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List of Publications by Year
in descending order

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31 papers	3,075 citations	471061 17 h-index	476904 29 g-index
31 all docs	31 docs citations	31 times ranked	3523 citing authors

#	Article	IF	Citations
1	Hemophilia B Gene Therapy with a High-Specific-Activity Factor IX Variant. New England Journal of Medicine, 2017, 377, 2215-2227.	13.9	549
2	In vivo genome editing restores haemostasis in a mouse model of haemophilia. Nature, 2011, 475, 217-221.	13.7	523
3	Entering the Modern Era of Gene Therapy. Annual Review of Medicine, 2019, 70, 273-288.	5.0	311
4	Overcoming Preexisting Humoral Immunity to AAV Using Capsid Decoys. Science Translational Medicine, 2013, 5, 194ra92.	5.8	267
5	In vivo genome editing of the albumin locus as a platform for protein replacement therapy. Blood, 2015, 126, 1777-1784.	0.6	256
6	High AAV vector purity results in serotype- and tissue-independent enhancement of transduction efficiency. Gene Therapy, 2010, 17, 503-510.	2.3	240
7	IgG-cleaving endopeptidase enables in vivo gene therapy in the presence of anti-AAV neutralizing antibodies. Nature Medicine, 2020, 26, 1096-1101.	15.2	193
8	Robust ZFN-mediated genome editing in adult hemophilic mice. Blood, 2013, 122, 3283-3287.	0.6	159
9	Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A. New England Journal of Medicine, 2021, 385, 1961-1973.	13.9	127
10	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
11	Adeno-associated viral vectors for the treatment of hemophilia. Human Molecular Genetics, 2016, 25, R36-R41.	1.4	56
12	Reversal of Type 1 Diabetes by Engineering a Glucose Sensor in Skeletal Muscle. Diabetes, 2006, 55, 1546-1553.	0.3	54
13	Liver Production of Sulfamidase Reverses Peripheral and Ameliorates CNS Pathology in Mucopolysaccharidosis IIIA Mice. Molecular Therapy, 2012, 20, 254-266.	3.7	51
14	Molecular signature of the immune and tissue response to non-coding plasmid DNA in skeletal muscle after electrotransfer. Gene Therapy, 2012, 19, 1177-1186.	2.3	27
15	Preclinical Evaluation of An Anti-HCV miRNA Cluster for Treatment of HCV Infection. Molecular Therapy, 2013, 21, 588-601.	3.7	25
16	Nonviral-Mediated Hepatic Expression of IGF-I Increases Treg Levels and Suppresses Autoimmune Diabetes in Mice. Diabetes, 2013, 62, 551-560.	0.3	25
17	Spk-9001: Adeno-Associated Virus Mediated Gene Transfer for Hemophilia B Achieves Sustained Mean Factor IX Activity Levels of >30% without Immunosuppression. Blood, 2016, 128, 3-3.	0.6	24
18	Experimental Variables that Affect Human Hepatocyte AAV Transduction in Liver Chimeric Mice. Molecular Therapy - Methods and Clinical Development, 2020, 18, 189-198.	1.8	19

#	Article	IF	Citations
19	Hepatic expression of GAA results in enhanced enzyme bioavailability in mice and non-human primates. Nature Communications, 2021, 12, 6393.	5.8	14
20	Skeletal Muscle Metabolism in the Pathology and Treatment of Type 1 Diabetes. Current Pharmaceutical Design, 2010, 16, 1002-1020.	0.9	11
21	Preclinical assessment of an optimized AAV-FVIII vector in mice and non-human primates for the treatment of hemophilia A. Molecular Therapy - Methods and Clinical Development, 2022, 24, 20-29.	1.8	7
22	An edible switch for gene therapy. Nature Biotechnology, 2016, 34, 824-825.	9.4	4
23	A Novel Strategy to Circumvent Pre-Existing Humoral Immunity to AAV. Blood, 2012, 120, 2050-2050.	0.6	3
24	Oracle or false prophet? Can we predict AAV efficacy based on preexisting antibody titers?. Research and Practice in Thrombosis and Haemostasis, 2019, 3, 149-151.	1.0	2
25	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
26	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
27	ZFN Mediated Targeting Of Albumin â€œSafe Harborâ€•Results In Therapeutic Levels Of Human Factor VIII In a Mouse Model Of Hemophilia A. Blood, 2013, 122, 720-720.	0.6	1
28	Adeno-Associated Viral Vector Delivery of Optimized Human Factor VIII Achieves Therapeutic Factor VIII Levels in Non-Human Primates. Blood, 2015, 126, 199-199.	0.6	1
29	In Vivo Genome Editing in Neonatal Mouse Liver Preferentially Utilizes Homology Directed Repair. Blood, 2015, 126, 4422-4422.	0.6	1
30	Aproximaciones de terapia gÃ©nica para la diabetes tipo 1. Avances En DiabetologÃa, 2010, 26, 6-12.	0.1	0
31	Robust Factor IX Expression Following ZFN-Mediated Genome Editing in An Adult Mouse Model of Hemophilia B. Blood, 2011, 118, 668-668.	0.6	0