

# Marina Cavazzana-Calvo

## List of Publications by Year in Descending Order

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The third column is the impact factor (IF) of the journal, and the fourth column is the number of citations of the article.

83  
papers

9,843  
citations

39  
h-index

88  
g-index

88  
ext. papers

11,630  
ext. citations

12.1  
avg, IF

5.34  
L-index

#	Paper	IF	Citations
83	Long-term safety and efficacy of lentiviral hematopoietic stem/progenitor cell gene therapy for Wiskott-Aldrich syndrome.. <i>Nature Medicine</i> , <b>2022</b> ,	50.5	5
82	Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT inborn errors working party analysis.. <i>Blood</i> , <b>2022</b> ,	2.2	3
81	Long-term outcomes of lentiviral gene therapy for the Hemoglobinopathies: the HGB-205 trial.. <i>Nature Medicine</i> , <b>2022</b> ,	50.5	8
80	Transient mTOR inhibition rescues 4-1BB CAR-Tregs from tonic signal-induced dysfunction. <i>Nature Communications</i> , <b>2021</b> , 12, 6446	17.4	2
79	Novel Lentiviral Vectors for Gene Therapy of Sickle Cell Disease Combining Gene Addition and Gene Silencing Strategies. <i>Blood</i> , <b>2021</b> , 138, 3973-3973	2.2	1
78	Hematopoietic cell transplantation in severe combined immunodeficiency: The SCETIDE 2006-2014 European cohort. <i>Journal of Allergy and Clinical Immunology</i> , <b>2021</b> ,	11.5	4
77	A combination of cyclophosphamide and interleukin-2 allows CD4+ T cells converted to Tregs to control scurfy syndrome. <i>Blood</i> , <b>2021</b> , 137, 2326-2336	2.2	2
76	Retrieval of vector integration sites from cell-free DNA. <i>Nature Medicine</i> , <b>2021</b> , 27, 1458-1470	50.5	5
75	A gain-of-function RAC2 mutation is associated with bone-marrow hypoplasia and an autosomal dominant form of severe combined immunodeficiency. <i>Haematologica</i> , <b>2021</b> , 106, 404-411	6.6	7
74	Effect of hydroxyurea exposure before puberty on sperm parameters in males with sickle cell disease. <i>Blood</i> , <b>2021</b> , 137, 826-829	2.2	9
73	Vascular access for optimal hematopoietic stem cell collection. <i>Journal of Clinical Apheresis</i> , <b>2021</b> , 36, 12-19	3.2	4
72	Alternative Promoter Encodes a Functional Munc13-4 Isoform Predominantly Expressed in Lymphocytes and Platelets. <i>Frontiers in Immunology</i> , <b>2020</b> , 11, 1154	8.4	2
71	Donor-targeted serotherapy as a rescue therapy for steroid-resistant acute GVHD after HLA-mismatched kidney transplantation. <i>American Journal of Transplantation</i> , <b>2020</b> , 20, 2243-2253	8.7	7
70	Editing a B globin repressor binding site restores fetal hemoglobin synthesis and corrects the sickle cell disease phenotype. <i>Science Advances</i> , <b>2020</b> , 6,	14.3	49
69	Bone Marrow Transplantation in Congenital Erythropoietic Porphyria: Sustained Efficacy but Unexpected Liver Dysfunction. <i>Biology of Blood and Marrow Transplantation</i> , <b>2020</b> , 26, 704-711	4.7	4
68	Seletalisib for Activated PI3K Syndromes: Open-Label Phase 1b and Extension Studies. <i>Journal of Immunology</i> , <b>2020</b> , 205, 2979-2987	5.3	6
67	Extensive multilineage analysis in patients with mixed chimerism after allogeneic transplantation for sickle cell disease: insight into hematopoiesis and engraftment thresholds for gene therapy. <i>Haematologica</i> , <b>2020</b> , 105, 1240-1247	6.6	11

66	Clonal tracking in gene therapy patients reveals a diversity of human hematopoietic differentiation programs. <i>Blood</i> , <b>2020</b> , 135, 1219-1231	2.2	22
65	Lentiviral and genome-editing strategies for the treatment of Hemoglobinopathies. <i>Blood</i> , <b>2019</b> , 134, 1203-1213	2.2	42
64	Innovative Curative Treatment of Beta Thalassemia: Cost-Efficacy Analysis of Gene Therapy Versus Allogeneic Hematopoietic Stem-Cell Transplantation. <i>Human Gene Therapy</i> , <b>2019</b> , 30, 753-761	4.8	20
63	Hematopoietic stem cell transplantation for CD40 ligand deficiency: Results from an EBMT/ESID-IEWP-SCETIDE-PIDTC study. <i>Journal of Allergy and Clinical Immunology</i> , <b>2019</b> , 143, 2238-2253	11.5	38
62	Risk factors and outcomes according to age at transplantation with an HLA-identical sibling for sickle cell disease. <i>Haematologica</i> , <b>2019</b> , 104, e543-e546	6.6	26
61	Gene therapy targeting haematopoietic stem cells for inherited diseases: progress and challenges. <i>Nature Reviews Drug Discovery</i> , <b>2019</b> , 18, 447-462	64.1	80
60	Gene therapy of hemoglobinopathies: progress and future challenges. <i>Human Molecular Genetics</i> , <b>2019</b> , 28, R24-R30	5.6	33
59	Successful in utero stem cell transplantation in X-linked severe combined immunodeficiency. <i>Blood Advances</i> , <b>2019</b> , 3, 237-241	7.8	5
58	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. <i>Frontiers in Pediatrics</i> , <b>2019</b> , 7, 443	3.4	34
57	Plerixafor enables safe, rapid, efficient mobilization of hematopoietic stem cells in sickle cell disease patients after exchange transfusion. <i>Haematologica</i> , <b>2018</b> , 103, 778-786	6.6	64
56	Gene Therapy in Patients with Transfusion-Dependent $\beta$ Thalassemia. <i>New England Journal of Medicine</i> , <b>2018</b> , 378, 1479-1493	59.2	347
55	Transfusion-related adverse events are decreased in pregnant women with sickle cell disease by a change in policy from systematic transfusion to prophylactic oxygen therapy at home: A retrospective survey by the international sickle cell disease observatory. <i>American Journal of Hematology</i> , <b>2018</b> , 89, 781-800	7.1	5
54	Long-term follow-up of IPEX syndrome patients after different therapeutic strategies: An international multicenter retrospective study. <i>Journal of Allergy and Clinical Immunology</i> , <b>2018</b> , 141, 1036-1049.e5	11.5	157
53	Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated editing of the human $\beta$ globin locus. <i>Blood</i> , <b>2018</b> , 131, 1960-1973	2.2	110
52	Disease Evolution and Response to Rapamycin in Activated Phosphoinositide 3-Kinase $\delta$ Syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase $\delta$ Syndrome Registry. <i>Frontiers in Immunology</i> , <b>2018</b> , 9, 543	8.4	88
51	Analysis of RBC Properties in Patients with SCD Treated with Lentiglobin Gene Therapy. <i>Blood</i> , <b>2018</b> , 132, 2195-2195	2.2	2
50	Generation of adult human T-cell progenitors for immunotherapeutic applications. <i>Journal of Allergy and Clinical Immunology</i> , <b>2018</b> , 141, 1491-1494.e4	11.5	11
49	Gene Therapy for Hemoglobinopathies. <i>Human Gene Therapy</i> , <b>2018</b> , 29, 1106-1113	4.8	25

48	INSPIRED: A Pipeline for Quantitative Analysis of Sites of New DNA Integration in Cellular Genomes. <i>Molecular Therapy - Methods and Clinical Development</i> , <b>2017</b> , 4, 39-49	6.4	42
47	INSPIRED: Quantification and Visualization Tools for Analyzing Integration Site Distributions. <i>Molecular Therapy - Methods and Clinical Development</i> , <b>2017</b> , 4, 17-26	6.4	36
46	Gene Therapy in a Patient with Sickle Cell Disease. <i>New England Journal of Medicine</i> , <b>2017</b> , 376, 848-855	59.2	418
45	Gene Therapy in a Patient with Sickle Cell Disease. <i>New England Journal of Medicine</i> , <b>2017</b> , 376, 2094	59.2	21
44	Gene Therapy for $\beta$ Hemoglobinopathies. <i>Molecular Therapy</i> , <b>2017</b> , 25, 1142-1154	11.7	69
43	Family cord blood banking for sickle cell disease: a twenty-year experience in two dedicated public cord blood banks. <i>Haematologica</i> , <b>2017</b> , 102, 976-983	6.6	6
42	Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation. <i>Blood</i> , <b>2017</b> , 129, 1548-1556	2.2	230
41	Gene Therapy Approaches to Hemoglobinopathies. <i>Hematology/Oncology Clinics of North America</i> , <b>2017</b> , 31, 835-852	3.1	40
40	Arterio-venous fistula for automated red blood cells exchange in patients with sickle cell disease: Complications and outcomes. <i>American Journal of Hematology</i> , <b>2017</b> , 92, 136-140	7.1	15
39	Gene Therapy with Hematopoietic Stem Cells: The Diseased Bone Marrow's Point of View. <i>Stem Cells and Development</i> , <b>2017</b> , 26, 71-76	4.4	17
38	Mammalian target of rapamycin inhibition counterbalances the inflammatory status of immune cells in patients with chronic granulomatous disease. <i>Journal of Allergy and Clinical Immunology</i> , <b>2017</b> , 139, 1641-1649.e6	11.5	21
37	Gene transfer into hematopoietic stem cells reduces HLH manifestations in a murine model of Munc13-4 deficiency. <i>Blood Advances</i> , <b>2017</b> , 1, 2781-2789	7.8	8
36	Cardiac iron overload in chronically transfused patients with thalassemia, sickle cell anemia, or myelodysplastic syndrome. <i>PLoS ONE</i> , <b>2017</b> , 12, e0172147	3.7	29
35	X-linked primary immunodeficiency associated with hemizygous mutations in the moesin (MSN) gene. <i>Journal of Allergy and Clinical Immunology</i> , <b>2016</b> , 138, 1681-1689.e8	11.5	45
34	Specific T cells for the treatment of cytomegalovirus and/or adenovirus in the context of hematopoietic stem cell transplantation. <i>Journal of Allergy and Clinical Immunology</i> , <b>2016</b> , 138, 920-924.e3	11.5	15
33	Clinical and immunologic phenotype associated with activated phosphoinositide 3-kinase $\beta$ syndrome 2: A cohort study. <i>Journal of Allergy and Clinical Immunology</i> , <b>2016</b> , 138, 210-218.e9	11.5	163
32	Gene Therapy for X-Linked Severe Combined Immunodeficiency: Where Do We Stand?. <i>Human Gene Therapy</i> , <b>2016</b> , 27, 108-16	4.8	65
31	Gene Therapy of the $\beta$ Hemoglobinopathies by Lentiviral Transfer of the $\beta$ (A(T87Q))-Globin Gene. <i>Human Gene Therapy</i> , <b>2016</b> , 27, 148-65	4.8	111

30	Hyperinflammation in patients with chronic granulomatous disease leads to impairment of hematopoietic stem cell functions. <i>Journal of Allergy and Clinical Immunology</i> , <b>2016</b> , 138, 219-228.e9	11.5	45
29	Interim Results from a Phase 1/2 Clinical Study of Lentiglobin Gene Therapy for Severe Sickle Cell Disease. <i>Blood</i> , <b>2016</b> , 128, 1176-1176	2.2	30
28	Gene-corrected human Munc13-4-deficient CD8+ T cells can efficiently restrict EBV-driven lymphoproliferation in immunodeficient mice. <i>Blood</i> , <b>2016</b> , 128, 2859-2862	2.2	16
27	Development of Lentiviral Vectors Simultaneously Expressing Multiple siRNAs Against CCR5, vif and tat/rev Genes for an HIV-1 Gene Therapy Approach. <i>Molecular Therapy - Nucleic Acids</i> , <b>2016</b> , 5, e312	10.7	14
26	An in vivo genetic reversion highlights the crucial role of Myb-Like, SWIRM, and MPN domains 1 (MYSM1) in human hematopoiesis and lymphocyte differentiation. <i>Journal of Allergy and Clinical Immunology</i> , <b>2015</b> , 136, 1619-1626.e5	11.5	44
25	Outcomes following gene therapy in patients with severe Wiskott-Aldrich syndrome. <i>JAMA - Journal of the American Medical Association</i> , <b>2015</b> , 313, 1550-63	27.4	245
24	Systematic neonatal screening for severe combined immunodeficiency and severe T-cell lymphopenia: Analysis of cost-effectiveness based on French real field data. <i>Journal of Allergy and Clinical Immunology</i> , <b>2015</b> , 135, 1589-93	11.5	22
23	AK2 deficiency compromises the mitochondrial energy metabolism required for differentiation of human neutrophil and lymphoid lineages. <i>Cell Death and Disease</i> , <b>2015</b> , 6, e1856	9.8	45
22	Early-onset hypogammaglobulinemia: A survey of 44 patients. <i>Journal of Allergy and Clinical Immunology</i> , <b>2015</b> , 136, 1097-9.e2	11.5	4
21	Faster T-cell development following gene therapy compared with haploidentical HSCT in the treatment of SCID-X1. <i>Blood</i> , <b>2015</b> , 125, 3563-9	2.2	52
20	Preclinical evaluation of efficacy and safety of an improved lentiviral vector for the treatment of $\beta$ -thalassemia and sickle cell disease. <i>Current Gene Therapy</i> , <b>2015</b> , 15, 64-81	4.3	78
19	Gene therapy for inherited immunodeficiency. <i>Expert Opinion on Biological Therapy</i> , <b>2014</b> , 14, 789-98	5.4	39
18	A modified $\beta$ retrovirus vector for X-linked severe combined immunodeficiency. <i>New England Journal of Medicine</i> , <b>2014</b> , 371, 1407-17	59.2	278
17	A human immunodeficiency caused by mutations in the PIK3R1 gene. <i>Journal of Clinical Investigation</i> , <b>2014</b> , 124, 3923-8	15.9	166
16	Variable correction of Artemis deficiency by I-Sce1-meganuclease-assisted homologous recombination in murine hematopoietic stem cells. <i>Gene Therapy</i> , <b>2014</b> , 21, 529-32	4	13
15	Stem cell transplantation for primary immunodeficiencies: the European experience. <i>Current Opinion in Allergy and Clinical Immunology</i> , <b>2014</b> , 14, 516-20	3.3	9
14	Characteristics and outcome of early-onset, severe forms of Wiskott-Aldrich syndrome. <i>Blood</i> , <b>2013</b> , 121, 1510-6	2.2	65
13	Human T-lymphoid progenitors generated in a feeder-cell-free Delta-like-4 culture system promote T-cell reconstitution in NOD/SCID/ $\beta$ (-/-) mice. <i>Stem Cells</i> , <b>2012</b> , 30, 1771-80	5.8	50

12	Long-term outcome and lineage-specific chimerism in 194 patients with Wiskott-Aldrich syndrome treated by hematopoietic cell transplantation in the period 1980-2009: an international collaborative study. <i>Blood</i> , <b>2011</b> , 118, 1675-84	2.2	236
11	Transfusion independence and HMGA2 activation after gene therapy of human $\beta$ -thalassaemia. <i>Nature</i> , <b>2010</b> , 467, 318-22	50.4	953
10	Transplantation of hematopoietic stem cells and long-term survival for primary immunodeficiencies in Europe: entering a new century, do we do better?. <i>Journal of Allergy and Clinical Immunology</i> , <b>2010</b> , 126, 602-10.e1-11	11.5	328
9	Efficacy of gene therapy for X-linked severe combined immunodeficiency. <i>New England Journal of Medicine</i> , <b>2010</b> , 363, 355-64	59.2	471
8	A self-inactivating lentiviral vector for SCID-X1 gene therapy that does not activate LMO2 expression in human T cells. <i>Blood</i> , <b>2010</b> , 116, 900-8	2.2	100
7	Hematopoietic stem cell gene therapy with a lentiviral vector in X-linked adrenoleukodystrophy. <i>Science</i> , <b>2009</b> , 326, 818-23	33.3	1153
6	Insertional oncogenesis in 4 patients after retrovirus-mediated gene therapy of SCID-X1. <i>Journal of Clinical Investigation</i> , <b>2008</b> , 118, 3132-42	15.9	1269
5	Long-term outcome following hematopoietic stem-cell transplantation in Wiskott-Aldrich syndrome: collaborative study of the European Society for Immunodeficiencies and European Group for Blood and Marrow Transplantation. <i>Blood</i> , <b>2008</b> , 111, 439-45	2.2	195
4	Failure of SCID-X1 gene therapy in older patients. <i>Blood</i> , <b>2005</b> , 105, 4255-7	2.2	105
3	Autoimmunity in Wiskott-Aldrich syndrome: risk factors, clinical features, and outcome in a single-center cohort of 55 patients. <i>Pediatrics</i> , <b>2003</b> , 111, e622-7	7.4	248
2	Sustained correction of X-linked severe combined immunodeficiency by ex vivo gene therapy. <i>New England Journal of Medicine</i> , <b>2002</b> , 346, 1185-93	59.2	952
1	Comparison of lenograstim vs filgrastim administration following chemotherapy for peripheral blood stem cell (PBSC) collection: a retrospective study of 126 patients. <i>Leukemia and Lymphoma</i> , <b>1999</b> , 35, 501-5	1.9	26