficiencies: Part 2. Current Opinion in Immunology, 2012, 24, 585-591.	5.5	61
144	Gene therapy for primary immunodeficiencies: part 1. Current Opinion in Immunology, 2012, 24, 580-584.	5.5	82

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145	Defective B cell tolerance in adenosine deaminase deficiency is corrected by gene therapy. Journal of Clinical Investigation, 2012, 122, 2141-2152.	8.2	55
146	In vivo T-cell dynamics during immune reconstitution after hematopoietic stem cell gene therapy in adenosine deaminase severe combined immune deficiency. Journal of Allergy and Clinical Immunology, 2011, 127, 1368-1375.e8.	2.9	13
147	Purine metabolism, immune reconstitution, and abdominal adipose tumor after gene therapy for adenosine deaminase deficiency. Journal of Allergy and Clinical Immunology, 2011, 127, 1417-1419.e3.	2.9	13
148	Early-onset monocyteâ€“Bâ€“natural killerâ€“dendritic cellsâ€™ deficiency successfully treated with hematopoietic stem cell transplantation. Journal of Allergy and Clinical Immunology, 2011, 128, 897-900.e1.	2.9	1
149	Lentiviral-mediated gene therapy leads to improvement of B-cell functionality in a murine model of Wiskott-Aldrich syndrome. Journal of Allergy and Clinical Immunology, 2011, 127, 1376-1384.e5.	2.9	34
150	Successful Treatment With Percutaneous Transhepatic Alcoholization of a Liver Abscess in a Child With Chronic Granulomatous Disease. Pediatric Infectious Disease Journal, 2011, 30, 819-820.	2.0	5
151	Integration profile of retroviral vector in gene therapy treated patients is cellâ€specific according to gene expression and chromatin conformation of target cell. EMBO Molecular Medicine, 2011, 3, 89-101.	6.9	95
152	Insertion Sites in Engrafted Cells Cluster Within a Limited Repertoire of Genomic Areas After Gammaretroviral Vector Gene Therapy. Molecular Therapy, 2011, 19, 2031-2039.	8.2	48
153	Update on gene therapy for adenosine deaminase-deficient severe combined immunodeficiency. Current Opinion in Allergy and Clinical Immunology, 2010, 10, 551-556.	2.3	56
154	Role of reduced intensity conditioning in T-cell and B-cell immune reconstitution after HLA-identical bone marrow transplantation in ADA-SCID. Haematologica, 2010, 95, 1778-1782.	3.5	16
155	Gene Therapy for Primary Immunodeficiencies. , 2010, , 213-231.		0
156	Gene Therapy for Adenosine Deaminase Deficiency. Immunology and Allergy Clinics of North America, 2010, 30, 249-260.	1.9	16
157	Revertant T lymphocytes in a patient with Wiskott-Aldrich syndrome: Analysis of function and distribution in lymphoid organs. Journal of Allergy and Clinical Immunology, 2010, 125, 439-448.e8.	2.9	31
158	Unpredictability of Intravenous Busulfan Pharmacokinetics in Children Undergoing Hematopoietic Stem Cell Transplantation for Advanced Beta Thalassemia: Limited Toxicity with a Dose-Adjustment Policy. Biology of Blood and Marrow Transplantation, 2010, 16, 622-628.	2.0	36
159	Ten years of gene therapy for primary immune deficiencies. Hematology American Society of Hematology Education Program, 2009, 2009, 682-689.	2.5	86
160	Evidence for Long-term Efficacy and Safety of Gene Therapy for Wiskottâ€“Aldrich Syndrome in Preclinical Models. Molecular Therapy, 2009, 17, 1073-1082.	8.2	77
161	The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. Journal of Experimental Medicine, 2009, 206, 735-742.	8.5	53
162	The Quality of Life of Children and Adolescents with X-Linked Agammaglobulinemia. Journal of Clinical Immunology, 2009, 29, 501-507.	3.8	34

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163	Hematopoietic stem cell gene therapy for adenosine deaminase deficient-SCID. Immunologic Research, 2009, 44, 150-159.	2.9	32
164	Comprehensive genomic access to vector integration in clinical gene therapy. Nature Medicine, 2009, 15, 1431-1436.	30.7	173
165	Gene Therapy for Immunodeficiency Due to Adenosine Deaminase Deficiency. New England Journal of Medicine, 2009, 360, 447-458.	27.0	944
166	New insights into the pathogenesis of adenosine deaminase-severe combined immunodeficiency and progress in gene therapy. Current Opinion in Allergy and Clinical Immunology, 2009, 9, 496-502.	2.3	40
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