Alessandro Aiuti

List of Publications by Year in Descending Order

217 papers	16,063 citations	62 h-index	123 g-index
233 ext. papers	19,526 ext. citations	9.5 avg, IF	6.11 L-index

#	Paper	IF	Citations
217	Human genetic and immunological determinants of critical COVID-19 pneumonia.. *Nature*, **2022**,	50.4	23
216	Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT inborn errors working party analysis.. *Blood*, **2022**,	2.2	3
215	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	40	11
214	Wiskott-Aldrich syndrome: Oral findings and microbiota in children and review of the literature.. *Clinical and Experimental Dental Research*, **2022**, 8, 28-36	1.9	2
213	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders.. *Nature Communications*, **2022**, 13, 1315	17.4	3
212	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	11.5	3
211	Expanded circulating hematopoietic stem/progenitor cells as novel cell source for the treatment of TCIRG1 osteopetrosis. *Haematologica*, **2021**, 106, 74-86	6.6	10
210	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*, **2021**, 1	5.7	0
209	Lentiviral-Mediated Gene Therapy for the Treatment of Adenosine Deaminase 2 Deficiency. *Blood*, **2021**, 138, 2937-2937	2.2	
208	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	5.4	24
207	SARS-CoV-2-related MIS-C: A key to the viral and genetic causes of Kawasaki disease?. *Journal of Experimental Medicine*, **2021**, 218,	16.6	45
206	Metachromatic leukodystrophy: A single-center longitudinal study of 45 patients. *Journal of Inherited Metabolic Disease*, **2021**, 44, 1151-1164	5.4	5
205	Retrieval of vector integration sites from cell-free DNA. *Nature Medicine*, **2021**, 27, 1458-1470	50.5	5
204	Oncogene-induced senescence in hematopoietic progenitors features myeloid restricted hematopoiesis, chronic inflammation and histiocytosis. *Nature Communications*, **2021**, 12, 4559	17.4	3
203	Hematopoietic Tumors in a Mouse Model of X-linked Chronic Granulomatous Disease after Lentiviral Vector-Mediated Gene Therapy. *Molecular Therapy*, **2021**, 29, 86-102	11.7	8
202	Coronavirus disease 2019 in patients with inborn errors of immunity: An international study. *Journal of Allergy and Clinical Immunology*, **2021**, 147, 520-531	11.5	142
201	Gene therapy using haematopoietic stem and progenitor cells. *Nature Reviews Genetics*, **2021**, 22, 216-234	50.1	39

200	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	2	4
199	Emapalumab treatment in an ADA-SCID patient with refractory hemophagocytic lymphohistiocytosis-related graft failure and disseminated bacillus Calmette-Guérin infection. *Haematologica*, **2021**, 106, 641-646	6.6	9
198	Immunosuppressive therapy in childhood-onset arrhythmogenic inflammatory cardiomyopathy. *PACE - Pacing and Clinical Electrophysiology*, **2021**, 44, 552-556	1.6	5
197	Update on Clinical Ex Vivo Hematopoietic Stem Cell Gene Therapy for Inherited Monogenic Diseases. *Molecular Therapy*, **2021**, 29, 489-504	11.7	12
196	From Your Nose to Your Toes: A Review of Severe Acute Respiratory Syndrome Coronavirus 2 Pandemic-Associated Pernio. *Journal of Investigative Dermatology*, **2021**, 141, 2791-2796	4.3	6
195	Lentiviral correction of enzymatic activity restrains macrophage inflammation in adenosine deaminase 2 deficiency. *Blood Advances*, **2021**, 5, 3174-3187	7.8	3
194	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,	28	91
193	X-linked recessive TLR7 deficiency in ~1% of men under 60 years old with life-threatening COVID-19. *Science Immunology*, **2021**, 6,	28	67
192	Peripheral blood stem and progenitor cell collection in pediatric candidates for gene therapy: a 10-year series. *Molecular Therapy - Methods and Clinical Development*, **2021**, 22, 76-83	6.4	0
191	Evidence of Treatment Benefits in Patients with Mucopolysaccharidosis Type I-Hurler in Long-term Follow-up Using a New Magnetic Resonance Imaging Scoring System. *Journal of Pediatrics*, **2021**,	3.6	1
190	A Global Effort to Define the Human Genetics of Protective Immunity to SARS-CoV-2 Infection. *Cell*, **2020**, 181, 1194-1199	56.2	113
189	Leukocyte and Dried Blood Spot Arylsulfatase A Assay by Tandem Mass Spectrometry. *Analytical Chemistry*, **2020**, 92, 6341-6348	7.8	8
188	New perspectives in gene therapy for inherited disorders. *Pediatric Allergy and Immunology*, **2020**, 31 Suppl 24, 5-7	4.2	3
187	Urogenital Abnormalities in Adenosine Deaminase Deficiency. *Journal of Clinical Immunology*, **2020**, 40, 610-618	5.7	4
186	Hematopoietic Stem Cells Are Endowed with Erythroid Signature in Beta-Thalassemia. *Blood*, **2020**, 136, 31-31	2.2	
185	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	5.7	7
184	NFKB2 regulates human Tfh and Tfr pool formation and germinal center potential. *Clinical Immunology*, **2020**, 210, 108309	9	6
183	Mild SARS-CoV-2 Infection After Gene Therapy in a Child With Wiskott-Aldrich Syndrome: A Case Report. *Frontiers in Immunology*, **2020**, 11, 603428	8.4	5

182	Autoantibodies against type I IFNs in patients with life-threatening COVID-19. *Science*, **2020**, 370,	33.3	1090
181	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	11.5	0
180	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	8.4	19
179	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent β-thalassemia. *Nature Medicine*, **2019**, 25, 234-241	50.5	110
178	Advances in stem cell research and therapeutic development. *Nature Cell Biology*, **2019**, 21, 801-811	23.4	90
177	Lentiviral gene therapy corrects platelet phenotype and function in patients with Wiskott-Aldrich syndrome. *Journal of Allergy and Clinical Immunology*, **2019**, 144, 825-838	11.5	29
176	Bone marrow harvesting from paediatric patients undergoing haematopoietic stem cell gene therapy. *Bone Marrow Transplantation*, **2019**, 54, 1995-2003	4.4	7
175	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	11.7	10
174	Targeted NGS Platforms for Genetic Screening and Gene Discovery in Primary Immunodeficiencies. *Frontiers in Immunology*, **2019**, 10, 316	8.4	22
173	A combined immunodeficiency with severe infections, inflammation, and allergy caused by ARPC1B deficiency. *Journal of Allergy and Clinical Immunology*, **2019**, 143, 2296-2299	11.5	59
172	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	14.6	95
171	A novel disorder involving dyshematopoiesis, inflammation, and HLH due to aberrant CDC42 function. *Journal of Experimental Medicine*, **2019**, 216, 2778-2799	16.6	71
170	Bone marrow stromal cells from β-thalassemia patients have impaired hematopoietic supportive capacity. *Journal of Clinical Investigation*, **2019**, 129, 1566-1580	15.9	24
169	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	2.2	4
168	Biological Properties of HSC: Scientific Basis for HSCT **2019**, 49-56		
167	In vivo dynamics of human hematopoietic stem cells: novel concepts and future directions. *Blood Advances*, **2019**, 3, 1916-1924	7.8	17
166	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. *Frontiers in Pediatrics*, **2019**, 7, 443	3.4	34
165	Penalized inference of the hematopoietic cell differentiation network via high-dimensional clonal tracking. *Applied Network Science*, **2019**, 4,	2.9	1

164	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	11.5	71
163	ALPS-Like Phenotype Caused by ADA2 Deficiency Rescued by Allogeneic Hematopoietic Stem Cell Transplantation. *Frontiers in Immunology*, **2018**, 9, 2767	8.4	22
162	Autonomous role of Wiskott-Aldrich syndrome platelet deficiency in inducing autoimmunity and inflammation. *Journal of Allergy and Clinical Immunology*, **2018**, 142, 1272-1284	11.5	17
161	Impaired X-CGD T cell compartment is gp91phox-NADPH oxidase independent. *Clinical Immunology* , **2018**, 193, 52-59	9	4
160	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	11.5	13
159	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	4.4	10
158	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	11.5	157
157	Gene Therapy for Adenosine Deaminase Deficiency: A Comprehensive Evaluation of Short- and Medium-Term Safety. *Molecular Therapy*, **2018**, 26, 917-931	11.7	35
156	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	4.2	23
155	JAK3 mutations in Italian patients affected by SCID: New molecular aspects of a long-known gene. *Molecular Genetics & Genomic Medicine*, **2018**, 6, 713-721	2.3	11
154	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	8.4	15
153	Disease Evolution and Response to Rapamycin in Activated Phosphoinositide 3-Kinase δ Syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase δ Syndrome Registry. *Frontiers in Immunology*, **2018**, 9, 543	8.4	88
152	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	11.2	11
151	Gene therapy for mucopolysaccharidoses: in vivo and ex vivo approaches. *Italian Journal of Pediatrics*, **2018**, 44, 130	3.2	19
150	T-cell defects in patients with germline mutations account for combined immunodeficiency. *Blood*, **2018**, 132, 2362-2374	2.2	59
149	Dynamics of genetically engineered hematopoietic stem and progenitor cells after autologous transplantation in humans. *Nature Medicine*, **2018**, 24, 1683-1690	50.5	62
148	Hematopoietic stem cell gene therapy for the cure of blood diseases: primary immunodeficiencies. *Rendiconti Lincei*, **2018**, 29, 755-764	1.7	2
147	Gene Therapy for Primary Immunodeficiencies **2018**, 413-431		

146	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	9	26
145	Good Laboratory Practice Preclinical Safety Studies for GSK2696273 (MLV Vector-Based Ex Vivo Gene Therapy for Adenosine Deaminase Deficiency Severe Combined Immunodeficiency) in NSG Mice. *Human Gene Therapy Clinical Development*, **2017**, 28, 17-27	3.2	11
144	Gene therapy for ADA-SCID, the first marketing approval of an gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. *EMBO Molecular Medicine*, **2017**, 9, 737-740	12	138
143	Efficient Ex Vivo Engineering and Expansion of Highly Purified Human Hematopoietic Stem and Progenitor Cell Populations for Gene Therapy. *Stem Cell Reports*, **2017**, 8, 977-990	8	92
142	A map of human circular RNAs in clinically relevant tissues. *Journal of Molecular Medicine*, **2017**, 95, 1179-1189	5.5	195
141	Twenty-Five Years of Gene Therapy for ADA-SCID: From Bubble Babies to an Approved Drug. *Human Gene Therapy*, **2017**, 28, 972-981	4.8	64
140	Severe Toxoplasma gondii infection in a member of a NFKB2-deficient family with T and B cell dysfunction. *Clinical Immunology*, **2017**, 183, 273-277	9	10
139	Biological and functional characterization of bone marrow-derived mesenchymal stromal cells from patients affected by primary immunodeficiency. *Scientific Reports*, **2017**, 7, 8153	4.9	12
138	Neonatal umbilical cord blood transplantation halts skeletal disease progression in the murine model of MPS-I. *Scientific Reports*, **2017**, 7, 9473	4.9	6
137	Multiparametric Whole Blood Dissection: A one-shot comprehensive picture of the human hematopoietic system. *Cytometry Part A: the Journal of the International Society for Analytical Cytology*, **2017**, 91, 952-965	4.6	8
136	Long-Term Outcome of Adenosine Deaminase-Deficient Patients-a Single-Center Experience. *Journal of Clinical Immunology*, **2017**, 37, 582-591	5.7	19
135	Gene therapy for lysosomal storage disorders: recent advances for metachromatic leukodystrophy and mucopolysaccaridosis I. *Journal of Inherited Metabolic Disease*, **2017**, 40, 543-554	5.4	50
134	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	5.7	26
133	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	11.5	43
132	Primary immunodeficiency diseases: Genomic approaches delineate heterogeneous Mendelian disorders. *Journal of Allergy and Clinical Immunology*, **2017**, 139, 232-245	11.5	164
131	Alterations in the brain adenosine metabolism cause behavioral and neurological impairment in ADA-deficient mice and patients. *Scientific Reports*, **2017**, 7, 40136	4.9	27
130	The Role of Conditioning in Hematopoietic Stem-Cell Gene Therapy. *Human Gene Therapy*, **2016**, 27, 741-748	4.8	27
129	Safer conditioning for blood stem cell transplants. *Nature Biotechnology*, **2016**, 34, 721-3	44.5	13

128	Bone marrow-derived CD34 fraction: A rich source of mesenchymal stromal cells for clinical application. *Cytotherapy*, **2016**, 18, 1560-1563	4.8	9
127	In Vivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases. *Cell Stem Cell*, **2016**, 19, 107-19	18	130
126	AQP8 transports NOX2-generated H2O2 across the plasma membrane to promote signaling in B cells. *Journal of Leukocyte Biology*, **2016**, 100, 1071-1079	6.5	45
125	A novel genomic inversion in Wiskott-Aldrich-associated autoinflammation. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 619-622.e7	11.5	11
124	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, 476-87	40	287
123	Combined immunodeficiency due to JAK3 mutation in a child presenting with skin granuloma. *Journal of Allergy and Clinical Immunology*, **2016**, 137, 948-51.e5	11.5	5
122	Incremental Innovation of Ex Vivo Hematopoietic Stem Cell Engineering to Expand Clinical Gene Therapy Applications. *Blood*, **2016**, 128, 4707-4707	2.2	
121	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	2.2	1
120	Immunotherapy of acute leukemia by chimeric antigen receptor-modified lymphocytes using an improved Sleeping Beauty transposon platform. *Oncotarget*, **2016**, 7, 51581-51597	3.3	33
119	Pioglitazone as a novel therapeutic approach in chronic granulomatous disease. *Journal of Allergy and Clinical Immunology*, **2016**, 137, 1913-1915.e2	11.5	19
118	Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. *Blood*, **2016**, 128, 45-54	2.2	133
117	Lentiviral Vector Gene Therapy Protects XCGD Mice From Acute Staphylococcus aureus Pneumonia and Inflammatory Response. *Molecular Therapy*, **2016**, 24, 1873-1880	11.7	7
116	B-cell reconstitution after lentiviral vector-mediated gene therapy in patients with Wiskott-Aldrich syndrome. *Journal of Allergy and Clinical Immunology*, **2015**, 136, 692-702.e2	11.5	34
115	Longitudinal Evaluation of Immune Reconstitution and B-cell Function After Hematopoietic Cell Transplantation for Primary Immunodeficiency. *Journal of Clinical Immunology*, **2015**, 35, 373-83	5.7	11
114	Clinical applications of gene therapy for primary immunodeficiencies. *Human Gene Therapy*, **2015**, 26, 210-9	4.8	65
113	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	17.5	114
112	Autoimmunity and regulatory T cells in 22q11.2 deletion syndrome patients. *Pediatric Allergy and Immunology*, **2015**, 26, 591-4	4.2	14
111	Tracking genetically engineered lymphocytes long-term reveals the dynamics of T cell immunological memory. *Science Translational Medicine*, **2015**, 7, 317ra198	17.5	65

110	Defective B-cell proliferation and maintenance of long-term memory in patients with chronic granulomatous disease. *Journal of Allergy and Clinical Immunology*, **2015**, 135, 753-61.e2	11.5	32
109	Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. *Journal of Clinical Investigation*, **2015**, 125, 3941-51	15.9	37
108	B-cell development and functions and therapeutic options in adenosine deaminase-deficient patients. *Journal of Allergy and Clinical Immunology*, **2014**, 133, 799-806.e10	11.5	27
107	Lentiviral vectors for the treatment of primary immunodeficiencies. *Journal of Inherited Metabolic Disease*, **2014**, 37, 525-33	5.4	15
106	Clinical features and follow-up in patients with 22q11.2 deletion syndrome. *Journal of Pediatrics*, **2014**, 164, 1475-80.e2	3.6	83
105	Wiskott-Aldrich Syndrome protein deficiency perturbs the homeostasis of B-cell compartment in humans. *Journal of Autoimmunity*, **2014**, 50, 42-50	15.5	63
104	Chronic granulomatous disease presenting with salmonella brain abscesses. *Pediatric Infectious Disease Journal*, **2014**, 33, 525-8	3.4	5
103	Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatosis. *Molecular Therapy*, **2014**, 22, 1472-1483	11.7	50
102	Etiology, clinical outcome, and laboratory features in children with neutropenia: analysis of 104 cases. *Pediatric Allergy and Immunology*, **2014**, 25, 283-9	4.2	14
101	Progress in gene therapy for primary immunodeficiencies using lentiviral vectors. *Current Opinion in Allergy and Clinical Immunology*, **2014**, 14, 527-34	3.3	22
100	Gene therapy for Wiskott-Aldrich Syndrome. *Current Gene Therapy*, **2014**, 14, 413-21	4.3	13
99	Lentiviral hematopoietic stem cell gene therapy benefits metachromatic leukodystrophy. *Science*, **2013**, 341, 1233158	33.3	837
98	Lentiviral hematopoietic stem cell gene therapy in patients with Wiskott-Aldrich syndrome. *Science*, **2013**, 341, 1233151	33.3	755
97	Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity. *Clinical Immunology*, **2013**, 146, 248-61	9	141
96	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	3.2	26
95	Preclinical safety and efficacy of human CD34(+) cells transduced with lentiviral vector for the treatment of Wiskott-Aldrich syndrome. *Molecular Therapy*, **2013**, 21, 175-84	11.7	63
94	Wiskott-Aldrich syndrome protein-mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. *Journal of Experimental Medicine*, **2013**, 210, 355-74	16.6	45
93	Serratia marcescens osteomyelitis in a newborn with chronic granulomatous disease. *Pediatric Infectious Disease Journal*, **2013**, 32, 926	3.4	14

92	Wiskott-Aldrich syndrome protein-mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. *Journal of Cell Biology*, **2013**, 200, i6-i6	7.3	
91	T-cell suicide gene therapy prompts thymic renewal in adults after hematopoietic stem cell transplantation. *Blood*, **2012**, 120, 1820-30	2.2	43
90	HIV-1 envelope-dependent restriction of CXCR4-using viruses in child but not adult untransformed CD4+ T-lymphocyte lines. *Blood*, **2012**, 119, 2013-23	2.2	3
89	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, 1428-39	2.2	79
88	Outcome of hematopoietic stem cell transplantation for adenosine deaminase-deficient severe combined immunodeficiency. *Blood*, **2012**, 120, 3615-24; quiz 3626	2.2	126
87	Retroviral integrations in gene therapy trials. *Molecular Therapy*, **2012**, 20, 709-16	11.7	91
86	Gene therapy for primary immunodeficiencies: Part 2. *Current Opinion in Immunology*, **2012**, 24, 585-91	7.8	51
85	Gene therapy for primary immunodeficiencies: Part 1. *Current Opinion in Immunology*, **2012**, 24, 580-4	7.8	73
84	Autoimmune dysregulation and purine metabolism in adenosine deaminase deficiency. *Frontiers in Immunology*, **2012**, 3, 265	8.4	81
83	Defective B cell tolerance in adenosine deaminase deficiency is corrected by gene therapy. *Journal of Clinical Investigation*, **2012**, 122, 2141-52	15.9	45
82	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, 1368-75.e8	11.5	13
81	Purine metabolism, immune reconstitution, and abdominal adipose tumor after gene therapy for adenosine deaminase deficiency. *Journal of Allergy and Clinical Immunology*, **2011**, 127, 1417-9.e3	11.5	11
80	Early-onset monocyte-B-natural killer-dendritic cells' deficiency successfully treated with hematopoietic stem cell transplantation. *Journal of Allergy and Clinical Immunology*, **2011**, 128, 897-900.e1	11.5	1
79	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, 1376-84.e5	11.5	32
78	Successful treatment with percutaneous transhepatic alcoholization of a liver abscess in a child with chronic granulomatous disease. *Pediatric Infectious Disease Journal*, **2011**, 30, 819-20	3.4	5
77	Integration profile of retroviral vector in gene therapy treated patients is cell-specific according to gene expression and chromatin conformation of target cell. *EMBO Molecular Medicine*, **2011**, 3, 89-101	12	81
76	Insertion sites in engrafted cells cluster within a limited repertoire of genomic areas after gammaretroviral vector gene therapy. *Molecular Therapy*, **2011**, 19, 2031-9	11.7	38
75	Gene Therapy for Primary Immunodeficiencies **2010**, 213-231		

74	Gene therapy for adenosine deaminase deficiency. *Immunology and Allergy Clinics of North America*, **2010**, 30, 249-60	3.3	11
73	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	11.5	28
72	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, 622-8	4.7	31
71	Update on gene therapy for adenosine deaminase-deficient severe combined immunodeficiency. *Current Opinion in Allergy and Clinical Immunology*, **2010**, 10, 551-6	3.3	46
70	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, 1778-82	6.6	16
69	Ten years of gene therapy for primary immune deficiencies. *Hematology American Society of Hematology Education Program*, **2009**, 682-9	3.1	70
68	Evidence for long-term efficacy and safety of gene therapy for Wiskott-Aldrich syndrome in preclinical models. *Molecular Therapy*, **2009**, 17, 1073-82	11.7	66
67	The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. *Journal of Experimental Medicine*, **2009**, 206, 735-42	16.6	48
66	The quality of life of children and adolescents with X-linked agammaglobulinemia. *Journal of Clinical Immunology*, **2009**, 29, 501-7	5.7	30
65	Hematopoietic stem cell gene therapy for adenosine deaminase deficient-SCID. *Immunologic Research*, **2009**, 44, 150-9	4.3	29
64	Comprehensive genomic access to vector integration in clinical gene therapy. *Nature Medicine*, **2009**, 15, 1431-6	50.5	135
63	Gene therapy for immunodeficiency due to adenosine deaminase deficiency. *New England Journal of Medicine*, **2009**, 360, 447-58	59.2	792
62	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	3.3	33
61	How I treat ADA deficiency. *Blood*, **2009**, 114, 3524-32	2.2	168
60	Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. *Blood*, **2009**, 113, 6288-95	2.2	184
59	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, 3546-56	2.2	61
58	ADA-deficient SCID is associated with a specific microenvironment and bone phenotype characterized by RANKL/OPG imbalance and osteoblast insufficiency. *Blood*, **2009**, 114, 3216-26	2.2	68
57	The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. *Journal of Cell Biology*, **2009**, 185, i1-i1	7.3	

56	Clinical improvement and normalized Th1 cytokine profile in early and long-term interferon-alpha treatment in a suspected case of hyper-IgE syndrome. *Pediatric Allergy and Immunology*, **2008**, 19, 564-8	4.2	4
55	Innate-like effector differentiation of human invariant NKT cells driven by IL-7. *Journal of Immunology*, **2008**, 180, 4415-24	5.3	25
54	Altered intracellular and extracellular signaling leads to impaired T-cell functions in ADA-SCID patients. *Blood*, **2008**, 111, 4209-19	2.2	57
53	Molecular purging of multiple myeloma cells by ex-vivo culture and retroviral transduction of mobilized-blood CD34+ cells. *Journal of Translational Medicine*, **2007**, 5, 35	8.5	7
52	Lentiviral vectors targeting WASp expression to hematopoietic cells, efficiently transduce and correct cells from WAS patients. *Gene Therapy*, **2007**, 14, 415-28	4	90
51	Management options for adenosine deaminase deficiency; proceedings of the EBMT satellite workshop (Hamburg, March 2006). *Clinical Immunology*, **2007**, 123, 139-47	9	75
50	Burkitt's lymphoma in a patient with adenosine deaminase deficiency-severe combined immunodeficiency treated with polyethylene glycol-adenosine deaminase. *Journal of Pediatrics*, **2007**, 151, 93-5	3.6	26
49	WASP regulates suppressor activity of human and murine CD4(+)CD25(+)FOXP3(+) natural regulatory T cells. *Journal of Experimental Medicine*, **2007**, 204, 369-80	16.6	149
48	Current understanding of the Wiskott-Aldrich syndrome and prospects for gene therapy. *Expert Review of Clinical Immunology*, **2007**, 3, 205-15	5.1	
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