Marina Cavazzana-Calvo

List of Publications by Citations

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	Insertional oncogenesis in 4 patients after retrovirus-mediated gene therapy of SCID-X1. *Journal of Clinical Investigation*, **2008**, 118, 3132-42	15.9	1269
82	Hematopoietic stem cell gene therapy with a lentiviral vector in X-linked adrenoleukodystrophy. *Science*, **2009**, 326, 818-23	33.3	1153
81	Transfusion independence and HMGA2 activation after gene therapy of human β-thalassaemia. *Nature*, **2010**, 467, 318-22	50.4	953
80	Sustained correction of X-linked severe combined immunodeficiency by ex vivo gene therapy. *New England Journal of Medicine*, **2002**, 346, 1185-93	59.2	952
79	Efficacy of gene therapy for X-linked severe combined immunodeficiency. *New England Journal of Medicine*, **2010**, 363, 355-64	59.2	471
78	Gene Therapy in a Patient with Sickle Cell Disease. *New England Journal of Medicine*, **2017**, 376, 848-855	59.2	418
77	Gene Therapy in Patients with Transfusion-Dependent β-Thalassemia. *New England Journal of Medicine*, **2018**, 378, 1479-1493	59.2	347
76	Transplantation of hematopoietic stem cells and long-term survival for primary immunodeficiencies in Europe: entering a new century, do we do better?. *Journal of Allergy and Clinical Immunology*, **2010**, 126, 602-10.e1-11	11.5	328
75	A modified γ-retrovirus vector for X-linked severe combined immunodeficiency. *New England Journal of Medicine*, **2014**, 371, 1407-17	59.2	278
74	Autoimmunity in Wiskott-Aldrich syndrome: risk factors, clinical features, and outcome in a single-center cohort of 55 patients. *Pediatrics*, **2003**, 111, e622-7	7.4	248
73	Outcomes following gene therapy in patients with severe Wiskott-Aldrich syndrome. *JAMA - Journal of the American Medical Association*, **2015**, 313, 1550-63	27.4	245
72	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. *Blood*, **2011**, 118, 1675-84	2.2	236
71	Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation. *Blood*, **2017**, 129, 1548-1556	2.2	230
70	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. *Blood*, **2008**, 111, 439-45	2.2	195
69	A human immunodeficiency caused by mutations in the PIK3R1 gene. *Journal of Clinical Investigation*, **2014**, 124, 3923-8	15.9	166
68	Clinical and immunologic phenotype associated with activated phosphoinositide 3-kinase δ syndrome 2: A cohort study. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 210-218.e9	11.5	163
67	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

66	Gene Therapy of the β-Hemoglobinopathies by Lentiviral Transfer of the HBB(T87Q)-Globin Gene. *Human Gene Therapy*, **2016**, 27, 148-65	4.8	111
65	Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated editing of the human β-globin locus. *Blood*, **2018**, 131, 1960-1973	2.2	110
64	Failure of SCID-X1 gene therapy in older patients. *Blood*, **2005**, 105, 4255-7	2.2	105
63	A self-inactivating lentiviral vector for SCID-X1 gene therapy that does not activate LMO2 expression in human T cells. *Blood*, **2010**, 116, 900-8	2.2	100
62	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
61	Gene therapy targeting haematopoietic stem cells for inherited diseases: progress and challenges. *Nature Reviews Drug Discovery*, **2019**, 18, 447-462	64.1	80
60	Preclinical evaluation of efficacy and safety of an improved lentiviral vector for the treatment of β-thalassemia and sickle cell disease. *Current Gene Therapy*, **2015**, 15, 64-81	4.3	78
59	Gene Therapy for β-Hemoglobinopathies. *Molecular Therapy*, **2017**, 25, 1142-1154	11.7	69
58	Gene Therapy for X-Linked Severe Combined Immunodeficiency: Where Do We Stand?. *Human Gene Therapy*, **2016**, 27, 108-16	4.8	65
57	Characteristics and outcome of early-onset, severe forms of Wiskott-Aldrich syndrome. *Blood*, **2013**, 121, 1510-6	2.2	65
56	Plerixafor enables safe, rapid, efficient mobilization of hematopoietic stem cells in sickle cell disease patients after exchange transfusion. *Haematologica*, **2018**, 103, 778-786	6.6	64
55	Faster T-cell development following gene therapy compared with haploidentical HSCT in the treatment of SCID-X1. *Blood*, **2015**, 125, 3563-9	2.2	52
54	Human T-lymphoid progenitors generated in a feeder-cell-free Delta-like-4 culture system promote T-cell reconstitution in NOD/SCID/IL2Rγ(-/-) mice. *Stem Cells*, **2012**, 30, 1771-80	5.8	50
53	Editing a γ-globin repressor binding site restores fetal hemoglobin synthesis and corrects the sickle cell disease phenotype. *Science Advances*, **2020**, 6,	14.3	49
52	AK2 deficiency compromises the mitochondrial energy metabolism required for differentiation of human neutrophil and lymphoid lineages. *Cell Death and Disease*, **2015**, 6, e1856	9.8	45
51	X-linked primary immunodeficiency associated with hemizygous mutations in the moesin (MSN) gene. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 1681-1689.e8	11.5	45
50	Hyperinflammation in patients with chronic granulomatous disease leads to impairment of hematopoietic stem cell functions. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 219-228.e9	11.5	45
49	An in vivo genetic reversion highlights the crucial role of Myb-Like, SWIRM, and MPN domains 1 (MYSM1) in human hematopoiesis and lymphocyte differentiation. *Journal of Allergy and Clinical Immunology*, **2015**, 136, 1619-1626.e5	11.5	44

48	INSPIIRED: A Pipeline for Quantitative Analysis of Sites of New DNA Integration in Cellular Genomes. *Molecular Therapy - Methods and Clinical Development*, **2017**, 4, 39-49	6.4	42
47	Lentiviral and genome-editing strategies for the treatment of hemoglobinopathies. *Blood*, **2019**, 134, 1203-1213	2.2	42
46	Gene Therapy Approaches to Hemoglobinopathies. *Hematology/Oncology Clinics of North America*, **2017**, 31, 835-852	3.1	40
45	Gene therapy for inherited immunodeficiency. *Expert Opinion on Biological Therapy*, **2014**, 14, 789-98	5.4	39
44	Hematopoietic stem cell transplantation for CD40 ligand deficiency: Results from an EBMT/ESID-IEWP-SCETIDE-PIDTC study. *Journal of Allergy and Clinical Immunology*, **2019**, 143, 2238-2253	11.5	38
43	INSPIIRED: Quantification and Visualization Tools for Analyzing Integration Site Distributions. *Molecular Therapy - Methods and Clinical Development*, **2017**, 4, 17-26	6.4	36
42	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. *Frontiers in Pediatrics*, **2019**, 7, 443	3.4	34
41	Gene therapy of hemoglobinopathies: progress and future challenges. *Human Molecular Genetics*, **2019**, 28, R24-R30	5.6	33
40	Interim Results from a Phase 1/2 Clinical Study of Lentiglobin Gene Therapy for Severe Sickle Cell Disease. *Blood*, **2016**, 128, 1176-1176	2.2	30
39	Cardiac iron overload in chronically transfused patients with thalassemia, sickle cell anemia, or myelodysplastic syndrome. *PLoS ONE*, **2017**, 12, e0172147	3.7	29
38	Risk factors and outcomes according to age at transplantation with an HLA-identical sibling for sickle cell disease. *Haematologica*, **2019**, 104, e543-e546	6.6	26
37	Comparison of lenograstim vs filgrastim administration following chemotherapy for peripheral blood stem cell (PBSC) collection: a retrospective study of 126 patients. *Leukemia and Lymphoma*, **1999**, 35, 501-5	1.9	26
36	Gene Therapy for Hemoglobinopathies. *Human Gene Therapy*, **2018**, 29, 1106-1113	4.8	25
35	Systematic neonatal screening for severe combined immunodeficiency and severe T-cell lymphopenia: Analysis of cost-effectiveness based on French real field data. *Journal of Allergy and Clinical Immunology*, **2015**, 135, 1589-93	11.5	22
34	Clonal tracking in gene therapy patients reveals a diversity of human hematopoietic differentiation programs. *Blood*, **2020**, 135, 1219-1231	2.2	22
33	Gene Therapy in a Patient with Sickle Cell Disease. *New England Journal of Medicine*, **2017**, 376, 2094	59.2	21
32	Mammalian target of rapamycin inhibition counterbalances the inflammatory status of immune cells in patients with chronic granulomatous disease. *Journal of Allergy and Clinical Immunology*, **2017**, 139, 1641-1649.e6	11.5	21
31	Innovative Curative Treatment of Beta Thalassemia: Cost-Efficacy Analysis of Gene Therapy Versus Allogenic Hematopoietic Stem-Cell Transplantation. *Human Gene Therapy*, **2019**, 30, 753-761	4.8	20

30	Gene Therapy with Hematopoietic Stem Cells: The Diseased Bone Marrow's Point of View. *Stem Cells and Development*, **2017**, 26, 71-76	4.4	17
29	Gene-corrected human Munc13-4-deficient CD8+ T cells can efficiently restrict EBV-driven lymphoproliferation in immunodeficient mice. *Blood*, **2016**, 128, 2859-2862	2.2	16
28	Specific T cells for the treatment of cytomegalovirus and/or adenovirus in the context of hematopoietic stem cell transplantation. *Journal of Allergy and Clinical Immunology*, **2016**, 138, 920-924.e3	11.5	15
27	Arterio-venous fistula for automated red blood cells exchange in patients with sickle cell disease: Complications and outcomes. *American Journal of Hematology*, **2017**, 92, 136-140	7.1	15
26	Development of Lentiviral Vectors Simultaneously Expressing Multiple siRNAs Against CCR5, vif and tat/rev Genes for an HIV-1 Gene Therapy Approach. *Molecular Therapy - Nucleic Acids*, **2016**, 5, e312	10.7	14
25	Variable correction of Artemis deficiency by I-Sce1-meganuclease-assisted homologous recombination in murine hematopoietic stem cells. *Gene Therapy*, **2014**, 21, 529-32	4	13
24	Extensive multilineage analysis in patients with mixed chimerism after allogeneic transplantation for sickle cell disease: insight into hematopoiesis and engraftment thresholds for gene therapy. *Haematologica*, **2020**, 105, 1240-1247	6.6	11
23	Generation of adult human T-cell progenitors for immunotherapeutic applications. *Journal of Allergy and Clinical Immunology*, **2018**, 141, 1491-1494.e4	11.5	11
22	Stem cell transplantation for primary immunodeficiencies: the European experience. *Current Opinion in Allergy and Clinical Immunology*, **2014**, 14, 516-20	3.3	9
21	Effect of hydroxyurea exposure before puberty on sperm parameters in males with sickle cell disease. *Blood*, **2021**, 137, 826-829	2.2	9
20	Gene transfer into hematopoietic stem cells reduces HLH manifestations in a murine model of Munc13-4 deficiency. *Blood Advances*, **2017**, 1, 2781-2789	7.8	8
19	Long-term outcomes of lentiviral gene therapy for the hemoglobinopathies: the HGB-205 trial.. *Nature Medicine*, **2022**,	50.5	8
18	Donor-targeted serotherapy as a rescue therapy for steroid-resistant acute GVHD after HLA-mismatched kidney transplantation. *American Journal of Transplantation*, **2020**, 20, 2243-2253	8.7	7
17	A gain-of-function RAC2 mutation is associated with bone-marrow hypoplasia and an autosomal dominant form of severe combined immunodeficiency. *Haematologica*, **2021**, 106, 404-411	6.6	7
16	Family cord blood banking for sickle cell disease: a twenty-year experience in two dedicated public cord blood banks. *Haematologica*, **2017**, 102, 976-983	6.6	6
15	Seletalisib for Activated PI3K Syndromes: Open-Label Phase 1b and Extension Studies. *Journal of Immunology*, **2020**, 205, 2979-2987	5.3	6
14	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. *American Journal of Hematology*, **2018**, 93, 794-802	7.1	5
13	Long-term safety and efficacy of lentiviral hematopoietic stem/progenitor cell gene therapy for Wiskott-Aldrich syndrome.. *Nature Medicine*, **2022**,	50.5	5

12	Retrieval of vector integration sites from cell-free DNA. *Nature Medicine*, **2021**, 27, 1458-1470	50.5	5
11	Successful in utero stem cell transplantation in X-linked severe combined immunodeficiency. *Blood Advances*, **2019**, 3, 237-241	7.8	5
10	Early-onset hypogammaglobulinemia: A survey of 44 patients. *Journal of Allergy and Clinical Immunology*, **2015**, 136, 1097-9.e2	11.5	4
9	Hematopoietic cell transplantation in severe combined immunodeficiency: The SCETIDE 2006-2014 European cohort. *Journal of Allergy and Clinical Immunology*, **2021**,	11.5	4
8	Bone Marrow Transplantation in Congenital Erythropoietic Porphyria: Sustained Efficacy but Unexpected Liver Dysfunction. *Biology of Blood and Marrow Transplantation*, **2020**, 26, 704-711	4.7	4
7	Vascular access for optimal hematopoietic stem cell collection. *Journal of Clinical Apheresis*, **2021**, 36, 12-19	3.2	4
6	Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT inborn errors working party analysis.. *Blood*, **2022**,	2.2	3
5	Alternative Promoter Encodes a Functional Munc13-4 Isoform Predominantly Expressed in Lymphocytes and Platelets. *Frontiers in Immunology*, **2020**, 11, 1154	8.4	2
4	Analysis of RBC Properties in Patients with SCD Treated with Lentiglobin Gene Therapy. *Blood*, **2018**, 132, 2195-2195	2.2	2
3	Transient mTOR inhibition rescues 4-1BB CAR-Tregs from tonic signal-induced dysfunction. *Nature Communications*, **2021**, 12, 6446	17.4	2
2	A combination of cyclophosphamide and interleukin-2 allows CD4+ T cells converted to Tregs to control scurfy syndrome. *Blood*, **2021**, 137, 2326-2336	2.2	2
1	Novel Lentiviral Vectors for Gene Therapy of Sickle Cell Disease Combining Gene Addition and Gene Silencing Strategies. *Blood*, **2021**, 138, 3973-3973	2.2	1