

# William W Hauswirth

## List of Publications by Year in descending order

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283  
papers

24,297  
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7568

77  
h-index

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139  
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287  
all docs

287  
docs citations

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times ranked

14057  
citing authors

| #  | ARTICLE  | IF   | CITATIONS |
|----|--|------|-----------|
| 1  | Gene therapy restores vision in a canine model of childhood blindness. <i>Nature Genetics</i> , 2001, 28, 92-95.   | 21.4 | 1,130     |
| 2  | Treatment of Leber Congenital Amaurosis Due to RPE65 Mutations by Ocular Subretinal Injection of Adeno-Associated Virus Gene Vector: Short-Term Results of a Phase I Trial. <i>Human Gene Therapy</i> , 2008, 19, 979-990.                       | 2.7  | 880       |
| 3  | Human gene therapy for RPE65 isomerase deficiency activates the retinoid cycle of vision but with slow rod kinetics. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2008, 105, 15112-15117.            | 7.1  | 639       |
| 4  | Gene Therapy for Leber Congenital Amaurosis Caused by RPE65 Mutations. <i>JAMA Ophthalmology</i> , 2012, 130, 9.   | 2.4  | 580       |
| 5  | DICER1 deficit induces Alu RNA toxicity in age-related macular degeneration. <i>Nature</i> , 2011, 471, 325-330.   | 27.8 | 573       |
| 6  | DICER1 Loss and Alu RNA Induce Age-Related Macular Degeneration via the NLRP3 Inflammasome and MyD88. <i>Cell</i> , 2012, 149, 847-859.  | 28.9 | 526       |
| 7  | Production and purification of serotype 1, 2, and 5 recombinant adeno-associated viral vectors. <i>Methods</i> , 2002, 28, 158-167.  | 3.8  | 514       |
| 8  | Long-Term Restoration of Rod and Cone Vision by Single Dose rAAV-Mediated Gene Transfer to the Retina in a Canine Model of Childhood Blindness. <i>Molecular Therapy</i> , 2005, 12, 1072-1082.  | 8.2  | 421       |
| 9  | Human retinal gene therapy for Leber congenital amaurosis shows advancing retinal degeneration despite enduring visual improvement. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2013, 110, E517-25. | 7.1  | 401       |
| 10 | Ribozyme rescue of photoreceptor cells in a transgenic rat model of autosomal dominant retinitis pigmentosa. <i>Nature Medicine</i> , 1998, 4, 967-971.  | 30.7 | 396       |
| 11 | High-efficiency Transduction of the Mouse Retina by Tyrosine-mutant AAV Serotype Vectors. <i>Molecular Therapy</i> , 2009, 17, 463-471.  | 8.2  | 355       |
| 12 | Gene Therapy with Brain-Derived Neurotrophic Factor As a Protection: Retinal Ganglion Cells in a Rat Glaucoma Model. , 2003, 44, 4357.   |      | 336       |
| 13 | Improvement and Decline in Vision with Gene Therapy in Childhood Blindness. <i>New England Journal of Medicine</i> , 2015, 372, 1920-1926.   | 27.0 | 333       |
| 14 | Human RPE65 Gene Therapy for Leber Congenital Amaurosis: Persistence of Early Visual Improvements and Safety at 1 Year. <i>Human Gene Therapy</i> , 2009, 20, 999-1004.  | 2.7  | 305       |
| 15 | Gene therapy for red-green colour blindness in adult primates. <i>Nature</i> , 2009, 461, 784-787.   | 27.8 | 282       |
| 16 | Effects of Adeno-associated Virus-vectored Ciliary Neurotrophic Factor on Retinal Structure and Function in Mice with a P216L rds/peripherin Mutation. <i>Experimental Eye Research</i> , 2002, 74, 719-735.                                     | 2.6  | 267       |
| 17 | Restoration of visual function in P23H rhodopsin transgenic rats by gene delivery of BiP/Grp78. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2010, 107, 5961-5966.                                   | 7.1  | 265       |
| 18 | Virally delivered Channelrhodopsin-2 Safely and Effectively Restores Visual Function in Multiple Mouse Models of Blindness. <i>Molecular Therapy</i> , 2011, 19, 1220-1229.  | 8.2  | 261       |

| #  | ARTICLE   | IF   | CITATIONS |
|----|---|------|-----------|
| 19 | Rescue of a mitochondrial deficiency causing Leber hereditary optic neuropathy. <i>Annals of Neurology</i> , 2002, 52, 534-542.   | 5.3  | 253       |
| 20 | TrkB Gene Transfer Protects Retinal Ganglion Cells from Axotomy-Induced Death <i>In Vivo</i> . <i>Journal of Neuroscience</i> , 2002, 22, 3977-3986.  | 3.6  | 245       |
| 21 | A Comprehensive Review of Retinal Gene Therapy. <i>Molecular Therapy</i> , 2013, 21, 509-519.   | 8.2  | 245       |
| 22 | Functional genomic screening identifies dual leucine zipper kinase as a key mediator of retinal ganglion cell death. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2013, 110, 4045-4050.     | 7.1  | 239       |
| 23 | Gene therapy rescues photoreceptor blindness in dogs and paves the way for treating human X-linked retinitis pigmentosa. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2012, 109, 2132-2137. | 7.1  | 237       |
| 24 | Gene therapy rescues cone function in congenital achromatopsia. <i>Human Molecular Genetics</i> , 2010, 19, 2581-2593.  | 2.9  | 235       |
| 25 | Novel Properties of Tyrosine-mutant AAV2 Vectors in the Mouse Retina. <i>Molecular Therapy</i> , 2011, 19, 293-301.   | 8.2  | 234       |
| 26 | Nucleotide sequence evidence for rapid genotypic shifts in the bovine mitochondrial DNA D-loop. <i>Nature</i> , 1983, 306, 400-402.   | 27.8 | 231       |
| 27 | Dicer Inactivation Leads to Progressive Functional and Structural Degeneration of the Mouse Retina. <i>Journal of Neuroscience</i> , 2008, 28, 4878-4887.   | 3.6  | 204       |
| 28 | SOD2 Knockdown Mouse Model of Early AMD. , 2007, 48, 4407.  |      | 201       |
| 29 | Restoration of cone vision in a mouse model of achromatopsia. <i>Nature Medicine</i> , 2007, 13, 685-687.   | 30.7 | 200       |
| 30 | Vision 1 Year after Gene Therapy for Leber's Congenital Amaurosis. <i>New England Journal of Medicine</i> , 2009, 361, 725-727.   | 27.0 | 197       |
| 31 | Safety of Recombinant Adeno-Associated Virus Type 2 RPE65 Vector Delivered by Ocular Subretinal Injection. <i>Molecular Therapy</i> , 2006, 13, 1074-1084.  | 8.2  | 196       |
| 32 | Effect of CNTF on Retinal Ganglion Cell Survival in Experimental Glaucoma. , 2009, 50, 2194.  |      | 195       |
| 33 | Treatment of retinitis pigmentosa due to MERTK mutations by ocular subretinal injection of adeno-associated virus gene vector: results of a phase I trial. <i>Human Genetics</i> , 2016, 135, 327-343.                                  | 3.8  | 195       |
| 34 | Gene Therapy Restores Vision-Dependent Behavior as Well as Retinal Structure and Function in a Mouse Model of RPE65 Leber Congenital Amaurosis. <i>Molecular Therapy</i> , 2006, 13, 565-572.   | 8.2  | 185       |
| 35 | Results at 2 Years after Gene Therapy for RPE65-Deficient Leber Congenital Amaurosis and Severe Early-Childhood Onset Retinal Dystrophy. <i>Ophthalmology</i> , 2016, 123, 1606-1620.   | 5.2  | 184       |
| 36 | Origin and termination of adeno-associated virus DNA replication. <i>Virology</i> , 1977, 78, 488-499.  | 2.4  | 183       |

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|----|--|------|-----------|
| 37 | Glial Cell Line Derived Neurotrophic Factor Delays Photoreceptor Degeneration in a Transgenic Rat Model of Retinitis Pigmentosa. <i>Molecular Therapy</i> , 2001, 4, 622-629.  | 8.2  | 173       |
| 38 | Gene Therapy for Leber Hereditary Optic Neuropathy. <i>Ophthalmology</i> , 2017, 124, 1621-1634.   | 5.2  | 172       |
| 39 | AAV-mediated gene transfer of pigment epithelium-derived factor inhibits choroidal neovascularization. <i>Investigative Ophthalmology and Visual Science</i> , 2002, 43, 1994-2000.  | 3.3  | 168       |
| 40 | Prolonged Recovery of Retinal Structure/Function after Gene Therapy in an Rs1h-Deficient Mouse Model of X-Linked Juvenile Retinoschisis. <i>Molecular Therapy</i> , 2005, 12, 644-651.   | 8.2  | 166       |
| 41 | Dual AAV-mediated gene therapy restores hearing in a DFNB9 mouse model. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2019, 116, 4496-4501.   | 7.1  | 162       |
| 42 | Retinal degeneration 12 (rd12): a new, spontaneously arising mouse model for human Leber congenital amaurosis (LCA). <i>Molecular Vision</i> , 2005, 11, 152-62.   | 1.1  | 159       |
| 43 | Gene delivery to mitochondria by targeting modified adenoassociated virus suppresses Leber's hereditary optic neuropathy in a mouse model. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2012, 109, E1238-47. | 7.1  | 153       |
| 44 | [48] Production and purification of recombinant adeno-associated virus. <i>Methods in Enzymology</i> , 2000, 316, 743-761.   | 1.0  | 152       |
| 45 | Baculoviral IAP Repeat-Containing-4 Protects Optic Nerve Axons in a Rat Glaucoma Model. <i>Molecular Therapy</i> , 2002, 5, 780-787.   | 8.2  | 151       |
| 46 | Anatomical, cellular and molecular analysis of 8,000-yr-old human brain tissue from the Windover archaeological site. <i>Nature</i> , 1986, 323, 803-806.  | 27.8 | 147       |
| 47 | ACE2 and Ang-(1-7) Confer Protection Against Development of Diabetic Retinopathy. <i>Molecular Therapy</i> , 2012, 20, 28-36.  | 8.2  | 143       |
| 48 | Targeting Photoreceptors via Intravitreal Delivery Using Novel, Capsid-Mutated AAV Vectors. <i>PLoS ONE</i> , 2013, 8, e62097.   | 2.5  | 143       |
| 49 | Safety in Nonhuman Primates of Ocular AAV2-RPE65, a Candidate Treatment for Blindness in Leber Congenital Amaurosis. <i>Human Gene Therapy</i> , 2006, 17, 845-858.  | 2.7  | 142       |
| 50 | Intraocular route of AAV2 vector administration defines humoral immune response and therapeutic potential. <i>Molecular Vision</i> , 2008, 14, 1760-9.   | 1.1  | 140       |
| 51 | Long-term Retinal Function and Structure Rescue Using Capsid Mutant AAV8 Vector in the rd10 Mouse, a Model of Recessive Retinitis Pigmentosa. <i>Molecular Therapy</i> , 2011, 19, 234-242.  | 8.2  | 135       |
| 52 | IL-10 Suppresses Chemokines, Inflammation, and Fibrosis in a Model of Chronic Renal Disease. <i>Journal of the American Society of Nephrology: JASN</i> , 2005, 16, 3651-3660.   | 6.1  | 134       |
| 53 | AAV-Mediated Gene Therapy for Retinal Degeneration in the rd10 Mouse Containing a Recessive PDE $\beta$ Mutation. , 2008, 49, 4278.  |      | 133       |
| 54 | Title is missing!. <i>Nature Genetics</i> , 2001, 28, 92-95.   | 21.4 | 132       |

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|----|--|------|-----------|
| 55 | Ancient HLA genes from 7,500-year-old archaeological remains. <i>Nature</i> , 1991, 349, 785-788.  | 27.8 | 126       |
| 56 | Adeno-associated virus type-2 expression of pigmented epithelium-derived factor or Kringle 1 of angiostatin reduce retinal neovascularization. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2002, 99, 8909-8914. | 7.1  | 124       |
| 57 | Mitochondrial Protein Nitration Primes Neurodegeneration in Experimental Autoimmune Encephalomyelitis. <i>Journal of Biological Chemistry</i> , 2006, 281, 31950-31962.  | 3.4  | 123       |
| 58 | Pharmacological and rAAV Gene Therapy Rescue of Visual Functions in a Blind Mouse Model of Leber Congenital Amaurosis. <i>PLoS Medicine</i> , 2005, 2, e333.   | 8.4  | 120       |
| 59 | Suppression of Mitochondrial Oxidative Stress Provides Long-term Neuroprotection in Experimental Optic Neuritis. , 2007, 48, 681.  |      | 115       |
| 60 | Mutation-independent rhodopsin gene therapy by knockdown and replacement with a single AAV vector. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2018, 115, E8547-E8556.  | 7.1  | 114       |
| 61 | Canine and Human Visual Cortex Intact and Responsive Despite Early Retinal Blindness from RPE65 Mutation. <i>PLoS Medicine</i> , 2007, 4, e230.  | 8.4  | 107       |
| 62 | The Mutant Human ND4 Subunit of Complex I Induces Optic Neuropathy in the Mouse. , 2007, 48, 1.  |      | 107       |
| 63 | Ribozyme gene therapy: applications for molecular medicine. <i>Trends in Molecular Medicine</i> , 2001, 7, 221-228.  | 6.7  | 106       |
| 64 | Dual Adeno-Associated Virus Vectors Result in Efficient <i>In Vitro</i> and <i>In Vivo</i> Expression of an Oversized Gene, <i>MYO7A</i> . <i>Human Gene Therapy Methods</i> , 2014, 25, 166-177.  | 2.1  | 105       |
| 65 | AAV-Mediated Cone Rescue in a Naturally Occurring Mouse Model of CNGA3-Achromatopsia. <i>PLoS ONE</i> , 2012, 7, e35250.   | 2.5  | 105       |
| 66 | AAV Delivery of Wild-Type Rhodopsin Preserves Retinal Function in a Mouse Model of Autosomal Dominant Retinitis Pigmentosa. <i>Human Gene Therapy</i> , 2011, 22, 567-575.   | 2.7  | 104       |
| 67 | Interleukin 10 attenuates neointimal proliferation and inflammation in aortic allografts by a heme oxygenase-dependent pathway. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2005, 102, 7251-7256.               | 7.1  | 101       |
| 68 | Diabetic eNOS-Knockout Mice Develop Accelerated Retinopathy. , 2010, 51, 5240.   |      | 101       |
| 69 | Lentiviral Gene Transfer of Rpe65 Rescues Survival and Function of Cones in a Mouse Model of Leber Congenital Amaurosis. <i>PLoS Medicine</i> , 2006, 3, e347.   | 8.4  | 100       |
| 70 | The Human Rhodopsin Kinase Promoter in an AAV5 Vector Confers Rod- and Cone-Specific Expression in the Primate Retina. <i>Human Gene Therapy</i> , 2012, 23, 1101-1115.  | 2.7  | 99        |
| 71 | Adeno-Associated Virus-Vectored Gene Therapy for Retinal Disease. <i>Human Gene Therapy</i> , 2005, 16, 649-663.   | 2.7  | 98        |
| 72 | ERK1/2 activation is a therapeutic target in age-related macular degeneration. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2012, 109, 13781-13786.  | 7.1  | 98        |

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|----|--|------|-----------|
| 73 | Gene Augmentation Therapy Restores Retinal Function and Visual Behavior in a Sheep Model of CNGA3 Achromatopsia. <i>Molecular Therapy</i> , 2015, 23, 1423-1433.   | 8.2  | 93        |
| 74 | Adeno-associated virus DNA replication: Nonunit-length molecules. <i>Virology</i> , 1979, 93, 57-68.   | 2.4  | 92        |
| 75 | Advancing Therapeutic Strategies for Inherited Retinal Degeneration: Recommendations From the Monaciano Symposium. <i>Investigative Ophthalmology and Visual Science</i> , 2015, 56, 918-931.                                    | 3.3  | 92        |
| 76 | Effect of Late-stage Therapy on Disease Progression in AAV-mediated Rescue of Photoreceptor Cells in the Retinoschisin-deficient Mouse. <i>Molecular Therapy</i> , 2008, 16, 1010-1017.  | 8.2  | 91        |
| 77 | Residual Foveal Cone Structure in <i>CNGB3</i> -Associated Achromatopsia. , 2016, 57, 3984.  |      | 90        |
| 78 | Complement C3-Targeted Gene Therapy Restricts Onset and Progression of Neurodegeneration in Chronic Mouse Glaucoma. <i>Molecular Therapy</i> , 2018, 26, 2379-2396.  | 8.2  | 89        |
| 79 | Functional and Behavioral Restoration of Vision by Gene Therapy in the Guanylate Cyclase-1 (GC1) Knockout Mouse. <i>PLoS ONE</i> , 2010, 5, e11306.  | 2.5  | 89        |
| 80 | Comparative analysis of in vivo and in vitro AAV vector transduction in the neonatal mouse retina: Effects of serotype and site of administration. <i>Vision Research</i> , 2008, 48, 377-385.                                   | 1.4  | 87        |
| 81 | Efficient Transduction of Vascular Endothelial Cells with Recombinant Adeno-Associated Virus Serotype 1 and 5 Vectors. <i>Human Gene Therapy</i> , 2005, 16, 235-247.  | 2.7  | 84        |
| 82 | Inhibition of Choroidal Neovascularization in a Nonhuman Primate Model by Intravitreal Administration of an AAV2 Vector Expressing a Novel Anti-VEGF Molecule. <i>Molecular Therapy</i> , 2011, 19, 260-265.                     | 8.2  | 84        |
| 83 | Preclinical Potency and Safety Studies of an AAV2-Mediated Gene Therapy Vector for the Treatment of <i>MERTK</i> Associated Retinitis Pigmentosa. <i>Human Gene Therapy Clinical Development</i> , 2013, 24, 23-28.              | 3.1  | 84        |
| 84 | Suppression of complex I gene expression induces optic neuropathy. <i>Annals of Neurology</i> , 2003, 53, 198-205.   | 5.3  | 83        |
| 85 | Safety and Effects of the Vector for the Leber Hereditary Optic Neuropathy Gene Therapy Clinical Trial. <i>JAMA Ophthalmology</i> , 2014, 132, 409.  | 2.5  | 83        |
| 86 | Length heterogeneity of a conserved displacement-loop sequence in human mitochondrial DNA. <i>Nucleic Acids Research</i> , 1985, 13, 8093-8104.  | 14.5 | 80        |
| 87 | Gene therapy prevents photoreceptor death and preserves retinal function in a Bardet-Biedl syndrome mouse model. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2011, 108, 6276-6281.  | 7.1  | 80        |
| 88 | DICER1/ <i>Alu</i> RNA dysmetabolism induces Caspase-8-mediated cell death in age-related macular degeneration. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2014, 111, 16082-16087. | 7.1  | 79        |
| 89 | Efficiency and Safety of AAV-Mediated Gene Delivery of the Human ND4 Complex I Subunit in the Mouse Visual System. , 2009, 50, 4205.   |      | 76        |
| 90 | Long-Term Rescue of Retinal Structure and Function by Rhodopsin RNA Replacement with a Single Adeno-Associated Viral Vector in P23H <i>RHO</i> Transgenic Mice. <i>Human Gene Therapy</i> , 2012, 23, 356-366.                   | 2.7  | 76        |

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|-----|--|-----|-----------|
| 91  | ER Stress Is Involved in T17M Rhodopsin-Induced Retinal Degeneration. , 2012, 53, 3792.  |     | 75        |
| 92  | Successful arrest of photoreceptor and vision loss expands the therapeutic window of retinal gene therapy to later stages of disease. Proceedings of the National Academy of Sciences of the United States of America, 2015, 112, E5844-53.                          | 7.1 | 75        |
| 93  | Photoreceptor avascular privilege is shielded by soluble VEGF receptor-1. ELife, 2013, 2, e00324.  | 6.0 | 75        |
| 94  | Intraocular CNTF Reduces Vision in Normal Rats in a Dose-Dependent Manner. , 2007, 48, 5756.   |     | 74        |
| 95  | Transient Photoreceptor Deconstruction by CNTF Enhances rAAV-Mediated Cone Functional Rescue in Late Stage CNGB3-Achromatopsia. Molecular Therapy, 2013, 21, 1131-1141.  | 8.2 | 74        |
| 96  | SOD2 gene transfer protects against optic neuropathy induced by deficiency of complex I. Annals of Neurology, 2004, 56, 182-191.   | 5.3 | 73        |
| 97  | Brain-Derived Neurotrophic Factor Gene Transfer With Adeno-Associated Viral and Lentiviral Vectors Prevents Rubrospinal Neuronal Atrophy and Stimulates Regeneration-Associated Gene Expression After Acute Cervical Spinal Cord Injury. Spine, 2007, 32, 1164-1173. | 2.0 | 73        |
| 98  | XIAP Protection of Photoreceptors in Animal Models of Retinitis Pigmentosa. PLoS ONE, 2007, 2, e314.   | 2.5 | 73        |
| 99  | Genetically Targeted Binary Labeling of Retinal Neurons. Journal of Neuroscience, 2014, 34, 7845-7861.   | 3.6 | 72        |
| 100 | Adeno-Associated Virus Type 2 and Hepatocellular Carcinoma?. Human Gene Therapy, 2015, 26, 779-781.  | 2.7 | 71        |
| 101 | Extracellular signal-regulated kinase 1/2 mediates survival, but not axon regeneration, of adult injured central nervous system neurons in vivo. Journal of Neurochemistry, 2005, 93, 72-83.   | 3.9 | 70        |
| 102 | Molecular and Cellular Alterations Induced by Sustained Expression of Ciliary Neurotrophic Factor in a Mouse Model of Retinitis Pigmentosa. , 2007, 48, 1389.  |     | 70        |
| 103 | Light-Driven Cone Arrestin Translocation in Cones of Postnatal Guanylate Cyclase-1 Knockout Mouse Retina Treated with AAV-GC1. , 2006, 47, 3745.   |     | 68        |
| 104 | Leber Hereditary Optic Neuropathy Gene Therapy Clinical Trial Recruitment. JAMA Ophthalmology, 2010, 128, 1129.  | 2.4 | 68        |
| 105 | Highly Efficient Delivery of Adeno-Associated Viral Vectors to the Primate Retina. Human Gene Therapy, 2016, 27, 580-597.  | 2.7 | 68        |
| 106 | Molecular Anthropology Meets Genetic Medicine to Treat Blindness in the North African Jewish Population: Human Gene Therapy Initiated in Israel. Human Gene Therapy, 2010, 21, 1749-1757.  | 2.7 | 65        |
| 107 | Whirlin Replacement Restores the Formation of the USH2 Protein Complex in Whirlin Knockout Photoreceptors. , 2011, 52, 2343.   |     | 64        |
| 108 | Modeling and Preventing Progressive Hearing Loss in Usher Syndrome III. Scientific Reports, 2017, 7, 13480.  | 3.3 | 63        |

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|-----|--|-----|-----------|
| 109 | <i>BEST1</i> gene therapy corrects a diffuse retina-wide microdetachment modulated by light exposure. Proceedings of the National Academy of Sciences of the United States of America, 2018, 115, E2839-E2848.               | 7.1 | 62        |
| 110 | AAV-Mediated Gene Therapy in the Guanylate Cyclase (RetGC1/RetGC2) Double Knockout Mouse Model of Leber Congenital Amaurosis. Human Gene Therapy, 2013, 24, 189-202.   | 2.7 | 60        |
| 111 | Optimization of Retinal Gene Therapy for X-Linked Retinitis Pigmentosa Due to RPGR Mutations. Molecular Therapy, 2017, 25, 1866-1880.  | 8.2 | 60        |
| 112 | Cone-Specific Promoters for Gene Therapy of Achromatopsia and Other Retinal Diseases. Human Gene Therapy, 2016, 27, 72-82.   | 2.7 | 59        |
| 113 | Increased Sensitivity to Light-Induced Damage in a Mouse Model of Autosomal Dominant Retinal Disease. , 2007, 48, 1942.  |     | 58        |
| 114 | Long-term Preservation of Cone Photoreceptors and Restoration of Cone Function by Gene Therapy in the Guanylate Cyclase-1 Knockout (GC1KO) Mouse. , 2011, 52, 7098.  |     | 58        |
| 115 | Gene Therapy Rescues Cone Structure and Function in the 3-Month-Old <i>rd12</i> Mouse: A Model for Midcourse RPE65 Leber Congenital Amaurosis. , 2011, 52, 7.  |     | 58        |
| 116 | Long-Term Structural Outcomes of Late-Stage RPE65 Gene Therapy. Molecular Therapy, 2020, 28, 266-278.  | 8.2 | 56        |
| 117 | Mechanistic Basis for the Failure of Cone Transducin to Translocate: Why Cones Are Never Blinded by Light. Journal of Neuroscience, 2010, 30, 6815-6824.   | 3.6 | 54        |
| 118 | CCN2/CTGF regulates neovessel formation <i>via</i> targeting structurally conserved cystine knot motifs in multiple angiogenic regulators. FASEB Journal, 2012, 26, 3365-3379.   | 0.5 | 54        |
| 119 | Safety and Biodistribution Evaluation in Cynomolgus Macaques of rAAV2tYF-PR1.7-hCNGB3, a Recombinant AAV Vector for Treatment of Achromatopsia. Human Gene Therapy Clinical Development, 2016, 27, 37-48.                    | 3.1 | 53        |
| 120 | Long-term Suppression of Neurodegeneration in Chronic Experimental Optic Neuritis: Antioxidant Gene Therapy. , 2007, 48, 5360.   |     | 52        |
| 121 | LHON Gene Therapy Vector Prevents Visual Loss and Optic Neuropathy Induced by G11778A Mutant Mitochondrial DNA: Biodistribution and Toxicology Profile. Investigative Ophthalmology and Visual Science, 2014, 55, 7739-7753. | 3.3 | 52        |
| 122 | Structural and Functional Protection of Photoreceptors from MNU-Induced Retinal Degeneration by the X-Linked Inhibitor of Apoptosis. , 2003, 44, 2757.   |     | 51        |
| 123 | Use of Mitochondrial Antioxidant Defenses for Rescue of Cells With a Leber Hereditary Optic Neuropathy-Causing Mutation. JAMA Ophthalmology, 2007, 125, 268.   | 2.4 | 51        |
| 124 | Clarín-1, Encoded by the Usher Syndrome III Causative Gene, Forms a Membranous Microdomain. Journal of Biological Chemistry, 2009, 284, 18980-18993.   | 3.4 | 51        |
| 125 | Ab-Extero AAV-Mediated Gene Delivery to the Suprachoroidal Space Using a 250 Micron Flexible Microcatheter. PLoS ONE, 2011, 6, e17140.   | 2.5 | 50        |
| 126 | Rd3 gene delivery restores guanylate cyclase localization and rescues photoreceptors in the Rd3 mouse model of Leber congenital amaurosis 12. Human Molecular Genetics, 2013, 22, 3894-3905.                                 | 2.9 | 50        |



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|-----|--|-----|-----------|
| 127 | REPEATABILITY AND LONGITUDINAL ASSESSMENT OF FOVEAL CONE STRUCTURE IN CNGB3-ASSOCIATED ACHROMATOPSIA. <i>Retina</i> , 2017, 37, 1956-1966.   | 1.7 | 50        |
| 128 | Tyrosine-Mutant AAV8 Delivery of Human <i>MERTK</i> Provides Long-Term Retinal Preservation in RCS Rats. , 2012, 53, 1895.   |     | 48        |
| 129 | Rationally Engineered AAV Capsids Improve Transduction and Volumetric Spread in the CNS. <i>Molecular Therapy - Nucleic Acids</i> , 2017, 8, 184-197.  | 5.1 | 48        |
| 130 | Patients and animal models of CNG $\beta$ 1-deficient retinitis pigmentosa support gene augmentation approach. <i>Journal of Clinical Investigation</i> , 2017, 128, 190-206.                                | 8.2 | 48        |
| 131 | Recombinant AAV-Mediated BEST1 Transfer to the Retinal Pigment Epithelium: Analysis of Serotype-Dependent Retinal Effects. <i>PLoS ONE</i> , 2013, 8, e75666.  | 2.5 | 48        |
| 132 | Self-complementary AAV5 vector facilitates quicker transgene expression in $\beta$ photoreceptor and retinal pigment epithelial cells of normal mouse. <i>Experimental Eye Research</i> , 2010, 90, 546-554. | 2.6 | 47        |
| 133 | XIAP Therapy Increases Survival of Transplanted Rod Precursors in a Degenerating Host Retina. , 2011, 52, 1567.  |     | 47        |
| 134 | Stability and Safety of an AAV Vector for Treating <i>RPGR-ORF15</i> X-Linked Retinitis Pigmentosa. <i>Human Gene Therapy</i> , 2015, 26, 593-602.   | 2.7 | 47        |
| 135 | Induction of Rapid and Highly Efficient Expression of the Human ND4 Complex I Subunit in the Mouse Visual System by Self-complementary Adeno-Associated Virus. <i>JAMA Ophthalmology</i> , 2010, 128, 876.   | 2.4 | 46        |
| 136 | Long-term RNA interference gene therapy in a dominant retinitis pigmentosa mouse model. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2011, 108, 18476-18481.     | 7.1 | 46        |
| 137 | Imaging the response of the retina to electrical stimulation with genetically encoded calcium indicators. <i>Journal of Neurophysiology</i> , 2013, 109, 1979-1988.  | 1.8 | 45        |
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| 271 | Overexpression of Type 3 Iodothyronine Deiodinase Reduces Cone Death in the Leber Congenital Amaurosis Model Mice. <i>Advances in Experimental Medicine and Biology</i> , 2018, 1074, 125-131.              | 1.6 | 4         |
| 272 | Co-Expression of Wild-Type and Mutant S163R C1QTNF5 in Retinal Pigment Epithelium. <i>Advances in Experimental Medicine and Biology</i> , 2018, 1074, 61-66.  | 1.6 | 4         |
| 273 | Bovine opsin gene expression exhibits a late fetal to adult regulatory switch. <i>Journal of Neuroscience Research</i> , 1995, 40, 728-736.   | 2.9 | 3         |
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| 275 | Safety and Biodistribution Evaluation in CNGB3-deficient Mice of rAAV2tYF-PR1.7-hCNGB3, a Recombinant AAV Vector for Treatment of Achromatopsia. <i>Human Gene Therapy Clinical Development</i> , 2016, , . | 3.1 | 1         |
| 276 | Safety and Biodistribution Evaluation in Cynomolgus Macaques of rAAV2tYF-PR1.7-hCNGB3, a Recombinant AAV Vector for Treatment of Achromatopsia. <i>Human Gene Therapy Clinical Development</i> , 0, , .     | 3.1 | 1         |
| 277 | AAV-mediated sFLT4 gene therapy ameliorates retinal lesions in Ccl2/Cx3cr1 deficient mice. <i>FASEB Journal</i> , 2010, 24, 568.8.  | 0.5 | 1         |
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| 281 | Reply. <i>Ophthalmology</i> , 2018, 125, e15-e16.   | 5.2 | 0         |
| 282 | AAV9 mediates more specific cardiac gene transfer in the rat than AAV2, AAV5, AAV7, and AAV8. <i>FASEB Journal</i> , 2009, 23, 939.12.  | 0.5 | 0         |
| 283 | NADPH Oxidase in Choroidal Neovascularization. , 2012, , 307-320.   |     | 0         |