Katherine A High

List of Publications by Year in descending order

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KATHERINE & HICH

#	Article	IF	CITATIONS
1	Factor IX assay discrepancies in the setting of liver gene therapy using a hyperfunctional variant factor IXâ€Padua. Journal of Thrombosis and Haemostasis, 2021, 19, 1212-1218.	1.9	17
2	89â€Five-year update for the Phase III voretigene neparvovec study in biallelic RPE65 mutation–associated inherited retinal disease. , 2021, , .		1
3	Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A. New England Journal of Medicine, 2021, 385, 1961-1973.	13.9	127
4	Turning genes into medicines—what have we learned from gene therapy drug development in the past decade?. Nature Communications, 2020, 11, 5821.	5.8	18
5	Long-Term Follow-Up of the First in Human Intravascular Delivery of AAV for Gene Transfer: AAV2-hFIX16 for Severe Hemophilia B. Molecular Therapy, 2020, 28, 2073-2082.	3.7	123
6	Gene Therapy. New England Journal of Medicine, 2019, 381, 455-464.	13.9	343
7	Efficacy, Safety, and Durability of Voretigene Neparvovec-rzyl in RPE65 Mutation–Associated Inherited Retinal Dystrophy. Ophthalmology, 2019, 126, 1273-1285.	2.5	239
8	Entering the Modern Era of Gene Therapy. Annual Review of Medicine, 2019, 70, 273-288.	5.0	311
9	Gene therapy comes of age. Science, 2018, 359, .	6.0	936
10	Novel mobility test to assess functional vision in patients with inherited retinal dystrophies. Clinical and Experimental Ophthalmology, 2018, 46, 247-259.	1.3	97
11	Hepatoâ€entrained B220 ⁺ <scp>CD</scp> 11c ⁺ <scp>NK</scp> 1.1 ⁺ cells regulate preâ€metastatic niche formation in theÂlung. EMBO Molecular Medicine, 2018, 10, .	3.3	11
12	Voretigene neparvovec-rzyl for the treatment of biallelic <i>RPE65</i> mutation–associated retinal dystrophy. Expert Opinion on Orphan Drugs, 2018, 6, 457-464.	0.5	9
13	Overcoming the Host Immune Response to Adeno-Associated Virus Gene Delivery Vectors: The Race Between Clearance, Tolerance, Neutralization, and Escape. Annual Review of Virology, 2017, 4, 511-534.	3.0	147
14	Efficacy and safety of voretigene neparvovec (AAV2-hRPE65v2) in patients with RPE65 -mediated inherited retinal dystrophy: a randomised, controlled, open-label, phase 3 trial. Lancet, The, 2017, 390, 849-860.	6.3	1,250
15	Hemophilia B Gene Therapy with a High-Specific-Activity Factor IX Variant. New England Journal of Medicine, 2017, 377, 2215-2227.	13.9	549
16	Successful Phenotype Improvement following Gene Therapy for Severe Hemophilia A in Privately Owned Dogs. PLoS ONE, 2016, 11, e0151800.	1.1	25
17	Safety and durability of effect of contralateral-eye administration of AAV2 gene therapy in patients with childhood-onset blindness caused by RPE65 mutations: a follow-on phase 1 trial. Lancet, The, 2016, 388, 661-672.	6.3	377
18	Sustained correction of FVII deficiency in dogs using AAV-mediated expression of zymogen FVII. Blood, 2016, 127, 565-571.	0.6	19

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19	In vivo genome editing of the albumin locus as a platform for protein replacement therapy. Blood, 2015, 126, 1777-1784.	0.6	256
20	AAV capsid CD8+ T-cell epitopes are highly conserved across AAV serotypes. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15029.	1.8	59
21	In Vivo Genome Editing in Neonatal Mouse Liver Preferentially Utilizes Homology Directed Repair. Blood, 2015, 126, 4422-4422.	0.6	1
22	Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B. New England Journal of Medicine, 2014, 371, 1994-2004.	13.9	1,063
23	Cellular Localization and Characterization of Cytosolic Binding Partners for Gla Domain-containing Proteins PRRG4 and PRRG2. Journal of Biological Chemistry, 2013, 288, 25908-25914.	1.6	13
24	Pharmacological Modulation of Humoral Immunity in a Nonhuman Primate Model of AAV Gene Transfer for Hemophilia B. Molecular Therapy, 2012, 20, 1410-1416.	3.7	90
25	Factor IX expression in skeletal muscle of a severe hemophilia B patient 10 years after AAV-mediated gene transfer. Blood, 2012, 119, 3038-3041.	0.6	183
26	The gene therapy journey for hemophilia: are we there yet?. Blood, 2012, 120, 4482-4487.	0.6	57
27	The gene therapy journey for hemophilia: are we there yet?. Hematology American Society of Hematology Education Program, 2012, 2012, 375-81.	0.9	20
28	A Novel Strategy to Circumvent Pre-Existing Humoral Immunity to AAV. Blood, 2012, 120, 2050-2050.	0.6	3
29	Insights Into the Mechanism of Zymogen Protein C Protection Against Cancer Progression. Blood, 2012, 120, 3350-3350.	0.6	1
30	In Vivo Genome Editing of Liver Albumin for Therapeutic Gene Expression: Rescue of Hemophilic Mice Via Integration of Factor 9. Blood, 2012, 120, 751-751.	0.6	1
31	Stable Factor IX Activity Following AAV-Mediated Gene Transfer in Patients with Severe Hemophilia B. Blood, 2012, 120, 752-752.	0.6	2
32	In Vivo Model to Evaluate Loss of Liver-Derived Factor IX Expression Caused by AAV Capsid-Specific CD8+ T Cells. Blood, 2012, 120, 2046-2046.	0.6	0
33	In vivo detection of hemorrhage rate in dog models of hemophilia and VWD and at human femoral arteriotomy by ARFI ultrasound. , 2011, , .		0
34	Adenovirus-Associated Virus Vector–Mediated Gene Transfer in Hemophilia B. New England Journal of Medicine, 2011, 365, 2357-2365.	13.9	1,606
35	Assessing the potential for AAV vector genotoxicity in a murine model. Blood, 2011, 117, 3311-3319.	0.6	196
36	ARFI ultrasound for in vivo monitoring of soft-tissue bleeding and hemostasis in a dog model of		0

hemophilia., 2010,,.

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37	Rabbit Anti-Thymocyte Globulin (rATG) Administrated Concomitantly with Liver Delivery of AAV2-hFIX Can Promote Inhibitor Formation In Rhesus Macaques Blood, 2010, 116, 3765-3765.	0.6	2
38	Peptide-Induced Antigen-Specific CD4+CD25+FoxP3+ T Cells Suppress Cytotoxicity T Cell Responses Directed Against the AAV Capsid Blood, 2010, 116, 3769-3769.	0.6	1
39	Intrinsically Hyperactive and Hyperproliferative CD8+ T Cells In Cmah-/- Mice as a Model of Human Gene Transfer Responses Blood, 2010, 116, 3773-3773.	0.6	2
40	Phenotypic Correction of a Mouse Model of Hemophilia B by In Vivo Genetic Correction of the F9 Gene. Blood, 2010, 116, LBA-5-LBA-5.	0.6	1
41	Zymogen Protein C as a Novel Modulator of Cancer Progression In Murine Models. Blood, 2010, 116, 718-718.	0.6	0
42	Host and Vector-dependent Effects on the Risk of Germline Transmission of AAV Vectors. Molecular Therapy, 2009, 17, 1022-1030.	3.7	33
43	Long-term correction of inhibitor-prone hemophilia B dogs treated with liver-directed AAV2-mediated factor IX gene therapy. Blood, 2009, 113, 797-806.	0.6	247
44	Suppression of CTL Responses against AAV-Capsid Epitopes by Peptide-Induced Regulatory T Cells Blood, 2009, 114, 377-377.	0.6	4
45	FIX-R338L (FIX Padua) as a Successful Alternative for the Treatment of Canine Severe Hemophilia B Blood, 2009, 114, 694-694.	0.6	2
46	Assessment of Insertional Mutagenesis Risk Following AAV Vector-Mediated Factor IX Gene Transfer in Mice Blood, 2009, 114, 2465-2465.	0.6	0
47	Efficacy and Safety of Continuous Expression of An Improved Variant of FVIIa On Murine Hemostasis with or without Inhibitors to Human Factor IX Blood, 2009, 114, 2466-2466.	0.6	0
48	Proteasome Inhibitors Decrease AAV2 Capsid-Derived Peptide Epitope Presentation On MHC Class I Following Transduction Blood, 2009, 114, 695-695.	0.6	0
49	Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2240-2248.	13.9	1,941
50	Nonsense Suppression Approaches in Treating Hemophilia. Blood, 2008, 112, 512-512.	0.6	2
51	Immunosuppression Modulates Immune Responses to AAV Capsid in Human Subjects Undergoing Intramuscular Gene Transfer for Lipoprotein Lipase Deficiency. Blood, 2008, 112, 822-822.	0.6	16
52	Successful and Safe Treatment of Canine Hemophilia by Continuous Expression of Canine FVIIa: a Model for FVIII/FIX Gene-Based Bypassing Agents. Blood, 2008, 112, Iba-4-Iba-4.	0.6	0
53	Engineered Factor IX with Augmented Clotting Activities in a Hemophilia B Mouse Model Blood, 2008, 112, 2025-2025.	0.6	0
54	CD8+ T-cell responses to adeno-associated virus capsid in humans. Nature Medicine, 2007, 13, 419-422.	15.2	629

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55	Safety of Recombinant Adeno-Associated Viral Vectors in a Large Animal Model Blood, 2007, 110, 2586-2586.	0.6	13
56	Quantifying Capsid Peptide:MHC I Complexes Following Adeno-Associated Virus (AAV) Transduction. Blood, 2007, 110, 3737-3737.	0.6	0
57	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. Nature Medicine, 2006, 12, 342-347.	15.2	1,865
58	The leak stops here: platelets as delivery vehicles for coagulation factors. Journal of Clinical Investigation, 2006, 116, 1840-1842.	3.9	9
59	A Novel Splenocyte Approach for Characterizing T Cell Responses to Adeno-Associated Virus in the Normal Population: Implications on Gene Transfer Blood, 2006, 108, 3258-3258.	0.6	Ο
60	Six-Year Follow-Up of Inhibitor Prone Hemophilia B Dogs Treated with Muscle and Liver-Directed AAV2 Mediated Factor IX Gene Therapy Blood, 2006, 108, 3282-3282.	0.6	0
61	A Murine FVIIa Variant with Increased Tissue Factor-Independent Intrinsic Activity Corrects the Murine Hemophilia B Phenotype Following AAV Administration: Implications for Therapeutic AAV Vector Doses Blood, 2006, 108, 3276-3276.	0.6	0
62	Anemia and Gene Therapy — A Matter of Control. New England Journal of Medicine, 2005, 352, 1146-1147.	13.9	0
63	Murine Model of Suppressed Intrinsic Pathway Using a FX Variant: FX Roma Blood, 2005, 106, 728-728.	0.6	0
64	Lessons from Transgenic Mice Expressing Supra-Physiological Levels of Activated Murine Factor VII Blood, 2005, 106, 1945-1945.	0.6	0
65	Characterization of the Immune Response to Canine Factor IX Following AAV-Mediated Intravascular Gene Delivery to Skeletal Muscle in Hemophilia B Dogs Blood, 2005, 106, 1297-1297.	0.6	0
66	AAV8-Factor IX Hepatic Gene Transfer with Transient Immunosuppression Is Safe but Is Abrogated by Low Titers of Neutralizing Antibody in Rhesus Macaques Blood, 2005, 106, 5532-5532.	0.6	0
67	Suppression of Inhibitor Formation to F.IX in Gene Transfer through Immune Deviation Induced by Mucosal Peptide Administration Blood, 2005, 106, 211-211.	0.6	1
68	A Novel Method of Regional Intravenous Delivery of AAV Vector to Skeletal Muscle Results in Correction of Canine Hemophilia B Phenotype Blood, 2004, 104, 3179-3179.	0.6	5
69	A Novel Role of Coagulation Proteases on Viral-Based Gene Transfer Efficacy Blood, 2004, 104, 691-691.	0.6	1
70	Expression of Blood Coagulation Factor X Is Not Liver-Restricted Blood, 2004, 104, 1939-1939.	0.6	0
71	In Vivo Evidence of Modulation of the Hemophilia Phenotype by the Factor V Leiden Blood, 2004, 104, 693-693.	0.6	0
72	Gene Transfer as an Approach to Treating Hemophilia. Seminars in Thrombosis and Hemostasis, 2003, 29, 107-120.	1.5	50

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73	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. Blood, 2003, 101, 2963-2972.	0.6	707
74	The risks of germline gene transfer. Hastings Center Report, 2003, 33, 3.	0.7	3
75	A frequent human coagulation Factor VII mutation (A294V, c152) in loop 140s affects the interaction with activators, tissue factor and substrates. Biochemical Journal, 2002, 363, 411-416.	1.7	36
76	In vivo gene transfer moves one step closer to success. Blood, 2002, 100, 1523-1524.	0.6	0
77	AAV-mediated gene transfer for hemophilia. Genetics in Medicine, 2002, 4, 56S-61S.	1.1	21
78	Gene Therapy for Hemophilia. , 2002, , 385-410.		0
79	Adeno-Associated Virus —Mediated Gene Transfer for Hemophilia B. International Journal of Hematology, 2002, 76, 310-318.	0.7	13
80	Geneâ€Based Approaches to the Treatment of Hemophilia. Annals of the New York Academy of Sciences, 2002, 961, 63-64.	1.8	10
81	AAV-Mediated Gene Transfer for Hemophilia. Annals of the New York Academy of Sciences, 2001, 953a, 64-74.	1.8	41
82	Gene Transfer as an Approach to Treating Hemophilia. Circulation Research, 2001, 88, 137-144.	2.0	52
83	Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector. Nature Genetics, 2000, 24, 257-261.	9.4	971
84	Human Factor IX Corrects the Bleeding Diathesis of Mice With Hemophilia B. Blood, 1998, 91, 784-790.	0.6	119
85	Genomic Sequence and Transcription Start Site for the Human Î ³ -Glutamyl Carboxylase. Blood, 1997, 89, 4058-4062.	0.6	51
86	Activation of a recombinant human factor VII structural analogue alters its affinity of binding to tissue factor. , 1996, 53, 66-71.		6