# Federico Mingozzi

List of Publications by Year
in descending order

Source: https://exaly.com/author-pdf/5557417/publications.pdf

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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 | Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2240-2248. | 27.0 | 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. | 30.7 | 1,865 |
| 3 | Adenovirus-Associated Virus Vectorâ€“Mediated Gene Transfer in Hemophilia B. New England Journal of Medicine, 2011, 365, 2357-2365. | 27.0 | 1,606 |
| 4 | 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 |
| 5 | Therapeutic in vivo gene transfer for genetic disease using AAV: progress and challenges. Nature Reviews Genetics, 2011, 12, 341-355. | 16.3 | 797 |
| 6 | Immune responses to AAV vectors: overcoming barriers to successful gene therapy. Blood, 2013, 122, 23-36. | 1.4 | 703 |
| 7 | CD8+ T-cell responses to adeno-associated virus capsid in humans. Nature Medicine, 2007, 13, 419-422. | 30.7 | 629 |
| 8 | 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 |
| 9 | AAV Vector Immunogenicity in Humans: A Long Journey to Successful Gene Transfer. Molecular Therapy, 2020, 28, 723-746. | 8.2 | 363 |
| 10 | 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 |
| 11 | 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 |
| 12 | Overcoming Preexisting Humoral Immunity to AAV Using Capsid Decoys. Science Translational Medicine, 2013, 5, 194ra92. | 12.4 | 267 |
| 13 | Immune Responses to AAV in Clinical Trials. Current Gene Therapy, 2011, 11, 321-330. | 2.0 | 253 |
| 14 | 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 |
| 15 | 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 |
| 16 | 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 |
| 17 | Human Immune Responses to Adeno-Associated Virus (AAV) Vectors. Frontiers in Immunology, 2020, 11, 670. | 4.8 | 198 |
| 18 | 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 |

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|---|---|---|---|
| 19 | Antigen-selective modulation of AAV immunogenicity with tolerogenic rapamycin nanoparticles enables successful vector re-administration. Nature Communications, 2018, 9, 4098. | 12.8 | 184 |
| 20 | Immune Responses to AAV in Clinical Trials. Current Gene Therapy, 2007, 7, 316-324. | 2.0 | 176 |
| 21 | Unraveling the Complex Story of Immune Responses to AAV Vectors Trial After Trial. Human Gene Therapy, 2017, 28, 1061-1074. | 2.7 | 170 |
| 22 | Engineered AAV vector minimizes in vivo targeting of transduced hepatocytes by capsid-specific CD8+ T cells. Blood, 2013, 121, 2224-2233. | 1.4 | 149 |
| 23 | 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 |
| 24 | 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 |
| 25 | Bioengineered AAV Capsids with Combined High Human Liver Transduction InÂVivo and Unique Humoral Seroreactivity. Molecular Therapy, 2018, 26, 289-303. | 8.2 | 130 |
| 26 | Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A. New England Journal of Medicine, 2021, 385, 1961-1973. | 27.0 | 127 |
| 27 | Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid Î±-glucosidase. Science Translational Medicine, 2017, 9, . | 12.4 | 103 |
| 28 | Engineering adeno-associated viral vectors to evade innate immune and inflammatory responses. Science Translational Medicine, 2021, 13, . | 12.4 | 99 |
| 29 | 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 |
| 30 | Enhanced liver gene transfer and evasion of preexisting humoral immunity with exosome-enveloped AAV vectors. Blood Advances, 2017, 1, 2019-2031. | 5.2 | 90 |
| 31 | 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 |
| 32 | Systemic AAV8-Mediated Gene Therapy Drives Whole-Body Correction of Myotubular Myopathy in Dogs. Molecular Therapy, 2017, 25, 839-854. | 8.2 | 81 |
| 33 | Exposure to wild-type AAV drives distinct capsid immunity profiles in humans. Journal of Clinical Investigation, 2018, 128, 5267-5279. | 8.2 | 76 |
| 34 | Improved Hepatic Gene Transfer by Using an Adeno-Associated Virus Serotype 5 Vector. Journal of Virology, 2002, 76, 10497-10502. | 3.4 | 69 |
| 35 | AAV capsid CD8+ T-cell epitopes are highly conserved across AAV serotypes. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15029. | 4.1 | 59 |
| 36 | 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 |

| # | Article | IF | Citations |
|---|---|---|---|
| 37 | 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 |
| 38 | Emerging Immunogenicity and Genotoxicity Considerations of Adeno-Associated Virus Vector Gene Therapy for Hemophilia. Journal of Clinical Medicine, 2021, 10, 2471. | 2.4 | 47 |
| 39 | Early Phase Clinical Immunogenicity of Valoctocogene Roxaparvovec, an AAV5-Mediated Gene Therapy for Hemophilia A. Molecular Therapy, 2021, 29, 597-610. | 8.2 | 42 |
| 40 | Progress and challenges of gene therapy for Pompe disease. Annals of Translational Medicine, 2019, 7, 287-287. | 1.7 | 35 |
| 41 | Rescue of GSDIII Phenotype with Gene Transfer Requires Liver- and Muscle-Targeted GDE Expression. Molecular Therapy, 2018, 26, 890-901. | 8.2 | 24 |
| 42 | 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 |
| 43 | Gene Therapy for Pompe Disease: The Time is now. Human Gene Therapy, 2019, 30, 1245-1262. | 2.7 | 20 |
| 44 | Singleâ€domain antibodies targeting antithrombin reduce bleeding in hemophilic mice with or without inhibitors. EMBO Molecular Medicine, 2020, 12, e11298. | 6.9 | 20 |
| 45 | 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 |
| 46 | Rescue of Advanced Pompe Disease in Mice with Hepatic Expression of Secretable Acid Î±-Glucosidase. Molecular Therapy, 2020, 28, 2056-2072. | 8.2 | 16 |
| 47 | 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 |
| 48 | 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 |
| 49 | Hepatic expression of GAA results in enhanced enzyme bioavailability in mice and non-human primates. Nature Communications, 2021, 12, 6393. | 12.8 | 14 |
| 50 | Safety of Recombinant Adeno-Associated Viral Vectors in a Large Animal Model.. Blood, 2007, 110, 2586-2586. | 1.4 | 13 |
| 51 | Development of a dual hybrid AAV vector for endothelial-targeted expression of von Willebrand factor. Gene Therapy, 2023, 30, 245-254. | 4.5 | 11 |
| 52 | 77. Antigen-Specific Modulation of Capsid Immunogenicity with Tolerogenic Nanoparticles Results in Successful AAV Vector Readministration. Molecular Therapy, 2016, 24, S34. | 8.2 | 8 |
| 53 | 251st ENMC international workshop: Polyglucosan storage myopathies 13â€“15 December 2019, Hoofddorp, the Netherlands. Neuromuscular Disorders, 2021, 31, 466-477. | 0.6 | 4 |
| 54 | Suppression of CTL Responses against AAV-Capsid Epitopes by Peptide-Induced Regulatory T Cells.. Blood, 2009, 114, 377-377. | 1.4 | 4 |

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|---|---|---|---|
| 55 | Adeno-Associated Viral Vector Mediated Gene Transfer for Hemophilia B. Blood, 2011, 118, 5-5. | 1.4 | 4 |
| 56 | A Novel Strategy to Circumvent Pre-Existing Humoral Immunity to AAV. Blood, 2012, 120, 2050-2050. | 1.4 | 3 |
| 57 | Gene Therapy in Pediatric Liver Disease. , 2019, , 799-829. | | 2 |
| 58 | 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 |
| 59 | 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 |
| 60 | 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 |
| 61 | 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 |
| 62 | 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 |
| 63 | Identification of the AAV2 Capsid CD8+ T Cell Epitope in C57BL/6 Mice.. Blood, 2004, 104, 3188-3188. | 1.4 | 1 |
| 64 | 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 |
| 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 | 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 |
| 67 | 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 |
| 68 | 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 |
| 69 | Quantifying Capsid Peptide:MHC I Complexes Following Adeno-Associated Virus (AAV) Transduction. Blood, 2007, 110, 3737-3737. | 1.4 | 0 |
| 70 | Assessment of Insertional Mutagenesis Risk Following AAV Vector-Mediated Factor IX Gene Transfer in Mice.. Blood, 2009, 114, 2465-2465. | 1.4 | 0 |
| 71 | Proteasome Inhibitors Decrease AAV2 Capsid-Derived Peptide Epitope Presentation On MHC Class I Following Transduction.. Blood, 2009, 114, 695-695. | 1.4 | 0 |
| 72 | Tolerance Induction To FIX Padua With AAV Liver Gene Transfer In Inhibitor-Prone Hemophilia B Dogs. Blood, 2013, 122, 4203-4203. | 1.4 | 0 |