

# Punam Malik

## List of Publications by Year in descending order

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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	Pulmonary macrophage transplantation therapy. <i>Nature</i> , 2014, 514, 450-454.	27.8	249
2	Mechanism of monocyte activation and expression of proinflammatory cytochemokines by placenta growth factor. <i>Blood</i> , 2003, 102, 1515-1524.	1.4	228
3	Successful correction of the human $\beta^0$ -thalassemia major phenotype using a lentiviral vector. <i>Blood</i> , 2004, 104, 3445-3453.	1.4	184
4	Placenta growth factor activates monocytes and correlates with sickle cell disease severity. <i>Blood</i> , 2003, 102, 1506-1514.	1.4	141
5	Beyond the Definitions of the Phenotypic Complications of Sickle Cell Disease: An Update on Management. <i>Scientific World Journal</i> , The, 2012, 2012, 1-55.	2.1	125
6	CRISPR-Cas9 fusion to dominant-negative 53BP1 enhances HDR and inhibits NHEJ specifically at Cas9 target sites. <i>Nature Communications</i> , 2019, 10, 2866.	12.8	124
7	Improved Human $\beta^0$ -globin Expression from Self-inactivating Lentiviral Vectors Carrying the Chicken Hypersensitive Site-4 (cHS4) Insulator Element. <i>Molecular Therapy</i> , 2007, 15, 1863-1871.	8.2	120
8	Therapeutic strategies for sickle cell disease: towards a multi-agent approach. <i>Nature Reviews Drug Discovery</i> , 2019, 18, 139-158.	46.4	116
9	High-level erythroid-specific gene expression in primary human and murine hematopoietic cells with self-inactivating lentiviral vectors. <i>Blood</i> , 2001, 98, 2664-2672.	1.4	106
10	Integrated Genomic Analysis of Diverse Induced Pluripotent Stem Cells from the Progenitor Cell Biology Consortium. <i>Stem Cell Reports</i> , 2016, 7, 110-125.	4.8	101
11	Cardiomyopathy With Restrictive Physiology in Sickle Cell Disease. <i>JACC: Cardiovascular Imaging</i> , 2016, 9, 243-252.	5.3	97
12	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. <i>Blood</i> , 2009, 114, 1174-1185.	1.4	94
13	Association between diffuse myocardial fibrosis and diastolic dysfunction in sickle cell anemia. <i>Blood</i> , 2017, 130, 205-213.	1.4	86
14	Placenta growth factor augments endothelin-1 and endothelin-B receptor expression via hypoxia-inducible factor-1. <i>Blood</i> , 2008, 112, 856-865.	1.4	78
15	High levels of placenta growth factor in sickle cell disease promote pulmonary hypertension. <i>Blood</i> , 2010, 116, 109-112.	1.4	77
16	Genotoxic Potential of Lineage-specific Lentivirus Vectors Carrying the $\beta^0$ -Globin Locus Control Region. <i>Molecular Therapy</i> , 2009, 17, 1929-1937.	8.2	74
17	Pathophysiology and therapy for haemoglobinopathies; Part I: sickle cell disease. <i>Expert Reviews in Molecular Medicine</i> , 2006, 8, 1-23.	3.9	71
18	Involvement of miR-30c and miR-301a in immediate induction of plasminogen activator inhibitor-1 by placental growth factor in human pulmonary endothelial cells. <i>Biochemical Journal</i> , 2011, 434, 473-482.	3.7	68

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19	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
20	Mechanism of Reduction in Titers From Lentivirus Vectors Carrying Large Inserts in the 3'LTR. Molecular Therapy, 2009, 17, 1527-1536.	8.2	62
21	Biomarkers for early detection of sickle nephropathy. American Journal of Hematology, 2011, 86, 559-566.	4.1	60
22	The 3' 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
23	Gene Therapy for Hemoglobinopathies. Hematology/Oncology Clinics of North America, 2014, 28, 199-216.	2.2	52
24	Genetic diminution of circulating prothrombin ameliorates multiorgan pathologies in sickle cell disease mice. Blood, 2015, 126, 1844-1855.	1.4	51
25	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
26	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
27	Placenta growth factor induces 5-lipoxygenase-activating protein to increase leukotriene formation in sickle cell disease. Blood, 2009, 113, 1129-1138.	1.4	40
28	Role of the coagulation system in the pathogenesis of sickle cell disease. Blood Advances, 2019, 3, 3170-3180.	5.2	38
29	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
30	Losartan for the nephropathy of sickle cell anemia: A phase 2, multicenter trial. American Journal of Hematology, 2017, 92, E520-E528.	4.1	36
31	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
32	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
33	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
34	Placenta growth factor augments airway hyperresponsiveness via leukotrienes and IL-13. Journal of Clinical Investigation, 2015, 126, 571-584.	8.2	33
35	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
36	Peroxisome Proliferator-activated Receptor- $\alpha$ -mediated Transcription of miR-199a2 Attenuates Endothelin-1 Expression via Hypoxia-inducible Factor-1 $\alpha$ . Journal of Biological Chemistry, 2014, 289, 36031-36047.	3.4	29

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37	Drug Therapies for the Management of Sickle Cell Disease. <i>F1000Research</i> , 2020, 9, 592.	1.6	29
38	Progression of albuminuria in patients with sickle cell anemia: a multicenter, longitudinal study. <i>Blood Advances</i> , 2020, 4, 1501-1511.	5.2	28
39	Erythropoietin-mediated expression of placenta growth factor is regulated via activation of hypoxia-inducible factor-1 $\alpha$ and post-transcriptionally by miR-214 in sickle cell disease. <i>Biochemical Journal</i> , 2015, 468, 409-423.	3.7	24
40	Genetic Variants Associated with Therapy-Related Cardiomyopathy among Childhood Cancer Survivors of African Ancestry. <i>Cancer Research</i> , 2021, 81, 2556-2565.	0.9	24
41	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. <i>Blood</i> , 2018, 132, 1021-1021.	1.4	23
42	Patient Perspectives on Gene Transfer Therapy for Sickle Cell Disease. <i>Advances in Therapy</i> , 2017, 34, 2007-2021.	2.9	22
43	MicroRNA 648 Targets ET-1 mRNA and Is Cotranscriptionally Regulated with <i>MICAL3</i> by PAX5. <i>Molecular and Cellular Biology</i> , 2015, 35, 514-528.	2.3	21
44	End points for sickle cell disease clinical trials: renal and cardiopulmonary, cure, and low-resource settings. <i>Blood Advances</i> , 2019, 3, 4002-4020.	5.2	21
45	Gene Therapy for Hemoglobinopathies: Tremendous Successes and Remaining Caveats. <i>Molecular Therapy</i> , 2016, 24, 668-670.	8.2	19
46	Peroxisome proliferator-activated receptor- $\alpha$ -mediated transcription of <i>miR-301a</i> and <i>miR-454</i> and their host gene <i>SKA2</i> regulates endothelin-1 and PAI-1 expression in sickle cell disease. <i>Bioscience Reports</i> , 2015, 35, .	2.4	18
47	Diastolic dysfunction is associated with exercise impairment in patients with sickle cell anemia. <i>Pediatric Blood and Cancer</i> , 2018, 65, e27113.	1.5	16
48	Elimination of the fibrinogen integrin $\alpha$ 2 $\beta$ 2-binding motif improves renal pathology in mice with sickle cell anemia. <i>Blood Advances</i> , 2019, 3, 1519-1532.	5.2	16
49	FT-4202, an oral PKR activator, has potent antisickling effects and improves RBC survival and Hb levels in SCA mice. <i>Blood Advances</i> , 2021, 5, 2385-2390.	5.2	16
50	Early initiation of hydroxyurea (hydroxycarbamide) using individualised, pharmacokinetics-guided dosing can produce sustained and nearly pan-cellular expression of fetal haemoglobin in children with sickle cell anaemia. <i>British Journal of Haematology</i> , 2021, 194, 617-625.	2.5	16
51	Vasculopathy-associated hyperangiotensinemia mobilizes haematopoietic stem cells/progenitors through endothelial AT2R and cytoskeletal dysregulation. <i>Nature Communications</i> , 2015, 6, 5914.	12.8	15
52	Purification of baculovirus vectors using heparin affinity chromatography. <i>Molecular Therapy - Methods and Clinical Development</i> , 2016, 3, 16071.	4.1	15
53	Angiotensin receptor signaling in sickle cell anemia has a renoprotective effect on urine concentrating ability but results in sickle glomerulopathy. <i>American Journal of Hematology</i> , 2018, 93, E177-E181.	4.1	15
54	Safe and Effective <i>In Vivo</i> Targeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development. <i>Human Gene Therapy</i> , 2021, 32, 31-42.	2.7	15

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55	A Versatile Tool for the Quantification of CRISPR/Cas9-Induced Genome Editing Events in Human Hematopoietic Cell Lines and Hematopoietic Stem/Progenitor Cells. <i>Journal of Molecular Biology</i> , 2019, 431, 102-110.	4.2	14
56	Production and purification of high-titer foamy virus vector for the treatment of leukocyte adhesion deficiency. <i>Molecular Therapy - Methods and Clinical Development</i> , 2016, 3, 16004.	4.1	13
57	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. <i>Blood</i> , 2020, 136, 20-21.	1.4	13
58	Activated Transcription Factor 3 in Association with Histone Deacetylase 6 Negatively Regulates MicroRNA 199a2 Transcription by Chromatin Remodeling and Reduces Endothelin-1 Expression. <i>Molecular and Cellular Biology</i> , 2016, 36, 2838-2854.	2.3	12
59	Foamy Virus Vector Carries a Strong Insulator in Its Long Terminal Repeat Which Reduces Its Genotoxic Potential. <i>Journal of Virology</i> , 2018, 92, .	3.4	12
60	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. <i>Blood</i> , 2020, 136, 19-20.	1.4	12
61	Left atrial dysfunction in sickle cell anemia is associated with diffuse myocardial fibrosis, increased right ventricular pressure and reduced exercise capacity. <i>Scientific Reports</i> , 2020, 10, 1767.	3.3	11
62	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. <i>Blood</i> , 2021, 138, 3970-3970.	1.4	11
63	Pigtailed macaques as a model to study long-term safety of lentivirus vector-mediated gene therapy for hemoglobinopathies. <i>Molecular Therapy - Methods and Clinical Development</i> , 2014, 1, 14055.	4.1	10
64	Cerebral Metastasis of Hepatoblastoma: A Review. <i>Journal of Pediatric Hematology/Oncology</i> , 2016, 38, 279-282.	0.6	10
65	High Level of Perforin Expression Is Required for Effective Correction of Hemophagocytic Lymphohistiocytosis. <i>Human Gene Therapy</i> , 2016, 27, 847-859.	2.7	10
66	A Multi-Center, Phase-2 Trial of Losartan for the Nephropathy of Sickle Cell Anemia. <i>Blood</i> , 2016, 128, 265-265.	1.4	10
67	A reappraisal of the mechanisms underlying the cardiac complications of sickle cell anemia. <i>Pediatric Blood and Cancer</i> , 2017, 64, e26607.	1.5	8
68	CRISPR/Cas9 in allergic and immunologic diseases. <i>Expert Review of Clinical Immunology</i> , 2017, 13, 5-9.	3.0	8
69	Placenta growth factor mediated gene regulation in sickle cell disease. <i>Blood Reviews</i> , 2018, 32, 61-70.	5.7	8
70	Increased Oxidative Stress In Sickle Cell Disease Activates The Renin-Angiotensin-TGF- $\beta$ 2 Pathway To Mediate Sickle Nephropathy. <i>Blood</i> , 2013, 122, 2211-2211.	1.4	8
71	Hyperangiotensinemia Induces Stem Cell/Progenitor Mobilization and De-Adhesion From BM Endothelial Cells Through AT2R Signaling and Inhibition of RhoA Activity. <i>Blood</i> , 2012, 120, 3466-3466.	1.4	8
72	NRASQ61R mutation in human endothelial cells causes vascular malformations. <i>Angiogenesis</i> , 2022, 25, 331-342.	7.2	8

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73	Genetic Therapies for Sickle Cell Disease. <i>Pediatric Clinics of North America</i> , 2018, 65, 465-480.	1.8	7
74	Effective hematopoietic stem cell-based gene therapy in a murine model of hereditary pulmonary alveolar proteinosis. <i>Haematologica</i> , 2020, 105, 1147-1157.	3.5	7
75	A Phase I Trial Of Zileuton In Sickle Cell Disease. <i>Blood</i> , 2013, 122, 993-993.	1.4	7
76	Production and Purification of Baculovirus for Gene Therapy Application. <i>Journal of Visualized Experiments</i> , 2018, , .	0.3	6
77	Abnormal submaximal cardiopulmonary exercise parameters predict impaired peak exercise performance in sickle cell anemia patients. <i>Pediatric Blood and Cancer</i> , 2019, 66, e27703.	1.5	6
78	Longitudinal effect of disease-modifying therapy on tricuspid regurgitant velocity in children with sickle cell anemia. <i>Blood Advances</i> , 2021, 5, 89-98.	5.2	6
79	Towards access for all: 1st Working Group Report for the Global Gene Therapy Initiative (GGTI). <i>Gene Therapy</i> , 2023, 30, 216-221.	4.5	6
80	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. <i>Blood</i> , 2019, 134, 616-616.	1.4	6
81	Implementation of near-universal hydroxyurea uptake among children with sickle cell anemia: A single-center experience. <i>Pediatric Blood and Cancer</i> , 2021, 68, e29008.	1.5	5
82	Gene therapy for hemoglobin disorders - a mini-review. , 2016, 1, 25-31.		5
83	Assessment of Cardiac Abnormalities in Sickle Cell Disease Patients Using Cardiac Magnetic Resonance Imaging (CMR). <i>Blood</i> , 2021, 138, 3110-3110.	1.4	4
84	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. <i>Blood</i> , 2011, 118, 164-164.	1.4	3
85	Rapid and automated quantitation of dense red blood cells: A robust biomarker of hydroxyurea treatment response. <i>Blood Cells, Molecules, and Diseases</i> , 2021, 90, 102576.	1.4	2
86	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. <i>Blood</i> , 2020, 136, 21-22.	1.4	2
87	Individualized Dosing of Hydroxyurea for Children with Sickle Cell Anemia Using a Population Pharmacokinetic-Based Model: The TREAT Study. <i>Blood</i> , 2016, 128, 3652-3652.	1.4	2
88	Successful HPV Vaccination in Adolescents with Sickle Cell Disease Following a Quality Improvement Bundle Intervention. <i>Blood</i> , 2021, 138, 914-914.	1.4	2
89	Successful use of veno-venous extracorporeal membrane oxygenation for acute chest syndrome in a child with sickle cell disease and SARS-CoV-2. <i>Pediatric Pulmonology</i> , 2022, , .	2.0	2
90	Pediatric Cardio-Oncology Medicine: A New Approach in Cardiovascular Care. <i>Children</i> , 2021, 8, 1200.	1.5	2

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91	Cardiac pathophysiology in sickle cell disease. Journal of Thrombosis and Thrombolysis, 2021, 52, 248-259.	2.1	1
92	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
93	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
94	Genetically Engineered Cures: Gene Therapy for Sickle Cell Disease. , 2007, , 295-309.		1
95	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
96	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
97	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
98	Sickle Cell Disease Is Associated with Reduced Adenosine Deaminase Catalytic Activity, Resulting in Altered Adenosine Metabolism. Blood, 2011, 118, 1078-1078.	1.4	0
99	Gene Therapy for Hemophagocytic Lymphohistiocytosis (HLH): Fixing a Critical "Circuit Breaker"™ in the Immune System.. Blood, 2012, 120, 3158-3158.	1.4	0
100	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
101	Patient Perceptions Of Treatments In SCD: Implications For Gene Transfer Therapy. Blood, 2013, 122, 5555-5555.	1.4	0
102	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
103	Foamy Virus Backbone Has Insulator Properties Which Remarkably Reduce Its Genotoxicity Potential. Blood, 2016, 128, 1002-1002.	1.4	0
104	Progression of Albuminuria in Sickle Cell Anemia: A Multicenter, Longitudinal Study. Blood, 2019, 134, 1004-1004.	1.4	0
105	Longitudinal Effect of Hydroxyurea Therapy on Left Ventricular Diastolic Function in Sickle Cell Anemia. Blood, 2019, 134, 1006-1006.	1.4	0
106	Association of Thrombospondin-1 Gene Polymorphism with Elevated Tricuspid Regurgitant Velocity in Sickle Cell Anemia. Blood, 2021, 138, 2027-2027.	1.4	0
107	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
108	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

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109	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