

# Fabio Candotti

## List of Publications by Year in descending order

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182  
papers

10,786  
citations

38720

50  
h-index

33869

99  
g-index

210  
all docs

210  
docs citations

210  
times ranked

12095  
citing authors

#	ARTICLE	IF	CITATIONS
1	Activated STING in a Vascular and Pulmonary Syndrome. <i>New England Journal of Medicine</i> , 2014, 371, 507-518.	13.9	1,074
2	Mutations of Jak-3 gene in patients with autosomal severe combined immune deficiency (SCID). <i>Nature</i> , 1995, 377, 65-68.	13.7	864
3	Actionable Diagnosis of Neuroleptospirosis by Next-Generation Sequencing. <i>New England Journal of Medicine</i> , 2014, 370, 2408-2417.	13.9	760
4	Early-Onset Stroke and Vasculopathy Associated with Mutations in ADA2. <i>New England Journal of Medicine</i> , 2014, 370, 911-920.	13.9	687
5	Engraftment Potential of Human Amnion and Chorion Cells Derived from Term Placenta. <i>Transplantation</i> , 2004, 78, 1439-1448.	0.5	318
6	The altered landscape of the human skin microbiome in patients with primary immunodeficiencies. <i>Genome Research</i> , 2013, 23, 2103-2114.	2.4	236
7	Gene therapy for adenosine deaminase-deficient severe combined immune deficiency: clinical comparison of retroviral vectors and treatment plans. <i>Blood</i> , 2012, 120, 3635-3646.	0.6	222
8	How I treat ADA deficiency. <i>Blood</i> , 2009, 114, 3524-3532.	0.6	206
9	Persistence and expression of the adenosine deaminase gene for 12 years and immune reaction to gene transfer components: long-term results of the first clinical gene therapy trial. <i>Blood</i> , 2003, 101, 2563-2569.	0.6	203
10	Human adenylate kinase 2 deficiency causes a profound hematopoietic defect associated with sensorineural deafness. <i>Nature Genetics</i> , 2009, 41, 106-111.	9.4	198
11	Immune Response to Fetal Calf Serum by Two Adenosine Deaminase-Deficient Patients After T Cell Gene Therapy. <i>Human Gene Therapy</i> , 2002, 13, 1605-1610.	1.4	162
12	Clinical Manifestations and Pathophysiological Mechanisms of the Wiskott-Aldrich Syndrome. <i>Journal of Clinical Immunology</i> , 2018, 38, 13-27.	2.0	156
13	American society of gene therapy (ASGT) ad hoc subcommittee on retroviral-mediated gene transfer to hematopoietic stem cells. <i>Molecular Therapy</i> , 2003, 8, 180-187.	3.7	147
14	X-SCID transgene leukaemogenicity. <i>Nature</i> , 2006, 443, E5-E6.	13.7	144
15	Structural and Functional Basis for JAK3-Deficient Severe Combined Immunodeficiency. <i>Blood</i> , 1997, 90, 3996-4003.	0.6	138
16	Transfer of the HSV-tk Gene into Donor Peripheral Blood Lymphocytes for In Vivo Modulation of Donor Anti-Tumor Immunity after Allogeneic Bone Marrow Transplantation. The San Raffaele Hospital, Milan, Italy. <i>Human Gene Therapy</i> , 1995, 6, 813-819.	1.4	137
17	Somatic mosaicism in Wiskott-Aldrich syndrome suggests in vivo reversion by a DNA slippage mechanism. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2001, 98, 8697-8702.	3.3	137
18	Unexpected Effects of FERM Domain Mutations on Catalytic Activity of Jak3. <i>Molecular Cell</i> , 2001, 8, 959-969.	4.5	127

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19	Jak3, severe combined immunodeficiency, and a new class of immunosuppressive drugs. <i>Immunological Reviews</i> , 2005, 203, 127-142.	2.8	126
20	Complex Effects of Naturally Occurring Mutations in the JAK3 Pseudokinase Domain: Evidence for Interactions between the Kinase and Pseudokinase Domains. <i>Molecular and Cellular Biology</i> , 2000, 20, 947-956.	1.1	125
21	Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. <i>New England Journal of Medicine</i> , 2021, 384, 2002-2013.	13.9	122
22	Signaling via IL-2 and IL-4 in JAK3-Deficient Severe Combined Immunodeficiency Lymphocytes: JAK3-Dependent and Independent Pathways. <i>Immunity</i> , 1996, 5, 605-615.	6.6	120
23	Improving cellular therapy for primary immune deficiency diseases: Recognition, diagnosis, and management. <i>Journal of Allergy and Clinical Immunology</i> , 2009, 124, 1152-1160.e12.	1.5	110
24	Jak3 and the pathogenesis of severe combined immunodeficiency. <i>Molecular Immunology</i> , 2004, 41, 727-737.	1.0	109
25	Nuclear Role of WASp in the Pathogenesis of Dysregulated T <sub>H</sub> 1 Immunity in Human Wiskott-Aldrich Syndrome. <i>Science Translational Medicine</i> , 2010, 2, 37ra44.	5.8	109
26	Type I interferonopathies in pediatric rheumatology. <i>Pediatric Rheumatology</i> , 2016, 14, 35.	0.9	104
27	A novel form of complete IL-12/IL-23 receptor Å1 deficiency with cell surface-expressed nonfunctional receptors. <i>Blood</i> , 2004, 104, 2095-2101.	0.6	103
28	B cellâ€intrinsic deficiency of the Wiskott-Aldrich syndrome protein (WASp) causes severe abnormalities of the peripheral B-cell compartment in mice. <i>Blood</i> , 2012, 119, 2819-2828.	0.6	99
29	Of genes and phenotypes: the immunological and molecular spectrum of combined immune deficiency. Defects of the gc-JAK3 signaling pathway as a model. <i>Immunological Reviews</i> , 2000, 178, 39-48.	2.8	97
30	Impaired in vitro regulatory T cell function associated with Wiskottâ€Aldrich syndrome. <i>Clinical Immunology</i> , 2007, 124, 41-48.	1.4	95
31	Autoimmunity in Wiskott-Aldrich syndrome. <i>Current Opinion in Rheumatology</i> , 2003, 15, 446-453.	2.0	89
32	Gene Therapy Fulfilling Its Promise. <i>New England Journal of Medicine</i> , 2009, 360, 518-521.	13.9	88
33	Efficacy and Adverse Events During Janus Kinase Inhibitor Treatment of SAVI Syndrome. <i>Journal of Clinical Immunology</i> , 2019, 39, 476-485.	2.0	85
34	Cytokines and their role in lymphoid development, differentiation and homeostasis. <i>Current Opinion in Allergy and Clinical Immunology</i> , 2002, 2, 495-506.	1.1	81
35	Efficient Gene Transfer to Human Peripheral Blood Monocyte-Derived Dendritic Cells Using Human Immunodeficiency Virus Type 1-Based Lentiviral Vectors. <i>Human Gene Therapy</i> , 2000, 11, 1901-1909.	1.4	80
36	In vitro correction of JAK3-deficient severe combined immunodeficiency by retroviral-mediated gene transduction.. <i>Journal of Experimental Medicine</i> , 1996, 183, 2687-2692.	4.2	71

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37	Clinical efficacy of gene-modified stem cells in adenosine deaminase-deficient immunodeficiency. <i>Journal of Clinical Investigation</i> , 2017, 127, 1689-1699.	3.9	70
38	Second-site mutation in the Wiskott-Aldrich syndrome (WAS) protein gene causes somatic mosaicism in two WAS siblings. <i>Journal of Clinical Investigation</i> , 2003, 111, 1389-1397.	3.9	69
39	Altered BCR and TLR signals promote enhanced positive selection of autoreactive transitional B cells in Wiskott-Aldrich syndrome. <i>Journal of Experimental Medicine</i> , 2015, 212, 1663-1677.	4.2	67
40	NBAS mutations cause a multisystem disorder involving bone, connective tissue, liver, immune system, and retina. <i>American Journal of Medical Genetics, Part A</i> , 2015, 167, 2902-2912.	0.7	66
41	Individualized Iterative Phenotyping for Genome-wide Analysis of Loss-of-Function Mutations. <i>American Journal of Human Genetics</i> , 2015, 96, 913-925.	2.6	66
42	Primary Immune Deficiency Treatment Consortium (PIDTC) report. <i>Journal of Allergy and Clinical Immunology</i> , 2014, 133, 335-347.e11.	1.5	65
43	Multicentric dermatofibrosarcoma protuberans in patients with adenosine deaminase-deficient severe combined immune deficiency. <i>Journal of Allergy and Clinical Immunology</i> , 2012, 129, 762-769.e1.	1.5	64
44	Unexpected and variable phenotypes in a family with JAK3 deficiency. <i>Genes and Immunity</i> , 2001, 2, 422-432.	2.2	63
45	Lymphoid Development and Function in X-Linked Severe Combined Immunodeficiency Mice after Stem Cell Gene Therapy. <i>Molecular Therapy</i> , 2000, 1, 145-153.	3.7	59
46	Efficient Methods for Targeted Mutagenesis in Zebrafish Using Zinc-Finger Nucleases: Data from Targeting of Nine Genes Using CompoZr or CoDA ZFNs. <i>PLoS ONE</i> , 2013, 8, e57239.	1.1	58
47	Nuclear Role of WASp in Gene Transcription Is Uncoupled from Its ARP2/3-Dependent Cytoplasmic Role in Actin Polymerization. <i>Journal of Immunology</i> , 2014, 193, 150-160.	0.4	57
48	Somatic mosaicism in primary immune deficiencies. <i>Current Opinion in Allergy and Clinical Immunology</i> , 2008, 8, 510-514.	1.1	56
49	FOXP3+ Tregs require WASP to restrain Th2-mediated food allergy. <i>Journal of Clinical Investigation</i> , 2016, 126, 4030-4044.	3.9	53
50	Retrovirus-Mediated WASP Gene Transfer Corrects Wiskott-Aldrich Syndrome T-Cell Dysfunction. <i>Human Gene Therapy</i> , 2002, 13, 1039-1046.	1.4	52
51	Bovine apolipoprotein B-100 is a dominant immunogen in therapeutic cell populations cultured in fetal calf serum in mice and humans. <i>Blood</i> , 2007, 110, 501-508.	0.6	51
52	Differential contribution of Wiskott-Aldrich syndrome protein to selective advantage in T- and B-cell lineages. <i>Blood</i> , 2004, 103, 676-678.	0.6	50
53	Reticular dysgenesis-associated AK2 protects hematopoietic stem and progenitor cell development from oxidative stress. <i>Journal of Experimental Medicine</i> , 2015, 212, 1185-1202.	4.2	49
54	Systemic autoimmunity and defective Fas ligand secretion in the absence of the Wiskott-Aldrich syndrome protein. <i>Blood</i> , 2010, 116, 740-747.	0.6	48

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55	Revertant somatic mosaicism in the Wiskott-Aldrich syndrome. <i>Immunologic Research</i> , 2009, 44, 127-131.	1.3	47
56	Advances in the understanding of cytokine signal transduction: the role of Jaks and STATs in immunoregulation and the pathogenesis of immunodeficiency. <i>Journal of Clinical Immunology</i> , 1997, 17, 431-447.	2.0	45
57	Myeloid dysplasia and bone marrow hypocellularity in adenosine deaminase-deficient severe combined immune deficiency. <i>Blood</i> , 2011, 118, 2688-2694.	0.6	45
58	Hematopoietic Cell Transplantation Cures Adenosine Deaminase 2 Deficiency: Report on 30 Patients. <i>Journal of Clinical Immunology</i> , 2021, 41, 1633-1647.	2.0	43
59	Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. <i>Journal of Clinical Investigation</i> , 2015, 125, 3941-3951.	3.9	43
60	Development of Autologous T Lymphocytes in Two Males with X-Linked Severe Combined Immune Deficiency: Molecular and Cellular Characterization. <i>Clinical Immunology</i> , 2000, 95, 39-50.	1.4	42
61	Expansion of Hepatic and Hematopoietic Stem Cells Utilizing Mouse Embryonic Liver Explants. <i>Cell Transplantation</i> , 2001, 10, 81-89.	1.2	41
62	Retrovirus-mediated WASP gene transfer corrects defective actin polymerization in B cell lines from Wiskott-Aldrich syndrome patients carrying $\beta$ -null mutations. <i>Gene Therapy</i> , 1999, 6, 1170-1174.	2.3	40
63	CXCL12 Signaling Is Independent of Jak2 and Jak3. <i>Journal of Biological Chemistry</i> , 2005, 280, 17408-17414.	1.6	40
64	Lessons from the Wiskott-Aldrich Syndrome. <i>New England Journal of Medicine</i> , 2006, 355, 1759-1761.	13.9	39
65	Measurement of Proliferative Responses of Cultured Lymphocytes. <i>Current Protocols in Immunology</i> , 2011, 94, Unit7.10.	3.6	39
66	Retroviral Transfer of Acid Glucosidase cDNA to Enzyme-Deficient Myoblasts Results in Phenotypic Spread of the Genotypic Correction by Both Secretion and Fusion. <i>Human Gene Therapy</i> , 1997, 8, 1555-1563.	1.4	38
67	Analysis of T-cell repertoire diversity in Wiskott-Aldrich syndrome. <i>Blood</i> , 2005, 106, 3895-3897.	0.6	38
68	Development of Autologous, Oligoclonal, Poorly Functioning T Lymphocytes in a Patient With Autosomal Recessive Severe Combined Immunodeficiency Caused by Defects of the Jak3 Tyrosine Kinase. <i>Blood</i> , 1998, 91, 949-955.	0.6	37
69	In vivo retroviral gene transfer by direct intrafemoral injection results in correction of the SCID phenotype in Jak3 knock-out animals. <i>Blood</i> , 2003, 102, 843-848.	0.6	37
70	Prolonged pancytopenia in a gene therapy patient with ADA-deficient SCID and trisomy 8 mosaicism: a case report. <i>Blood</i> , 2007, 109, 503-506.	0.6	36
71	Hierarchy of Protein Tyrosine Kinases in Interleukin-2 (IL-2) Signaling: Activation of Syk Depends on Jak3; However, Neither Syk nor Lck Is Required for IL-2-Mediated STAT Activation. <i>Molecular and Cellular Biology</i> , 2000, 20, 4371-4380.	1.1	35
72	Somatic mosaicism in the Wiskott-Aldrich syndrome: Molecular and functional characterization of genotypic revertants. <i>Clinical Immunology</i> , 2010, 135, 72-83.	1.4	35

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73	The expression of Wiskott-Aldrich syndrome protein (WASP) is dependent on WASP-interacting protein (WIP). <i>International Immunology</i> , 2006, 19, 185-192.	1.8	34
74	Recent advances in gene therapy for severe congenital immunodeficiency diseases. <i>Current Opinion in Hematology</i> , 2008, 15, 375-380.	1.2	34
75	High incidence of lymphomas in a subgroup of wiskott-aldrich syndrome patients. <i>British Journal of Haematology</i> , 2003, 121, 529-530.	1.2	33
76	Multiple patients with revertant mosaicism in a single Wiskott-Aldrich syndrome family. <i>Blood</i> , 2004, 104, 1270-1272.	0.6	32
77	Gene Therapy for Severe Combined Immunodeficiency Caused by Adenosine Deaminase Deficiency: Improved Retroviral Vectors for Clinical Trials. <i>Acta Haematologica</i> , 1999, 101, 89-96.	0.7	30
78	Cartilage hair hypoplasia mutations that lead to <i>RMRP</i> promoter inefficiency or RNA transcript instability. <i>American Journal of Medical Genetics, Part A</i> , 2007, 143A, 2675-2681.	0.7	30
79	Unprecedented diversity of genotypic revertants in lymphocytes of a patient with Wiskott-Aldrich syndrome. <i>Blood</i> , 2008, 111, 5064-5067.	0.6	30
80	Self-inactivating Retroviral Vector-mediated Gene Transfer Induces Oncogene Activation and Immortalization of Primary Murine Bone Marrow Cells. <i>Molecular Therapy</i> , 2009, 17, 1910-1918.	3.7	29
81	Combined Immunodeficiencies Due to Defects in Signal Transduction: Defects of the $\hat{I}^3c$ -JAK3 Signaling Pathway as a Model. <i>Immunobiology</i> , 2000, 202, 106-119.	0.8	28
82	Molecular and phenotypic abnormalities of B lymphocytes in patients with Wiskott-Aldrich syndrome. <i>Journal of Allergy and Clinical Immunology</i> , 2014, 133, 896-899.e4.	1.5	28
83	Long-term outcomes after gene therapy for adenosine deaminase severe combined immune deficiency. <i>Blood</i> , 2021, 138, 1304-1316.	0.6	28
84	Cytoreductive conditioning intensity predicts clonal diversity in ADA-SCID retroviral gene therapy patients. <i>Blood</i> , 2017, 129, 2624-2635.	0.6	27
85	Transcriptomic Signature Differences Between SARS-CoV-2 and Influenza Virus Infected Patients. <i>Frontiers in Immunology</i> , 2021, 12, 666163.	2.2	27
86	Interleukin-4 Signaling in B Lymphocytes from Patients with X-linked Severe Combined Immunodeficiency. <i>Journal of Biological Chemistry</i> , 1997, 272, 7314-7319.	1.6	26
87	Outcomes in Two Japanese Adenosine Deaminase-Deficiency Patients Treated by Stem Cell Gene Therapy with No Cytoreductive Conditioning. <i>Journal of Clinical Immunology</i> , 2015, 35, 384-398.	2.0	25
88	Broad spectrum of autoantibodies in patients with Wiskott-Aldrich syndrome and X-linked thrombocytopenia. <i>Journal of Allergy and Clinical Immunology</i> , 2015, 136, 1401-1404.e3.	1.5	25
89	SLAM-associated Protein Deficiency Causes Imbalanced Early Signal Transduction and Blocks Downstream Activation in T Cells from X-linked Lymphoproliferative Disease Patients. <i>Journal of Biological Chemistry</i> , 2003, 278, 29593-29599.	1.6	24
90	Foamy Virus Vector-mediated Gene Correction of a Mouse Model of Wiskott-Aldrich Syndrome. <i>Molecular Therapy</i> , 2012, 20, 1270-1279.	3.7	24

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91	Gene transfer into hematopoietic stem cells as treatment for primary immunodeficiency diseases. <i>International Journal of Hematology</i> , 2014, 99, 383-392.	0.7	24
92	N-WASP is required for B-cell-mediated autoimmunity in Wiskott-Aldrich syndrome. <i>Blood</i> , 2016, 127, 216-220.	0.6	24
93	Molecular aspects of primary immunodeficiencies: lessons from cytokine and other signaling pathways. <i>Journal of Clinical Investigation</i> , 2002, 109, 1261-1269.	3.9	24
94	Molecular Modeling of the Jak3 Kinase Domains and Structural Basis for Severe Combined Immunodeficiency. <i>Clinical Immunology</i> , 2000, 96, 108-118.	1.4	23
95	Reconstitution of lymphoid development and function in ZAP-70-deficient mice following gene transfer into bone marrow cells. <i>Blood</i> , 2002, 100, 1248-1256.	0.6	23
96	Structure-Function Analysis of the WIP Role in T Cell Receptor-stimulated NFAT Activation. <i>Journal of Biological Chemistry</i> , 2007, 282, 30303-30310.	1.6	22
97	Elevated IgE and atopy in patients treated for early-onset ADA-SCID. <i>Journal of Allergy and Clinical Immunology</i> , 2013, 132, 1444-1446.e5.	1.5	22
98	A Novel Function of RNAs Arising From the Long Terminal Repeat of Human Endogenous Retrovirus 9 in Cell Cycle Arrest. <i>Journal of Virology</i> , 2013, 87, 25-36.	1.5	22
99	Lack of dominant-negative effects of a truncated $\hat{\beta}c$ on retroviral-mediated gene correction of immunodeficient mice. <i>Blood</i> , 2001, 97, 1618-1624.	0.6	21
100	Comparison of Five Retrovirus Vectors Containing the Human IL-2 Receptor $\hat{\beta}3$ Chain Gene for Their Ability to Restore T and B Lymphocytes in the X-Linked Severe Combined Immunodeficiency Mouse Model. <i>Molecular Therapy</i> , 2001, 3, 565-573.	3.7	20
101	Defective inhibition of B-cell proliferation by Wiskott-Aldrich syndrome protein-deficient regulatory T cells. <i>Blood</i> , 2011, 117, 6608-6611.	0.6	20
102	Platelets from WAS patients show an increased susceptibility to <i>ex vivo</i> phagocytosis. <i>Platelets</i> , 2013, 24, 288-296.	1.1	19
103	Adenosine Deaminase (ADA)-Deficient Severe Combined Immune Deficiency (SCID) in the US Immunodeficiency Network (USIDNet) Registry. <i>Journal of Clinical Immunology</i> , 2020, 40, 1124-1131.	2.0	19
104	Severe combined immune deficiencies due to defects of the common $\gamma$ chain-JAK3 signaling pathway. <i>Seminars in Immunopathology</i> , 1998, 19, 401-415.	4.0	18
105	Use of a herpes thymidine kinase/neomycin phosphotransferase chimeric gene for metabolic suicide gene transfer. <i>Cancer Gene Therapy</i> , 2000, 7, 574-580.	2.2	17
106	In Vivo Competitive Studies between Normal and Common $\hat{\beta}3$ Chain-Defective Bone Marrow Cells: Implications for Gene Therapy. <i>Human Gene Therapy</i> , 2000, 11, 2051-2056.	1.4	17
107	Functional Interaction of Common $\hat{\beta}3$ -Chain and Growth Hormone Receptor Signaling Apparatus. <i>Journal of Immunology</i> , 2006, 177, 6889-6895.	0.4	17
108	Development of IgA nephropathy-like glomerulonephritis associated with Wiskott-Aldrich syndrome protein deficiency. <i>Clinical Immunology</i> , 2012, 142, 160-166.	1.4	17

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109	Molecular aspects of primary immunodeficiencies: lessons from cytokine and other signaling pathways. <i>Journal of Clinical Investigation</i> , 2002, 109, 1261-1269.	3.9	16
110	Measurement of Proliferative Responses of Cultured Lymphocytes. <i>Current Protocols in Immunology</i> , 2008, 82, Unit 7.10.1-7.10.24.	3.6	15
111	Assessment of Immature Platelet Fraction in the Diagnosis of Wiskott-Aldrich Syndrome. <i>Frontiers in Pediatrics</i> , 2015, 3, 49.	0.9	15
112	Aberrant glycosylation of IgA in Wiskott-Aldrich syndrome and X-linked thrombocytopenia. <i>Journal of Allergy and Clinical Immunology</i> , 2013, 131, 587-590.e3.	1.5	14
113	Advances of gene therapy for primary immunodeficiencies. <i>F1000Research</i> , 2016, 5, 310.	0.8	14
114	Flow Cytometry Analysis of Adenosine Deaminase (ADA) Expression: A Simple and Reliable Tool for the Assessment of ADA-Deficient Patients Before and After Gene Therapy. <i>Human Gene Therapy</i> , 2002, 13, 425-432.	1.4	13
115	Mosaicism—Switch or Spectrum?. <i>Science</i> , 2010, 330, 46-47.	6.0	13
116	Somatic Mosaicism Caused by Monoallelic Reversion of a Mutation in T Cells of a Patient with ADA-SCID and the Effects of Enzyme Replacement Therapy on the Revertant Phenotype. <i>Scandinavian Journal of Immunology</i> , 2011, 74, 471-481.	1.3	13
117	Gene Therapy in Infants with Severe Combined Immunodeficiency. <i>BioDrugs</i> , 2002, 16, 229-239.	2.2	12
118	Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Results in an Expanded Cohort. <i>Blood</i> , 2019, 134, 3345-3345.	0.6	12
119	THE POTENTIAL FOR THERAPY OF IMMUNE DISORDERS WITH GENE THERAPY. <i>Pediatric Clinics of North America</i> , 2000, 47, 1389-1407.	0.9	11
120	Biosynthetic Ganciclovir Triphosphate: Its Isolation and Characterization from Ganciclovir-Treated Herpes Simplex Thymidine Kinase-Transduced Murine Cells. <i>Biochemical and Biophysical Research Communications</i> , 2001, 289, 525-530.	1.0	11
121	Retroviral-mediated gene transfer restores IL-12 and IL-23 signaling pathways in T cells from IL-12 receptor $\beta$ 2-deficient patients. <i>Molecular Therapy</i> , 2004, 9, 895-901.	3.7	11
122	Immune Responses to Gene-Modified T Cells. <i>Current Gene Therapy</i> , 2007, 7, 361-368.	0.9	11
123	Impulse oscillometry identifies peripheral airway dysfunction in children with adenosine deaminase deficiency. <i>Orphanet Journal of Rare Diseases</i> , 2015, 10, 159.	1.2	10
124	In vitro functional correction of Hermansky-Pudlak Syndrome type-1 by lentiviral-mediated gene transfer. <i>Molecular Genetics and Metabolism</i> , 2015, 114, 62-65.	0.5	10
125	Age-Dependent Defects of Regulatory B Cells in Wiskott-Aldrich Syndrome Gene Knockout Mice. <i>PLoS ONE</i> , 2015, 10, e0139729.	1.1	10
126	Pharmacokinetics and organ distribution of N-methanocarbothymidine, a novel thymidine analog, in mice bearing tumors transduced with the herpes simplex thymidine kinase gene. <i>Cancer Chemotherapy and Pharmacology</i> , 2002, 50, 360-366.	1.1	8



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127	C-8. Immunological and Metabolic Correction After Lentiviral Vector Gene Therapy for ADA Deficiency. <i>Molecular Therapy</i> , 2015, 23, S102-S103.	3.7	8
128	Busulfan Pharmacokinetics in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Gene Therapy. <i>Biology of Blood and Marrow Transplantation</i> , 2020, 26, 1819-1827.	2.0	8
129	Intra-uterine growth restriction induced by maternal low-protein diet causes long-term alterations of thymic structure and function in adult male rat offspring. <i>British Journal of Nutrition</i> , 2020, 123, 892-900.	1.2	8
130	Severe combined immune deficiency. , 2020, , 153-205.		7
131	Novel Discoveries in Immune Dysregulation in Inborn Errors of Immunity. <i>Frontiers in Immunology</i> , 2021, 12, 725587.	2.2	7
132	A convenient method for positive selection of retroviral producing cells generating vectors devoid of selectable markers. <i>Journal of Virological Methods</i> , 2004, 118, 61-67.	1.0	6
133	Gene therapy for Wiskott-Aldrich syndrome: here to stay. <i>Lancet Haematology</i> , the, 2019, 6, e230-e231.	2.2	6
134	Immunodeficiency Due to Defects of Purine Metabolism. , 2013, , 188-230.		6
135	Gene Therapy Model of X-linked Severe Combined Immunodeficiency Using a Modified Foamy Virus Vector. <i>PLoS ONE</i> , 2013, 8, e71594.	1.1	6
136	Platelets From WAS Patients Are More Susceptible Than Controls to Phagocytosis by Activated THP-1 Cells. <i>Blood</i> , 2011, 118, 2222-2222.	0.6	6
137	JAK3-DEFICIENT SEVERE COMBINED IMMUNODEFICIENCY. <i>Immunology and Allergy Clinics of North America</i> , 2000, 20, 97-111.	0.7	5
138	Gene therapy for immunodeficiency. <i>Current Allergy and Asthma Reports</i> , 2001, 1, 407-415.	2.4	5
139	Peptide library-based evaluation of T-cell receptor breadth detects defects in global and regulatory activation in human immunologic diseases. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2013, 110, 8164-8169.	3.3	5
140	Detection of Reactive Oxygen Species Using MitoSOX and CellROX in Zebrafish. <i>Bio-protocol</i> , 2016, 6, .	0.2	5
141	Application of Molecular Analysis to Genetic Counseling in the Wiskott-Aldrich Syndrome (WAS). <i>DNA and Cell Biology</i> , 1993, 12, 645-649.	0.9	4
142	Gene therapy for primary immune deficiencies. <i>Current Opinion in Allergy and Clinical Immunology</i> , 2001, 1, 497-501.	1.1	4
143	A model for reticular dysgenesis shows impaired sensory organ development and hair cell regeneration linked to cellular stress. <i>DMM Disease Models and Mechanisms</i> , 2019, 12, .	1.2	4
144	Immune deficiency, autoimmune disease and intellectual disability: A pleiotropic disorder caused by biallelic variants in the <i>TPP2</i> gene. <i>Clinical Genetics</i> , 2021, 99, 780-788.	1.0	4

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145	Critical role of WASp in germinal center tolerance through regulation of B cell apoptosis and diversification. <i>Cell Reports</i> , 2022, 38, 110474.	2.9	4
146	THE USE OF GENE THERAPY FOR IMMUNODEFICIENCY DISEASE. <i>Immunology and Allergy Clinics of North America</i> , 1996, 16, 683-726.	0.7	3
147	Primary immunodeficiencies and the rheumatologist. <i>Current Opinion in Rheumatology</i> , 2003, 15, 413-416.	2.0	3
148	Gene therapy of primary immunodeficiencies. <i>Seminars in Immunopathology</i> , 1998, 19, 493-508.	4.0	2
149	The long terminal repeat negative control region is a critical element for insertional oncogenesis after gene transfer into hematopoietic progenitors with Moloney murine leukemia viral vectors. <i>Gene Therapy</i> , 2016, 23, 815-818.	2.3	2
150	Signaling networks in B cell development and related therapeutic strategies. <i>Journal of Leukocyte Biology</i> , 2022, 111, 877-891.	1.5	2
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