Punam Malik

List of Publications by Year
in descending order

109 papers	3,554 citations	147801 31 h-index	149698 56 g-index
114 all docs	114 docs citations	114 times ranked	4853 citing authors

#	Article	IF	Citations
1	Towards access for all: 1st Working Group Report for the Global Gene Therapy Initiative (GGTI). Gene Therapy, 2023, 30, 216-221.	4.5	6
2	Successful use of venoâ€venous extracorporeal membrane oxygenation for acute chest syndrome in a child with sickle cell disease and SARSâ€CoVâ€2. Pediatric Pulmonology, 2022, , .	2.0	2
3	NRASQ61R mutation in human endothelial cells causes vascular malformations. Angiogenesis, 2022, 25, 331-342.	7.2	8
4	Genetic Variants Associated with Therapy-Related Cardiomyopathy among Childhood Cancer Survivors of African Ancestry. Cancer Research, 2021, 81, 2556-2565.	0.9	24
5	Longitudinal effect of disease-modifying therapy on tricuspid regurgitant velocity in children with sickle cell anemia. Blood Advances, 2021, 5, 89-98.	5.2	6
6	Implementation of nearâ€universal hydroxyurea uptake among children with sickle cell anemia: A singleâ€center experience. Pediatric Blood and Cancer, 2021, 68, e29008.	1.5	5
7	Cardiac pathophysiology in sickle cell disease. Journal of Thrombosis and Thrombolysis, 2021, 52, 248-259.	2.1	1
8	FT-4202, an oral PKR activator, has potent antisickling effects and improves RBC survival and Hb levels in SCA mice. Blood Advances, 2021, 5, 2385-2390.	5.2	16
9	Early initiation of hydroxyurea (hydroxycarbamide) using individualised, pharmacokineticsâ€guided dosing can produce sustained and nearly pancellular expression of fetal haemoglobin in children with sickle cell anaemia. British Journal of Haematology, 2021, 194, 617-625.	2.5	16
10	Rapid and automated quantitation of dense red blood cells: A robust biomarker of hydroxyurea treatment response. Blood Cells, Molecules, and Diseases, 2021, 90, 102576.	1.4	2
11	Safe and EffectiveIn VivoTargeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development. Human Gene Therapy, 2021, 32, 31-42.	2.7	15
12	Association of Thrombospondin-1 Gene Polymorphism with Elevated Tricuspid Regurgitant Velocity in Sickle Cell Anemia. Blood, 2021, 138, 2027-2027.	1.4	0
13	Successful HPV Vaccination in Adolescents with Sickle Cell Disease Following a Quality Improvement Bundle Intervention. Blood, 2021, 138, 914-914.	1.4	2
14	Assessment of Cardiac Abnormalities in Sickle Cell Disease Patients Using Cardiac Magnetic Resonance Imaging (CMR). Blood, 2021, 138, 3110-3110.	1.4	4
15	Safety and Efficacy of Aru-1801 in Patients with Sickle Cell Disease: Early Results from the Phase 1/2 Momentum Study of a Modified Gamma Globin Gene Therapy and Reduced Intensity Conditioning. Blood, 2021, 138, 3970-3970.	1.4	11
16	Pediatric Cardio-Oncology Medicine: A New Approach in Cardiovascular Care. Children, 2021, 8, 1200.	1.5	2
17	Effective hematopoietic stem cell-based gene therapy in a murine model of hereditary pulmonary alveolar proteinosis. Haematologica, 2020, 105, 1147-1157.	3.5	7
18	Left atrial dysfunction in sickle cell anemia is associated with diffuse myocardial fibrosis, increased right ventricular pressure and reduced exercise capacity. Scientific Reports, 2020, 10, 1767.	3.3	11

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19	Progression of albuminuria in patients with sickle cell anemia: a multicenter, longitudinal study. Blood Advances, 2020, 4, 1501-1511.	5.2	28
20	FT-4202, an Allosteric Activator of Pyruvate Kinase-R, Demonstrates Proof of Mechanism and Proof of Concept after a Single Dose and after Multiple Daily Doses in a Phase 1 Study of Patients with Sickle Cell Disease. Blood, 2020, 136, 19-20.	1.4	12
21	Oral Administration of FT-4202, an Allosteric Activator of Pyruvate Kinase-R, Has Potent Anti-Sickling Effects in a Sickle Cell Anemia (SCA) Mouse Model, Resulting in Improved RBC Survival and Hemoglobin Levels. Blood, 2020, 136, 21-22.	1.4	2
22	Early Results from a Phase 1/2 Study of Aru-1801 Gene Therapy for Sickle Cell Disease (SCD): Manufacturing Process Enhancements Improve Efficacy of a Modified Gamma Globin Lentivirus Vector and Reduced Intensity Conditioning Transplant. Blood, 2020, 136, 20-21.	1.4	13
23	Drug Therapies for the Management of Sickle Cell Disease. F1000Research, 2020, 9, 592.	1.6	29
24	Angiotensin Signaling Is Essential for Stress Erythropoiesis but Results in Retention of Dysfunctional Mitochondria in Erythrocytes That Generate Excessive Reactive Oxygen Species. Blood, 2020, 136, 31-32.	1.4	1
25	Rapid and Automated Quantitation of Dense Red Blood Cells: A Robust Biomarker of Therapeutic Response to Early Initiation of Hydroxyurea in Young Children with Sickle Cell Anemia. Blood, 2020, 136, 16-17.	1.4	0
26	Bone Marrow (BM) Delivery of Genetically-Modified (gm) Adult CD34+ Hematopoietic Stem and Progenitor Cells (HSPC) Improves Homing and Engraftment of Short-Term Progenitors over Long-Term Repopulating Hematopoietic Stem Cells. Blood, 2020, 136, 22-23.	1.4	0
27	Increased Hydroxyurea Prescribing Practices over Ten Years with Improved Clinical Outcomes in Children with Sickle Cell Anemia: A Single Center's Experience. Blood, 2020, 136, 34-34.	1.4	0
28	A Versatile Tool for the Quantification of CRISPR/Cas9-Induced Genome Editing Events in Human Hematopoietic Cell Lines and Hematopoietic Stem/Progenitor Cells. Journal of Molecular Biology, 2019, 431, 102-110.	4.2	14
29	CRISPR-Cas9 fusion to dominant-negative 53BP1 enhances HDR and inhibits NHEJ specifically at Cas9 target sites. Nature Communications, 2019, 10, 2866.	12.8	124
30	Robust clinical and laboratory response to hydroxyurea using pharmacokinetically guided dosing for young children with sickle cell anemia. American Journal of Hematology, 2019, 94, 871-879.	4.1	51
31	Abnormal submaximal cardiopulmonary exercise parameters predict impaired peak exercise performance in sickle cell anemia patients. Pediatric Blood and Cancer, 2019, 66, e27703.	1.5	6
32	Role of the coagulation system in the pathogenesis of sickle cell disease. Blood Advances, 2019, 3, 3170-3180.	5.2	38
33	Elimination of the fibrinogen integrin Î±MÎ²2-binding motif improves renal pathology in mice with sickle cell anemia. Blood Advances, 2019, 3, 1519-1532.	5.2	16
34	End points for sickle cell disease clinical trials: renal and cardiopulmonary, cure, and low-resource settings. Blood Advances, 2019, 3, 4002-4020.	5.2	21
35	Therapeutic strategies for sickle cell disease: towards a multi-agent approach. Nature Reviews Drug Discovery, 2019, 18, 139-158.	46.4	116
36	Phase 1 Single (SAD) and Multiple Ascending Dose (MAD) Studies of the Safety, Tolerability, Pharmacokinetics (PK) and Pharmacodynamics (PD) of FT-4202, an Allosteric Activator of Pyruvate Kinase-R, in Healthy and Sickle Cell Disease Subjects. Blood, 2019, 134, 616-616.	1.4	6

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37	Progression of Albuminuria in Sickle Cell Anemia: A Multicenter, Longitudinal Study. Blood, 2019, 134, 1004-1004.	1.4	0
38	Longitudinal Effect of Hydroxyurea Therapy on Left Ventricular Diastolic Function in Sickle Cell Anemia. Blood, 2019, 134, 1006-1006.	1.4	0
39	Angiotensin receptor signaling in sickle cell anemia has a renoâ€protective effect on urine concentrating ability but results in sickle glomerulopathy. American Journal of Hematology, 2018, 93, E177-E181.	4.1	15
40	Production and Purification of Baculovirus for Gene Therapy Application. Journal of Visualized Experiments, 2018, , .	0.3	6
41	Foamy Virus Vector Carries a Strong Insulator in Its Long Terminal Repeat Which Reduces Its Genotoxic Potential. Journal of Virology, 2018, 92, .	3.4	12
42	Placenta growth factor mediated gene regulation in sickle cell disease. Blood Reviews, 2018, 32, 61-70.	5.7	8
43	Diastolic dysfunction is associated with exercise impairment in patients with sickle cell anemia. Pediatric Blood and Cancer, 2018, 65, e27113.	1.5	16
44	Genetic Therapies for Sickle Cell Disease. Pediatric Clinics of North America, 2018, 65, 465-480.	1.8	7
45	Gene Therapy for Sickle Cell Anemia Using a Modified Gamma Globin Lentivirus Vector and Reduced Intensity Conditioning Transplant Shows Promising Correction of the Disease Phenotype. Blood, 2018, 132, 1021-1021.	1.4	23
46	p190-B RhoGAP and intracellular cytokine signals balance hematopoietic stem and progenitor cell self-renewal and differentiation. Nature Communications, 2017, 8, 14382.	12.8	35
47	A reappraisal of the mechanisms underlying the cardiac complications of sickle cell anemia. Pediatric Blood and Cancer, 2017, 64, e26607.	1.5	8
48	Association between diffuse myocardial fibrosis and diastolic dysfunction in sickle cell anemia. Blood, 2017, 130, 205-213.	1.4	86
49	Losartan for the nephropathy of sickle cell anemia: A phaseâ€2, multicenter trial. American Journal of Hematology, 2017, 92, E520-E528.	4.1	36
50	Patient Perspectives on Gene Transfer Therapy for Sickle Cell Disease. Advances in Therapy, 2017, 34, 2007-2021.	2.9	22
51	CRISPR/Cas9 in allergic and immunologic diseases. Expert Review of Clinical Immunology, 2017, 13, 5-9.	3.0	8
52	Cerebral Metastasis of Hepatoblastoma: A Review. Journal of Pediatric Hematology/Oncology, 2016, 38, 279-282.	0.6	10
53	The potential of gene therapy approaches for the treatment of hemoglobinopathies: achievements and challenges. Therapeutic Advances in Hematology, 2016, 7, 302-315.	2.5	33
54	Purification of baculovirus vectors using heparin affinity chromatography. Molecular Therapy - Methods and Clinical Development, 2016, 3, 16071.	4.1	15

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55	Gene Therapy for Hemoglobinopathies: Tremendous Successes and Remaining Caveats. Molecular Therapy, 2016, 24, 668-670.	8.2	19
56	Integrated Genomic Analysis of Diverse Induced Pluripotent Stem Cells from the Progenitor Cell Biology Consortium. Stem Cell Reports, 2016, 7, 110-125.	4.8	101
57	High Level of Perforin Expression Is Required for Effective Correction of Hemophagocytic Lymphohistiocytosis. Human Gene Therapy, 2016, 27, 847-859.	2.7	10
58	Activated Transcription Factor 3 in Association with Histone Deacetylase 6 Negatively Regulates MicroRNA 199a2 Transcription by Chromatin Remodeling and Reduces Endothelin-1 Expression. Molecular and Cellular Biology, 2016, 36, 2838-2854.	2.3	12
59	Production and purification of high-titer foamy virus vector for the treatment of leukocyte adhesion deficiency. Molecular Therapy - Methods and Clinical Development, 2016, 3, 16004.	4.1	13
60	Sickle cell anemia mice develop a unique cardiomyopathy with restrictive physiology. Proceedings of the National Academy of Sciences of the United States of America, 2016, 113, E5182-91.	7.1	65
61	Cardiomyopathy With Restrictive Physiology in Sickle CellÂDisease. JACC: Cardiovascular Imaging, 2016, 9, 243-252.	5.3	97
62	Individualized Dosing of Hydroxyurea for Children with Sickle Cell Anemia Using a Population Pharmacokinetic-Based Model: The TREAT Study. Blood, 2016, 128, 3652-3652.	1.4	2
63	Reactive Oxygen Species Produced by NADPH Oxidase Contribute to Cardiac Pathology in a Mouse Model of Sickle Cell Disease. Blood, 2016, 128, 853-853.	1.4	0
64	Diffuse Myocardial Fibrosis Is a Common Feature of Sickle Cell Anemia That Is Associated with Diastolic Dysfunction and Restrictive Cardiac Physiology. Blood, 2016, 128, 8-8.	1.4	1
65	Foamy Virus Backbone Has Insulator Properties Which Remarkably Reduce Its Genotoxicity Potential. Blood, 2016, 128, 1002-1002.	1.4	0
66	A Multi-Center, Phase-2 Trial of Losartan for the Nephropathy of Sickle Cell Anemia. Blood, 2016, 128, 265-265.	1.4	10
67	Gene therapy for hemoglobin disorders - a mini-review. , 2016, 1, 25-31.		5
68	Peroxisome proliferator-activated receptor-Î±-mediated transcription of miR-301a and miR-454 and their host gene SKA2 regulates endothelin-1 and PAI-1 expression in sickle cell disease. Bioscience Reports, 2015, 35, .	2.4	18
69	Genetic diminution of circulating prothrombin ameliorates multiorgan pathologies in sickle cell disease mice. Blood, 2015, 126, 1844-1855.	1.4	51
70	Erythropoietin-mediated expression of placenta growth factor is regulated via activation of hypoxia-inducible factor-1Î± and post-transcriptionally by miR-214 in sickle cell disease. Biochemical Journal, 2015, 468, 409-423.	3.7	24
71	Vasculopathy-associated hyperangiotensinemia mobilizes haematopoietic stem cells/progenitors through endothelial AT2R and cytoskeletal dysregulation. Nature Communications, 2015, 6, 5914.	12.8	15
72	Perforin Gene Transfer Into Hematopoietic Stem Cells Improves Immune Dysregulation in Murine Models of Perforin Deficiency. Molecular Therapy, 2015, 23, 737-745.	8.2	41

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73	MicroRNA 648 Targets ET-1 mRNA and Is Cotranscriptionally Regulated with MICAL3 by PAX5. Molecular and Cellular Biology, 2015, 35, 514-528.	2.3	21
74	Placenta growth factor augments airway hyperresponsiveness via leukotrienes and IL-13. Journal of Clinical Investigation, 2015, 126, 571-584.	8.2	33
75	Pigtailed macaques as a model to study long-term safety of lentivirus vector-mediated gene therapy for hemoglobinopathies. Molecular Therapy - Methods and Clinical Development, 2014, 1, 14055.	4.1	10
76	Peroxisome Proliferator-activated Receptor-Î±-mediated Transcription of miR-199a2 Attenuates Endothelin-1 Expression via Hypoxia-inducible Factor-1Î±. Journal of Biological Chemistry, 2014, 289, 36031-36047.	3.4	29
77	Gene Therapy for Hemoglobinopathies. Hematology/Oncology Clinics of North America, 2014, 28, 199-216.	2.2	52
78	Pulmonary macrophage transplantation therapy. Nature, 2014, 514, 450-454.	27.8	249
79	Increased Oxidative Stress In Sickle Cell Disease Activates The Renin-Angiotensin-TGF-Î² Pathway To Mediate Sickle Nephropathy. Blood, 2013, 122, 2211-2211.	1.4	8
80	A Phase I Trial Of Zileuton In Sickle Cell Disease. Blood, 2013, 122, 993-993.	1.4	7
81	Diminished Multi-Organ Pathologies and Inflammation Associated With Sickle Cell Disease In Mice With Genetically Limited Prothrombin Levels. Blood, 2013, 122, 729-729.	1.4	0
82	Safety Of a Gamma Globin Expressing Lentivirus Vector In a Non-Human Primate Model For Gene Therapy Of Sickle Cell Disease. Blood, 2013, 122, 2896-2896.	1.4	1
83	Patient Perceptions Of Treatments In SCD: Implications For Gene Transfer Therapy. Blood, 2013, 122, 5555-5555.	1.4	0
84	Beyond the Definitions of the Phenotypic Complications of Sickle Cell Disease: An Update on Management. Scientific World Journal, The, 2012, 2012, 1-55.	2.1	125
85	Gene Therapy for Hemophagocytic Lymphohistiocytosis (HLH): Fixing a Criticial â€˜Circuit Breakerâ€™ in the Immune System.. Blood, 2012, 120, 3158-3158.	1.4	0
86	Hyperangiotensinemia Induces Stem Cell/Progenitor Mobilization and De-Adhesion From BM Endothelial Cells Through AT2R Signaling and Inhibition of RhoA Activity. Blood, 2012, 120, 3466-3466.	1.4	8
87	Involvement of miR-30c and miR-301a in immediate induction of plasminogen activator inhibitor-1 by placental growth factor in human pulmonary endothelial cells. Biochemical Journal, 2011, 434, 473-482.	3.7	68
88	Biomarkers for early detection of sickle nephropathy. American Journal of Hematology, 2011, 86, 559-566.	4.1	60
89	A Phase 2 Clinical Study of HQK-1001 (2,2-dimethylbutyrate, sodium salt), a Fetal Hemoglobin Inducer, in Patients with Sickle Cell Disease. Blood, 2011, 118, 1066-1066.	1.4	1
90	Somatic Gene Therapy for X-Linked Severe Combined Immunodeficiency Using a Self-Inactivating Modified Gammaretroviral Vector Results in An Improved Preclinical Safety Profile and Early Clinical Efficacy in a Human Patient. Blood, 2011, 118, 164-164.	1.4	3

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91	Use of the in Vitro Immortalization Assay to Quantify the Impact of Integration Spectrum and Vector Design on Insertional Mutagenesis. Blood, 2011, 118, 3123-3123.	1.4	1
92	Sickle Cell Disease Is Associated with Reduced Adenosine Deaminase Catalytic Activity, Resulting in Altered Adenosine Metabolism. Blood, 2011, 118, 1078-1078.	1.4	0
93	High levels of placenta growth factor in sickle cell disease promote pulmonary hypertension. Blood, 2010, 116, 109-112.	1.4	77
94	Placenta Growth Factor (PlGF), a Novel Inducer of Plasminogen Activator Inhibitor-1 (PAI-1) in Sickle Cell Disease (SCD). Journal of Biological Chemistry, 2010, 285, 16713-16722.	3.4	35
95	Genetic Therapy for Beta-Thalassemia: From the Bench to the Bedside. Hematology American Society of Hematology Education Program, 2010, 2010, 445-450.	2.5	36
96	Mechanism of Reduction in Titers From Lentivirus Vectors Carrying Large Inserts in the 3â€²LTR. Molecular Therapy, 2009, 17, 1527-1536.	8.2	62
97	Genotoxic Potential of Lineage-specific Lentivirus Vectors Carrying the Î²-Globin Locus Control Region. Molecular Therapy, 2009, 17, 1929-1937.	8.2	74
98	Placenta growth factor induces 5-lipoxygenaseâ€“activating protein to increase leukotriene formation in sickle cell disease. Blood, 2009, 113, 1129-1138.	1.4	40
99	A novel human gamma-globin gene vector for genetic correction of sickle cell anemia in a humanized sickle mouse model: critical determinants for successful correction. Blood, 2009, 114, 1174-1185.	1.4	94
100	The 3â€² Region of the Chicken Hypersensitive Site-4 Insulator Has Properties Similar to Its Core and Is Required for Full Insulator Activity. PLoS ONE, 2009, 4, e6995.	2.5	58
101	Placenta growth factor augments endothelin-1 and endothelin-B receptor expression via hypoxia-inducible factor-1Î±. Blood, 2008, 112, 856-865.	1.4	78
102	Improved Human Î²-globin Expression from Self-inactivating Lentiviral Vectors Carrying the Chicken Hypersensitive Site-4 (cHS4) Insulator Element. Molecular Therapy, 2007, 15, 1863-1871.	8.2	120
103	Genetically Engineered Cures: Gene Therapy for Sickle Cell Disease. , 2007, , 295-309.		1
104	Pathophysiology and therapy for haemoglobinopathies; Part I: sickle cell disease. Expert Reviews in Molecular Medicine, 2006, 8, 1-23.	3.9	71
105	Self-Inactivating Lentiviral Vectors Resist Proviral Methylation but Do Not Confer Position-Independent Expression in Hematopoietic Stem Cells. Molecular Therapy, 2004, 10, 249-259.	8.2	30
106	Successful correction of the human Î²-thalassemia major phenotype using a lentiviral vector. Blood, 2004, 104, 3445-3453.	1.4	184
107	Placenta growth factor activates monocytes and correlates with sickle cell disease severity. Blood, 2003, 102, 1506-1514.	1.4	141
108	Mechanism of monocyte activation and expression of proinflammatory cytochemokines by placenta growth factor. Blood, 2003, 102, 1515-1524.	1.4	228

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109	High-level erythroid-specific gene expression in primary human and murine hematopoietic cells with self-inactivating lentiviral vectors. Blood, 2001, 98, 2664-2672.	1.4	106