Alessandra Biffi

List of Publications by Year
in descending order

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76	4,539	218677	106344
papers	citations	26 h-index	65 g-index

77	77	77	5778
all docs	docs citations	times ranked	citing authors

#	Article	IF	Citations
1	Low frequency of treatable pediatric disease alleles in gnomAD: An opportunity for future genomic screening of newborns. Human Genetics and Genomics Advances, 2022, 3, 100059.	1.7	3
2	Either IL-7 activation of JAK-STAT or BEZ inhibition of PI3K-AKT-mTOR pathways dominates the single-cell phosphosignature of <i>ex vivo</i> treated pediatric T-cell acute lymphoblastic leukemia cells. Haematologica, 2022, 107, 1293-1310.	3.5	8
3	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.	13.7	109
4	Pain Coping Strategies in Pediatric Patients with Acute Leukemias in the First Month of Therapy: Effects of Treatments and Implications on Procedural Analgesia. Cancers, 2022, 14, 1473.	3.7	1
5	Pain coping strategies in paediatric patients newly diagnosed with leukaemia compared with healthy peers. European Journal of Cancer Care, 2022, 31, .	1.5	3
6	NUP214â€“ABL1 fusion in childhood Tâ€ALL. Pediatric Blood and Cancer, 2022, 69, e29643.	1.5	4
7	New Indications for Hematopoietic Stem Cell Gene Therapy in Lysosomal Storage Disorders. Frontiers in Oncology, 2022, 12, .	2.8	6
8	Bone Remodeling in an Mps-1h Girl after Hematopoietic Stem Cell Transplantation along with Enzymatic Replacement Therapy. Endocrine, Metabolic and Immune Disorders - Drug Targets, 2022, 22, 1425-1432.	1.2	0
9	QOL-33. Adaptive behaviour of patients treated for malignant brain tumor in the first three years of life. Neuro-Oncology, 2022, 24, i141-i141.	1.2	0
10	QOL-32. Patients treated for malignant brain tumor in the first three years of life: clinical sequelae. Neuro-Oncology, 2022, 24, i141-i141.	1.2	0
11	miR-939 acts as tumor suppressor by modulating JUNB transcriptional activity in pediatric anaplastic large cell lymphoma. Haematologica, 2021, 106, 610-613.	3.5	9
12	Post-Transcriptional Genetic Silencing ofBCL11Ato Treat Sickle Cell Disease. New England Journal of Medicine, 2021, 384, 205-215.	27.0	250
13	Use of letermovir in off-label indications: Infectious Diseases Working Party of European Society of Blood and Marrow Transplantation retrospective study. Bone Marrow Transplantation, 2021, 56, 1171-1179.	2.4	30
14	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.	1.3	6
15	Histiocytic sarcoma arising in a child affected by Burkitt lymphoma, with t(8;14)(q24;q32) positivity in both tumors. Pediatric Hematology and Oncology, 2021, 38, 1-7.	0.8	2
16	Droplet Digital PCR Improves IG-/TR-based MRD Risk Definition in Childhood B-cell Precursor Acute Lymphoblastic Leukemia. HemaSphere, 2021, 5, e543.	2.7	20
17	Childhood cancer in Italy: background, goals, and achievements of the Italian Paediatric Hematology Oncology Association (AIEOP). Tumori, 2021, 107, 370-375.	1.1	11
18	Low miR-214-5p Expression Correlates With Aggressive Subtypes of Pediatric ALCL With Non-Common Histology. Frontiers in Oncology, 2021, 11, 663221.	2.8	2

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19	Metachromatic leukodystrophy: A singleâ€center longitudinal study of 45 patients. Journal of Inherited Metabolic Disease, 2021, 44, 1151-1164.	3.6	27
20	Ruxolitinib as a Novel Therapeutic Option for Poor Prognosis T-LBL Pediatric Patients. Cancers, 2021, 13, 3724.	3.7	2
21	Safety and efficacy of brincidofovir for Adenovirus infection in children receiving allogeneic stem cell transplantation: an AIEOP retrospective analyses. Bone Marrow Transplantation, 2021, 56, 3104-3107.	2.4	5
22	Integrated CGH/WES Analyses Advance Understanding of Aggressive Neuroblastoma Evolution: A Case Study. Cells, 2021, 10, 2695.	4.1	3
23	Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy for Metachromatic Leukodystrophy (MLD): Clinical Outcomes from 38 Patients. , 2021, 52, .		0
24	Quality of Life in Children, Adolescents and Young Adults with Sickle Cell Disease and Their Caregivers during Standard of Care and after Bone Marrow Transplantation: A Single Center Report. Blood, 2021, 138, 3032-3032.	1.4	0
25	The Role of Hemoglobin and Hemolysis on Transcranial Doppler Velocities in Children with Sickle Cell Disease: Data from a Natural History Cohort. Blood, 2021, 138, 3092-3092.	1.4	0
26	Mucormycosis with peculiar aortic involvement in a child with acute lymphoblastic leukemia. Pediatric Hematology and Oncology, 2020, 37, 164-169.	0.8	2
27	Pediatric IgG4â€related lymphadenopathy: A rare condition associated with autoimmunity and lymphoproliferative disorders. Pediatric Allergy and Immunology, 2020, 31, 332-336.	2.6	3
28	A novel germline variant in <scp>PIK3R1</scp> results in <scp>SHORT</scp> syndrome associated with <scp>TAL</scp>/<scp>LMO</scp> Tâ€cell acute lymphoblastic leukemia. American Journal of Hematology, 2020, 95, E335-E338.	4.1	11
29	Pediatric Patients Treated for Leukemia Back to School: A Mixed-Method Analysis of Narratives about Daily Life and Illness Experience. Behavioral Sciences (Basel, Switzerland), 2020, 10, 107.	2.1	10
30	Lipophilic dye-compatible brain clearing technique allowing correlative magnetic resonance/high-resolution fluorescence imaging in rat models of glioblastoma. Scientific Reports, 2020, 10, 17974.	3.3	3
31	Hospitalâ€based home care for children with cancer during the COVIDâ€19 pandemic in northeastern Italy. Pediatric Blood and Cancer, 2020, 67, e28501.	1.5	2
32	COVID-19 Pandemic: Perspective of an Italian Tertiary Care Pediatric Center. Healthcare (Switzerland), 2020, 8, 311.	2.0	5
33	The Changing Face of Adrenoleukodystrophy. Endocrine Reviews, 2020, 41, 577-593.	20.1	38
34	Health Locus of Control in Parents of Children with Leukemia and Associations with Their Life Perceptions and Depression Symptomatology. Children, 2020, 7, 40.	1.5	7
35	RNY4 in Circulating Exosomes of Patients With Pediatric Anaplastic Large Cell Lymphoma: An Active Player?. Frontiers in Oncology, 2020, 10, 238.	2.8	12
36	Minimal residual disease analysis in childhood mature Bâ€cell leukaemia/lymphoma treated with AIEOP LNHâ€97 protocol with/without antiâ€CD20 administration. British Journal of Haematology, 2020, 189, e108-e111.	2.5	8

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37	How we deal with the COVIDâ€19 epidemic in an Italian paediatric oncoâ€haematology clinic located in a region with a high density of cases. British Journal of Haematology, 2020, 189, 640-642.	2.5	19
38	The combined use of enzyme activity and metabolite assays as a strategy for newborn screening of mucopolysaccharidosis type I. Clinical Chemistry and Laboratory Medicine, 2020, 58, 2063-2072.	2.3	12
39	Gene-Based Approaches to Inherited Neurometabolic Diseases. Human Gene Therapy, 2019, 30, 1222-1235.	2.7	28
40	The Developmental Pathways of Preschool Children with Acute Lymphoblastic Leukemia: Communicative and Social Sequelae One Year after Treatment. Children, 2019, 6, 92.	1.5	5
41	Bone marrow harvesting from paediatric patients undergoing haematopoietic stem cell gene therapy. Bone Marrow Transplantation, 2019, 54, 1995-2003.	2.4	9
42	A comprehensive single cell transcriptional landscape of human hematopoietic progenitors. Nature Communications, 2019, 10, 2395.	12.8	247
43	Pre-clinical Safety and Efficacy of Lentiviral Vector-Mediated ExÂVivo Stem Cell Gene Therapy for the Treatment of Mucopolysaccharidosis IIIA. Molecular Therapy - Methods and Clinical Development, 2019, 13, 399-413.	4.1	37
44	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.	8.2	17
45	Assessing the Impact of Cyclosporin A on Lentiviral Transduction and Preservation of Human Hematopoietic Stem Cells in Clinically RelevantEx VivoGene Therapy Settings. Human Gene Therapy, 2019, 30, 1133-1146.	2.7	8
46	Highly efficient therapeutic gene editing of human hematopoietic stem cells. Nature Medicine, 2019, 25, 776-783.	30.7	344
47	Biodegradable polymeric nanoparticles administered in the cerebrospinal fluid: Brain biodistribution, preferential internalization in microglia and implications for cell-selective drug release. Biomaterials, 2019, 209, 25-40.	11.4	37
48	Simultaneous Flow Cytometric Characterization of Multiple Cell Types Retrieved from Mouse Brain/Spinal Cord Through Different Homogenization Methods. Journal of Visualized Experiments, 2019, , .	0.3	4
49	Gene Therapy as a Curative Option for Î²-Thalassemia. New England Journal of Medicine, 2018, 378, 1551-1552.	27.0	17
50	Metallothioneins are neuroprotective agents in lysosomal storage disorders. Annals of Neurology, 2018, 83, 418-432.	5.3	10
51	Successful hematopoietic stem cell mobilization and apheresis collection using plerixafor alone in sickle cell patients. Blood Advances, 2018, 2, 2505-2512.	5.2	62
52	Prostaglandin E2 Stimulates the Expansion of Regulatory Hematopoietic Stem and Progenitor Cells in Type 1 Diabetes. Frontiers in Immunology, 2018, 9, 1387.	4.8	15
53	Metachromatic Leukodystrophy. JAMA Neurology, 2018, 75, 1027.	9.0	2
54	Highly Efficient Therapeutic Gene Editing of BCL11A enhancer in Human Hematopoietic Stem Cells from ÃŸ-Hemoglobinopathy Patients for Fetal Hemoglobin Induction. Blood, 2018, 132, 3482-3482.	1.4	2

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55	Hematopoietic Stem Cell Gene Therapy for Storage Disease: Current and New Indications. Molecular Therapy, 2017, 25, 1155-1162.	8.2	68
56	Microglial dysfunction as a key pathological change in adrenomyeloneuropathy. Annals of Neurology, 2017, 82, 813-827.	5.3	37
57	Hematopoietic Gene Therapies for Metabolic and Neurologic Diseases. Hematology/Oncology Clinics of North America, 2017, 31, 869-881.	2.2	10
58	Intracerebroventricular delivery of hematopoietic progenitors results in rapid and robust engraftment of microglia-like cells. Science Advances, 2017, 3, e1701211.	10.3	38
59	Preclinical Testing of the Safety and Tolerability of Lentiviral Vectorâ€“Mediated Above-Normal Alpha-L-Iduronidase Expression in Murine and Human Hematopoietic Cells Using Toxicology and Biodistribution Good Laboratory Practice Studies. Human Gene Therapy, 2016, 27, 813-829.	2.7	40
60	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, 476-487.	13.7	393
61	Gene therapy for lysosomal storage disorders: a good start. Human Molecular Genetics, 2016, 25, R65-R75.	2.9	44
62	Design of a regulated lentiviral vector for hematopoietic stem cell gene therapy of globoid cell leukodystrophy. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15038.	4.1	29
63	Hematopoietic stem cell transplantation for metachromatic leukodystrophy. Expert Opinion on Orphan Drugs, 2015, 3, 911-919.	0.8	4
64	Metallothioneins as dynamic markers for brain disease in lysosomal disorders. Annals of Neurology, 2014, 75, 127-137.	5.3	17
65	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. Science, 2013, 341, 1233158.	12.6	998
66	Brain conditioning is instrumental for successful microglia reconstitution following hematopoietic stem cell transplantation. Proceedings of the National Academy of Sciences of the United States of America, 2012, 109, 15018-15023.	7.1	168
67	Maintenance of a functional hematopoietic stem cell niche through galactocerebrosidase and other enzymes. Current Opinion in Hematology, 2011, 18, 214-219.	2.5	5
68	Lentiviral vector common integration sites in preclinical models and a clinical trial reflect a benign integration bias and not oncogenic selection. Blood, 2011, 117, 5332-5339.	1.4	201
69	The galactocerebrosidase enzyme contributes to the maintenance of a functional hematopoietic stem cell niche. Blood, 2010, 116, 1857-1866.	1.4	50
70	Gene therapy augments the efficacy of hematopoietic cell transplantation and fully corrects mucopolysaccharidosis type I phenotype in the mouse model. Blood, 2010, 116, 5130-5139.	1.4	159
71	Identification of Hematopoietic Stem Cellâ€“Specific miRNAs Enables Gene Therapy of Globoid Cell Leukodystrophy. Science Translational Medicine, 2010, 2, 58ra84.	12.4	180
72	Monitoring disease evolution and treatment response in lysosomal disorders by the peripheral benzodiazepine receptor ligand PK11195. Neurobiology of Disease, 2009, 34, 51-62.	4.4	12

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73	Gene therapy of metachromatic leukodystrophy reverses neurological damage and deficits in mice. Journal of Clinical Investigation, 2006, 116, 3070-3082.	8.2	197
74	Gene Therapy of Storage Disorders by Retroviral and Lentiviral Vectors. Human Gene Therapy, 2005, 16, 1133-1142.	2.7	39
75	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. Journal of Clinical Investigation, 2004, 113, 1118-1129.	8.2	117
76	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. Journal of Clinical Investigation, 2004, 113, 1118-1129.	8.2	256