Paul J Orchard

List of Publications by Year
in descending order

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Version: 2024-02-01

117 papers	5,574 citations	126907 33 h-index	82547 72 g-index
118 all docs	118 docs citations	118 times ranked	5760 citing authors

#	Article	IF	Citations
1	Defects in TCIRG1 subunit of the vacuolar proton pump are responsible for a subset of human autosomal recessive osteopetrosis. Nature Genetics, 2000, 25, 343-346.	21.4	629
2	Clinical responses to bone marrow transplantation in children with severe osteogenesis imperfecta. Blood, 2001, 97, 1227-1231.	1.4	540
3	Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. New England Journal of Medicine, 2017, 377, 1630-1638.	27.0	412
4	Indications for Autologous and Allogeneic Hematopoietic CellÂTransplantation: Guidelines from the American Society forÂBlood and Marrow Transplantation. Biology of Blood and Marrow Transplantation, 2015, 21, 1863-1869.	2.0	342
5	Cerebral X-linked adrenoleukodystrophy: the international hematopoietic cell transplantation experience from 1982 to 1999. Blood, 2004, 104, 881-888.	1.4	334
6	Long-term outcome of Hurler syndrome patients after hematopoietic cell transplantation: an international multicenter study. Blood, 2015, 125, 2164-2172.	1.4	262
7	Outcomes after allogeneic hematopoietic cell transplantation for childhood cerebral adrenoleukodystrophy: the largest single-institution cohort report. Blood, 2011, 118, 1971-1978.	1.4	236
8	Outcomes of transplantation using various hematopoietic cell sources in children with Hurler syndrome after myeloablative conditioning. Blood, 2013, 121, 3981-3987.	1.4	183
9	Diagnosis and Management of Osteopetrosis: Consensus Guidelines From the Osteopetrosis Working Group. Journal of Clinical Endocrinology and Metabolism, 2017, 102, 3111-3123.	3.6	170
10	Indications for Hematopoietic Cell Transplantation and Immune Effector Cell Therapy: Guidelines from the American Society for Transplantation and Cellular Therapy. Biology of Blood and Marrow Transplantation, 2020, 26, 1247-1256.	2.0	139
11	Rapid Induction of Cerebral Organoids From Human Induced Pluripotent Stem Cells Using a Chemically Defined Hydrogel and Defined Cell Culture Medium. Stem Cells Translational Medicine, 2016, 5, 970-979.	3.3	116
12	Hematopoietic stem cell transplantation for infantile osteopetrosis. Blood, 2015, 126, 270-276.	1.4	89
13	Hematopoietic Cell Therapy for Metabolic Disease. Journal of Pediatrics, 2007, 151, 340-346.	1.8	81
14	Survival and Functional Outcomes in Boys with Cerebral Adrenoleukodystrophy with and without Hematopoietic Stem Cell Transplantation. Biology of Blood and Marrow Transplantation, 2019, 25, 538-548.	2.0	81
15	CNS Langerhans cell histiocytosis: Common hematopoietic origin for LCHâ€associated neurodegeneration and mass lesions. Cancer, 2018, 124, 2607-2620.	4.1	73
16	Unrelated donor bone marrow transplantation for children and adolescents with aplastic anaemia or myelodysplasia. British Journal of Haematology, 1997, 96, 749-756.	2.5	72
17	Transplant Outcomes in Leukodystrophies. Seminars in Hematology, 2010, 47, 70-78.	3.4	64
18	Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. Orphanet Journal of Rare Diseases, 2019, 14, 137.	2.7	62

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19	Transplantation in inborn errors of metabolism: current considerations and future perspectives. British Journal of Haematology, 2014, 167, 293-303.	2.5	60
20	Neurocognition across the spectrum of mucopolysaccharidosis type I: Age, severity, and treatment. Molecular Genetics and Metabolism, 2015, 116, 61-68.	1.1	59
21	Enzyme Replacement is Associated with Better Cognitive Outcomes after Transplant in Hurler Syndrome. Journal of Pediatrics, 2013, 162, 375-380.e1.	1.8	58
22	A report on stateâ€wide implementation of newborn screening for Xâ€linked Adrenoleukodystrophy. American Journal of Medical Genetics, Part A, 2019, 179, 1205-1213.	1.2	56
23	Neurocognitive Trajectory of Boys Who Received a Hematopoietic Stem Cell Transplant at an Early Stage of Childhood Cerebral Adrenoleukodystrophy. JAMA Neurology, 2017, 74, 710.	9.0	55
24	Long-term outcomes of systemic therapies for Hurler syndrome: an international multicenter comparison. Genetics in Medicine, 2018, 20, 1423-1429.	2.4	54
25	Treatment of brain disease in the mucopolysaccharidoses. Molecular Genetics and Metabolism, 2017, 122, 25-34.	1.1	52
26	Enzyme replacement therapy prior to haematopoietic stem cell transplantation in Mucopolysaccharidosis Type I: 10year combined experience of 2 centres. Molecular Genetics and Metabolism, 2016, 117, 373-377.	1.1	51
27	Intranasal Adeno-Associated Virus Mediated Gene Delivery and Expression of Human Iduronidase in the Central Nervous System: A Noninvasive and Effective Approach for Prevention of Neurologic Disease in Mucopolysaccharidosis Type I. Human Gene Therapy, 2017, 28, 576-587.	2.7	50
28	Mucopolysaccharidosis Type I: A Review of the Natural History and Molecular Pathology. Cells, 2020, 9, 1838.	4.1	48
29	Health-related quality of life in mucopolysaccharidosis: looking beyond biomedical issues. Orphanet Journal of Rare Diseases, 2016, 11, 119.	2.7	41
30	<scp>MRI</scp> surveillance of boys with Xâ€linked adrenoleukodystrophy identified by newborn screening: Metaâ€analysis and consensus guidelines. Journal of Inherited Metabolic Disease, 2021, 44, 728-739.	3.6	39
31	Long-Term Survival and Late Deaths after Hematopoietic Cell Transplantation for Primary Immunodeficiency Diseases and Inborn Errors of Metabolism. Biology of Blood and Marrow Transplantation, 2012, 18, 1438-1445.	2.0	37
32	Choice of conditioning regimens for bone marrow transplantation in severe aplastic anemia. Blood Advances, 2019, 3, 3123-3131.	5.2	37
33	An exploratory study of brain function and structure in mucopolysaccharidosis type I: Long term observations following hematopoietic cell transplantation (HCT). Molecular Genetics and Metabolism, 2012, 107, 116-121.	1.1	36
34	Mucopolysaccharidosis Type I: Current Treatments, Limitations, and Prospects for Improvement. Biomolecules, 2021, 11, 189.	4.0	36
35	Clinical-Scale Selection of Anti-CD3/CD28â€“Activated T Cells After Transduction with a Retroviral Vector Expressing Herpes Simplex Virus Thymidine Kinase and Truncated Nerve Growth Factor Receptor. Human Gene Therapy, 2002, 13, 979-988.	2.7	35
36	Primary hepatic lymphoma in an adolescent treated with hepatic lobectomy and chemotherapy. Cancer, 1990, 65, 2222-2226.	4.1	34

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37	Intensity of MRI Gadolinium Enhancement in Cerebral Adrenoleukodystrophy: A Biomarker for Inflammation and Predictor of Outcome following Transplantation in Higher Risk Patients. American Journal of Neuroradiology, 2016, 37, 367-372.	2.4	33
38	Chitotriosidase as a biomarker of cerebral adrenoleukodystrophy. Journal of Neuroinflammation, 2011, 8, 144.	7.2	32
39	Pharmacokinetics and Model-Based Dosing to Optimize Fludarabine Therapy in Pediatric Hematopoietic Cell Transplant Recipients. Biology of Blood and Marrow Transplantation, 2017, 23, 1701-1713.	2.0	32
40	Cerebral Spinal Fluid levels of Cytokines are elevated in Patients with Metachromatic Leukodystrophy. Scientific Reports, 2016, 6, 24579.	3.3	31
41	Allele-Level HLA Matching Impacts Key Outcomes Following Umbilical Cord Blood Transplantation for Inherited Metabolic Disorders. Biology of Blood and Marrow Transplantation, 2017, 23, 119-125.	2.0	31
42	Recommendations for the management of MPS VI: systematic evidence- and consensus-based guidance. Orphanet Journal of Rare Diseases, 2019, 14, 118.	2.7	30
43	Interaction of N-acetylcysteine and Cysteine in Human Plasma. Journal of Pharmaceutical Sciences, 2012, 101, 4653-4659.	3.3	28
44	Mortality after hematopoietic stem cell transplantation for severe mucopolysaccharidosis type I: the 30â€year University of Minnesota experience. Journal of Inherited Metabolic Disease, 2017, 40, 271-280.	3.6	28
45	Unexpected coronary artery findings in mucopolysaccharidosis. Report of four cases and literature review. Cardiovascular Pathology, 2014, 23, 145-151.	1.6	27
46	Low Bone Mineral Content and Challenges in Interpretation of Dual-Energy X-Ray Absorptiometry in Children With Mucopolysaccharidosis Types I, II, and VI. Journal of Clinical Densitometry, 2014, 17, 200-206.	1.2	27
47	Second allogeneic hematopoietic cell transplantation for graft failure: Poor outcomes for neutropenic graft failure. American Journal of Hematology, 2015, 90, 892-896.	4.1	27
48	Elevated cerebral spinal fluid biomarkers in children with mucopolysaccharidosis I-H. Scientific Reports, 2016, 6, 38305.	3.3	25
49	Progression of Hip Dysplasia in Mucopolysaccharidosis Type I Hurler After Successful Hematopoietic Stem Cell Transplantation. Journal of Bone and Joint Surgery - Series A, 2016, 98, 386-395.	3.0	25
50	Intrathecal enzyme replacement for Hurler syndrome: biomarker association with neurocognitive outcomes. Genetics in Medicine, 2019, 21, 2552-2560.	2.4	25
51	Hip Dysplasia in Patients With Hurler Syndrome (Mucopolysaccharidosis Type 1H). Journal of Pediatric Orthopaedics, 2013, 33, 635-643.	1.2	24
52	Comparative Effectiveness of Intracerebroventricular, Intrathecal, and Intranasal Routes of AAV9 Vector Administration for Genetic Therapy of Neurologic Disease in Murine Mucopolysaccharidosis Type I. Frontiers in Molecular Neuroscience, 2021, 14, 618360.	2.9	21
53	Successful donor engraftment and repair of the blood-brain barrier in cerebral adrenoleukodystrophy. Blood, 2019, 133, 1378-1381.	1.4	20
54	Biomarkers of bone remodeling in children with mucopolysaccharidosis types I, II, and VI. Journal of Pediatric Rehabilitation Medicine, 2014, 7, 159-165.	0.5	19

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55	Effect of recombinant human growth hormone on changes in height, bone mineral density, and body composition over 1â€“2 years in children with Hurler or Hunter syndrome. Molecular Genetics and Metabolism, 2014, 111, 101-106.	1.1	18
56	Differences in MPS I and MPS II Disease Manifestations. International Journal of Molecular Sciences, 2021, 22, 7888.	4.1	18
57	Leukodystrophy and Gene Therapy with a Dimmer Switch. New England Journal of Medicine, 2011, 364, 572-573.	27.0	14
58	Mechanisms of Antioxidant Induction with High-Dose N-Acetylcysteine in Childhood Cerebral Adrenoleukodystrophy. CNS Drugs, 2015, 29, 1041-1047.	5.9	13
59	N-Acetylcysteine Reverses the Mitochondrial Dysfunction Induced by Very Long-Chain Fatty Acids in Murine Oligodendrocyte Model of Adrenoleukodystrophy. Biomedicines, 2021, 9, 1826.	3.2	13
60	Association Between the Magnitude of Intravenous Busulfan Exposure and Development of Hepatic Veno-Occlusive Disease in Children and Young Adults Undergoing Myeloablative Allogeneic Hematopoietic Cell Transplantation. Transplantation and Cellular Therapy, 2022, 28, 196-202.	1.2	12
61	Treatment of thoracolumbar kyphosis in patients with mucopolysaccharidosis type I: results of an international consensus procedure. Orphanet Journal of Rare Diseases, 2019, 14, 17.	2.7	11
62	Cerebral adrenoleukodystrophy is associated with loss of tolerance to profilin. European Journal of Immunology, 2019, 49, 947-953.	2.9	11
63	Clinical trial of laronidase in Hurler syndrome after hematopoietic cell transplantation. Pediatric Research, 2020, 87, 104-111.	2.3	11
64	Reduced-Toxicity (BuFlu) Conditioning Is Better Tolerated but Has a Higher Second Transplantation Rate Compared to Myeloablative Conditioning (BuCy) in Children with Inherited Metabolic Disorders. Biology of Blood and Marrow Transplantation, 2020, 26, 486-492.	2.0	11
65	Neurocognitive benchmarks following transplant for emerging cerebral adrenoleukodystrophy. Neurology, 2020, 95, e591-e600.	1.1	11
66	Variables affecting outcomes after allogeneic hematopoietic stem cell transplant for cerebral adrenoleukodystrophy. Blood Advances, 2022, 6, 1512-1524.	5.2	11
67	Association between APOE4 and biomarkers in cerebral adrenoleukodystrophy. Scientific Reports, 2019, 9, 7858.	3.3	10
68	N-acetylcysteine Provides Cytoprotection in Murine Oligodendrocytes through Heme Oxygenase-1 Activity. Biomedicines, 2020, 8, 240.	3.2	10
69	The Frequency of Carpal Tunnel Syndrome in Hurler Syndrome After Peritransplant Enzyme Replacement Therapy: A Retrospective Comparison. Journal of Hand Surgery, 2017, 42, 573.e1-573.e8.	1.6	9
70	Quality of life among boys with adrenoleukodystrophy following hematopoietic stem cell transplant. Child Neuropsychology, 2018, 24, 986-998.	1.3	9
71	Late Mortality after Allogeneic Blood or Marrow Transplantation for Inborn Errors of Metabolism: A Report from the Blood or Marrow Transplant Survivor Study-2 (BMTSS-2). Biology of Blood and Marrow Transplantation, 2019, 25, 328-334.	2.0	9
72	Consensus opinion on immune-mediated cytopenias after hematopoietic cell transplant for inherited metabolic disorders. Bone Marrow Transplantation, 2021, 56, 1238-1247.	2.4	9

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73	Metabolic Syndrome and Cardiovascular Risk Factors after Hematopoietic Cell Transplantation in Severe Mucopolysaccharidosis Type I (Hurler Syndrome). Biology of Blood and Marrow Transplantation, 2018, 24, 1289-1293.	2.0	8
74	TCIRG1 Transgenic Rescue of Osteoclast Function Using Induced Pluripotent Stem Cells Derived from Patients with Infantile Malignant Autosomal Recessive Osteopetrosis. Journal of Bone and Joint Surgery - Series A, 2019, 101, 1939-1947.	3.0	8
75	A Phase 2 Trial of KIR-Mismatched Unrelated Donor Transplantation Using in Vivo T Cell Depletion with Antithymocyte Globulin in Acute Myelogenous Leukemia: Children's Oncology Group AAML05P1 Study. Biology of Blood and Marrow Transplantation, 2020, 26, 712-717.	2.0	8
76	White matter alteration and cerebellar atrophy are hallmarks of brain MRI in alpha-mannosidosis. Molecular Genetics and Metabolism, 2021, 132, 189-197.	1.1	8
77	Multi-Institutional Assessments of Transplantation for Metabolic Disorders. Biology of Blood and Marrow Transplantation, 2013, 19, S58-S63.	2.0	7
78	Postâ€transplant adaptive function in childhood cerebral adrenoleukodystrophy. Annals of Clinical and Translational Neurology, 2018, 5, 252-261.	3.7	7
79	Post-transplant laronidase augmentation for children with Hurler syndrome: biochemical outcomes. Scientific Reports, 2019, 9, 14105.	3.3	7
80	Volume of Gadolinium Enhancement and Successful Repair of the Blood-Brain Barrier in Cerebral Adrenoleukodystrophy. Biology of Blood and Marrow Transplantation, 2020, 26, 1894-1899.	2.0	7
81	Dyskeratosis Congenita: Low Regimen-Related Toxicity Following Hematopoietic Cell Transplantation (HCT) Using a Reduced Intensity Conditioning Regimen.. Blood, 2007, 110, 2005-2005.	1.4	7
82	Evaluation of Neurofilament Light Chain as a Biomarker of Neurodegeneration in X-Linked Childhood Cerebral Adrenoleukodystrophy. Cells, 2022, 11, 913.	4.1	7
83	Population Pharmacokinetics of Clofarabine as Part of Pretransplantation Conditioning in Pediatric Subjects before Hematopoietic Cell Transplantation. Biology of Blood and Marrow Transplantation, 2019, 25, 1603-1610.	2.0	6
84	Hematopoietic cell transplantation for severe MPS I in the first six months of life: The heart of the matter. Molecular Genetics and Metabolism, 2019, 126, 117-120.	1.1	6
85	Failure of intrathecal allogeneic mesenchymal stem cells to halt progressive demyelination in two boys with cerebral adrenoleukodystrophy. Stem Cells Translational Medicine, 2020, 9, 554-558.	3.3	6
86	Outcome After Cord Blood Transplantation Using Busulfan Pharmacokinetics-Targeted Myeloablative Conditioning for Hurler Syndrome. Transplantation and Cellular Therapy, 2021, 27, 91.e1-91.e4.	1.2	6
87	Abnormal polyamine metabolism is unique to the neuropathic forms of MPS: potential for biomarker development and insight into pathogenesis. Human Molecular Genetics, 2017, 26, 3837-3849.	2.9	5
88	Quantitative brain MRI morphology in severe and attenuated forms of mucopolysaccharidosis type I. Molecular Genetics and Metabolism, 2022, 135, 122-132.	1.1	5
89	G-CSF Mobilized Human Mesenchymal Stem Cells Are Found in the Peripheral Blood and Have Telomere Limited Growth Potential.. Blood, 2006, 108, 4246-4246.	1.4	4
90	Hematopoietic stem cell transplant for Hurler syndrome: does using bone marrow or umbilical cord blood make a difference?. Blood Advances, 2022, 6, 6023-6027.	5.2	4

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91	Long-Term Results of Carpal Tunnel and Trigger Finger Releases in a Patient with Hurler Syndrome. JBJS Case Connector, 2015, 5, e27.	0.3	3
92	Population Pharmacokinetic Analysis of Nâ€Acetylcysteine in Pediatric Patients With Inherited Metabolic Disorders Undergoing Hematopoietic Stem Cell Transplant. Journal of Clinical Pharmacology, 2021, 61, 1638-1645.	2.0	3
93	Differential outcomes for frontal versus posterior demyelination in childhood cerebral adrenoleukodystrophy. Journal of Inherited Metabolic Disease, 2021, 44, 1434-1440.	3.6	3
94	Pulmonary Risk Factors in Allogeneic Transplantation for Hurler Syndrome.. Blood, 2004, 104, 2154-2154.	1.4	3
95	The Changing Patterns of Graft Failure in MPS1H, Hurler Syndrome: A Review of 30-Years Experience. Blood, 2016, 128, 4700-4700.	1.4	3
96	Busulfan dose Recommendation in Inherited Metabolic Disorders: Population Pharmacokinetic Analysis. Transplantation and Cellular Therapy, 2022, 28, 104.e1-104.e7.	1.2	3
97	Primary Adrenal Insufficiency in a Boy with Type I Diabetes: The Importance of Considering X-linked Adrenoleukodystrophy. Journal of the Endocrine Society, 2022, 6, bvac039.	0.2	3
98	Translational and Clinical Pharmacology Considerations in Drug Repurposing for Xâ€linked Adrenoleukodystrophyâ€A Rare Peroxisomal Disorder. British Journal of Clinical Pharmacology, 2021, , .	2.4	2
99	Transfection of the mouse ICAM-1 gene into murine neuroblastoma enhances susceptibility to lysis, reduces in vivo tumorigenicity and decreases ICAM-2-dependent killing. Cancer Immunology, Immunotherapy, 1994, 38, 135-141.	4.2	2
100	Hematopoietic cell transplantation for sialidosis type I. Molecular Genetics and Metabolism Reports, 2022, 30, 100832.	1.1	2
101	Isoprostanoid Plasma Levels Are Relevant to Cerebral Adrenoleukodystrophy Disease. Life, 2022, 12, 146.	2.4	2
102	Quantifying medical manifestations in hurler syndrome with the infant physical symptom score: Associations with long-term physical and adaptive outcomes. Molecular Genetics and Metabolism, 2022, , .	1.1	2
103	Isokinetic muscle strength differences in patients with mucopolysaccharidosis I, II, and VI. Journal of Pediatric Rehabilitation Medicine, 2014, 7, 353-360.	0.5	1
104	Fast, sensitive method for trisaccharide biomarker detection in mucopolysaccharidosis type 1. Scientific Reports, 2018, 8, 3681.	3.3	1
105	Successful hematopoietic cell transplantation following cardiac transplantation in two pediatric patients. Pediatric Transplantation, 2018, 22, e13103.	1.0	1
106	Cellular Therapy in Rare Childhood Neurologic Disease: Lessons, Outcomes, and Access. Journal of Child Neurology, 2018, 33, 877-881.	1.4	1
107	Stable Gene Transfer and Expression in Human Primary T-Cells by the Sleeping Beauty Transposon System.. Blood, 2005, 106, 5539-5539.	1.4	1
108	Glycosaminoglycans as Anticoagulants in Mucopolysaccharidosis Type I (MPS I).. Blood, 2007, 110, 2160.5-2160.5.	1.4	1

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109	High-Exposure, Targeted Daily Busulfan and Fludarabine-Based Conditioning for Children Undergoing Hematopoietic Stem Cell Transplantation for Inherited Metabolic Disorders: Outcomes at a Single Center. Blood, 2016, 128, 2191-2191.	1.4	1
110	Unusual Cortical Phenotype After Hematopoietic Stem Cell Transplantation in a Patient With Osteopetrosis. JBMR Plus, 2022, 6, .	2.7	1
111	Glycoprotein nonmetastatic melanoma protein B (GNMPB) as a novel biomarker for cerebral adrenoleukodystrophy. Scientific Reports, 2022, 12, 7985.	3.3	1
112	Adrenal Insufficiency in an Adolescent Boy With Type 1 Diabetes Mellitus - the Importance of Considering X-Linked Adrenoleukodystrophy. Journal of the Endocrine Society, 2021, 5, A110-A110.	0.2	0
113	Glycosaminoglycans as Anticoagulants in Mucopolysaccharidosis Type I (MPS I).. Blood, 2007, 110, 3951-3951.	1.4	0
114	Brain Sparing Conditioning Regimen and Umbilical Cord Blood Transplantation for Inherited High Risk Neurologic Metabolic Diseases.. Blood, 2007, 110, 3009-3009.	1.4	0
115	Patient and Parental Perspectives on Long-Term Adaptive, Behavioral and Functional Outcomes Following Allogeneic Hematopoietic Cell Transplantation for Inherited Metabolic Disorders. Blood, 2015, 126, 4351-4351.	1.4	0
116	Neurocognitive Trajectory of Patients with Childhood Cerebral Adrenoleukodystrophy Who Received Allogeneic Hematopoietic Cell Transplantation at an Early Stage of Cerebral Disease. Blood, 2016, 128, 4682-4682.	1.4	0
117	Burden of Morbidity after Allogeneic Blood or Marrow Transplantation for Inborn Errors of Metabolism: A BMT Survivor Study Report. Transplantation and Cellular Therapy, 2022, 28, 157.e1-157.e9.	1.2	0