Alessandro Aiuti

List of Publications by Citations

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	A highly efficacious lymphocyte chemoattractant, stromal cell-derived factor 1 (SDF-1). *Journal of Experimental Medicine*, **1996**, 184, 1101-9	16.6	1265
216	The chemokine SDF-1 is a chemoattractant for human CD34+ hematopoietic progenitor cells and provides a new mechanism to explain the mobilization of CD34+ progenitors to peripheral blood. *Journal of Experimental Medicine*, **1997**, 185, 111-20	16.6	1204
215	Autoantibodies against type I IFNs in patients with life-threatening COVID-19. *Science*, **2020**, 370,	33.3	1090
214	Correction of ADA-SCID by stem cell gene therapy combined with nonmyeloablative conditioning. *Science*, **2002**, 296, 2410-3	33.3	947
213	Lentiviral hematopoietic stem cell gene therapy benefits metachromatic leukodystrophy. *Science*, **2013**, 341, 1233158	33.3	837
212	Gene therapy for immunodeficiency due to adenosine deaminase deficiency. *New England Journal of Medicine*, **2009**, 360, 447-58	59.2	792
211	Lentiviral hematopoietic stem cell gene therapy in patients with Wiskott-Aldrich syndrome. *Science*, **2013**, 341, 1233151	33.3	755
210	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
209	Hot spots of retroviral integration in human CD34+ hematopoietic cells. *Blood*, **2007**, 110, 1770-8	2.2	211
208	Multilineage hematopoietic reconstitution without clonal selection in ADA-SCID patients treated with stem cell gene therapy. *Journal of Clinical Investigation*, **2007**, 117, 2233-40	15.9	203
207	A map of human circular RNAs in clinically relevant tissues. *Journal of Molecular Medicine*, **2017**, 95, 1179-1189	5.1	195
206	Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. *Blood*, **2009**, 113, 6288-95	2.2	184
205	Immune reconstitution in ADA-SCID after PBL gene therapy and discontinuation of enzyme replacement. *Nature Medicine*, **2002**, 8, 423-5	50.5	173
204	How I treat ADA deficiency. *Blood*, **2009**, 114, 3524-32	2.2	168
203	Primary immunodeficiency diseases: Genomic approaches delineate heterogeneous Mendelian disorders. *Journal of Allergy and Clinical Immunology*, **2017**, 139, 232-245	11.5	164
202	Wiskott-Aldrich syndrome protein regulates lipid raft dynamics during immunological synapse formation. *Immunity*, **2002**, 17, 157-66	32.3	158
201	Expression of CXCR4, the receptor for stromal cell-derived factor-1 on fetal and adult human lympho-hematopoietic progenitors. *European Journal of Immunology*, **1999**, 29, 1823-31	6.1	158

200	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
199	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
198	Safety of retroviral gene marking with a truncated NGF receptor. *Nature Medicine*, **2003**, 9, 367-9	50.5	149
197	SAP controls the cytolytic activity of CD8+ T cells against EBV-infected cells. *Blood*, **2005**, 105, 4383-9	2.2	145
196	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
195	Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity. *Clinical Immunology*, **2013**, 146, 248-61	9	141
194	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
193	Comprehensive genomic access to vector integration in clinical gene therapy. *Nature Medicine*, **2009**, 15, 1431-6	50.5	135
192	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
191	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
190	Outcome of hematopoietic stem cell transplantation for adenosine deaminase-deficient severe combined immunodeficiency. *Blood*, **2012**, 120, 3615-24; quiz 3626	2.2	126
189	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
188	Human CD34+ Cells Express CXCR4 and Its Ligand Stromal Cell Derived Factor-1. Implications for Infection by T-Cell Tropic Human Immunodeficiency Virus. *Blood*, **1999**, 94, 62-73	2.2	114
187	A Global Effort to Define the Human Genetics of Protective Immunity to SARS-CoV-2 Infection. *Cell*, **2020**, 181, 1194-1199	56.2	113
186	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent thalassemia. *Nature Medicine*, **2019**, 25, 234-241	50.5	110
185	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
184	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
183	Lentiviral vector-mediated gene transfer in T cells from Wiskott-Aldrich syndrome patients leads to functional correction. *Molecular Therapy*, **2004**, 10, 903-15	11.7	92

182	Retroviral integrations in gene therapy trials. *Molecular Therapy*, **2012**, 20, 709-16	11.7	91
181	Defective Th1 cytokine gene transcription in CD4+ and CD8+ T cells from Wiskott-Aldrich syndrome patients. *Journal of Immunology*, **2006**, 177, 7451-61	5.3	91
180	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
179	Advances in stem cell research and therapeutic development. *Nature Cell Biology*, **2019**, 21, 801-811	23.4	90
178	Lentiviral vectors targeting WASp expression to hematopoietic cells, efficiently transduce and correct cells from WAS patients. *Gene Therapy*, **2007**, 14, 415-28	4	90
177	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
176	Clinical features and follow-up in patients with 22q11.2 deletion syndrome. *Journal of Pediatrics*, **2014**, 164, 1475-80.e2	3.6	83
175	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
174	Autoimmune dysregulation and purine metabolism in adenosine deaminase deficiency. *Frontiers in Immunology*, **2012**, 3, 265	8.4	81
173	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
172	Management options for adenosine deaminase deficiency; proceedings of the EBMT satellite workshop (Hamburg, March 2006). *Clinical Immunology*, **2007**, 123, 139-47	9	75
171	Efficacy of gene therapy for Wiskott-Aldrich syndrome using a WAS promoter/cDNA-containing lentiviral vector and nonlethal irradiation. *Human Gene Therapy*, **2006**, 17, 303-13	4.8	75
170	Skewed T-cell receptor repertoire, decreased thymic output, and predominance of terminally differentiated T cells in ataxia telangiectasia. *Blood*, **2002**, 100, 4082-9	2.2	74
169	Gene therapy for primary immunodeficiencies: Part 1. *Current Opinion in Immunology*, **2012**, 24, 580-4	7.8	73
168	A novel disorder involving dyshematopoiesis, inflammation, and HLH due to aberrant CDC42 function. *Journal of Experimental Medicine*, **2019**, 216, 2778-2799	16.6	71
167	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
166	Ten years of gene therapy for primary immune deficiencies. *Hematology American Society of Hematology Education Program*, **2009**, 682-9	3.1	70
165	Ex vivo gene therapy with lentiviral vectors rescues adenosine deaminase (ADA)-deficient mice and corrects their immune and metabolic defects. *Blood*, **2006**, 108, 2979-88	2.2	69

164	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
163	X-linked recessive TLR7 deficiency in ~1% of men under 60 years old with life-threatening COVID-19. *Science Immunology*, **2021**, 6,	28	67
162	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
161	Clinical applications of gene therapy for primary immunodeficiencies. *Human Gene Therapy*, **2015**, 26, 210-9	4.8	65
160	Tracking genetically engineered lymphocytes long-term reveals the dynamics of T cell immunological memory. *Science Translational Medicine*, **2015**, 7, 317ra198	17.5	65
159	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
158	Wiskott-Aldrich Syndrome protein deficiency perturbs the homeostasis of B-cell compartment in humans. *Journal of Autoimmunity*, **2014**, 50, 42-50	15.5	63
157	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
156	Dynamics of genetically engineered hematopoietic stem and progenitor cells after autologous transplantation in humans. *Nature Medicine*, **2018**, 24, 1683-1690	50.5	62
155	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
154	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
153	T-cell defects in patients with germline mutations account for combined immunodeficiency. *Blood*, **2018**, 132, 2362-2374	2.2	59
152	Altered intracellular and extracellular signaling leads to impaired T-cell functions in ADA-SCID patients. *Blood*, **2008**, 111, 4209-19	2.2	57
151	Transcriptional Targeting of Retroviral Vectors to the Erythroblastic Progeny of Transduced Hematopoietic Stem Cells. *Blood*, **1999**, 93, 3276-3285	2.2	57
150	Developmental expression of the T-box transcription factor T-bet/Tbx21 during mouse embryogenesis. *Mechanisms of Development*, **2002**, 116, 157-60	1.7	56
149	Assessment of thymic output in common variable immunodeficiency patients by evaluation of T cell receptor excision circles. *Clinical and Experimental Immunology*, **2002**, 129, 346-53	6.2	55
148	Gene therapy for primary immunodeficiencies: Part 2. *Current Opinion in Immunology*, **2012**, 24, 585-91	7.8	51
147	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

146	Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatosis. *Molecular Therapy*, **2014**, 22, 1472-1483	11.7	50
145	The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. *Journal of Experimental Medicine*, **2009**, 206, 735-42	16.6	48
144	Cell-surface marking of CD(34+)-restricted phenotypes of human hematopoietic progenitor cells by retrovirus-mediated gene transfer. *Human Gene Therapy*, **1997**, 8, 1611-23	4.8	48
143	Biased T-cell receptor repertoires in patients with chromosome 22q11.2 deletion syndrome (DiGeorge syndrome/velocardiofacial syndrome). *Clinical and Experimental Immunology*, **2003**, 132, 323-31	6.2	47
142	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
141	Bone marrow clonogenic capability, cytokine production, and thymic output in patients with common variable immunodeficiency. *Journal of Immunology*, **2005**, 174, 5074-81	5.3	46
140	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
139	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
138	Defective B cell tolerance in adenosine deaminase deficiency is corrected by gene therapy. *Journal of Clinical Investigation*, **2012**, 122, 2141-52	15.9	45
137	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
136	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
135	T-cell suicide gene therapy prompts thymic renewal in adults after hematopoietic stem cell transplantation. *Blood*, **2012**, 120, 1820-30	2.2	43
134	Gene therapy for adenosine deaminase deficiency. *Current Opinion in Allergy and Clinical Immunology*, **2003**, 3, 461-6	3.3	41
133	Gene therapy using haematopoietic stem and progenitor cells. *Nature Reviews Genetics*, **2021**, 22, 216-234	30.1	39
132	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
131	Recovery of hematopoietic activity in bone marrow from human immunodeficiency virus type 1-infected patients during highly active antiretroviral therapy. *AIDS Research and Human Retroviruses*, **2000**, 16, 1471-9	1.6	38
130	Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. *Journal of Clinical Investigation*, **2015**, 125, 3941-51	15.9	37
129	Gene Therapy for Adenosine Deaminase Deficiency: A Comprehensive Evaluation of Short- and Medium-Term Safety. *Molecular Therapy*, **2018**, 26, 917-931	11.7	35

128	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
127	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. *Frontiers in Pediatrics*, **2019**, 7, 443	3.4	34
126	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
125	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
124	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
123	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
122	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
121	The quality of life of children and adolescents with X-linked agammaglobulinemia. *Journal of Clinical Immunology*, **2009**, 29, 501-7	5.7	30
120	Hematopoietic support and cytokine expression of murine-stable hepatocyte cell lines (MMH). *Hepatology*, **1998**, 28, 1645-54	11.2	30
119	Capillary electrophoresis in diagnosis and monitoring of adenosine deaminase deficiency. *Clinical Chemistry*, **2003**, 49, 1830-8	5.5	30
118	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
117	Hematopoietic stem cell gene therapy for adenosine deaminase deficient-SCID. *Immunologic Research*, **2009**, 44, 150-9	4.3	29
116	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
115	Immunodysregulation of HIV disease at bone marrow level. *Autoimmunity Reviews*, **2005**, 4, 486-90	13.6	28
114	The Role of Conditioning in Hematopoietic Stem-Cell Gene Therapy. *Human Gene Therapy*, **2016**, 27, 741-748	4.8	27
113	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
112	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
111	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

110	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
109	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
108	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
107	Innate-like effector differentiation of human invariant NKT cells driven by IL-7. *Journal of Immunology*, **2008**, 180, 4415-24	5.3	25
106	Induction of CD4+ T cell depletion in mice doubly transgenic for HIV gp120 and human CD4. *European Journal of Immunology*, **1997**, 27, 1319-24	6.1	24
105	Bone marrow stromal cells from Thalassemia patients have impaired hematopoietic supportive capacity. *Journal of Clinical Investigation*, **2019**, 129, 1566-1580	15.9	24
104	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
103	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
102	Human genetic and immunological determinants of critical COVID-19 pneumonia.. *Nature*, **2022**,	50.4	23
101	Targeted NGS Platforms for Genetic Screening and Gene Discovery in Primary Immunodeficiencies. *Frontiers in Immunology*, **2019**, 10, 316	8.4	22
100	ALPS-Like Phenotype Caused by ADA2 Deficiency Rescued by Allogeneic Hematopoietic Stem Cell Transplantation. *Frontiers in Immunology*, **2018**, 9, 2767	8.4	22
99	Progress in gene therapy for primary immunodeficiencies using lentiviral vectors. *Current Opinion in Allergy and Clinical Immunology*, **2014**, 14, 527-34	3.3	22
98	Control of human coagulation by recombinant serine proteases. Blood clotting is activated by recombinant factor XII deleted of five regulatory domains. *FEBS Journal*, **1992**, 208, 23-30		20
97	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
96	Long-Term Outcome of Adenosine Deaminase-Deficient Patients-a Single-Center Experience. *Journal of Clinical Immunology*, **2017**, 37, 582-591	5.7	19
95	Pioglitazone as a novel therapeutic approach in chronic granulomatous disease. *Journal of Allergy and Clinical Immunology*, **2016**, 137, 1913-1915.e2	11.5	19
94	Gene therapy for mucopolysaccharidoses: in vivo and ex vivo approaches. *Italian Journal of Pediatrics*, **2018**, 44, 130	3.2	19
93	Improvement of interleukin 2 production, clonogenic capability and restoration of stromal cell function in human immunodeficiency virus-type-1 patients after highly active antiretroviral therapy. *British Journal of Haematology*, **2002**, 118, 864-74	4.5	18

92	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
91	In vivo dynamics of human hematopoietic stem cells: novel concepts and future directions. *Blood Advances*, **2019**, 3, 1916-1924	7.8	17
90	Advances in gene therapy for ADA-deficient SCID. *Current Opinion in Molecular Therapeutics*, **2002**, 4, 515-22		17
89	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
88	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
87	Lentiviral vectors for the treatment of primary immunodeficiencies. *Journal of Inherited Metabolic Disease*, **2014**, 37, 525-33	5.4	15
86	Autoimmunity and regulatory T cells in 22q11.2 deletion syndrome patients. *Pediatric Allergy and Immunology*, **2015**, 26, 591-4	4.2	14
85	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
84	Human CD26 expression in transgenic mice affects murine T-cell populations and modifies their subset distribution. *Human Immunology*, **2002**, 63, 719-30	2.3	14
83	Serratia marcescens osteomyelitis in a newborn with chronic granulomatous disease. *Pediatric Infectious Disease Journal*, **2013**, 32, 926	3.4	14
82	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
81	Safer conditioning for blood stem cell transplants. *Nature Biotechnology*, **2016**, 34, 721-3	44.5	13
80	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
79	Decreased apoptosis of bone marrow progenitor cells in HIV-1-infected patients during highly active antiretroviral therapy. *Aids*, **2004**, 18, 1335-7	3.5	13
78	IL-3 or IL-7 increases ex vivo gene transfer efficiency in ADA-SCID BM CD34+ cells while maintaining in vivo lymphoid potential. *Molecular Therapy*, **2004**, 10, 1096-108	11.7	13
77	Optimisation of retroviral supernatant production conditions for the genetic modification of human CD34+ cells. *Journal of Gene Medicine*, **2001**, 3, 219-27	3.5	13
76	Gene therapy for Wiskott-Aldrich Syndrome. *Current Gene Therapy*, **2014**, 14, 413-21	4.3	13
75	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

74	Mobilized blood CD34+ cells transduced and selected with a clinically applicable protocol reconstitute lymphopoiesis in SCID-Hu mice. *Human Gene Therapy*, **2004**, 15, 305-11	4.8	12
73	Interleukin 7 production by bone marrow-derived stromal cells in HIV-1-infected patients during highly active antiretroviral therapy. *Aids*, **2002**, 16, 2231-2	3.5	12
72	Lack of evidence for a superantigen in lymphocytes from HIV-discordant monozygotic twins. *Aids*, **1994**, 8, 443-9	3.5	12
71	Update on Clinical Ex Vivo Hematopoietic Stem Cell Gene Therapy for Inherited Monogenic Diseases. *Molecular Therapy*, **2021**, 29, 489-504	11.7	12
70	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
69	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
68	A novel genomic inversion in Wiskott-Aldrich-associated autoinflammation. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 619-622.e7	11.5	11
67	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
66	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
65	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
64	Gene therapy for adenosine deaminase deficiency. *Immunology and Allergy Clinics of North America*, **2010**, 30, 249-60	3.3	11
63	Gene therapy for adenosine-deaminase-deficient severe combined immunodeficiency. *Best Practice and Research in Clinical Haematology*, **2004**, 17, 505-16	4.2	11
62	Membrane expression of HLA-Cw4 free chains in activated T cells of transgenic mice. *Immunogenetics*, **1995**, 42, 368-75	3.2	11
61	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
60	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
59	Expanded circulating hematopoietic stem/progenitor cells as novel cell source for the treatment of TCIRG1 osteopetrosis. *Haematologica*, **2021**, 106, 74-86	6.6	10
58	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
57	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

56	HIV type 1 protease inhibitors enhance bone marrow progenitor cell activity in normal subjects and in HIV type 1-infected patients. *AIDS Research and Human Retroviruses*, **2005**, 21, 51-7	1.6	10
55	Bone marrow-derived CD34 fraction: A rich source of mesenchymal stromal cells for clinical application. *Cytotherapy*, **2016**, 18, 1560-1563	4.8	9
54	Recovery of haematopoietic abnormalities in HIV-1 infected patients treated with HAART. *Aids*, **1999**, 13, 2486-8	3.5	9
53	Emapalumab treatment in an ADA-SCID patient with refractory hemophagocytic lymphohistiocytosis-related graft failure and disseminated bacillus Calmette-Guﬁn infection. *Haematologica*, **2021**, 106, 641-646	6.6	9
52	Leukocyte and Dried Blood Spot Arylsulfatase A Assay by Tandem Mass Spectrometry. *Analytical Chemistry*, **2020**, 92, 6341-6348	7.8	8
51	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
50	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
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