Federico Mingozzi

List of Publications by Year
in descending order

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72 papers	14,047 citations	87888 38 h-index	114465 63 g-index
73 all docs	73 docs citations	73 times ranked	8987 citing authors

#	Article	IF	Citations
1	Development of a dual hybrid AAV vector for endothelial-targeted expression of von Willebrand factor. Gene Therapy, 2023, 30, 245-254.	4.5	11
2	Early Phase Clinical Immunogenicity of Valoctocogene Roxaparvovec, an AAV5-Mediated Gene Therapy for Hemophilia A. Molecular Therapy, 2021, 29, 597-610.	8.2	42
3	251st ENMC international workshop: Polyglucosan storage myopathies 13â€“15 December 2019, Hoofddorp, the Netherlands. Neuromuscular Disorders, 2021, 31, 466-477.	0.6	4
4	Engineering adeno-associated viral vectors to evade innate immune and inflammatory responses. Science Translational Medicine, 2021, 13, .	12.4	99
5	Emerging Immunogenicity and Genotoxicity Considerations of Adeno-Associated Virus Vector Gene Therapy for Hemophilia. Journal of Clinical Medicine, 2021, 10, 2471.	2.4	47
6	A novel therapeutic strategy for skeletal disorders: Proof of concept of gene therapy for X-linked hypophosphatemia. Science Advances, 2021, 7, eabj5018.	10.3	2
7	Hepatic expression of GAA results in enhanced enzyme bioavailability in mice and non-human primates. Nature Communications, 2021, 12, 6393.	12.8	14
8	Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A. New England Journal of Medicine, 2021, 385, 1961-1973.	27.0	127
9	AAV Vector Immunogenicity in Humans: A Long Journey to Successful Gene Transfer. Molecular Therapy, 2020, 28, 723-746.	8.2	363
10	Gene therapy with secreted acid alpha-glucosidase rescues Pompe disease in a novel mouse model with early-onset spinal cord and respiratory defects. EBioMedicine, 2020, 61, 103052.	6.1	14
11	Singleâ€domain antibodies targeting antithrombin reduce bleeding in hemophilic mice with or without inhibitors. EMBO Molecular Medicine, 2020, 12, e11298.	6.9	20
12	IgG-cleaving endopeptidase enables in vivo gene therapy in the presence of anti-AAV neutralizing antibodies. Nature Medicine, 2020, 26, 1096-1101.	30.7	193
13	Rescue of Advanced Pompe Disease in Mice with Hepatic Expression of Secretable Acid Î±-Glucosidase. Molecular Therapy, 2020, 28, 2056-2072.	8.2	16
14	Human Immune Responses to Adeno-Associated Virus (AAV) Vectors. Frontiers in Immunology, 2020, 11, 670.	4.8	198
15	Gene Therapy for Pompe Disease: The Time is now. Human Gene Therapy, 2019, 30, 1245-1262.	2.7	20
16	Gene Therapy in Pediatric Liver Disease. , 2019, , 799-829.		2
17	AAV Gene Transfer with Tandem Promoter Design Prevents Anti-transgene Immunity and Provides Persistent Efficacy in Neonate Pompe Mice. Molecular Therapy - Methods and Clinical Development, 2019, 12, 85-101.	4.1	52
18	Progress and challenges of gene therapy for Pompe disease. Annals of Translational Medicine, 2019, 7, 287-287.	1.7	35

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19	Rescue of GSDIII Phenotype with Gene Transfer Requires Liver- and Muscle-Targeted GDE Expression. Molecular Therapy, 2018, 26, 890-901.	8.2	24
20	Bioengineered AAV Capsids with Combined High Human Liver Transduction InÂVivo and Unique Humoral Seroreactivity. Molecular Therapy, 2018, 26, 289-303.	8.2	130
21	Antigen-selective modulation of AAV immunogenicity with tolerogenic rapamycin nanoparticles enables successful vector re-administration. Nature Communications, 2018, 9, 4098.	12.8	184
22	Exposure to wild-type AAV drives distinct capsid immunity profiles in humans. Journal of Clinical Investigation, 2018, 128, 5267-5279.	8.2	76
23	Systemic AAV8-Mediated Gene Therapy Drives Whole-Body Correction of Myotubular Myopathy in Dogs. Molecular Therapy, 2017, 25, 839-854.	8.2	81
24	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.	6.7	147
25	Unraveling the Complex Story of Immune Responses to AAV Vectors Trial After Trial. Human Gene Therapy, 2017, 28, 1061-1074.	2.7	170
26	Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid Î±-glucosidase. Science Translational Medicine, 2017, 9, .	12.4	103
27	Enhanced liver gene transfer and evasion of preexisting humoral immunity with exosome-enveloped AAV vectors. Blood Advances, 2017, 1, 2019-2031.	5.2	90
28	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.	13.7	377
29	Long-term exposure to Myozyme results in a decrease of anti-drug antibodies in late-onset Pompe disease patients. Scientific Reports, 2016, 6, 36182.	3.3	22
30	77. Antigen-Specific Modulation of Capsid Immunogenicity with Tolerogenic Nanoparticles Results in Successful AAV Vector Readministration. Molecular Therapy, 2016, 24, S34.	8.2	8
31	A translationally optimized AAV-UGT1A1 vector drives safe and long-lasting correction of Crigler-Najjar syndrome. Molecular Therapy - Methods and Clinical Development, 2016, 3, 16049.	4.1	50
32	AAV capsid CD8+ T-cell epitopes are highly conserved across AAV serotypes. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15029.	4.1	59
33	AAV liver expression of FIX-Padua prevents and eradicates FIX inhibitor without increasing thrombogenicity in hemophilia B dogs and mice. Blood, 2015, 125, 1553-1561.	1.4	143
34	Determination of Anti-Adeno-Associated Virus Vector Neutralizing Antibody Titer with an In Vitro Reporter System. Human Gene Therapy Methods, 2015, 26, 45-53.	2.1	82
35	Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B. New England Journal of Medicine, 2014, 371, 1994-2004.	27.0	1,063
36	Overcoming Preexisting Humoral Immunity to AAV Using Capsid Decoys. Science Translational Medicine, 2013, 5, 194ra92.	12.4	267

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37	Engineered AAV vector minimizes in vivo targeting of transduced hepatocytes by capsid-specific CD8+ T cells. Blood, 2013, 121, 2224-2233.	1.4	149
38	Immune responses to AAV vectors: overcoming barriers to successful gene therapy. Blood, 2013, 122, 23-36.	1.4	703
39	IL12-Mediated Liver Inflammation Reduces the Formation of AAV Transcriptionally Active Forms but Has No Effect over Preexisting AAV Transgene Expression. PLoS ONE, 2013, 8, e67748.	2.5	18
40	Tolerance Induction To FIX Padua With AAV Liver Gene Transfer In Inhibitor-Prone Hemophilia B Dogs. Blood, 2013, 122, 4203-4203.	1.4	0
41	Pharmacological Modulation of Humoral Immunity in a Nonhuman Primate Model of AAV Gene Transfer for Hemophilia B. Molecular Therapy, 2012, 20, 1410-1416.	8.2	90
42	A Novel Strategy to Circumvent Pre-Existing Humoral Immunity to AAV. Blood, 2012, 120, 2050-2050.	1.4	3
43	Adenovirus-Associated Virus Vectorâ€“Mediated Gene Transfer in Hemophilia B. New England Journal of Medicine, 2011, 365, 2357-2365.	27.0	1,606
44	Immune Responses to AAV in Clinical Trials. Current Gene Therapy, 2011, 11, 321-330.	2.0	253
45	Therapeutic in vivo gene transfer for genetic disease using AAV: progress and challenges. Nature Reviews Genetics, 2011, 12, 341-355.	16.3	797
46	Adeno-Associated Viral Vector Mediated Gene Transfer for Hemophilia B. Blood, 2011, 118, 5-5.	1.4	4
47	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.	1.4	2
48	Peptide-Induced Antigen-Specific CD4+CD25+FoxP3+ T Cells Suppress Cytotoxicity T Cell Responses Directed Against the AAV Capsid.. Blood, 2010, 116, 3769-3769.	1.4	1
49	Intrinsically Hyperactive and Hyperproliferative CD8+ T Cells In Cmah-/- Mice as a Model of Human Gene Transfer Responses.. Blood, 2010, 116, 3773-3773.	1.4	2
50	AAV-1â€“mediated gene transfer to skeletal muscle in humans results in dose-dependent activation of capsid-specific T cells. Blood, 2009, 114, 2077-2086.	1.4	248
51	Suppression of CTL Responses against AAV-Capsid Epitopes by Peptide-Induced Regulatory T Cells.. Blood, 2009, 114, 377-377.	1.4	4
52	Assessment of Insertional Mutagenesis Risk Following AAV Vector-Mediated Factor IX Gene Transfer in Mice.. Blood, 2009, 114, 2465-2465.	1.4	0
53	Proteasome Inhibitors Decrease AAV2 Capsid-Derived Peptide Epitope Presentation On MHC Class I Following Transduction.. Blood, 2009, 114, 695-695.	1.4	0
54	Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2240-2248.	27.0	1,941

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55	Immunosuppression Modulates Immune Responses to AAV Capsid in Human Subjects Undergoing Intramuscular Gene Transfer for Lipoprotein Lipase Deficiency. Blood, 2008, 112, 822-822.	1.4	16
56	Immune Responses to AAV in Clinical Trials. Current Gene Therapy, 2007, 7, 316-324.	2.0	176
57	Modulation of tolerance to the transgene product in a nonhuman primate model of AAV-mediated gene transfer to liver. Blood, 2007, 110, 2334-2341.	1.4	218
58	CD8+ T-cell responses to adeno-associated virus capsid in humans. Nature Medicine, 2007, 13, 419-422.	30.7	629
59	Safety of Recombinant Adeno-Associated Viral Vectors in a Large Animal Model.. Blood, 2007, 110, 2586-2586.	1.4	13
60	Quantifying Capsid Peptide:MHC I Complexes Following Adeno-Associated Virus (AAV) Transduction. Blood, 2007, 110, 3737-3737.	1.4	0
61	Effects of transient immunosuppression on adenoassociated, virus-mediated, liver-directed gene transfer in rhesus macaques and implications for human gene therapy. Blood, 2006, 108, 3321-3328.	1.4	295
62	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. Nature Medicine, 2006, 12, 342-347.	30.7	1,865
63	AAV-2 Capsid-Specific CD8+ T Cells Limit the Duration of Gene Therapy in Humans and Cross-React with AAV-8 Capsid.. Blood, 2006, 108, 455-455.	1.4	2
64	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.	1.4	0
65	Proteaseâ^'Activated Receptor 2 (PARâ^'2) as a Novel Target To Prevent Inhibitor Formation to FIX.. Blood, 2006, 108, 763-763.	1.4	1
66	Protease-Activated Receptor-2 (PAR-2) as a Novel Target for Modulating Immune Responses to Neo Antigens Following In Vivo Gene Transfer.. Blood, 2005, 106, 1296-1296.	1.4	1
67	T Cell Responses to AAV Vector Capsid Limit the Duration of Transgene Expression in Humans after Liver-Directed Gene Therapy.. Blood, 2005, 106, 3055-3055.	1.4	0
68	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.	1.4	0
69	Identification of the AAV2 Capsid CD8+ T Cell Epitope in C57BL/6 Mice.. Blood, 2004, 104, 3188-3188.	1.4	1
70	Induction of immune tolerance to coagulation factor IX antigen by in vivo hepatic gene transfer. Journal of Clinical Investigation, 2003, 111, 1347-1356.	8.2	242
71	Induction of immune tolerance to coagulation factor IX antigen by in vivo hepatic gene transfer. Journal of Clinical Investigation, 2003, 111, 1347-1356.	8.2	363
72	Improved Hepatic Gene Transfer by Using an Adeno-Associated Virus Serotype 5 Vector. Journal of Virology, 2002, 76, 10497-10502.	3.4	69