

Alessandra Biffi

List of Publications by Year in descending order

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76
papers

4,539
citations

218677

26
h-index

106344

65
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all docs

77
docs citations

77
times ranked

5778
citing authors

#	ARTICLE	IF	CITATIONS
1	Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy. <i>Science</i> , 2013, 341, 1233-1238.	12.6	998
2	Lentiviral haematopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. <i>Lancet</i> , 2016, 388, 476-487.	13.7	393
3	Highly efficient therapeutic gene editing of human hematopoietic stem cells. <i>Nature Medicine</i> , 2019, 25, 776-783.	30.7	344
4	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. <i>Journal of Clinical Investigation</i> , 2004, 113, 1118-1129.	8.2	256
5	Post-Transcriptional Genetic Silencing of <i>BCL11A</i> to Treat Sickle Cell Disease. <i>New England Journal of Medicine</i> , 2021, 384, 205-215.	27.0	250
6	A comprehensive single cell transcriptional landscape of human hematopoietic progenitors. <i>Nature Communications</i> , 2019, 10, 2395.	12.8	247
7	Lentiviral vector common integration sites in preclinical models and a clinical trial reflect a benign integration bias and not oncogenic selection. <i>Blood</i> , 2011, 117, 5332-5339.	1.4	201
8	Gene therapy of metachromatic leukodystrophy reverses neurological damage and deficits in mice. <i>Journal of Clinical Investigation</i> , 2006, 116, 3070-3082.	8.2	197
9	Identification of Hematopoietic Stem Cell-Specific miRNAs Enables Gene Therapy of Globoid Cell Leukodystrophy. <i>Science Translational Medicine</i> , 2010, 2, 58ra84.	12.4	180
10	Brain conditioning is instrumental for successful microglia reconstitution following hematopoietic stem cell transplantation. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2012, 109, 15018-15023.	7.1	168
11	Gene therapy augments the efficacy of hematopoietic cell transplantation and fully corrects mucopolysaccharidosis type I phenotype in the mouse model. <i>Blood</i> , 2010, 116, 5130-5139.	1.4	159
12	Correction of metachromatic leukodystrophy in the mouse model by transplantation of genetically modified hematopoietic stem cells. <i>Journal of Clinical Investigation</i> , 2004, 113, 1118-1129.	8.2	117
13	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. <i>Lancet</i> , 2022, 399, 372-383.	13.7	109
14	Hematopoietic Stem Cell Gene Therapy for Storage Disease: Current and New Indications. <i>Molecular Therapy</i> , 2017, 25, 1155-1162.	8.2	68
15	Successful hematopoietic stem cell mobilization and apheresis collection using plerixafor alone in sickle cell patients. <i>Blood Advances</i> , 2018, 2, 2505-2512.	5.2	62
16	The galactocerebrosidase enzyme contributes to the maintenance of a functional hematopoietic stem cell niche. <i>Blood</i> , 2010, 116, 1857-1866.	1.4	50
17	Gene therapy for lysosomal storage disorders: a good start. <i>Human Molecular Genetics</i> , 2016, 25, R65-R75.	2.9	44
18	Preclinical Testing of the Safety and Tolerability of Lentiviral Vector-Mediated Above-Normal Alpha-L-Iduronidase Expression in Murine and Human Hematopoietic Cells Using Toxicology and Biodistribution Good Laboratory Practice Studies. <i>Human Gene Therapy</i> , 2016, 27, 813-829.	2.7	40

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19	Gene Therapy of Storage Disorders by Retroviral and Lentiviral Vectors. <i>Human Gene Therapy</i> , 2005, 16, 1133-1142.	2.7	39
20	Intracerebroventricular delivery of hematopoietic progenitors results in rapid and robust engraftment of microglia-like cells. <i>Science Advances</i> , 2017, 3, e1701211.	10.3	38
21	The Changing Face of Adrenoleukodystrophy. <i>Endocrine Reviews</i> , 2020, 41, 577-593.	20.1	38
22	Microglial dysfunction as a key pathological change in adrenomyeloneuropathy. <i>Annals of Neurology</i> , 2017, 82, 813-827.	5.3	37
23	Pre-clinical Safety and Efficacy of Lentiviral Vector-Mediated Ex Vivo Stem Cell Gene Therapy for the Treatment of Mucopolysaccharidosis IIIA. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 13, 399-413.	4.1	37
24	Biodegradable polymeric nanoparticles administered in the cerebrospinal fluid: Brain biodistribution, preferential internalization in microglia and implications for cell-selective drug release. <i>Biomaterials</i> , 2019, 209, 25-40.	11.4	37
25	Use of letemovir in off-label indications: Infectious Diseases Working Party of European Society of Blood and Marrow Transplantation retrospective study. <i>Bone Marrow Transplantation</i> , 2021, 56, 1171-1179.	2.4	30
26	Design of a regulated lentiviral vector for hematopoietic stem cell gene therapy of globoid cell leukodystrophy. <i>Molecular Therapy - Methods and Clinical Development</i> , 2015, 2, 15038.	4.1	29
27	Gene-Based Approaches to Inherited Neurometabolic Diseases. <i>Human Gene Therapy</i> , 2019, 30, 1222-1235.	2.7	28
28	Metachromatic leukodystrophy: A single-center longitudinal study of 45 patients. <i>Journal of Inherited Metabolic Disease</i> , 2021, 44, 1151-1164.	3.6	27
29	Droplet Digital PCR Improves IG/TR-based MRD Risk Definition in Childhood B-cell Precursor Acute Lymphoblastic Leukemia. <i>HemaSphere</i> , 2021, 5, e543.	2.7	20
30	How we deal with the COVID-19 epidemic in an Italian paediatric oncology-haematology clinic located in a region with a high density of cases. <i>British Journal of Haematology</i> , 2020, 189, 640-642.	2.5	19
31	Metallothioneins as dynamic markers for brain disease in lysosomal disorders. <i>Annals of Neurology</i> , 2014, 75, 127-137.	5.3	17
32	Gene Therapy as a Curative Option for β^2 -Thalassemia. <i>New England Journal of Medicine</i> , 2018, 378, 1551-1552.	27.0	17
33	Targeting a Pre-existing Anti-transgene T Cell Response for Effective Gene Therapy of MPS-I in the Mouse Model of the Disease. <i>Molecular Therapy</i> , 2019, 27, 1215-1227.	8.2	17
34	Prostaglandin E2 Stimulates the Expansion of Regulatory Hematopoietic Stem and Progenitor Cells in Type 1 Diabetes. <i>Frontiers in Immunology</i> , 2018, 9, 1387.	4.8	15
35	Monitoring disease evolution and treatment response in lysosomal disorders by the peripheral benzodiazepine receptor ligand PK11195. <i>Neurobiology of Disease</i> , 2009, 34, 51-62.	4.4	12
36	RNY4 in Circulating Exosomes of Patients With Pediatric Anaplastic Large Cell Lymphoma: An Active Player?. <i>Frontiers in Oncology</i> , 2020, 10, 238.	2.8	12

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37	The combined use of enzyme activity and metabolite assays as a strategy for newborn screening of mucopolysaccharidosis type I. <i>Clinical Chemistry and Laboratory Medicine</i> , 2020, 58, 2063-2072.	2.3	12
38	A novel germline variant in <i>PIK3R1</i> results in <i>SHORT</i> syndrome associated with <i>TAL</i> <i>cell</i> acute lymphoblastic leukemia. <i>American Journal of Hematology</i> , 2020, 95, E335-E338.	4.1	11
39	Childhood cancer in Italy: background, goals, and achievements of the Italian Paediatric Hematology Oncology Association (AIEOP). <i>Tumori</i> , 2021, 107, 370-375.	1.1	11
40	Hematopoietic Gene Therapies for Metabolic and Neurologic Diseases. <i>Hematology/Oncology Clinics of North America</i> , 2017, 31, 869-881.	2.2	10
41	Metallothioneins are neuroprotective agents in lysosomal storage disorders. <i>Annals of Neurology</i> , 2018, 83, 418-432.	5.3	10
42	Pediatric Patients Treated for Leukemia Back to School: A Mixed-Method Analysis of Narratives about Daily Life and Illness Experience. <i>Behavioral Sciences (Basel, Switzerland)</i> , 2020, 10, 107.	2.1	10
43	Bone marrow harvesting from paediatric patients undergoing haematopoietic stem cell gene therapy. <i>Bone Marrow Transplantation</i> , 2019, 54, 1995-2003.	2.4	9
44	miR-939 acts as tumor suppressor by modulating JUNB transcriptional activity in pediatric anaplastic large cell lymphoma. <i>Haematologica</i> , 2021, 106, 610-613.	3.5	9
45	Assessing the Impact of Cyclosporin A on Lentiviral Transduction and Preservation of Human Hematopoietic Stem Cells in Clinically Relevant Ex Vivo Gene Therapy Settings. <i>Human Gene Therapy</i> , 2019, 30, 1133-1146.	2.7	8
46	Minimal residual disease analysis in childhood mature <i>B</i> -cell leukaemia/lymphoma treated with AIEOP LNH97 protocol with/without anti-CD20 administration. <i>British Journal of Haematology</i> , 2020, 189, e108-e111.	2.5	8
47	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. <i>Haematologica</i> , 2022, 107, 1293-1310.	3.5	8
48	Health Locus of Control in Parents of Children with Leukemia and Associations with Their Life Perceptions and Depression Symptomatology. <i>Children</i> , 2020, 7, 40.	1.5	7
49	Toward Reference Intervals of ARSA Activity in the Cerebrospinal Fluid: Implication for the Clinical Practice of Metachromatic Leukodystrophy. <i>Journal of Applied Laboratory Medicine</i> , 2021, 6, 354-366.	1.3	6
50	New Indications for Hematopoietic Stem Cell Gene Therapy in Lysosomal Storage Disorders. <i>Frontiers in Oncology</i> , 2022, 12, .	2.8	6
51	Maintenance of a functional hematopoietic stem cell niche through galactocerebrosidase and other enzymes. <i>Current Opinion in Hematology</i> , 2011, 18, 214-219.	2.5	5
52	The Developmental Pathways of Preschool Children with Acute Lymphoblastic Leukemia: Communicative and Social Sequelae One Year after Treatment. <i>Children</i> , 2019, 6, 92.	1.5	5
53	COVID-19 Pandemic: Perspective of an Italian Tertiary Care Pediatric Center. <i>Healthcare (Switzerland)</i> , 2020, 8, 311.	2.0	5
54	Safety and efficacy of brincidofovir for Adenovirus infection in children receiving allogeneic stem cell transplantation: an AIEOP retrospective analyses. <i>Bone Marrow Transplantation</i> , 2021, 56, 3104-3107.	2.4	5

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55	Hematopoietic stem cell transplantation for metachromatic leukodystrophy. Expert Opinion on Orphan Drugs, 2015, 3, 911-919.	0.8	4
56	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
57	<i>NUP214“ABL1</i> fusion in childhood TéALL. Pediatric Blood and Cancer, 2022, 69, e29643.	1.5	4
58	Pediatric IgG4érelated lymphadenopathy: A rare condition associated with autoimmunity and lymphoproliferative disorders. Pediatric Allergy and Immunology, 2020, 31, 332-336.	2.6	3
59	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
60	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
61	Integrated CGH/WES Analyses Advance Understanding of Aggressive Neuroblastoma Evolution: A Case Study. Cells, 2021, 10, 2695.	4.1	3
62	Pain coping strategies in paediatric patients newly diagnosed with leukaemia compared with healthy peers. European Journal of Cancer Care, 2022, 31, .	1.5	3
63	Metachromatic Leukodystrophy. JAMA Neurology, 2018, 75, 1027.	9.0	2
64	Mucormycosis with peculiar aortic involvement in a child with acute lymphoblastic leukemia. Pediatric Hematology and Oncology, 2020, 37, 164-169.	0.8	2
65	Hospitalébased home care for children with cancer during the COVIDé19 pandemic in northeastern Italy. Pediatric Blood and Cancer, 2020, 67, e28501.	1.5	2
66	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
67	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
68	Ruxolitinib as a Novel Therapeutic Option for Poor Prognosis T-LBL Pediatric Patients. Cancers, 2021, 13, 3724.	3.7	2
69	Highly Efficient Therapeutic Gene Editing of BCL11A enhancer in Human Hematopoietic Stem Cells from A&Y-Hemoglobinopathy Patients for Fetal Hemoglobin Induction. Blood, 2018, 132, 3482-3482.	1.4	2
70	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
71	Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy for Metachromatic Leukodystrophy (MLD): Clinical Outcomes from 38 Patients. , 2021, 52, .		0
72	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

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73	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
74	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
75	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
76	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