## Katherine A High

List of Publications by Year in descending order

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#	Article	IF	CITATIONS
1	Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2240-2248.	13.9	1,941
2	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
3	Adenovirus-Associated Virus Vector–Mediated Gene Transfer in Hemophilia B. New England Journal of Medicine, 2011, 365, 2357-2365.	13.9	1,606
4	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
5	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
6	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
7	Gene therapy comes of age. Science, 2018, 359, .	6.0	936
8	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. Blood, 2003, 101, 2963-2972.	0.6	707
9	CD8+ T-cell responses to adeno-associated virus capsid in humans. Nature Medicine, 2007, 13, 419-422.	15.2	629
10	Hemophilia B Gene Therapy with a High-Specific-Activity Factor IX Variant. New England Journal of Medicine, 2017, 377, 2215-2227.	13.9	549
11	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
12	Gene Therapy. New England Journal of Medicine, 2019, 381, 455-464.	13.9	343
13	Entering the Modern Era of Gene Therapy. Annual Review of Medicine, 2019, 70, 273-288.	5.0	311
14	In vivo genome editing of the albumin locus as a platform for protein replacement therapy. Blood, 2015, 126, 1777-1784.	0.6	256
15	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
16	Efficacy, Safety, and Durability of Voretigene Neparvovec-rzyl in RPE65 Mutation–Associated Inherited Retinal Dystrophy. Ophthalmology, 2019, 126, 1273-1285.	2.5	239
17	Assessing the potential for AAV vector genotoxicity in a murine model. Blood, 2011, 117, 3311-3319.	0.6	196
18	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

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19	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
20	Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A. New England Journal of Medicine, 2021, 385, 1961-1973.	13.9	127
21	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
22	Human Factor IX Corrects the Bleeding Diathesis of Mice With Hemophilia B. Blood, 1998, 91, 784-790.	0.6	119
23	Novel mobility test to assess functional vision in patients with inherited retinal dystrophies. Clinical and Experimental Ophthalmology, 2018, 46, 247-259.	1.3	97
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	AAV capsid CD8+ T-cell epitopes are highly conserved across AAV serotypes. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15029.	1.8	59
26	The gene therapy journey for hemophilia: are we there yet?. Blood, 2012, 120, 4482-4487.	0.6	57
27	Gene Transfer as an Approach to Treating Hemophilia. Circulation Research, 2001, 88, 137-144.	2.0	52
28	Genomic Sequence and Transcription Start Site for the Human Î <sup>3</sup> -Glutamyl Carboxylase. Blood, 1997, 89, 4058-4062.	0.6	51
29	Gene Transfer as an Approach to Treating Hemophilia. Seminars in Thrombosis and Hemostasis, 2003, 29, 107-120.	1.5	50
30	AAV-Mediated Gene Transfer for Hemophilia. Annals of the New York Academy of Sciences, 2001, 953a, 64-74.	1.8	41
31	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
32	Host and Vector-dependent Effects on the Risk of Germline Transmission of AAV Vectors. Molecular Therapy, 2009, 17, 1022-1030.	3.7	33
33	Successful Phenotype Improvement following Gene Therapy for Severe Hemophilia A in Privately Owned Dogs. PLoS ONE, 2016, 11, e0151800.	1.1	25
34	AAV-mediated gene transfer for hemophilia. Genetics in Medicine, 2002, 4, 56S-61S.	1.1	21
35	The gene therapy journey for hemophilia: are we there yet?. Hematology American Society of Hematology Education Program, 2012, 2012, 375-81.	0.9	20
36	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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37	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
38	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
39	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
40	Adeno-Associated Virus —Mediated Gene Transfer for Hemophilia B. International Journal of Hematology, 2002, 76, 310-318.	0.7	13
41	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
42	Safety of Recombinant Adeno-Associated Viral Vectors in a Large Animal Model Blood, 2007, 110, 2586-2586.	0.6	13
43	Hepatoâ€entrained B220 <sup>+</sup> <scp>CD</scp> 11c <sup>+</sup> <scp>NK</scp> 1.1 <sup>+</sup> cells regulate preâ€metastatic niche formation in theÂlung. EMBO Molecular Medicine, 2018, 10, .	3.3	11
44	Geneâ€Based Approaches to the Treatment of Hemophilia. Annals of the New York Academy of Sciences, 2002, 961, 63-64.	1.8	10
45	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
46	The leak stops here: platelets as delivery vehicles for coagulation factors. Journal of Clinical Investigation, 2006, 116, 1840-1842.	3.9	9
47	Activation of a recombinant human factor VII structural analogue alters its affinity of binding to tissue factor. , 1996, 53, 66-71.		6
48	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
49	Suppression of CTL Responses against AAV-Capsid Epitopes by Peptide-Induced Regulatory T Cells Blood, 2009, 114, 377-377.	0.6	4
50	A Novel Strategy to Circumvent Pre-Existing Humoral Immunity to AAV. Blood, 2012, 120, 2050-2050.	0.6	3
51	The risks of germline gene transfer. Hastings Center Report, 2003, 33, 3.	0.7	3
52	Nonsense Suppression Approaches in Treating Hemophilia. Blood, 2008, 112, 512-512.	0.6	2
53	FIX-R338L (FIX Padua) as a Successful Alternative for the Treatment of Canine Severe Hemophilia B Blood, 2009, 114, 694-694.	0.6	2
54	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

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55	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
56	Stable Factor IX Activity Following AAV-Mediated Gene Transfer in Patients with Severe Hemophilia B. Blood, 2012, 120, 752-752.	0.6	2
57	A Novel Role of Coagulation Proteases on Viral-Based Gene Transfer Efficacy Blood, 2004, 104, 691-691.	0.6	1
58	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
59	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
60	Insights Into the Mechanism of Zymogen Protein C Protection Against Cancer Progression. Blood, 2012, 120, 3350-3350.	0.6	1
61	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
62	In Vivo Genome Editing in Neonatal Mouse Liver Preferentially Utilizes Homology Directed Repair. Blood, 2015, 126, 4422-4422.	0.6	1
63	89â€Five-year update for the Phase III voretigene neparvovec study in biallelic RPE65 mutation–associated inherited retinal disease. , 2021, , .		1
64	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
65	In vivo gene transfer moves one step closer to success. Blood, 2002, 100, 1523-1524.	0.6	Ο
66	Gene Therapy for Hemophilia. , 2002, , 385-410.		0
67	Anemia and Gene Therapy — A Matter of Control. New England Journal of Medicine, 2005, 352, 1146-1147.	13.9	Ο
68	ARFI ultrasound for in vivo monitoring of soft-tissue bleeding and hemostasis in a dog model of hemophilia. , 2010, , .		0
69	In vivo detection of hemorrhage rate in dog models of hemophilia and VWD and at human femoral arteriotomy by ARFI ultrasound. , 2011, , .		Ο
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	Murine Model of Suppressed Intrinsic Pathway Using a FX Variant: FX Roma Blood, 2005, 106, 728-728.	0.6	0

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73	Lessons from Transgenic Mice Expressing Supra-Physiological Levels of Activated Murine Factor VII Blood, 2005, 106, 1945-1945.	0.6	0
74	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
75	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
76	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	0
77	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
78	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
79	Quantifying Capsid Peptide:MHC I Complexes Following Adeno-Associated Virus (AAV) Transduction. Blood, 2007, 110, 3737-3737.	0.6	0
80	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
81	Engineered Factor IX with Augmented Clotting Activities in a Hemophilia B Mouse Model Blood, 2008, 112, 2025-2025.	0.6	0
82	Assessment of Insertional Mutagenesis Risk Following AAV Vector-Mediated Factor IX Gene Transfer in Mice Blood, 2009, 114, 2465-2465.	0.6	0
83	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
84	Proteasome Inhibitors Decrease AAV2 Capsid-Derived Peptide Epitope Presentation On MHC Class I Following Transduction Blood, 2009, 114, 695-695.	0.6	0
85	Zymogen Protein C as a Novel Modulator of Cancer Progression In Murine Models. Blood, 2010, 116, 718-718.	0.6	0
86	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