

# Gene Therapy in a Patient with Sickle Cell Disease

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Citation Report

#	ARTICLE	IF	CITATIONS
1	Managing cell and human identity. <i>Science</i> , 2017, 356, 139-140.	6.0	3
2	Sickle Cell Disease. <i>New England Journal of Medicine</i> , 2017, 376, 1561-1573.	13.9	898
3	Personalized Therapeutics and Pharmacogenomics: Integral to Personalized Health Care. <i>Pharmaceutical Research</i> , 2017, 34, 1535-1538.	1.7	2
4	Bone Marrow as a Hematopoietic Stem Cell Source for Gene Therapy in Sickle Cell Disease: Evidence from Rhesus and SCD Patients. <i>Human Gene Therapy Clinical Development</i> , 2017, 28, 136-144.	3.2	23
5	A guide to approaching regulatory considerations for lentiviral-mediated gene therapies. <i>Human Gene Therapy Methods</i> , 0, , .	2.1	1
6	Sickle cell disease: tipping the balance of genomic research to catalyse discoveries in Africa. <i>Lancet</i> , The, 2017, 389, 2355-2358.	6.3	11
8	False-positive HIV nucleic acid amplification testing during CAR T-cell therapy. <i>Diagnostic Microbiology and Infectious Disease</i> , 2017, 88, 305-307.	0.8	18
9	Gene Therapy in a Patient with Sickle Cell Disease. <i>New England Journal of Medicine</i> , 2017, 376, 2093-2094.	13.9	21
10	Eliminating HIV-1 Packaging Sequences from Lentiviral Vector Proviruses Enhances Safety and Expedites Gene Transfer for Gene Therapy. <i>Molecular Therapy</i> , 2017, 25, 1790-1804.	3.7	32
11	Gene Therapy for $\beta^2$ -Hemoglobinopathies. <i>Molecular Therapy</i> , 2017, 25, 1142-1154.	3.7	94
12	Treating sickle cell disease by targeting HbS polymerization. <i>Blood</i> , 2017, 129, 2719-2726.	0.6	170
13	Scalable Lentiviral Vector Production Using Stable HEK293SF Producer Cell Lines. <i>Human Gene Therapy Methods</i> , 2017, 28, 330-339.	2.1	80
14	Pathways to pulmonary hypertension in sickle cell disease: the search for prevention and early intervention. <i>Expert Review of Hematology</i> , 2017, 10, 875-890.	1.0	9
15	Promises and Challenges in Hematopoietic Stem Cell Gene Therapy. <i>Human Gene Therapy</i> , 2017, 28, 782-799.	1.4	6
16	Curative approaches for sickle cell disease: A review of allogeneic and autologous strategies. <i>Blood Cells, Molecules, and Diseases</i> , 2017, 67, 155-168.	0.6	11
17	Gene Therapy Approaches to Hemoglobinopathies. <i>Hematology/Oncology Clinics of North America</i> , 2017, 31, 835-852.	0.9	49
18	Function and Safety of Lentivirus-Mediated Gene Transfer for <i>CSF2RA</i> -Deficiency. <i>Human Gene Therapy Methods</i> , 2017, 28, 318-329.	2.1	16
20	The changing landscape of gene editing in hematopoietic stem cells: a step towards Cas9 clinical translation. <i>Current Opinion in Hematology</i> , 2017, 24, 481-488.	1.2	56

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21	A Guide to Approaching Regulatory Considerations for Lentiviral-Mediated Gene Therapies. <i>Human Gene Therapy Methods</i> , 2017, 28, 163-176.	2.1	68
22	Engraftment and in vivo proliferation advantage of gene-corrected mobilized CD34+ cells from Fanconi anemia patients. <i>Blood</i> , 2017, 130, 1535-1542.	0.6	42
23	What is the role of apheresis technology in stem cell transplantation?. <i>Transfusion and Apheresis Science</i> , 2017, 56, 788-794.	0.5	2
24	Are the risks of treatment to cure a child with severe sickle cell disease too high?. <i>BMJ: British Medical Journal</i> , 2017, 359, j5250.	2.4	7
25	Collaborative Research. <i>Clinical Nurse Specialist</i> , 2017, 31, 191-194.	0.3	1
26	Major Advances in the Development of Vectors for Clinical Gene Therapy of Hematopoietic Stem Cells from European Groups over the Last 25 Years. <i>Human Gene Therapy</i> , 2017, 28, 964-971.	1.4	3
28	Hurdles Associated with the Translational Use of Genetically Modified Cells. <i>Current Stem Cell Reports</i> , 2018, 4, 39-45.	0.7	0
29	Plerixafor enables safe, rapid, efficient mobilization of hematopoietic stem cells in sickle cell disease patients after exchange transfusion. <i>Haematologica</i> , 2018, 103, 778-786.	1.7	89
30	High-level embryonic globin production with efficient erythroid differentiation from a K562 erythroleukemia cell line. <i>Experimental Hematology</i> , 2018, 62, 7-16.e1.	0.2	10
31	Acute chest syndrome in sickle cell disease. <i>Hospital Practice (1995)</i> , 2018, 46, 144-151.	0.5	18
32	Haploidentical Peripheral Blood Stem Cell Transplantation Demonstrates Stable Engraftment in Adults with Sickle Cell Disease. <i>Biology of Blood and Marrow Transplantation</i> , 2018, 24, 1759-1765.	2.0	50
33	Gene Therapy in Patients with Transfusion-Dependent $\beta^2$ -Thalassemia. <i>New England Journal of Medicine</i> , 2018, 378, 1479-1493.	13.9	525
34	Fetal hemoglobin induction in sickle erythroid progenitors using a synthetic zinc finger DNA-binding domain. <i>Haematologica</i> , 2018, 103, e384-e387.	1.7	10
35	Hematopoietic stem cell transplantation in its 60s: A platform for cellular therapies. <i>Science Translational Medicine</i> , 2018, 10, .	5.8	125
36	Sickle Cell Anemia and Its Phenotypes. <i>Annual Review of Genomics and Human Genetics</i> , 2018, 19, 113-147.	2.5	66
37	Preclinical Development of a Lentiviral Vector for Gene Therapy of X-Linked Severe Combined Immunodeficiency. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 9, 257-269.	1.8	38
38	ESCMID Study Group for Infections in Compromised Hosts (ESGICH) Consensus Document on the safety of targeted and biological therapies: an infectious diseases perspective (Introduction). <i>Clinical Microbiology and Infection</i> , 2018, 24, S2-S9.	2.8	52
39	Nucleic acid based therapies: developing frontier for precision medicine. <i>BMJ: British Medical Journal</i> , 2018, 360, k223.	2.4	0

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40	Emerging Therapies. Hematology/Oncology Clinics of North America, 2018, 32, 343-352.	0.9	5
41	Adolescents's experiences of living with sickle cell disease: An integrative narrative review of the literature. International Journal of Nursing Studies, 2018, 80, 20-28.	2.5	24
42	Progress in Science, Progress in Society. , 2018, , .		0
43	A long noncoding RNA from the HBS1L-MYB intergenic region on chr6q23 regulates human fetal hemoglobin expression. Blood Cells, Molecules, and Diseases, 2018, 69, 1-9.	0.6	45
44	Gene therapy comes of age. Science, 2018, 359, .	6.0	936
45	New Perspectives in Genetic Therapies. , 2018, , 71-78.		0
46	Gene therapy and gene editing strategies for hemoglobinopathies. Blood Cells, Molecules, and Diseases, 2018, 70, 87-101.	0.6	28
47	Achievements in 2017, Promises of 2018. Journal of Fetal Medicine, 2018, 5, 1-4.	0.1	0
48	Nongenetic Optical Methods for Measuring and Modulating Neuronal Response. ACS Nano, 2018, 12, 4086-4095.	7.3	35
49	Bone Marrow Transplantation after Nonmyeloablative Treosulfan Conditioning Is Curative in a Murine Model of Sickle Cell Disease. Biology of Blood and Marrow Transplantation, 2018, 24, 1554-1562.	2.0	2
50	Sickle cell disease. Nature Reviews Disease Primers, 2018, 4, 18010.	18.1	764
51	Turning the corner from observation to intervention in human genetics. Journal of Genetics and Genomics, 2018, 45, 57-59.	1.7	1
52	Gene therapy clinical trials worldwide to 2017: An update. Journal of Gene Medicine, 2018, 20, e3015.	1.4	612
53	Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated editing of the human $\beta$ -globin locus. Blood, 2018, 131, 1960-1973.	0.6	156
54	Molecular basis of $\beta$ -thalassemia and potential therapeutic targets. Blood Cells, Molecules, and Diseases, 2018, 70, 54-65.	0.6	138
55	Ex Vivo Selection of Transduced Hematopoietic Stem Cells for Gene Therapy of $\beta$ -Hemoglobinopathies. Molecular Therapy, 2018, 26, 480-495.	3.7	15
56	Molecular basis of $\alpha$ -thalassemia. Blood Cells, Molecules, and Diseases, 2018, 70, 43-53.	0.6	131
57	Ethical Challenges in Hematopoietic Cell Transplantation for Sickle Cell Disease. Biology of Blood and Marrow Transplantation, 2018, 24, 219-227.	2.0	26

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58	Sickle cell disease: a malady beyond a hemoglobin defect in cerebrovascular disease. Expert Review of Hematology, 2018, 11, 45-55.	1.0	15
59	Fetal haemoglobin induction in sickle cell disease. British Journal of Haematology, 2018, 180, 189-200.	1.2	47
60	Evolution of hemoglobin loci and their regulatory elements. Blood Cells, Molecules, and Diseases, 2018, 70, 2-12.	0.6	37
62	Hemoglobinopathies and hemolytic anemias. Hematologie, 2018, 24, 169-182.	0.0	0
63	Gene therapy for sickle cell disease. The Cochrane Library, 2018, 11, CD007652.	1.5	9
64	Sickle Cell Disease: Advances in Treatment. Ochsner Journal, 2018, 18, 377-389.	0.5	64
65	A New Era for Hemoglobinopathies: More Than One Curative Option. Current Gene Therapy, 2018, 17, 364-378.	0.9	9
66	A ten year review of the sickle cell program in Muhimbili National Hospital, Tanzania. BMC Hematology, 2018, 18, 33.	2.6	31
67	Getting the Most: Enhancing Efficacy by Promoting Erythropoiesis and Thrombopoiesis after Gene Therapy in Mice with Hurler Syndrome. Molecular Therapy - Methods and Clinical Development, 2018, 11, 52-64.	1.8	1
68	Advances in the Treatment of Sickle Cell Disease. Mayo Clinic Proceedings, 2018, 93, 1810-1824.	1.4	62
69	Pre-clinical Development of a Lentiviral Vector Expressing the Anti-sickling $\beta^2AS3$ Globin for Gene Therapy for Sickle Cell Disease. Molecular Therapy - Methods and Clinical Development, 2018, 11, 167-179.	1.8	16
70	Barcoding of Macaque Hematopoietic Stem and Progenitor Cells: A Robust Platform to Assess Vector Genotoxicity. Molecular Therapy - Methods and Clinical Development, 2018, 11, 143-154.	1.8	9
71	Gene and Cell Therapy: Tearing Down Walls. Human Gene Therapy, 2018, 29, 1071-1073.	1.4	0
72	Cryopreservation of Human Adipose-Derived Stem Cells for Use in Ex Vivo Regional Gene Therapy for Bone Repair. Human Gene Therapy Methods, 2018, 29, 269-277.	2.1	10
73	Multiple Integrated Non-clinical Studies Predict the Safety of Lentivirus-Mediated Gene Therapy for $\beta^2$ -Thalassemia. Molecular Therapy - Methods and Clinical Development, 2018, 11, 9-28.	1.8	21
74	Promise of gene therapy to treat sickle cell disease. Expert Opinion on Biological Therapy, 2018, 18, 1123-1136.	1.4	18
75	Sickle Cell Anaemia- A Synopsis of the Inherited Ailment. Archives of Medicine, 2018, 10, .	0.2	1
76	Cell engineering with microfluidic squeezing preserves functionality of primary immune cells in vivo. Proceedings of the National Academy of Sciences of the United States of America, 2018, 115, E10907-E10914.	3.3	129

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77	Risks associated with fertility preservation for women with sickle cell anemia. <i>Fertility and Sterility</i> , 2018, 110, 720-731.	0.5	28
78	Gene Therapy for Hemoglobinopathies. <i>Human Gene Therapy</i> , 2018, 29, 1106-1113.	1.4	34
79	How I treat the older adult with sickle cell disease. <i>Blood</i> , 2018, 132, 1750-1760.	0.6	31
80	Gene Therapy for Sickle Cell Disease<i></i>A Lentiviral Vector Comparison Study. <i>Human Gene Therapy</i> , 2018, 29, 1153-1166.	1.4	33
81	Preclinical Development of a Hematopoietic Stem and Progenitor Cell Bioengineered Factor VIII Lentiviral Vector Gene Therapy for Hemophilia A. <i>Human Gene Therapy</i> , 2018, 29, 1183-1201.	1.4	39
82	A microfluidic approach to study the effect of mechanical stress on erythrocytes in sickle cell disease. <i>Lab on A Chip</i> , 2018, 18, 2975-2984.	3.1	32
83	Successful hematopoietic stem cell mobilization and apheresis collection using plerixafor alone in sickle cell patients. <i>Blood Advances</i> , 2018, 2, 2505-2512.	2.5	62
84	Hematopoietic stem cell transplantation for sickle cell disease: Progress and challenges. <i>Pediatric Blood and Cancer</i> , 2018, 65, e27263.	0.8	30
86	Hemoglobin disorders: lentiviral gene therapy in the starting blocks to enter clinical practice. <i>Experimental Hematology</i> , 2018, 64, 12-32.	0.2	35
87	How I diagnose and treat venous thromboembolism in sickle cell disease. <i>Blood</i> , 2018, 132, 1761-1769.	0.6	29
88	Oncostatin M and Kit-Ligand Control Hematopoietic Stem Cell Fate during Zebrafish Embryogenesis. <i>Stem Cell Reports</i> , 2018, 10, 1920-1934.	2.3	26
89	Safety and efficacy of plerixafor dose escalation for the mobilization of CD34 <sup>+</sup> hematopoietic progenitor cells in patients with sickle cell disease: interim results. <i>Haematologica</i> , 2018, 103, 770-777.	1.7	47
90	Sickle Cell Clinical Research and Intervention Program (SCCRIP): A lifespan cohort study for sickle cell disease progression from the pediatric stage into adulthood. <i>Pediatric Blood and Cancer</i> , 2018, 65, e27228.	0.8	57
91	Treatment Options for Sickle Cell Disease. <i>Pediatric Clinics of North America</i> , 2018, 65, 427-443.	0.9	39
92	Genetic Therapies for Sickle Cell Disease. <i>Pediatric Clinics of North America</i> , 2018, 65, 465-480.	0.9	7
93	In Utero Therapy for Congenital Disorders Using Amniotic Fluid Stem Cells. , 2018, , 3-20.		2
94	Gene Therapy With Regulatory T Cells: A Beneficial Alliance. <i>Frontiers in Immunology</i> , 2018, 9, 554.	2.2	30
95	On the Binding Free Energy and Molecular Origin of Sickle Cell Hemoglobin Aggregation. <i>Journal of Physical Chemistry B</i> , 2018, 122, 7475-7483.	1.2	11

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96	The past, present and future management of sickle cell retinopathy within an African context. <i>Eye</i> , 2018, 32, 1304-1314.	1.1	9
97	Advances in new drug therapies for the management of sickle cell disease. <i>Expert Opinion on Orphan Drugs</i> , 2018, 6, 329-343.	0.5	13
98	Genetic therapies for sickle cell disease. <i>Seminars in Hematology</i> , 2018, 55, 76-86.	1.8	32
99	Erythropoiesis: insights into pathophysiology and treatments in 2017. <i>Molecular Medicine</i> , 2018, 24, 11.	1.9	76
100	How I safely transfuse patients with sickle-cell disease and manage delayed hemolytic transfusion reactions. <i>Blood</i> , 2018, 131, 2773-2781.	0.6	109
101	An Optimized Lentiviral Vector Efficiently Corrects the Human Sickle Cell Disease Phenotype. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 10, 268-280.	1.8	20
102	Foamy Virus Vectors Transduce Visceral Organs and Hippocampal Structures following In Vivo Delivery to Neonatal Mice. <i>Molecular Therapy - Nucleic Acids</i> , 2018, 12, 626-634.	2.3	7
103	Controlled Non-Viral Gene Delivery in Cartilage and Bone Repair: Current Strategies and Future Directions. <i>Advanced Therapeutics</i> , 2018, 1, 1800038.	1.6	18
104	Gene therapy for sickle cell disease: An update. <i>Cytotherapy</i> , 2018, 20, 899-910.	0.3	84
105	Retinal and Choroidal Vascular Diseases. <i>Current Practices in Ophthalmology</i> , 2018, , 91-131.	0.1	0
106	Ctrl-Alt-inDel: genome editing to reprogram a cell in the clinic. <i>Current Opinion in Genetics and Development</i> , 2018, 52, 48-56.	1.5	11
107	Priming Human Repopulating Hematopoietic Stem and Progenitor Cells for Cas9/sgRNA Gene Targeting. <i>Molecular Therapy - Nucleic Acids</i> , 2018, 12, 89-104.	2.3	84
108	Stem cell transplantation in sickle cell disease: therapeutic potential and challenges faced. <i>Expert Review of Hematology</i> , 2018, 11, 547-565.	1.0	34
109	Gene Editing on Center Stage. <i>Trends in Genetics</i> , 2018, 34, 600-611.	2.9	117
110	New Concepts in Transfusion Medicine. , 2018, , 177-184.		1
111	CRISPR in Sub-Saharan Africa: Applications and Education. <i>Trends in Biotechnology</i> , 2019, 37, 234-237.	4.9	6
112	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.	2.0	14
113	The clinical application of gene editing: ethical and social issues. <i>Personalized Medicine</i> , 2019, 16, 337-350.	0.8	25

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114	Accelerating the Science of SCD Therapies—Is a Cure Possible?. JAMA - Journal of the American Medical Association, 2019, 322, 921.	3.8	6
115	Resveratrol trimer enhances gene delivery to hematopoietic stem cells by reducing antiviral restriction at endosomes. Blood, 2019, 134, 1298-1311.	0.6	27
116	Therapeutically relevant engraftment of a CRISPR-Cas9–edited HSC-enriched population with HbF reactivation in nonhuman primates. Science Translational Medicine, 2019, 11, .	5.8	88
117	Gene therapy of hemoglobinopathies: progress and future challenges. Human Molecular Genetics, 2019, 28, R24-R30.	1.4	51
118	Gene therapy for primary immunodeficiency. Human Molecular Genetics, 2019, 28, R15-R23.	1.4	55
119	Transfusing children with hemoglobinopathies. Transfusion Clinique Et Biologique, 2019, 26, 147-149.	0.2	1
120	Gene therapy of hematological disorders: current challenges. Gene Therapy, 2019, 26, 296-307.	2.3	8
121	The study of genes and signal transduction pathways involved in mustard lung injury: A gene therapy approach. Gene, 2019, 714, 143968.	1.0	3
122	A Growing Population of Older Adults with Sickle Cell Disease. Clinics in Geriatric Medicine, 2019, 35, 349-367.	1.0	5
123	In Vivo Outcome of Homology-Directed Repair at the HBB Gene in HSC Using Alternative Donor Template Delivery Methods. Molecular Therapy - Nucleic Acids, 2019, 17, 277-288.	2.3	74
124	Enhancing Lentiviral and Alpharetroviral Transduction of Human Hematopoietic Stem Cells for Clinical Application. Molecular Therapy - Methods and Clinical Development, 2019, 14, 134-147.	1.8	37
125	Gene Therapy. New England Journal of Medicine, 2019, 381, 455-464.	13.9	343
126	Cinchona alkaloids as natural fetal hemoglobin inducing agents in human erythroleukemia cells. RSC Advances, 2019, 9, 17551-17559.	1.7	11
127	Use of Heterologous Vesiculovirus G Proteins Circumvents the Humoral Anti-envelope Immunity in Lentivector-Based In Vivo Gene Delivery. Molecular Therapy - Nucleic Acids, 2019, 17, 126-137.	2.3	19
128	Multifunctional co-loaded magnetic nanocapsules for enhancing targeted MR imaging and in vivo photodynamic therapy. Nanomedicine: Nanotechnology, Biology, and Medicine, 2019, 21, 102047.	1.7	10
129	Ethical development of stem-cell-based interventions. Nature Medicine, 2019, 25, 1037-1044.	15.2	29
130	Busulfan Combined with Immunosuppression Allows Efficient Engraftment of Gene-Modified Cells in a Rhesus Macaque Model. Molecular Therapy, 2019, 27, 1586-1596.	3.7	28
131	Gene editing of PKLR gene in human hematopoietic progenitors through 5' and 3' UTR modified TALEN mRNA. PLoS ONE, 2019, 14, e0223775.	1.1	23



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132	Production of foetal globin in adult monkeys. <i>Nature Biomedical Engineering</i> , 2019, 3, 857-859.	11.6	0
133	Reducing Health Care Disparities in Sickle Cell Disease: A Review. <i>Public Health Reports</i> , 2019, 134, 599-607.	1.3	123
134	A multiscale simulation framework for the manufacturing facility and supply chain of autologous cell therapies. <i>Cytotherapy</i> , 2019, 21, 1081-1093.	0.3	21
135	Adenovirus vectors in hematopoietic stem cell genome editing. <i>FEBS Letters</i> , 2019, 593, 3623-3648.	1.3	35
136	CURING HEMOGLOBINOPATHIES: CHALLENGES AND ADVANCES OF CONVENTIONAL AND NEW GENE THERAPY APPROACHES.. <i>Mediterranean Journal of Hematology and Infectious Diseases</i> , 2019, 11, e2019067.	0.5	16
137	T-cell deplete versus T-cell replete haploidentical hematopoietic stem cell transplantation for sickle cell disease: where are we?. <i>Expert Review of Hematology</i> , 2019, 12, 733-752.	1.0	6
138	Lentiviral and genome-editing strategies for the treatment of $\beta^2$ -hemoglobinopathies. <i>Blood</i> , 2019, 134, 1203-1213.	0.6	74
139	Layered Double Hydroxide Nanoparticles for Efficient Gene Delivery for Cancer Treatment. <i>Bioconjugate Chemistry</i> , 2019, 30, 2544-2554.	1.8	22
140	CRISPR technologies for stem cell engineering and regenerative medicine. <i>Biotechnology Advances</i> , 2019, 37, 107447.	6.0	59
141	Development of a forward-oriented therapeutic lentiviral vector for hemoglobin disorders. <i>Nature Communications</i> , 2019, 10, 4479.	5.8	21
142	Why, Who, When, and How? Rationale for Considering Allogeneic Stem Cell Transplantation in Children with Sickle Cell Disease. <i>Journal of Clinical Medicine</i> , 2019, 8, 1523.	1.0	9
143	Evaluation of Outcomes and Quality of Care in Children with Sickle Cell Disease Diagnosed by Newborn Screening: A Real-World Nation-Wide Study in France. <i>Journal of Clinical Medicine</i> , 2019, 8, 1594.	1.0	21
144	&lt;p&gt;Gene Therapy For Beta-Thalassemia: Updated Perspectives&lt;/p&gt;. <i>The Application of Clinical Genetics</i> , 2019, Volume 12, 167-180.	1.4	39
145	Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent $\alpha^0$ -thalassemia. <i>Nature Medicine</i> , 2019, 25, 234-241.	15.2	188
146	Genetic engineering of hematopoiesis: current stage of clinical translation and future perspectives. <i>EMBO Molecular Medicine</i> , 2019, 11, .	3.3	86
147	Gene Therapy for Beta-Hemoglobinopathies: Milestones, New Therapies and Challenges. <i>Molecular Diagnosis and Therapy</i> , 2019, 23, 173-186.	1.6	23
148	Restoration of correct $\beta^2$ IVS2-654-globin mRNA splicing and HbA production by engineered U7 snRNA in $\beta^2$ -thalassaemia/HbE erythroid cells. <i>Scientific Reports</i> , 2019, 9, 7672.	1.6	7
149	Gene Therapy. <i>Advances in Pediatrics</i> , 2019, 66, 37-54.	0.5	15

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150	Highly efficient editing of the $\beta$ -globin gene in patient-derived hematopoietic stem and progenitor cells to treat sickle cell disease. <i>Nucleic Acids Research</i> , 2019, 47, 7955-7972.	6.5	110
151	Gene Therapy in Pediatric Liver Disease. , 2019, , 799-829.		2
152	PGE2 and Poloxamer Synperonic F108 Enhance Transduction of Human HSPCs with a $\beta$ -Globin Lentiviral Vector. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 13, 390-398.	1.8	38
153	Sickle cell disease: Clinical presentation and management of a global health challenge. <i>Blood Reviews</i> , 2019, 37, 100580.	2.8	42
154	Sickle Cell Disease—Genetics, Pathophysiology, Clinical Presentation and Treatment. <i>International Journal of Neonatal Screening</i> , 2019, 5, 20.	1.2	80
155	Aberrant Clonal Hematopoiesis following Lentiviral Vector Transduction of HSPCs in a Rhesus Macaque. <i>Molecular Therapy</i> , 2019, 27, 1074-1086.	3.7	34
156	Lentiviral Vectors for the Treatment and Prevention of Cystic Fibrosis Lung Disease. <i>Genes</i> , 2019, 10, 218.	1.0	48
157	Effect of increased dose of total body irradiation on graft failure associated with HLA-haploidentical transplantation in patients with severe haemoglobinopathies: a prospective clinical trial. <i>Lancet Haematology</i> , 2019, 6, e183-e193.	2.2	111
158	Innovation in Chemistry, Manufacturing, and Controls—A Regulatory Perspective From Industry. <i>Journal of Pharmaceutical Sciences</i> , 2019, 108, 2207-2237.	1.6	30
159	CRISPR-Cas9 genome editing induces megabase-scale chromosomal truncations. <i>Nature Communications</i> , 2019, 10, 1136.	5.8	292
160	High-Efficiency Lentiviral Transduction of Human CD34+ Cells in High-Density Culture with Poloxamer and Prostaglandin E2. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 13, 187-196.	1.8	31
161	Genomic Medicine—Progress, Pitfalls, and Promise. <i>Cell</i> , 2019, 177, 45-57.	13.5	143
162	The Growing Development of DNA Nanostructures for Potential Healthcare—Related Applications. <i>Advanced Healthcare Materials</i> , 2019, 8, e1801546.	3.9	60
163	Gene therapy targeting haematopoietic stem cells for inherited diseases: progress and challenges. <i>Nature Reviews Drug Discovery</i> , 2019, 18, 447-462.	21.5	141
164	Enhanced Transduction of Macaca fascicularis Hematopoietic Cells with Chimeric Lentiviral Vectors. <i>Human Gene Therapy</i> , 2019, 30, 1306-1323.	1.4	3
165	Therapeutic gene editing in haematological disorders with CRISPR/Cas9. <i>British Journal of Haematology</i> , 2019, 185, 821-835.	1.2	32
166	Gene Therapy for Platelet Disorders. , 2019, , 1191-1205.		0
167	21st Century FOX: Toward Gene Therapy for the Regulatory T Cell Deficiency Syndrome IPEX. <i>Cell Stem Cell</i> , 2019, 24, 208-209.	5.2	0

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168	Chemistry, manufacturing and controls for gene modified hematopoietic stem cells. <i>Cytotherapy</i> , 2019, 21, 358-366.	0.3	5
169	Hematopoietic stem cell transplantation and cellular therapy in sickle cell disease: where are we now?. <i>Current Opinion in Hematology</i> , 2019, 26, 448-452.	1.2	16
170	How I treat sickle cell disease with hematopoietic cell transplantation. <i>Blood</i> , 2019, 134, 2249-2260.	0.6	10
171	A method for noninvasive prenatal diagnosis of monogenic autosomal recessive disorders. <i>Blood</i> , 2019, 134, 1190-1193.	0.6	14
172	Single-cell analysis of bone marrow-derived CD34+ cells from children with sickle cell disease and thalassemia. <i>Blood</i> , 2019, 134, 2111-2115.	0.6	21
173	Gene Therapy of the $\beta^2$ Hemoglobinopathies: Success and Challenges. <i>Hemoglobin</i> , 2019, 43, 301-301.	0.4	0
174	End points for sickle cell disease clinical trials: renal and cardiopulmonary, cure, and low-resource settings. <i>Blood Advances</i> , 2019, 3, 4002-4020.	2.5	21
175	Autologous Stem-Cell-Based Gene Therapy for Inherited Disorders: State of the Art and Perspectives. <i>Frontiers in Pediatrics</i> , 2019, 7, 443.	0.9	66
176	In-Vivo Gene Therapy with Foamy Virus Vectors. <i>Viruses</i> , 2019, 11, 1091.	1.5	16
177	The Sickle Cell Disease Ontology: enabling universal sickle cell-based knowledge representation. <i>Database: the Journal of Biological Databases and Curation</i> , 2019, 2019, .	1.4	14
178	High-throughput assessment of hemoglobin polymer in single red blood cells from sickle cell patients under controlled oxygen tension. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2019, 116, 25236-25242.	3.3	20
179	EHA Research Roadmap on Hemoglobinopathies and Thalassemia: An Update. <i>HemaSphere</i> , 2019, 3, e208.	1.2	13
180	Genetic risk assessment and haemoglobinopathy counselling: two case studies. <i>British Journal of Midwifery</i> , 2019, 27, 790-796.	0.1	0
181	Hemoglobinopathies (Sickle Cell Disease and Thalassemia). , 2019, , 595-601.		2
182	Gene therapy for blood diseases. <i>Current Opinion in Biotechnology</i> , 2019, 60, 39-45.	3.3	27
183	Engineering Globin Gene Expression. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 12, 102-110.	1.8	9
184	Entering the Modern Era of Gene Therapy. <i>Annual Review of Medicine</i> , 2019, 70, 273-288.	5.0	311
185	IND-Enabling Studies for a Clinical Trial to Genetically Program a Persistent Cancer-Targeted Immune System. <i>Clinical Cancer Research</i> , 2019, 25, 1000-1011.	3.2	9

#	ARTICLE	IF	CITATIONS
186	NEW THERAPEUTIC OPTIONS FOR THE TREATMENT OF SICKLE CELL DISEASE. Mediterranean Journal of Hematology and Infectious Diseases, 2019, 11, e2019002.	0.5	29
187	CD46 Null Packaging Cell Line Improves Measles Lentiviral Vector Production and Gene Delivery to Hematopoietic Stem and Progenitor Cells. Molecular Therapy - Methods and Clinical Development, 2019, 13, 27-39.	1.8	10
188	Hydroxyurea – An Essential Medicine for Sickle Cell Disease in Africa. New England Journal of Medicine, 2019, 380, 187-189.	13.9	17
189	CRISPR-based RNA editing: diagnostic applications and therapeutic options. Expert Review of Molecular Diagnostics, 2019, 19, 83-88.	1.5	15
190	Emerging Genetic Therapy for Sickle Cell Disease. Annual Review of Medicine, 2019, 70, 257-271.	5.0	90
191	Genomic Science – From 2001 to Present Day: What School Nurses Need to Know. NASN School Nurse (Print), 2019, 34, 235-239.	0.4	2
192	Improving Gene Editing Outcomes in Human Hematopoietic Stem and Progenitor Cells by Temporal Control of DNA Repair. Stem Cells, 2019, 37, 284-294.	1.4	70
193	Silencing of transgene expression in mammalian cells by DNA methylation and histone modifications in gene therapy perspective. Biotechnology and Genetic Engineering Reviews, 2019, 35, 1-25.	2.4	27
194	Immunosuppression overcomes insulin- and vector-specific immune responses that limit efficacy of AAV2/8-mediated insulin gene therapy in NOD mice. Gene Therapy, 2019, 26, 40-56.	2.3	8
195	Therapeutic strategies for sickle cell disease: towards a multi-agent approach. Nature Reviews Drug Discovery, 2019, 18, 139-158.	21.5	116
196	Emerging pharmacotherapeutic approaches for the management of sickle cell disease. Expert Opinion on Pharmacotherapy, 2019, 20, 173-186.	0.9	23
197	Hemostatic Aspects of Sickle Cell Disease. , 2019, , 819-842.		0
198	Modulation of immune responses in lentiviral vector-mediated gene transfer. Cellular Immunology, 2019, 342, 103802.	1.4	49
199	Advances in the gene therapy of monogenic blood cell diseases. Clinical Genetics, 2020, 97, 89-102.	1.0	18
200	Cardiovascular manifestations of sickle cell disease. European Heart Journal, 2020, 41, 1365-1373.	1.0	25
201	Extensive multilineage analysis in patients with mixed chimerism after allogeneic transplantation for sickle cell disease: insight into hematopoiesis and engraftment thresholds for gene therapy. Haematologica, 2020, 105, 1240-1247.	1.7	24
202	Improved Titer and Gene Transfer by Lentiviral Vectors Using Novel, Small $\beta$ -Globin Locus Control Region Elements. Molecular Therapy, 2020, 28, 328-340.	3.7	27
203	A Molecular Revolution in the Treatment of Hemophilia. Molecular Therapy, 2020, 28, 997-1015.	3.7	66

#	ARTICLE	IF	CITATIONS
204	Not all red cells sickle the same: Contributions of the reticulocyte to disease pathology in sickle cell anemia. <i>Blood Reviews</i> , 2020, 40, 100637.	2.8	21
205	Intestinal pathophysiological and microbial changes in sickle cell disease: Potential targets for therapeutic intervention. <i>British Journal of Haematology</i> , 2020, 188, 488-493.	1.2	17
206	ATM activity in T cells is critical for immune surveillance of lymphoma in vivo. <i>Leukemia</i> , 2020, 34, 771-786.	3.3	13
207	Paediatric haematologists' attitudes regarding haematopoietic cell transplantation as treatment for sickle cell disease. <i>British Journal of Haematology</i> , 2020, 188, 976-984.	1.2	4
208	Treating sickle cell anemia: A new era dawns. <i>American Journal of Hematology</i> , 2020, 95, 338-342.	2.0	15
209	Application of Genetic Engineering in Biotherapeutics Development. <i>Journal of Pharmaceutical Innovation</i> , 2020, 15, 232-254.	1.1	4
210	Emerging drugs in randomized controlled trials for sickle cell disease: are we on the brink of a new era in research and treatment?. <i>Expert Opinion on Investigational Drugs</i> , 2020, 29, 23-31.	1.9	15
212	Essential Current Concepts in Stem Cell Biology. <i>Learning Materials in Biosciences</i> , 2020, , .	0.2	2
213	Immunoresponse to Gene-Modified Hematopoietic Stem Cells. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 16, 42-49.	1.8	16
214	Gene Therapy of the Hemoglobinopathies. <i>HemaSphere</i> , 2020, 4, e479.	1.2	18
215	Gene therapy and gene correction: targets, progress, and challenges for treating human diseases. <i>Gene Therapy</i> , 2022, 29, 3-12.	2.3	53
217	Parents of Children with Sickle Cell Disease Are Interested in Preimplantation Genetic Testing. <i>Journal of Pediatrics</i> , 2020, 223, 178-182.e2.	0.9	11
218	Mutation-Specific Guide RNA for Compound Heterozygous Porphyria On-target Scarless Correction by CRISPR/Cas9 in Stem Cells. <i>Stem Cell Reports</i> , 2020, 15, 677-693.	2.3	6
219	A Lactose-Derived CRISPR/Cas9 Delivery System for Efficient Genome Editing In Vivo to Treat Orthotopic Hepatocellular Carcinoma. <i>Advanced Science</i> , 2020, 7, 2001424.	5.6	50
220	The mRNA-Binding Protein IGF2BP1 Restores Fetal Hemoglobin in Cultured Erythroid Cells from Patients with $\beta^2$ -Hemoglobin Disorders. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 429-440.	1.8	13
221	Creating New $\beta^2$ -Globin-Expressing Lentiviral Vectors by High-Resolution Mapping of Locus Control Region Enhancer Sequences. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 999-1013.	1.8	9
222	Lack of methylation on transgene leads to high level and persistent transgene expression in induced pluripotent stem cells. <i>Gene</i> , 2020, 758, 144958.	1.0	3
223	Comparison of US Federal and Foundation Funding of Research for Sickle Cell Disease and Cystic Fibrosis and Factors Associated With Research Productivity. <i>JAMA Network Open</i> , 2020, 3, e201737.	2.8	102

#	ARTICLE	IF	CITATIONS
224	Individual red blood cell fetal hemoglobin quantification allows to determine protective thresholds in sickle cell disease. <i>American Journal of Hematology</i> , 2020, 95, 1235-1245.	2.0	18
225	Whole blood viscosity and red blood cell adhesion: Potential biomarkers for targeted and curative therapies in sickle cell disease. <i>American Journal of Hematology</i> , 2020, 95, 1246-1256.	2.0	42
226	Pseudotyping Lentiviral Vectors: When the Clothes Make the Virus. <i>Viruses</i> , 2020, 12, 1311.	1.5	23
227	The role of haematopoietic stem cell transplantation for sickle cell disease in the era of targeted disease-modifying therapies and gene editing. <i>Lancet Haematology</i> , 2020, 7, e902-e911.	2.2	18
228	Myelodysplastic syndrome unrelated to lentiviral vector in a patient treated with gene therapy for sickle cell disease. <i>Blood Advances</i> , 2020, 4, 2058-2063.	2.5	93
229	Advances in gene therapy for hematologic disease and considerations for transfusion medicine. <i>Seminars in Hematology</i> , 2020, 57, 83-91.	1.8	5
230	Overview of the current status of gene therapy for primary immune deficiencies (PIDs). <i>Journal of Allergy and Clinical Immunology</i> , 2020, 146, 229-233.	1.5	8
231	Cytherapy Clinical Trials in Genetic Disorders of the Blood and Options for Reimbursement. , 2020, , 409-432.		0
232	Fetal hemoglobin in sickle cell anemia. <i>Blood</i> , 2020, 136, 2392-2400.	0.6	43
233	Fetal hemoglobin rescues ineffective erythropoiesis in sickle cell disease. <i>Haematologica</i> , 2021, 106, 2707-2719.	1.7	27
234	Innovative Therapies for Hemoglobin Disorders. <i>BioDrugs</i> , 2020, 34, 625-647.	2.2	7
235	Rare genetic causes of complex kidney and urological diseases. <i>Nature Reviews Nephrology</i> , 2020, 16, 641-656.	4.1	27
236	Gene therapy for sickle cell disease. <i>The Cochrane Library</i> , 2020, 2020, CD007652.	1.5	5
237	Design of efficacious somatic cell genome editing strategies for recessive and polygenic diseases. <i>Nature Communications</i> , 2020, 11, 6277.	5.8	7
238	Mechanisms of Genome Protection and Repair. <i>Advances in Experimental Medicine and Biology</i> , 2020, , .	0.8	2
239	Application of droplet digital PCR for the detection of vector copy number in clinical CAR/TCR T cell products. <i>Journal of Translational Medicine</i> , 2020, 18, 191.	1.8	19
240	Sickle cell disease as a vascular disorder. <i>Expert Review of Hematology</i> , 2020, 13, 645-653.	1.0	9
241	Microvascular thrombosis: experimental and clinical implications. <i>Translational Research</i> , 2020, 225, 105-130.	2.2	62

#	ARTICLE	IF	CITATIONS
242	An evolutionarily ancient mechanism for regulation of hemoglobin expression in vertebrate red cells. <i>Blood</i> , 2020, 136, 269-278.	0.6	16
243	Advances in Sickle Cell Disease Management. <i>Advances in Pediatrics</i> , 2020, 67, 57-71.	0.5	7
244	Î²T87Q-Globin Gene Therapy Reduces Sickle Hemoglobin Production, Allowing for Ex Vivo Anti-sickling Activity in Human Erythroid Cells. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 912-921.	1.8	13
245	Baseline and Disease-Induced Transcriptional Profiles in Children with Sickle Cell Disease. <i>Scientific Reports</i> , 2020, 10, 9013.	1.6	4
246	Recent Advances in the Treatment of Sickle Cell Disease. <i>Frontiers in Physiology</i> , 2020, 11, 435.	1.3	114
247	Transfusion in Sickle cell disease: best practice and understanding of its utility. <i>ISBT Science Series</i> , 2020, 15, 347-351.	1.1	1
248	Sickle Cell Hemoglobin. <i>Sub-Cellular Biochemistry</i> , 2020, 94, 297-322.	1.0	6
249	Alternative donor hematopoietic stem cell transplantation for sickle cell disease in Europe. <i>Hematology/ Oncology and Stem Cell Therapy</i> , 2020, 13, 181-188.	0.6	22
250	Pathophysiology and recent therapeutic insights of sickle cell disease. <i>Annals of Hematology</i> , 2020, 99, 925-935.	0.8	21
251	The role of HLA matching in unrelated donor hematopoietic stem cell transplantation for sickle cell disease in Europe. <i>Bone Marrow Transplantation</i> , 2020, 55, 1946-1954.	1.3	14
252	Emerging therapies in sickle cell disease. <i>British Journal of Haematology</i> , 2020, 190, 149-172.	1.2	33
253	Genetic Engineering of the Kidney to Permanently Silence MHC Transcripts During ex vivo Organ Perfusion. <i>Frontiers in Immunology</i> , 2020, 11, 265.	2.2	38
254	Preclinical Evaluation of a Novel Lentiviral Vector Driving Lineage-Specific BCL11A Knockdown for Sickle Cell Gene Therapy. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 589-600.	1.8	39
255	Hematopoietic stem cell transplantation for patients with sickle cell disease in the Eastern Mediterranean. <i>Hematology/ Oncology and Stem Cell Therapy</i> , 2020, 13, 106-110.	0.6	0
256	Curative options for sickle cell disease in Africa: Approach in Tanzania. <i>Hematology/ Oncology and Stem Cell Therapy</i> , 2020, 13, 66-70.	0.6	10
257	Haploidentical bone marrow transplant with posttransplant cyclophosphamide for sickle cell disease: An update. <i>Hematology/ Oncology and Stem Cell Therapy</i> , 2020, 13, 91-97.	0.6	10
258	A Comprehensive Review of the Treatment and Management of Pain in Sickle Cell Disease. <i>Current Pain and Headache Reports</i> , 2020, 24, 17.	1.3	6
259	Treating sickle cell anemia. <i>Science</i> , 2020, 367, 1198-1199.	6.0	44

#	ARTICLE	IF	CITATIONS
260	Ex vivo regional gene therapy with human adipose-derived stem cells for bone repair. <i>Bone</i> , 2020, 138, 115524.	1.4	16
261	Safe and efficient peripheral blood stem cell collection in patients with sickle cell disease using plerixafor. <i>Haematologica</i> , 2020, 105, e497.	1.7	29
262	Haptoglobin Therapeutics and Compartmentalization of Cell-Free Hemoglobin Toxicity. <i>Trends in Molecular Medicine</i> , 2020, 26, 683-697.	3.5	58
263	Ready for Repair? Gene Editing Enters the Clinic for the Treatment of Human Disease. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 18, 532-557.	1.8	67
264	Gene therapy for primary immune deficiencies. , 2020, , 1215-1228.		0
265	Priorities for Improving Outcomes for Nonmalignant Blood Diseases: A Report from the Blood and Marrow Transplant Clinical Trials Network. <i>Biology of Blood and Marrow Transplantation</i> , 2020, 26, e94-e100.	2.0	3
266	Improved health care utilization and costs in transplanted versus non-transplanted adults with sickle cell disease. <i>PLoS ONE</i> , 2020, 15, e0229710.	1.1	14
267	Editing a $\beta^3$ -globin repressor binding site restores fetal hemoglobin synthesis and corrects the sickle cell disease phenotype. <i>Science Advances</i> , 2020, 6, .	4.7	91
268	Curative options for sickle cell disease: haploidentical stem cell transplantation or gene therapy?. <i>British Journal of Haematology</i> , 2020, 189, 408-423.	1.2	29
269	Cardiac causes of hypoxia in sickle cell disease. <i>Progress in Pediatric Cardiology</i> , 2020, 56, 101192.	0.2	1
270	Lentiviral gene therapy for X-linked chronic granulomatous disease. <i>Nature Medicine</i> , 2020, 26, 200-206.	15.2	175
272	Enhanced Transduction of Human Hematopoietic Stem Cells by AAV6 Vectors: Implications in Gene Therapy and Genome Editing. <i>Molecular Therapy - Nucleic Acids</i> , 2020, 20, 451-458.	2.3	17
273	Development of new self-assembled cationic amino liposomes for efficient gene delivery. <i>Biomaterials Science</i> , 2020, 8, 3021-3025.	2.6	13
274	Current and future gene therapies for hemoglobinopathies. <i>Current Opinion in Hematology</i> , 2020, 27, 149-154.	1.2	9
275	Clonal tracking in gene therapy patients reveals a diversity of human hematopoietic differentiation programs. <i>Blood</i> , 2020, 135, 1219-1231.	0.6	50
276	CRISPR/Cas9 for the treatment of haematological diseases: a journey from bacteria to the bedside. <i>British Journal of Haematology</i> , 2021, 192, 33-49.	1.2	4
277	How will new genetic technologies, such as gene editing, change reproductive decision-making? Views of high-risk couples. <i>European Journal of Human Genetics</i> , 2021, 29, 39-50.	1.4	4
278	Lentiviral vector bioprocess economics for cell and gene therapy commercialization. <i>Biochemical Engineering Journal</i> , 2021, 167, 107868.	1.8	26



#	ARTICLE	IF	CITATIONS
279	CRISPR-Cas9 Gene Editing for Sickle Cell Disease and $\beta^2$ -Thalassemia. <i>New England Journal of Medicine</i> , 2021, 384, 252-260.	13.9	939
280	Post-Transcriptional Genetic Silencing of <i>BCL11A</i> to Treat Sickle Cell Disease. <i>New England Journal of Medicine</i> , 2021, 384, 205-215.	13.9	250
281	PLGA-Nanoparticles for Intracellular Delivery of the CRISPR-Complex to Elevate Fetal Globin Expression in Erythroid Cells. <i>Biomaterials</i> , 2021, 268, 120580.	5.7	29
282	Clinical management of sickle cell liver disease in children and young adults. <i>Archives of Disease in Childhood</i> , 2021, 106, 315-320.	1.0	10
283	Systematic Review/Meta-Analysis on Efficacy of Allogeneic Hematopoietic Cell Transplantation in Sickle Cell Disease: An International Effort on Behalf of the Pediatric Diseases Working Party of European Society for Blood and Marrow Transplantation and the Sickle Cell Transplantation International Consortium. <i>Transplantation and Cellular Therapy</i> , 2021, 27, 167.e1-167.e12.	0.6	8
284	Gene therapy using haematopoietic stem and progenitor cells. <i>Nature Reviews Genetics</i> , 2021, 22, 216-234.	7.7	151
285	Restoration of $\beta^2$ -Globin Expression with Optimally Designed Lentiviral Vector for $\beta^2$ -Thalassemia Treatment in Chinese Patients. <i>Human Gene Therapy</i> , 2021, 32, 481-494.	1.4	6
286	Liver-directed gene-based therapies for inborn errors of metabolism. <i>Expert Opinion on Biological Therapy</i> , 2021, 21, 229-240.	1.4	11
287	What are the key considerations when prescribing pharmacotherapy for sickle cell anemia?. <i>Expert Opinion on Pharmacotherapy</i> , 2021, 22, 5-8.	0.9	0
288	Hematopoietic Cell Transplant and Cellular Therapies for Sickle Cell Disease. , 2021, , 383-399.		0
289	A Novel Branched DNA-Based Flowcytometric Method for Single-Cell Characterization of Gene Therapy Products and Expression of Therapeutic Genes. <i>Frontiers in Immunology</i> , 2020, 11, 607991.	2.2	2
290	Microfluidic electrical impedance assessment of red blood cell-mediated microvascular occlusion. <i>Lab on A Chip</i> , 2021, 21, 1036-1048.	3.1	25
291	HSCT in Benign Hematological Disorders. <i>Organ and Tissue Transplantation</i> , 2021, , 115-163.	0.0	0
292	Hematopoietic Cell Transplantation for Sickle Cell Disease. <i>Frontiers in Pediatrics</i> , 2020, 8, 551170.	0.9	14
293	Sickle Cell Disease. <i>Annals of Internal Medicine</i> , 2021, 174, ITC1-ITC16.	2.0	38
294	A systematic review of quality of life in sickle cell disease and thalassemia after stem cell transplant or gene therapy. <i>Blood Advances</i> , 2021, 5, 570-583.	2.5	38
295	Therapeutic gene editing strategies using CRISPR-Cas9 for the $\beta^2$ -hemoglobinopathies. <i>Journal of Biomedical Research</i> , 2021, 35, 115.	0.7	6
296	Feasibility of establishing a network of community health workers to support care of people with sickle cell disease in Kumasi, Ghana. <i>Journal of Community Genetics</i> , 2021, 12, 155-161.	0.5	1

#	ARTICLE	IF	CITATIONS
297	Non-Myeloablative human leukocyte antigen-matched related donor transplantation in sickle cell disease: outcomes from three independent centres. <i>British Journal of Haematology</i> , 2021, 192, 761-768.	1.2	41
298	A plethora of gene therapies for hemoglobinopathies. <i>Nature Medicine</i> , 2021, 27, 202-204.	15.2	4
299	Hematopoietic Stem Cell-Targeted Gene-Addition and Gene-Editing Strategies for $\beta^2$ -hemoglobinopathies. <i>Cell Stem Cell</i> , 2021, 28, 191-208.	5.2	17
300	Evidence-based interventions implemented in low-and middle-income countries for sickle cell disease management: A systematic review of randomized controlled trials. <i>PLoS ONE</i> , 2021, 16, e0246700.	1.1	11
301	Update on Clinical Ex Vivo Hematopoietic Stem Cell Gene Therapy for Inherited Monogenic Diseases. <i>Molecular Therapy</i> , 2021, 29, 489-504.	3.7	46
302	Parameters affecting successful stem cell collections for genetic therapies in sickle cell disease. <i>Transfusion and Apheresis Science</i> , 2021, 60, 103059.	0.5	3
303	The Cas9 Hammer and Sickle: A Challenge for Genome Editors. <i>CRISPR Journal</i> , 2021, 4, 6-13.	1.4	11
304	Ineffective erythropoiesis in sickle cell disease: new insights and future implications. <i>Current Opinion in Hematology</i> , 2021, 28, 171-176.	1.2	10
305	Lentivirus-mediated gene therapy for Fabry disease. <i>Nature Communications</i> , 2021, 12, 1178.	5.8	58
306	CRISPR/Cas9 gene editing for curing sickle cell disease. <i>Transfusion and Apheresis Science</i> , 2021, 60, 103060.	0.5	32
307	Management of Sickle Cell Disease Complications Beyond Acute Chest Syndrome. <i>Journal of Blood Medicine</i> , 2021, Volume 12, 101-114.	0.7	7
308	Allogeneic stem cell transplantation with omidubicel in sickle cell disease. <i>Blood Advances</i> , 2021, 5, 843-852.	2.5	15
309	Biophysical and rheological biomarkers of red blood cell physiology and pathophysiology. <i>Current Opinion in Hematology</i> , 2021, 28, 138-149.	1.2	15
310	Molecular Medicine: Found in Translation. <i>Med</i> , 2021, 2, 122-136.	2.2	13
311	Gene therapy for hemoglobinopathies. <i>Transfusion and Apheresis Science</i> , 2021, 60, 103061.	0.5	6
312	Implications of hematopoietic stem cells heterogeneity for gene therapies. <i>Gene Therapy</i> , 2021, 28, 528-541.	2.3	12
313	Persistence of CRISPR/Cas9 Gene Edited Hematopoietic Stem Cells Following Transplantation: A Systematic Review and Meta-Analysis of Preclinical Studies. <i>Stem Cells Translational Medicine</i> , 2021, 10, 996-1007.	1.6	8
314	Recent Advances in Preclinical Research Using PAMAM Dendrimers for Cancer Gene Therapy. <i>International Journal of Molecular Sciences</i> , 2021, 22, 2912.	1.8	54

#	ARTICLE	IF	CITATIONS
315	Genome editing using CRISPR/Cas9 to treat hereditary hematological disorders. <i>Gene Therapy</i> , 2022, 29, 207-216.	2.3	10
316	Recent trends in cancer therapy: A review on the current state of gene delivery. <i>Life Sciences</i> , 2021, 269, 119087.	2.0	108
317	Gene therapy for infantile malignant osteopetrosis: review of pre-clinical research and proof-of-concept for phenotypic reversal. <i>Molecular Therapy - Methods and Clinical Development</i> , 2021, 20, 389-397.	1.8	5
318	In Vivo Survival and Differentiation of Friedreich Ataxia iPSC-Derived Sensory Neurons Transplanted in the Adult Dorsal Root Ganglia. <i>Stem Cells Translational Medicine</i> , 2021, 10, 1157-1169.	1.6	4
319	Cardiac pathophysiology in sickle cell disease. <i>Journal of Thrombosis and Thrombolysis</i> , 2021, 52, 248-259.	1.0	1
320	Transfusion and Cellular Therapy in Pediatric Sickle Cell Disease. <i>Clinics in Laboratory Medicine</i> , 2021, 41, 101-119.	0.7	6
321	Long-Term Follow-Up of Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. <i>Human Gene Therapy</i> , 2021, 32, 1260-1269.	1.4	21
322	Sustained fetal hemoglobin induction in vivo is achieved by <i>BCL11A</i> interference and coexpressed truncated erythropoietin receptor. <i>Science Translational Medicine</i> , 2021, 13, .	5.8	6
323	The Affinity of Hemoglobin for Oxygen Is Not Altered During COVID-19. <i>Frontiers in Physiology</i> , 2021, 12, 578708.	1.3	20
324	Preclinical evaluation for engraftment of CD34+ cells gene-edited at the sickle cell disease locus in xenograft mouse and non-human primate models. <i>Cell Reports Medicine</i> , 2021, 2, 100247.	3.3	15
326	Marginal zone B cells mediate a CD4 T-cell-dependent extrafollicular antibody response following RBC transfusion in mice. <i>Blood</i> , 2021, 138, 706-721.	0.6	34
327	Lentiviral vector ALS20 yields high hemoglobin levels with low genomic integrations for treatment of beta-globinopathies. <i>Molecular Therapy</i> , 2021, 29, 1625-1638.	3.7	10
328	Genetic therapies for the first molecular disease. <i>Journal of Clinical Investigation</i> , 2021, 131, .	3.9	17
329	Delta-globin gene expression improves sickle cell disease in a humanised mouse model. <i>British Journal of Haematology</i> , 2021, 193, 1228-1237.	1.2	7
330	Disease severity impacts plerixafor-mobilized stem cell collection in patients with sickle cell disease. <i>Blood Advances</i> , 2021, 5, 2403-2411.	2.5	24
331	Haematology in the UK: A 60-year personal perspective. <i>EJHaem</i> , 2021, 2, 569-576.	0.4	0
332	Advances in neuroimaging to improve care in sickle cell disease. <i>Lancet Neurology</i> , The, 2021, 20, 398-408.	4.9	6
333	Gene Therapy as the New Frontier for Sickle Cell Disease. <i>Current Medicinal Chemistry</i> , 2022, 29, 453-466.	1.2	6

#	ARTICLE	IF	CITATIONS
334	Inclusion of a short hairpin RNA targeting <i>BCL11A</i> into a $\beta$ -globin expressing vector allows concurrent synthesis of curative adult and fetal hemoglobin. <i>Haematologica</i> , 2021, 106, 2740-2745.	1.7	5
335	Strategies to Uplift Novel Mendelian Gene Discovery for Improved Clinical Outcomes. <i>Frontiers in Genetics</i> , 2021, 12, 674295.	1.1	23
337	Ex vivo expansion of hematopoietic stem cells: Finally transitioning from the lab to the clinic. <i>Blood Reviews</i> , 2021, 50, 100853.	2.8	20
338	Leukemia after gene therapy for sickle cell disease: insertional mutagenesis, busulfan, both, or neither. <i>Blood</i> , 2021, 138, 942-947.	0.6	49
339	CRISPR-Cas9 can cause chromothripsis. <i>Nature Genetics</i> , 2021, 53, 768-769.	9.4	7
340	Innovative Treatments for Rare Anemias. <i>HemaSphere</i> , 2021, 5, e576.	1.2	13
341	A systems pharmacology model for gene therapy in sickle cell disease. <i>CPT: Pharmacometrics and Systems Pharmacology</i> , 2021, 10, 696-708.	1.3	2
342	Base editing of hematopoietic stem cells rescues sickle cell disease in mice. <i>Nature</i> , 2021, 595, 295-302.	13.7	175
343	Cas9 protein delivery non-integrating lentiviral vectors for gene correction in sickle cell disease. <i>Molecular Therapy - Methods and Clinical Development</i> , 2021, 21, 121-132.	1.8	25
344	Development of $\beta$ -globin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease. <i>Science Translational Medicine</i> , 2021, 13, .	5.8	82
345	Research in Sickle Cell Disease: From Bedside to Bench to Bedside. <i>HemaSphere</i> , 2021, 5, e584.	1.2	16
346	Therapy Development by Genome Editing of Hematopoietic Stem Cells. <i>Cells</i> , 2021, 10, 1492.	1.8	15
347	Genome-based therapeutic interventions for $\beta$ -type hemoglobinopathies. <i>Human Genomics</i> , 2021, 15, 32.	1.4	10
348	Biomarkers for the central nervous system complications of sickle cell disease: are we there yet?. <i>Proteomics - Clinical Applications</i> , 2021, 15, 2100026.	0.8	0
349	Gene therapy for sickle cell disease: moving from the bench to the bedside. <i>Blood</i> , 2021, 138, 932-941.	0.6	37
350	Allogeneic hematopoietic stem cell transplant for sickle cell disease: The why, who, and what. <i>Blood Reviews</i> , 2021, 50, 100868.	2.8	7
351	Exploiting Single-Cell Tools in Gene and Cell Therapy. <i>Frontiers in Immunology</i> , 2021, 12, 702636.	2.2	21
352	Antibiotics to modify sickle cell disease vaso-occlusive crisis?. <i>Blood Reviews</i> , 2021, 50, 100867.	2.8	2

#	ARTICLE	IF	CITATIONS
353	Cardiovascular phenotypes predict clinical outcomes in sickle cell disease: An echocardiographyâ€based cluster analysis. American Journal of Hematology, 2021, 96, 1166-1175.	2.0	5
354	Gene therapy for Fabry disease: Progress, challenges, and outlooks on gene-editing. Molecular Genetics and Metabolism, 2021, 134, 117-131.	0.5	13
355	Murine bone marrow mesenchymal stromal cells have reduced hematopoietic maintenance ability in sickle cell disease. Blood, 2021, 138, 2570-2582.	0.6	12
357	Wild-type HIV infection after treatment with lentiviral gene therapy for $\beta^0$ -thalassemia. Blood Advances, 2021, 5, 2701-2706.	2.5	4
358	Characterization and Optimization of Chitosan-Coated Polybutylcyanoacrylate Nanoparticles for the Transfection-Guided Neural Differentiation of Mouse Induced Pluripotent Stem Cells. International Journal of Molecular Sciences, 2021, 22, 8741.	1.8	1
359	Designing Lentiviral Vectors for Gene Therapy of Genetic Diseases. Viruses, 2021, 13, 1526.	1.5	27
360	Gene therapies close in on a cure for sickle-cell disease. Nature, 2021, 596, S2-S4.	13.7	2
361	Combination of lentiviral and genome editing technologies for the treatment of sickle cell disease. Molecular Therapy, 2022, 30, 145-163.	3.7	6
363	Initial experimental experience of tripleâ€knockout pig red blood cells as potential sources for transfusion in alloimmunized patients with sickle cell disease. Transfusion, 2021, 61, 3104-3118.	0.8	10
364	Coordinated $\beta^0$ -globin expression and $\beta^+$ -globin reduction in a multiplex lentiviral gene therapy vector for $\beta^0$ -thalassemia. Molecular Therapy, 2021, 29, 2841-2853.	3.7	11
366	Preferential Expansion of Human CD34 <sup>+</sup> CD133 <sup>+</sup> CD90 <sup>+</sup> Hematopoietic Stem Cells Enhances Gene-Modified Cell Frequency for Gene Therapy. Human Gene Therapy, 2022, 33, 188-201.	1.4	5
367	Society for Maternal-Fetal Medicine Special Statement: Beyond the Scalpel: In Utero Fetal Gene Therapy and Curative Medicine. American Journal of Obstetrics and Gynecology, 2021, 225, B9-B18.	0.7	4
368	Clinically relevant gene editing in hematopoietic stem cells for the treatment of pyruvate kinase deficiency. Molecular Therapy - Methods and Clinical Development, 2021, 22, 237-248.	1.8	11
369	How I approach diseaseâ€modifying therapy in children with sickle cell disease in an era of novel therapies. Pediatric Blood and Cancer, 2021, 68, e29363.	0.8	2
370	Lifting the lid on perioperative goal-directed therapy. British Journal of Anaesthesia, 2021, 127, 508-510.	1.5	0
371	Genome editing in large animal models. Molecular Therapy, 2021, 29, 3140-3152.	3.7	18
372	Gene therapy process change evaluation framework: Transient transfection and stable producer cell line comparison. Biochemical Engineering Journal, 2021, 176, 108202.	1.8	10
373	Pediatric Bone Marrow Transplantation. Organ and Tissue Transplantation, 2021, , 577-616.	0.0	0

#	ARTICLE	IF	CITATIONS
374	Genome Editing for $\beta^2$ -Hemoglobinopathies: Advances and Challenges. Journal of Clinical Medicine, 2021, 10, 482.	1.0	17
375	Sickle cell disease: progress towards combination drug therapy. British Journal of Haematology, 2021, 194, 240-251.	1.2	17
376	Safe and Effective <i>In Vivo</i> Targeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development. Human Gene Therapy, 2021, 32, 31-42.	1.4	15
377	Introduction to Precision Medicine: Minority Populations and Cardiovascular Health. Contemporary Cardiology, 2021, , 13-22.	0.0	0
379	Studying ALS: Current Approaches, Effect on Potential Treatment Strategy. Advances in Experimental Medicine and Biology, 2020, 1241, 195-217.	0.8	16
380	Embryonic and Fetal Human Hemoglobins: Structures, Oxygen Binding, and Physiological Roles. Sub-Cellular Biochemistry, 2020, 94, 275-296.	1.0	7
381	Clinical Manifestations of Sickle Cell Disease Across the Lifespan. , 2018, , 3-39.		3
382	Gene Therapy: The Path Toward Becoming a Realistic Cure for Sickle Cell Disease. , 2018, , 303-328.		1
383	Gene therapy and gene editing. , 2020, , 463-477.		2
384	CRISPR-Cas systems: Overview, innovations and applications in human disease research and gene therapy. Computational and Structural Biotechnology Journal, 2020, 18, 2401-2415.	1.9	100
385	Immune mechanisms involved in sickle cell disease pathogenesis: current knowledge and perspectives. Immunology Letters, 2020, 224, 1-11.	1.1	16
386	Microfluidic assessment of red blood cell mediated microvascular occlusion. Lab on A Chip, 2020, 20, 2086-2099.	3.1	46
387	Hepatocyte ALOXE3 is induced during adaptive fasting and enhances insulin sensitivity by activating hepatic PPAR $\beta$ . JCI Insight, 2018, 3, .	2.3	21
388	Are genetic approaches still needed to cure sickle cell disease?. Journal of Clinical Investigation, 2019, 130, 7-9.	3.9	8
389	Reducing Health Care Disparities in Sickle Cell Disease: A Review. , 0, .		1
390	Optimal disease management and health monitoring in adults with sickle cell disease. Hematology American Society of Hematology Education Program, 2019, 2019, 505-512.	0.9	7
391	Drug Therapies for the Management of Sickle Cell Disease. F1000Research, 2020, 9, 592.	0.8	29
392	New insights on stem cells modeling and treatment of human diseases. Frontiers in Bioscience - Landmark, 2020, 25, 1568-1599.	3.0	3

#	ARTICLE	IF	CITATIONS
393	Curative Therapies for Sickle Cell Disease. <i>Ochsner Journal</i> , 2019, 19, 131-137.	0.5	20
394	Gene therapy in sickle cell disease: Possible utility and impact. <i>Cleveland Clinic Journal of Medicine</i> , 2020, 87, 28-29.	0.6	4
395	Therapeutic advances in sickle cell disease in the last decade. <i>Indian Journal of Medical Research</i> , 2017, 145, 708.	0.4	2
396	Editing outside the body: Ex vivo gene-modification for $\beta^2$ -hemoglobinopathy cellular therapy. <i>Molecular Therapy</i> , 2021, 29, 3163-3178.	3.7	12
397	Rounding up sickle cells with gene therapy. <i>Science Translational Medicine</i> , 2017, 9, .	5.8	0
398	Agir sur les gènes est-ce suffisant?. <i>Archives De Philosophie Du Droit</i> , 2017, Tome 59, 39-52.	0.0	1
399	Sickle Cell Disease and Hematopoietic Stem Cell Transplantation. , 2018, , .		2
402	Hematopoietic Stem Cell Gene Therapy for Inherited Monogenic Diseases and Its Implications for Future Gene Therapy Trials in Turke. <i>Trakya University Journal of Natural Sciences</i> , 0, , .	0.4	0
403	Sickle Cell Anemia: A review on the most severe form of Sickle Cell Disease. <i>Revista Bionatura</i> , 2019, 02, .	0.1	0
407	Biopharmaceutical molecules. , 2020, , 31-68.		1
408	Thérapie génique. , 2020, , 219-224.		0
409	Osmolality Threshold for Sickle Cell Erythrocyte Hemolysis. <i>Journal of Clinical Medical Research</i> , 2020, 01, .	0.1	0
410	Harnessing microbial iron chelators to develop innovative therapeutic agents. <i>Journal of Advanced Research</i> , 2022, 39, 89-101.	4.4	10
411	Sickle Cell Disease: A Primer for Primary Care Providers. <i>Pediatric Annals</i> , 2020, 49, e43-e49.	0.3	0
412	HSCT in Benign Hematological Disorders. <i>Organ and Tissue Transplantation</i> , 2021, , 1-49.	0.0	0
413	Pediatric Bone Marrow Transplantation. <i>Organ and Tissue Transplantation</i> , 2020, , 1-41.	0.0	0
414	Hematopoietic Stem Cells. <i>Learning Materials in Biosciences</i> , 2020, , 1-19.	0.2	0
415	Sickle cell disease: A primary care update. <i>Cleveland Clinic Journal of Medicine</i> , 2020, 87, 19-27.	0.6	21

#	ARTICLE	IF	CITATIONS
416	Sickle Cell Disease in the Adolescent Female. , 2020, , 217-225.		0
417	Recent advances in lentiviral vectors for gene therapy. Science China Life Sciences, 2021, 64, 1842-1857.	2.3	16
419	Gene therapy and editing in the treatment of hereditary blood disorders: Medical and ethical aspects. Clinical Ethics, 0, , 147775092110572.	0.5	0
420	Treatment decision-making in sickle cell disease patients. Journal of Community Genetics, 2022, 13, 143-151.	0.5	3
421	Lentiviral Transduction for Optimal LSC/HSC Manipulation. Methods in Molecular Biology, 2021, 2185, 299-306.	0.4	0
422	The Role of Gene Therapy in Cartilage Repair. Archives of Bone and Joint Surgery, 2019, 7, 79-90.	0.1	10
423	Enhancing therapeutic efficacy of in vivo platelet-targeted gene therapy in hemophilia A mice. Blood Advances, 2020, 4, 5722-5734.	2.5	3
426	Treating Rare Diseases in Africa: The Drugs Exist but the Need Is Unmet. Frontiers in Pharmacology, 2021, 12, 770640.	1.6	14
427	l-glutamine, crizanlizumab, voxelotor, and cell-based therapy for adult sickle cell disease: Hype or hope?. Blood Reviews, 2022, 53, 100925.	2.8	22
428	Biologic and Clinical Efficacy of LentiGlobin for Sickle Cell Disease. New England Journal of Medicine, 2022, 386, 617-628.	13.9	144
429	Acute Myeloid Leukemia Case after Gene Therapy for Sickle Cell Disease. New England Journal of Medicine, 2022, 386, 138-147.	13.9	86
431	Size and density measurements of single sickle red blood cells using microfluidic magnetic levitation. Lab on A Chip, 2022, 22, 683-696.	3.1	16
432	Long-term outcomes of lentiviral gene therapy for the $\beta^2$ -hemoglobinopathies: the HGB-205 trial. Nature Medicine, 2022, 28, 81-88.	15.2	53
434	Pharmacologic induction of PGC $\alpha 1 \pm$ stimulates fetal haemoglobin gene expression. British Journal of Haematology, 2022, , .	1.2	4
435	The EHA Research Roadmap: Hematopoietic Stem Cell Gene Therapy. HemaSphere, 2022, 6, e671.	1.2	8
436	Autologous, lentivirus-modified, T $\alpha$ rapa cell $\alpha$ micropharmacies $\alpha$ for lysosomal storage disorders. EMBO Molecular Medicine, 2022, 14, e14297.	3.3	5
437	A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. Nature Communications, 2022, 13, 1315.	5.8	61
438	Microfluidic Methods to Advance Mechanistic Understanding and Translational Research in Sickle Cell Disease. Translational Research, 2022, , .	2.2	0



#	ARTICLE	IF	CITATIONS
439	Gene Editing for Inherited Red Blood Cell Diseases. <i>Frontiers in Physiology</i> , 2022, 13, 848261.	1.3	5
440	A landscape analysis and discussion of value of gene therapies for sickle cell disease. <i>Expert Review of Pharmacoeconomics and Outcomes Research</i> , 2022, 22, 891-911.	0.7	7
441	Treatment of sickle cell disease: Beyond hydroxyurea. , 0, .		0
442	Elastic property of sickle and normal hemoglobin protein: Molecular dynamics. <i>AIP Advances</i> , 2022, 12, .	0.6	1
443	Gene therapy access: Global challenges, opportunities, and views from Brazil, South Africa, and India. <i>Molecular Therapy</i> , 2022, 30, 2122-2129.	3.7	12
444	Catching Them Early: Framework Parameters and Progress for Prenatal and Childhood Application of Advanced Therapies. <i>Pharmaceutics</i> , 2022, 14, 793.	2.0	4
445	Impact of hydroxyurea dose and adherence on hematologic outcomes for children with sickle cell anemia. <i>Pediatric Blood and Cancer</i> , 2022, , e29607.	0.8	3
446	Of mice and men: From hematopoiesis in mouse models to curative gene therapy for sickle cell disease. <i>Cell</i> , 2022, , .	13.5	3
447	Targeting fetal hemoglobin expression to treat $\beta^2$ hemoglobinopathies. <i>Expert Opinion on Therapeutic Targets</i> , 2022, 26, 347-359.	1.5	7
448	Validation of single-gene noninvasive prenatal testing for sickle cell disease. <i>American Journal of Hematology</i> , 2022, 97, .	2.0	9
454	High-level correction of the sickle mutation is amplified in vivo during erythroid differentiation. <i>IScience</i> , 2022, 25, 104374.	1.9	22
455	20 Years of Legislation - How Australia Has Responded to the Challenge of Regulating Genetically Modified Organisms in the Clinic. <i>Frontiers in Medicine</i> , 2022, 9, .	1.2	2
456	Protocol for "Genetic composition of sickle cell disease in the Arab population: A systematic review". <i>Health Science Reports</i> , 2022, 5, e450.	0.6	4
457	Targeting the Hematopoietic Stem Cell Niche in $\beta^2$ -Thalassemia and Sickle Cell Disease. <i>Pharmaceutics</i> , 2022, 15, 592.	1.7	5
458	Induction of Fetal Hemoglobin by Introducing Natural Hereditary Persistence of Fetal Hemoglobin Mutations in the $\beta^3$ -Globin Gene Promoters for Genome Editing Therapies for $\beta^2$ -Thalassemia. <i>Frontiers in Genetics</i> , 2022, 13, .	1.1	3
459	High-throughput analysis of hematopoietic stem cell engraftment after intravenous and intracerebroventricular dosing. <i>Molecular Therapy</i> , 2022, 30, 3209-3225.	3.7	4
460	From Mendel to a Mendelian disorder: towards a cure for sickle cell disease. <i>Nature Reviews Genetics</i> , 2022, 23, 389-390.	7.7	3
461	Long-Term Health Effects of Curative Therapies on Heart, Lungs, and Kidneys for Individuals with Sickle Cell Disease Compared to Those with Hematologic Malignancies. <i>Journal of Clinical Medicine</i> , 2022, 11, 3118.	1.0	3

#	ARTICLE	IF	CITATIONS
462	Development and clinical translation of ex vivo gene therapy. Computational and Structural Biotechnology Journal, 2022, 20, 2986-3003.	1.9	1
463	Protection is not always a good thing: The immune system's impact on gene therapy. Genetics and Molecular Biology, 2022, 45, .	0.6	2
464	A review on the current progress of layered double hydroxide application in biomedical sectors. European Physical Journal Plus, 2022, 137, .	1.2	4
465	Intraosseous delivery of platelet-targeted factor VIII lentiviral vector in humanized NBSCGW mice. Blood Advances, 2022, 6, 5556-5569.	2.5	2
466	Recent advances in sickle cell research - Tribute to Dr. Paul S Frenette -. Stem Cell Reports, 2022, 17, 1509-1535.	2.3	8
467	Metabolic Reprogramming in Sickle Cell Diseases: Pathophysiology and Drug Discovery Opportunities. International Journal of Molecular Sciences, 2022, 23, 7448.	1.8	2
468	Gene Therapy for Hemoglobinopathies. Hematology/Oncology Clinics of North America, 2022, 36, 769-795.	0.9	9
469	Hemoglobin: Physiology and Hemoglobinopathy. , 2022, , 45-51.		1
470	Emerging drugs for the treatment of sickle cell disease: a review of phase II/III trials. Expert Opinion on Emerging Drugs, 2022, 27, 211-224.	1.0	2
471	A high-throughput microfluidic device based on controlled incremental filtration to enable centrifugation-free, low extracorporeal volume leukapheresis. Scientific Reports, 2022, 12, .	1.6	4
472	OcclusionChip: A functional microcapillary occlusion assay complementary to ektacytometry for detection of small-fraction red blood cells with abnormal deformability. Frontiers in Physiology, 0, 13, .	1.3	5
473	Harnessing nucleic acid technologies for human health on earth and in space. Life Sciences in Space Research, 2022, 35, 113-126.	1.2	2
474	Hemoglobinopathies and Thalassemias. , 2023, , 143-172.		1
475	Replication dependent and independent mechanisms of GAA repeat instability. DNA Repair, 2022, 118, 103385.	1.3	4
477	Incidence, kinetics, and risk factors for intra- and extracranial cerebral arteriopathies in a newborn sickle cell disease cohort early assessed by transcranial and cervical color Doppler ultrasound. Frontiers in Neurology, 0, 13, .	1.1	1
478	IGF2-tagging of GAA promotes full correction of murine Pompe disease at a clinically relevant dosage of lentiviral gene therapy. Molecular Therapy - Methods and Clinical Development, 2022, 27, 109-130.	1.8	9
479	Lovo's gene therapy for sickle cell disease: Treatment process evolution and outcomes in the initial groups of the HGB-206 study. American Journal of Hematology, 2023, 98, 11-22.	2.0	18
480	Nomenclature for Cellular and Genetic Therapies: A Need for Standardization. Transplantation and Cellular Therapy, 2022, 28, 795-801.	0.6	1

#	ARTICLE	IF	CITATIONS
481	Gene Therapy Cargoes Based on Viral Vector Delivery. <i>Current Gene Therapy</i> , 2023, 23, 111-134.	0.9	5
482	Delivering genes with human immunodeficiency virus-derived vehicles: still state-of-the-art after 25 years. <i>Journal of Biomedical Science</i> , 2022, 29, .	2.6	8
483	Progress de la thérapie génique dans les maladies génétiques du système hématopoïétique. <i>Medicine/Sciences</i> , 2022, 38, 768-771.	0.0	0
484	Gene therapy for primary mitochondrial diseases: experimental advances and clinical challenges. <i>Nature Reviews Neurology</i> , 2022, 18, 689-698.	4.9	18
485	Promising therapeutic aspects in human genetic imprinting disorders. <i>Clinical Epigenetics</i> , 2022, 14, .	1.8	2
486	Site-specific genome editing in treatment of inherited diseases: possibility, progress, and perspectives. <i>Medical Review</i> , 2022, 2, 471-500.	0.3	6
487	Intraosseous injection of SMNP vectors enables CRISPR/Cas9-mediated knock-in of HBB gene into hematopoietic stem and progenitor cells. <i>Nano Today</i> , 2022, 47, 101659.	6.2	2
488	Overview of the Risk of Infection Associated with Biologic and Target Therapies. , 2022, , 3-15.		0
489	Kruppel-like factor 1 GATA1 fusion protein improves the sickle cell disease phenotype in mice both in vitro and in vivo. <i>Blood</i> , 2022, 140, 2276-2289.	0.6	4
490	Comparison of busulfan and total body irradiation conditioning on hematopoietic clonal dynamics following lentiviral gene transfer in rhesus macaques. <i>Molecular Therapy - Methods and Clinical Development</i> , 2023, 28, 62-75.	1.8	4
491	Different Therapeutic Interventions and Mechanisms of Action of Antisickling Agents Currently in Use in Sickle Cell Disease Management. <i>European Medical Journal Hematology</i> , 0, , 113-117.	0.0	1
492	Fetal hemoglobin per erythrocyte (<math>\langle \text{HbF} \rangle / \text{cell}</math>) after gene therapy for sickle cell anemia. <i>American Journal of Hematology</i> , 2023, 98, .	2.0	2
493	Development of curative therapies for sickle cell disease. <i>Frontiers in Medicine</i> , 0, 9, .	1.2	5
494	The Optimized $\beta^3$ -Globin Lentiviral Vector GGHI-mB-3D Leads to Nearly Therapeutic HbF Levels In Vitro in CD34+ Cells from Sickle Cell Disease Patients. <i>Viruses</i> , 2022, 14, 2716.	1.5	1
495	The Current State of Sickle Cell Disease Treatments [Part I]. , 2022, 1, 465-471.		0
496	Efficient and error-free correction of sickle mutation in human erythroid cells using prime editor-2. <i>Frontiers in Genome Editing</i> , 0, 4, .	2.7	0
497	Donor chimerism and immune reconstitution following haploidentical transplantation in sickle cell disease. <i>Frontiers in Immunology</i> , 0, 13, .	2.2	0
498	Genetic engineering and genome editing in plants, animals and humans: Facts and myths. <i>Gene</i> , 2023, 856, 147141.	1.0	2

#	ARTICLE	IF	CITATIONS
499	Long-term health outcomes following curative therapies for sickle cell disease. Hematology American Society of Hematology Education Program, 2022, 2022, 272-276.	0.9	1
500	Therapeutic adenine base editing of human hematopoietic stem cells. Nature Communications, 2023, 14, .	5.8	16
501	CRISPR/Cas9-mediated gene editing. A promising strategy in hematological disorders. Cytotherapy, 2023, 25, 277-285.	0.3	4
502	Process for production of chimeric antigen receptor-transducing lentivirus particles using infection with replicon particles containing self-replicating RNAs. Biochemical Engineering Journal, 2023, 191, 108814.	1.8	0
503	Gene editing for sickle cell disease and transfusion dependent thalassemias- A cure within reach. Seminars in Hematology, 2023, 60, 3-9.	1.8	4
504	Stem Cell-Based Therapeutic Approaches in Genetic Diseases. Advances in Experimental Medicine and Biology, 2023, , .	0.8	0
505	Polysaccharides from marine resources exhibit great potential in the treatment of tumor: A review. Carbohydrate Polymer Technologies and Applications, 2023, 5, 100308.	1.6	1
506	Novel lentiviral vectors for gene therapy of sickle cell disease combining gene addition and gene silencing strategies. Molecular Therapy - Nucleic Acids, 2023, 32, 229-246.	2.3	3
507	A disposable impedance-based sensor for in-line cell growth monitoring in CAR-T cell manufacturing. Bioelectrochemistry, 2023, 152, 108416.	2.4	4
508	A systematic review comparing allogeneic hematopoietic stem cell transplant to gene therapy in sickle cell disease. Hematology, 2023, 28, .	0.7	5
509	Allogeneic hematopoietic stem cell transplantation to cure sickle cell disease: A review. Frontiers in Medicine, 0, 10, .	1.2	7
510	Microfluidic concurrent assessment of red blood cell adhesion and microcapillary occlusion: potential hemorheological biomarkers in sickle cell disease. Sensors & Diagnostics, 2023, 2, 457-467.	1.9	0
511	Gene therapy approaches for sickle cell anemia. Transfusion and Apheresis Science, 2023, 62, 103677.	0.5	0
512	Heavy Metal. , 2023, , 553-559.		0
513	Viral Vectors in Gene Therapy: Where Do We Stand in 2023?. Viruses, 2023, 15, 698.	1.5	20
514	Gene Therapy for $\beta^0$ -Hemoglobinopathies: From Discovery to Clinical Trials. Viruses, 2023, 15, 713.	1.5	2
515	Challenges in the treatment of melanoma with BRAF and MEK inhibitors in patients with sickle cell disease: case report and review of the literature. Therapeutic Advances in Hematology, 2023, 14, 204062072311559.	1.1	0
516	The Potential Revolution of Cancer Treatment with CRISPR Technology. Cancers, 2023, 15, 1813.	1.7	7

#	ARTICLE	IF	CITATIONS
517	<scp>pH</scp> regulates hematopoietic stem cell potential viaÂpolyamines. EMBO Reports, 2023, 24, .	2.0	4
518	Development of in vitro gene editing therapy in disease treatment. , 0, 36, 124-131.		0
519	A Review of CRISPR Cas9 for SCA: Treatment Strategies and Could Target Î²-globin Gene and BCL11A Gene using CRISPR Cas9 Prevent the Patient from Sickle Cell Anemia?. Open Access Macedonian Journal of Medical Sciences, 2023, 11, 1-12.	0.1	0
520	Therapeutic perspective for children and young adults living with thalassemia and sickle cell disease. European Journal of Pediatrics, 2023, 182, 2509-2519.	1.3	7
521	Recent progress in the treatment of sickle cell disease: an up-to-date review. Beni-Suef University Journal of Basic and Applied Sciences, 2023, 12, .	0.8	0
523	Introduction: Biotechnologyâ€”An Ever Expanding Toolbox for Medicine. , 2017, , 1-26.		0
528	Sickle Cell Disease: Lessons Learned. , 2023, , 259-275.		0
531	Viral vectors engineered for gene therapy. International Review of Cell and Molecular Biology, 2023, , 1-41.	1.6	1
536	From target discovery to clinical drug development with human genetics. Nature, 2023, 620, 737-745.	13.7	14
545	Perspectives of current understanding and therapeutics of Diamond-Blackfan anemia. Leukemia, 0, , .	3.3	0
553	Cellular Therapies: A Description of the Types of Existing Cellular Therapies and Associated Toxicities. , 2023, , 55-67.		0
554	Looking ahead: ethical and social challenges of somatic gene therapy for sickle cell disease in Africa. Gene Therapy, 0, , .	2.3	0
559	Gene Therapy in Hematology. , 2024, , .		0
560	Applied Stem Cell Research in Sickle Cell Disease. , 2024, , .		0
566	Vector-Mediated Genotoxicity and Mutagenicity in Hematopoietic Stem Cell Gene Therapy. , 2024, , .		0