Efficacy and safety of voretigene neparvovec (AAV2-hR2-mediated inherited retinal dystrophy: a randomised, co

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Citation Report

#	Article	IF	CITATIONS
1	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
2	The Future Looks Brighter After 25 Years of Retinal Gene Therapy. Human Gene Therapy, 2017, 28, 982-987.	1.4	46
3	Gene therapy for RPE65 -mediated inherited retinal dystrophy completes phase 3. Lancet, The, 2017, 390, 823-824.	6.3	9
4	Gene Supplementation Rescues Rod Function and Preserves Photoreceptor and Retinal Morphology in Dogs, Leading the Way Toward Treating Human <i>PDE6A</i> Retinitis Pigmentosa. Human Gene Therapy, 2017, 28, 1189-1201.	1.4	27
5	Gene Therapy Briefs. Human Gene Therapy Clinical Development, 2017, 28, 167-174.	3.2	0
6	The pigmented epithelium, a bright partner against photoreceptor degeneration. Journal of Neurogenetics, 2017, 31, 203-215.	0.6	16
7	<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.	3.3	62
8	Retinal gene therapy. British Medical Bulletin, 2018, 126, 13-25.	2.7	52
9	Allele-Specific CRISPR-Cas9 Genome Editing of the Single-Base P23H Mutation for Rhodopsin-Associated Dominant Retinitis Pigmentosa. CRISPR Journal, 2018, 1, 55-64.	1.4	96
10	Multimodal Imaging for Differential Diagnosis of Bietti Crystalline Dystrophy. Ophthalmology Retina, 2018, 2, 1071-1077.	1.2	27
11	Tapping the RNA world for therapeutics. Nature Structural and Molecular Biology, 2018, 25, 357-364.	3.6	147
12	Prospect of retinal gene therapy following commercialization of voretigene neparvovec-rzyl for retinal dystrophy mediated by RPE65 mutation. Journal of Current Ophthalmology, 2018, 30, 1-2.	0.3	57
13	OPA1: How much do we know to approach therapy?. Pharmacological Research, 2018, 131, 199-210.	3.1	44
14	Gene therapy for Leber congenital amaurosis. Expert Review of Ophthalmology, 2018, 13, 11-15.	0.3	3
15	The First Approved Gene Therapy Product for Cancer Ad- <i>p53</i> (Gendicine): 12 Years in the Clinic. Human Gene Therapy, 2018, 29, 160-179.	1.4	225
16	Nucleic acid based therapies: developing frontier for precision medicine. BMJ: British Medical Journal, 2018, 360, k223.	2.4	O
17	Negative regulators that mediate ocular immune privilege. Journal of Leukocyte Biology, 2018, 103, 1179-1187.	1.5	60
18	Biosafety in Handling Gene Transfer Vectors. Current Protocols in Human Genetics, 2018, 96, 12.1.1-12.1.17.	3.5	1

#	Article	IF	Citations
19	Gene therapy for RPE65-mediated retinal dystrophies. Survey of Ophthalmology, 2018, 63, 445-446.	1.7	1
20	Gene therapy comes of age. Science, 2018, 359, .	6.0	936
21	Retinal Neuroprotection: Overcoming the Translational Roadblocks. American Journal of Ophthalmology, 2018, 192, xv-xxii.	1.7	12
22	Non-syndromic retinitis pigmentosa. Progress in Retinal and Eye Research, 2018, 66, 157-186.	7.3	565
23	Amelioration of Neurosensory Structure and Function in Animal and Cellular Models of a Congenital Blindness. Molecular Therapy, 2018, 26, 1581-1593.	3.7	19
24	Presentation of <i>TRPM1</i> -Associated Congenital Stationary Night Blindness in Children. JAMA Ophthalmology, 2018, 136, 389.	1.4	35
25	Gene Therapy for Retinal Degeneration. Cell, 2018, 173, 5.	13.5	58
26	Turning the corner from observation to intervention in human genetics. Journal of Genetics and Genomics, 2018, 45, 57-59.	1.7	1
27	CRISPR-Cas9 genome engineering: Treating inherited retinal degeneration. Progress in Retinal and Eye Research, 2018, 65, 28-49.	7. 3	64
28	Pharmacotherapy of retinal disease with visual cycle modulators. Expert Opinion on Pharmacotherapy, 2018, 19, 471-481.	0.9	31
29	Novel mobility test to assess functional vision in patients with inherited retinal dystrophies. Clinical and Experimental Ophthalmology, 2018, 46, 247-259.	1.3	97
30	Molecular genetics and emerging therapies for retinitis pigmentosa: Basic research and clinical perspectives. Progress in Retinal and Eye Research, 2018, 63, 107-131.	7.3	301
31	Photoreceptor Cell Replacement Therapy from Stem Cells. Fundamental Biomedical Technologies, 2018, , 1-16.	0.2	0
32	A Cross-Sectional and Longitudinal Study of Retinal Sensitivity in <i>RPE65</i> -Associated Leber Congenital Amaurosis., 2018, 59, 3330.		19
33	Fall Prevention: A Deliberative Nursing Process. Journal of Gerontology & Geriatric Research, 2018, 07,	0.1	0
34	Caring for Hereditary Childhood Retinal Blindness. Asia-Pacific Journal of Ophthalmology, 2018, 7, 183-191.	1.3	14
35	Cystic Fibrosis Gene Therapy: Looking Back, Looking Forward. Genes, 2018, 9, 538.	1.0	87
36	Addressing the Value of Gene Therapy and Enhancing Patient Access to Transformative Treatments. Molecular Therapy, 2018, 26, 2717-2726.	3.7	71

#	ARTICLE	IF	Citations
38	Gene Therapy for Cystic Fibrosis Lung Disease: Overcoming the Barriers to Translation to the Clinic. Frontiers in Pharmacology, 2018, 9, 1381.	1.6	34
39	Antigen-selective modulation of AAV immunogenicity with tolerogenic rapamycin nanoparticles enables successful vector re-administration. Nature Communications, 2018, 9, 4098.	5.8	184
40	Beneficial effects on vision in patients undergoing retinal gene therapy for choroideremia. Nature Medicine, 2018, 24, 1507-1512.	15.2	140
41	Severe Loss of Tritan Color Discrimination in <i>RPE65</i> Associated Leber Congenital Amaurosis., 2018, 59, 85.		15
43	Synthetic materials at the forefront of gene delivery. Nature Reviews Chemistry, 2018, 2, 258-277.	13.8	215
44	Emerging Technologies for Delivery of Biotherapeutics and Gene Therapy Across the Blood–Brain Barrier. BioDrugs, 2018, 32, 547-559.	2.2	64
45	Structural biology of 11- <i>cis-</i> retinaldehyde production in the classical visual cycle. Biochemical Journal, 2018, 475, 3171-3188.	1.7	18
46	GNE Myopathy: Etiology, Diagnosis, and Therapeutic Challenges. Neurotherapeutics, 2018, 15, 900-914.	2.1	63
47	Successful intracranial delivery of trastuzumab by gene-therapy for treatment of HER2-positive breast cancer brain metastases. Journal of Controlled Release, 2018, 291, 80-89.	4.8	27
48	Gene therapy for <i>RPE65</i> -related retinal disease. Ophthalmic Genetics, 2018, 39, 671-677.	0.5	85
49	Gene Therapy for Inherited Retinopathies: Update on Development Progress. Journal of Vitreoretinal Diseases, 2018, 2, 219-226.	0.2	2
50	Inherited Retinal Degenerations: Current Landscape and Knowledge Gaps. Translational Vision Science and Technology, 2018, 7, 6.	1.1	168
51	Human Cardiac Gene Therapy. Circulation Research, 2018, 123, 601-613.	2.0	75
52	Clinical Characterization of 66 Patients With Congenital Retinal Disease Due to the Deep-Intronic c.2991+1655A>G Mutation in <i>CEP290</i>		21
53	The Blunt End: Surgical Challenges of Gene Therapy for Inherited Retinal Diseases. American Journal of Ophthalmology, 2018, 196, xxv-xxix.	1.7	25
54	PROGRESSION OF SCOTOPIC SINGLE-FLASH ELECTRORETINOGRAPHY IN THE STAGES OF CAPN5 VITREORETINOPATHY. Retinal Cases and Brief Reports, 2021, 15, 473-478.	0.3	5
55	A novel adeno-associated virus capsid with enhanced neurotropism corrects a lysosomal transmembrane enzyme deficiency. Brain, 2018, 141, 2014-2031.	3.7	80
56	Preparation of Nonhuman Primate Eyes for Histological Evaluation After Retinal Gene Transfer. Human Gene Therapy Methods, 2018, 29, 115-123.	2.1	0

#	Article	IF	Citations
57	Translational Aspects of Adeno-Associated Virus–Mediated Cardiac Gene Therapy. Human Gene Therapy, 2018, 29, 1341-1351.	1.4	7
58	2DåŠå°Žä½"ã®æ°ªæ–¹å'ãƒ~ãƒ†ãƒæŽ¥å•ã,'ワンãƒãƒãƒã§. Nature Digest, 2018, 15, 33-35.	0.0	0
59	Restoring vision. Nature, 2018, 557, 359-367.	13.7	108
60	Spliceosome-Mediated Pre-mRNA trans-Splicing Can Repair CEP290 mRNA. Molecular Therapy - Nucleic Acids, 2018, 12, 294-308.	2.3	23
61	Efficient Gene Transfer to Kidney Mesenchymal Cells Using a Synthetic Adeno-Associated Viral Vector. Journal of the American Society of Nephrology: JASN, 2018, 29, 2287-2297.	3.0	38
62	Progress in Gene Therapy to Prevent Retinal Ganglion Cell Loss in Glaucoma and Leber's Hereditary Optic Neuropathy. Neural Plasticity, 2018, 2018, 1-11.	1.0	31
63	Choroideremia: molecular mechanisms and development of AAV gene therapy. Expert Opinion on Biological Therapy, 2018, 18, 807-820.	1.4	28
64	Clinical applications of retinal gene therapies. Precision Clinical Medicine, 2018, 1, 5-20.	1.3	11
65	Guiding Lights in Genome Editing for Inherited Retinal Disorders: Implications for Gene and Cell Therapy. Neural Plasticity, 2018, 2018, 1-15.	1.0	29
66	CRISPR/Cas9 genome surgery for retinal diseases. Drug Discovery Today: Technologies, 2018, 28, 23-32.	4.0	10
67	Retained Plasticity and Substantial Recovery of Rod-Mediated Visual Acuity at the Visual Cortex in Blind Adult Mice with Retinal Dystrophy. Molecular Therapy, 2018, 26, 2397-2406.	3.7	6
68	Mutations in known disease genes account for the majority of autosomal recessive retinal dystrophies. Clinical Genetics, 2018, 94, 554-563.	1.0	12
69	Non-viral ocular gene therapy, pEYS606, for the treatment of non-infectious uveitis: Preclinical evaluation of the medicinal product. Journal of Controlled Release, 2018, 285, 244-251.	4.8	24
70	Gene therapy in inherited retinal degenerative diseases, a review. Ophthalmic Genetics, 2018, 39, 560-568.	0.5	55
71	Special Issue Introduction: Inherited Retinal Disease: Novel Candidate Genes, Genotype–Phenotype Correlations, and Inheritance Models. Genes, 2018, 9, 215.	1.0	58
72	Low vision services: a practical guide for the clinician. Therapeutic Advances in Ophthalmology, 2018, 10, 251584141877626.	0.8	21
73	Acoustically targeted chemogenetics for the non-invasive control of neural circuits. Nature Biomedical Engineering, 2018, 2, 475-484.	11.6	91
74	An ode to gene edits that prevent deafness. Nature, 2018, 553, 162-163.	13.7	2

#	Article	IF	CITATIONS
75	Seeing the Light after 25 Years of Retinal Gene Therapy. Trends in Molecular Medicine, 2018, 24, 669-681.	3.5	93
76	Transfection by cationic gemini lipids and surfactants. MedChemComm, 2018, 9, 1404-1425.	3.5	28
77	Emerging Gene Therapy Treatments for Inherited Retinal Diseases. Ophthalmic Surgery Lasers and Imaging Retina, 2018, 49, 472-478.	0.4	2
78	Efficacy and effectiveness: The wrong use of different terms. European Journal of Internal Medicine, 2018, 54, e17-e18.	1.0	9
79	Helper-free Production of Laboratory Grade AAV and Purification by Iodixanol Density Gradient Centrifugation. Molecular Therapy - Methods and Clinical Development, 2018, 10, 1-7.	1.8	59
80	Herpes Simplex Virus Vectors for Gene Transfer to the Central Nervous System. Diseases (Basel,) Tj ETQq $1\ 1\ 0.78$	34314 rgB ⁻	Г/Qyerlock
81	Systemic AAV Micro-dystrophin Gene Therapy for Duchenne Muscular Dystrophy. Molecular Therapy, 2018, 26, 2337-2356.	3.7	306
82	Accurate Titration of Infectious AAV Particles Requires Measurement of Biologically Active Vector Genomes and Suitable Controls. Molecular Therapy - Methods and Clinical Development, 2018, 10, 223-236.	1.8	33
83	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
84	Neuraminidaseâ€mediated desialylation augments AAV9â€mediated gene expression in skeletal muscle. Journal of Gene Medicine, 2018, 20, e3049.	1.4	9
85	Cryoprecipitate augments the global transduction of the adeno-associated virus serotype 9 after a systemic administration. Journal of Controlled Release, 2018, 286, 415-424.	4.8	7
86	Breaking and Sealing Barriers in Retinal Gene Therapy. Molecular Therapy, 2018, 26, 2081-2082.	3.7	10
87	Efficient Gene Transfer to the Central Nervous System by Single-Stranded Anc80L65. Molecular Therapy - Methods and Clinical Development, 2018, 10, 197-209.	1.8	62
88	Stargardt macular dystrophy and evolving therapies. Expert Opinion on Biological Therapy, 2018, 18, 1049-1059.	1.4	40
89	Inherited Retinal Diseases. Current Practices in Ophthalmology, 2018, , 133-154.	0.1	0
90	NAD+ and sirtuins in retinal degenerative diseases: A look at future therapies. Progress in Retinal and Eye Research, 2018, 67, 118-129.	7.3	24
91	Results at 5 Years After Gene Therapy for RPE65-Deficient Retinal Dystrophy. Human Gene Therapy, 2018, 29, 1428-1437.	1.4	48
92	In vitro efficacy of a gene-activated nerve guidance conduit incorporating non-viral PEI-pDNA nanoparticles carrying genes encoding for NGF, GDNF and c-Jun. Acta Biomaterialia, 2018, 75, 115-128.	4.1	41

#	ARTICLE	IF	CITATIONS
93	Ocular gene therapy for choroideremia: clinical trials and future perspectives. Expert Review of Ophthalmology, 2018, 13, 129-138.	0.3	11
94	Viral delivery of genome-modifying proteins for cellular reprogramming. Current Opinion in Genetics and Development, 2018, 52, 92-99.	1.5	4
95	Contribution of noncoding pathogenic variants to RPGRIP1-mediated inherited retinal degeneration. Genetics in Medicine, 2019, 21, 694-704.	1.1	27
96	Age-Related Seroprevalence of Antibodies Against AAV-LK03 in a UK Population Cohort. Human Gene Therapy, 2019, 30, 79-87.	1.4	51
97	Unmet Needs in Ophthalmology: A European Vision Institute-Consensus Roadmap 2019–2025. Ophthalmic Research, 2019, 62, 123-133.	1.0	20
99	<i>CAPN5</i> genetic inactivation phenotype supports therapeutic inhibition trials. Human Mutation, 2019, 40, 2377-2392.	1.1	9
101	Cost-effectiveness of Voretigene Neparvovec-rzyl Therapy. JAMA Ophthalmology, 2019, 137, 1123.	1.4	4
102	Human gene therapy approaches for the treatment of Parkinson's disease: An overview of current and completed clinical trials. Parkinsonism and Related Disorders, 2019, 66, 16-24.	1.1	40
103	In vivo phenotypic and molecular characterization of retinal degeneration in mouse models of three ciliopathies. Experimental Eye Research, 2019, 186, 107721.	1.2	8
104	Targeting neuronal and glial cell types with synthetic promoter AAVs in mice, non-human primates and humans. Nature Neuroscience, 2019, 22, 1345-1356.	7.1	144
105	Recombinant Adeno-Associated Virus Gene Therapy in Light of Luxturna (and Zolgensma and Glybera): Where Are We, and How Did We Get Here?. Annual Review of Virology, 2019, 6, 601-621.	3.0	217
106	Enhancement of Adeno-Associated Virus-Mediated Gene Therapy Using Hydroxychloroquine in Murine and Human Tissues. Molecular Therapy - Methods and Clinical Development, 2019, 14, 77-89.	1.8	25
107	Infectivity Assessment of Recombinant Adeno-Associated Virus and Wild-Type Adeno-Associated Virus Exposed to Various Diluents and Environmental Conditions. Human Gene Therapy Methods, 2019, 30, 137-143.	2.1	8
108	Cost-effectiveness of Voretigene Neparvovec-rzyl vs Standard Care for <i>RPE65</i> Inherited Retinal Disease. JAMA Ophthalmology, 2019, 137, 1115.	1.4	43
109	Advances in gene therapy for cystic fibrosis lung disease. Human Molecular Genetics, 2019, 28, R88-R94.	1.4	72
110	Rescue of Rod Synapses by Induction of Ca _v Alpha 1F in the Mature Ca _v 1.4 Knock-Out Mouse Retina., 2019, 60, 3150.		11
111	Gene Therapy. New England Journal of Medicine, 2019, 381, 455-464.	13.9	343
112	Non-viral delivery of chemically modified mRNA to the retina: Subretinal versus intravitreal administration. Journal of Controlled Release, 2019, 307, 315-330.	4.8	32

#	Article	IF	CITATIONS
113	The photoreceptor cilium and its diseases. Current Opinion in Genetics and Development, 2019, 56, 22-33.	1.5	41
114	A novel, wearable, electronic visual aid to assist those with reduced peripheral vision. PLoS ONE, 2019, 14, e0223755.	1.1	5
115	Biodegradable Polymers for Gene Delivery. Molecules, 2019, 24, 3744.	1.7	100
116	Functional Genomics of the Retina to Elucidate its Construction and Deconstruction. International Journal of Molecular Sciences, 2019, 20, 4922.	1.8	7
117	Neonatal brain-directed gene therapy rescues a mouse model of neurodegenerative CLN6 Batten disease. Human Molecular Genetics, 2019, 28, 3867-3879.	1.4	21
118	Delivering genes across the blood-brain barrier: LY6A, a novel cellular receptor for AAV-PHP.B capsids. PLoS ONE, 2019, 14, e0225206.	1.1	145
120	An Update on Ocular Gene Therapy for Monogenic and Multifactorial Retinal Diseases. Journal of Vitreoretinal Diseases, 2019, 3, 366-377.	0.2	0
122	Generation and Characterization of Induced Pluripotent Stem Cells and Retinal Organoids From a Leber's Congenital Amaurosis Patient With Novel RPE65 Mutations. Frontiers in Molecular Neuroscience, 2019, 12, 212.	1.4	30
123	Efficacy, Safety, and Durability of Voretigene Neparvovec-rzyl in RPE65 Mutation–Associated Inherited Retinal Dystrophy. Ophthalmology, 2019, 126, 1273-1285.	2.5	239
125	Challenges to Routine Genetic Testing for Inherited Retinal Dystrophies. Ophthalmology, 2019, 126, 1466-1468.	2.5	6
126	Helper-Dependent Adenovirus Transduces the Human and Rat Retina but Elicits an Inflammatory Reaction When Delivered Subretinally in Rats. Human Gene Therapy, 2019, 30, 1371-1384.	1.4	19
128	Safety of Same-Eye Subretinal Sequential Readministration of AAV2-hRPE65v2 in Non-human Primates. Molecular Therapy - Methods and Clinical Development, 2019, 15, 133-148.	1.8	20
129	Sustained Rescue of Rod Function and Probable Non–Cell-Autonomous Rescue of Cones after Gene Therapy. Ophthalmology, 2019, 126, 1286-1287.	2.5	1
130	Non-antibiotic Small-Molecule Regulation of DHFR-Based Destabilizing Domains InÂVivo. Molecular Therapy - Methods and Clinical Development, 2019, 15, 27-39.	1.8	13
131	Development and Clinical Translation of Approved Gene Therapy Products for Genetic Disorders. Frontiers in Genetics, 2019, 10, 868.	1.1	168
132	Retina transduction by rAAV2 after intravitreal injection: comparison between mouse and rat. Gene Therapy, 2019, 26, 479-490.	2.3	14
133	Short-Term Local Expression of a PD-L1 Blocking Antibody from a Self-Replicating RNA Vector Induces Potent Antitumor Responses. Molecular Therapy, 2019, 27, 1892-1905.	3.7	28
134	Utility of In Vitro Mutagenesis of RPE65 Protein for Verification of Mutational Pathogenicity Before Gene Therapy. JAMA Ophthalmology, 2019, 137, 1381.	1.4	11

#	Article	IF	CITATIONS
135	AAV-PHP.B Administration Results in a Differential Pattern of CNS Biodistribution in Non-human Primates Compared with Mice. Molecular Therapy, 2019, 27, 2018-2037.	3.7	92
136	Estimation of impact of <i>RPE65-</i> mediated inherited retinal disease on quality of life and the potential benefits of gene therapy. British Journal of Ophthalmology, 2019, 103, 1610-1614.	2.1	38
137	Safety and efficacy evaluations of an adeno-associated virus variant for preparing IL10-secreting human neural stem cell-based therapeutics. Gene Therapy, 2019, 26, 135-150.	2.3	5
138	Factors governing the transduction efficiency of adeno-associated virus in the retinal ganglion cells following intravitreal injection. Gene Therapy, 2019, 26, 109-120.	2.3	10
139	Viral Vectors for Gene Therapy. Methods in Molecular Biology, 2019, , .	0.4	2
140	Adeno-associated virus vector as a platform for gene therapy delivery. Nature Reviews Drug Discovery, 2019, 18, 358-378.	21.5	1,267
141	Suprachoroidally injected pharmacological agents for the treatment of chorioâ€retinal diseases: a targeted approach. Acta Ophthalmologica, 2019, 97, 460-472.	0.6	51
142	Systemic Delivery of Adeno-Associated Viral Vectors in Mice and Dogs. Methods in Molecular Biology, 2019, 1937, 281-294.	0.4	5
143	Luxturna: FDA documents reveal the value of a costly gene therapy. Drug Discovery Today, 2019, 24, 949-954.	3.2	133
144	Gene therapy for retinal dystrophy. Nature Medicine, 2019, 25, 198-199.	15.2	13
145	Retinal Gene Distribution and Functionality Implicated in Inherited Retinal Degenerations Can Reveal Disease-Relevant Pathways for Pharmacologic Intervention. Pharmaceuticals, 2019, 12, 74.	1.7	5
146	Recent advances in slow and sustained drug release for retina drug delivery. Expert Opinion on Drug Delivery, 2019, 16, 679-686.	2.4	15
147	C3- and CR3-dependent microglial clearance protects photoreceptors in retinitis pigmentosa. Journal of Experimental Medicine, 2019, 216, 1925-1943.	4.2	82
148	Has retinal gene therapy come of age? From bench to bedside and back to bench. Human Molecular Genetics, 2019, 28, R108-R118.	1.4	41
149	Ocular gene therapies in clinical practice: viral vectors and nonviral alternatives. Drug Discovery Today, 2019, 24, 1685-1693.	3.2	78
150	Spectrum of Disease Severity and Phenotype in Choroideremia Carriers. American Journal of Ophthalmology, 2019, 207, 77-86.	1.7	21
151	Rescue of spinal muscular atrophy mouse models with AAV9-Exon-specific U1 snRNA. Nucleic Acids Research, 2019, 47, 7618-7632.	6.5	37
152	Genome Editing as a Treatment for the Most Prevalent Causative Genes of Autosomal Dominant Retinitis Pigmentosa. International Journal of Molecular Sciences, 2019, 20, 2542.	1.8	40

#	Article	IF	CITATIONS
153	Depicting brighter possibilities for treating blindness. Science Translational Medicine, 2019, 11, .	5.8	24
154	TLR9-Activating CpG-B ODN but Not TLR7 Agonists Triggers Antibody Formation to Factor IX in Muscle Gene Transfer. Human Gene Therapy Methods, 2019, 30, 81-92.	2.1	22
155	Therapeutic perspectives for structural and functional abnormalities of cilia. Cellular and Molecular Life Sciences, 2019, 76, 3695-3709.	2.4	9
156	Gene delivery to cone photoreceptors by subretinal injection of rAAV2/6 in the mouse retina. Biochemical and Biophysical Research Communications, 2019, 515, 222-227.	1.0	4
157	Important Considerations in Modeling the Cost-Effectiveness for the First Food and Drug Administration–Approved Gene Therapy and Implications for Future One-Time Therapies. Value in Health, 2019, 22, 970-971.	0.1	6
158	Defining and Managing High-Priced Cures: Healthcare Payers' Opinions. Value in Health, 2019, 22, 648-655.	0.1	7
159	Gene Therapy in Pediatric Liver Disease. , 2019, , 799-829.		2
160	A novel RP2 missense mutation Q158P identified in an X-linked retinitis pigmentosa family impaired RP2 protein stability. Gene, 2019, 707, 86-92.	1.0	8
161	Viral-mediated gene therapy and genetically modified therapeutics: A primer on biosafety handling for the health-system pharmacist. American Journal of Health-System Pharmacy, 2019, 76, 795-802.	0.5	15
162	Optimizing Donor Cellular Dissociation and Subretinal Injection Parameters for Stem Cell-Based Treatments. Stem Cells Translational Medicine, 2019, 8, 797-809.	1.6	21
164	Adhesive thermosensitive gels for local delivery of viral vectors. Biotechnology and Bioengineering, 2019, 116, 2353-2363.	1.7	6
165	Soluble CX3CL1 gene therapy improves cone survival and function in mouse models of retinitis pigmentosa. Proceedings of the National Academy of Sciences of the United States of America, 2019, 116, 10140-10149.	3.3	35
166	CRISPR Activation Enhances InÂVitro Potency of AAV Vectors Driven by Tissue-Specific Promoters. Molecular Therapy - Methods and Clinical Development, 2019, 13, 380-389.	1.8	35
167	Leber Congenital Amaurosis (LCA): Potential for Improvement of Vision. , 2019, 60, 1680.		50
168	New Frontiers of Corneal Gene Therapy. Human Gene Therapy, 2019, 30, 923-945.	1.4	18
169	Adeno-associated virus vectored immunoprophylaxis to prevent HIV in healthy adults: a phase 1 randomised controlled trial. Lancet HIV, the, 2019 , 6, $e230$ - $e239$.	2.1	84
170	Adeno Associated Virus (AAV) as a Tool for Clinical and Experimental Delivery of Target Genes into the Mammalian Retina. Biological and Pharmaceutical Bulletin, 2019, 42, 343-347.	0.6	15
171	Therapeutic AAV Gene Transfer to the Nervous System: A Clinical Reality. Neuron, 2019, 101, 839-862.	3.8	234

#	Article	IF	Citations
173	Molybdenum cofactor deficiency type B knock-in mouse models carrying patient-identical mutations and their rescue by singular AAV injections. Human Genetics, 2019, 138, 355-361.	1.8	3
174	Cell Therapy for Retinal Dystrophies: From Cell Suspension Formulation to Complex Retinal Tissue Bioengineering. Stem Cells International, 2019, 2019, 1-14.	1.2	24
175	The Growing Development of DNA Nanostructures for Potential Healthcareâ€Related Applications. Advanced Healthcare Materials, 2019, 8, e1801546.	3.9	60
176	Reading deficits in diabetic patients treated with panretinal photocoagulation and good visual acuity. Acta Ophthalmologica, 2019, 97, e1013-e1018.	0.6	3
177	Characterizing variants of unknown significance in rhodopsin: A functional genomics approach. Human Mutation, 2019, 40, 1127-1144.	1.1	22
178	Detailed clinical characterisation, unique features and natural history of autosomal recessive <i>RDH12</i> -associated retinal degeneration. British Journal of Ophthalmology, 2019, 103, bjophthalmol-2018-313580.	2.1	20
179	Lipid nanoparticles for delivery of messenger RNA to the back of the eye. Journal of Controlled Release, 2019, 303, 91-100.	4.8	134
180	Monomethyl Fumarate Protects the Retina From Light-Induced Retinopathy. , 2019, 60, 1275.		18
181	A 2-Year Longitudinal Study of Normal Cone Photoreceptor Density. , 2019, 60, 1420.		11
182	A light in the dark: state of the art and perspectives in optogenetics and optopharmacology for restoring vision. Future Medicinal Chemistry, 2019, 11, 463-487.	1.1	7
183	Ciliary Neurotrophic Factor Treatment Improves Retinal Structure and Function in Macular Telangiectasia Type 2. Ophthalmology, 2019, 126, 550-551.	2.5	4
184	Novel stem cell and gene therapy in diabetic retinopathy, age related macular degeneration, and retinitis pigmentosa. International Journal of Retina and Vitreous, 2019, 5, 7.	0.9	20
185	Trans-ocular Electric Current InÂVivo Enhances AAV-Mediated Retinal Gene Transduction after Intravitreal Vector Administration. Molecular Therapy - Methods and Clinical Development, 2019, 13, 77-85.	1.8	12
186	Understanding Ocular Inflammation in Eyes Treated With Intravitreal Gene Therapy. JAMA Ophthalmology, 2019, 137, 407.	1.4	2
187	Immune Response and Intraocular Inflammation in Patients With Leber Hereditary Optic Neuropathy Treated With Intravitreal Injection of Recombinant Adeno-Associated Virus 2 Carrying the <i>ND4</i> Gene. JAMA Ophthalmology, 2019, 137, 399.	1.4	68
188	Comparative AAV-eGFP Transgene Expression Using Vector Serotypes $1\hat{a}\in$ 9, 7m8, and 8b in Human Pluripotent Stem Cells, RPEs, and Human and Rat Cortical Neurons. Stem Cells International, 2019, 2019, 1-11.	1.2	24
189	Design of AAV Vectors for Delivery of RNAi. Methods in Molecular Biology, 2019, 1950, 3-18.	0.4	6
190	Effects of Lycii Fructus and Salviae Miltiorrhizae on the Syndrome of Deficiency with Blood Stasis in RCS (rdy-/-, p-/-) Rats with Retinitis Pigmentosa: An Intervention Study. Digital Chinese Medicine, 2019, 2, 157-165.	0.5	2

#	ARTICLE	IF	CITATIONS
191	Autosomal Dominant Retinitis Pigmentosa Due to Class B Rhodopsin Mutations: An Objective Outcome for Future Treatment Trials. International Journal of Molecular Sciences, 2019, 20, 5344.	1.8	11
192	Bioanalysis of adeno-associated virus gene therapy therapeutics: regulatory expectations. Bioanalysis, 2019, 11, 2011-2024.	0.6	15
193	Investigation and Restoration of BEST1 Activity in Patient-derived RPEs with Dominant Mutations. Scientific Reports, 2019, 9, 19026.	1.6	27
194	Emerging Treatments for Leber's Hereditary Optic Neuropathy and Other Genetic Causes of Visual Loss. Seminars in Neurology, 2019, 39, 732-738.	0.5	0
195	Progress in the development of novel therapies for choroideremia. Expert Review of Ophthalmology, 2019, 14, 277-285.	0.3	12
196	Wide-field fundus autofluorescence imaging in patients with hereditary retinal degeneration: a literature review. International Journal of Retina and Vitreous, 2019, 5, 23.	0.9	16
197	Awakening the regenerative potential of the mammalian retina. Development (Cambridge), 2019, 146, .	1.2	22
198	Comprehensive AAV capsid fitness landscape reveals a viral gene and enables machine-guided design. Science, 2019, 366, 1139-1143.	6.0	217
199	Outcome Measures Used in Ocular Gene Therapy Trials: A Scoping Review of Current Practice. Frontiers in Pharmacology, 2019, 10, 1076.	1.6	24
200	DNA complexes as an efficient gene anticancer drug delivery therapy. , 2019, , 485-549.		0
201	Restoring vision at the fovea. Current Opinion in Behavioral Sciences, 2019, 30, 210-216.	2.0	6
202	A User's Guide to the Inverted Terminal Repeats of Adeno-Associated Virus. Human Gene Therapy Methods, 2019, 30, 206-213.	2.1	33
203	Gene Therapy. Advances in Biochemical Engineering/Biotechnology, 2019, 171, 321-368.	0.6	12
204	Retinal neuroprotection. Current Opinion in Ophthalmology, 2019, 30, 199-205.	1.3	27
205	Surgical Technique for Subretinal Gene Therapy in Humans with Inherited Retinal Degeneration. Retina, 2019, 39, S2-S8.	1.0	45
206	Current Concepts and Emerging Gene Therapies for Inherited Retinal Diseases. International Ophthalmology Clinics, 2019, 59, 83-110.	0.3	2
207	Mechanistic Insights into Factor VIII Immune Tolerance Induction via Prenatal Cell Therapy in Hemophilia A. Current Stem Cell Reports, 2019, 5, 145-161.	0.7	3
208	A systematic capsid evolution approach performed in vivo for the design of AAV vectors with tailored properties and tropism. Proceedings of the National Academy of Sciences of the United States of America, 2019, 116, 27053-27062.	3.3	107

#	Article	IF	CITATIONS
209	Multiplexed activation of endogenous genes by CRISPRa elicits potent antitumor immunity. Nature Immunology, 2019, 20, 1494-1505.	7.0	83
210	Retinitis Pigmentosa: Review of Current Treatment. International Ophthalmology Clinics, 2019, 59, 263-280.	0.3	47
211	Vision Restoration in Outer Retinal Degenerations: Current Approaches and Future Directions. International Ophthalmology Clinics, 2019, 59, 59-69.	0.3	1
212	CHOROIDEREMIA. Retina, 2019, 39, 2059-2069.	1.0	23
213	Gene therapy for progressive familial intrahepatic cholestasis type 3 in a clinically relevant mouse model. Nature Communications, 2019, 10, 5694.	5.8	30
214	Treatment with Stem Cells from Human Exfoliated Deciduous Teeth and Their Derived Conditioned Medium Improves Retinal Visual Function and Delays the Degeneration of Photoreceptors. Stem Cells and Development, 2019, 28, 1514-1526.	1.1	14
215	The use of nonhuman primates in studies of noise injury and treatment. Journal of the Acoustical Society of America, 2019, 146, 3770-3789.	0.5	18
216	Cytokines in Pain: Harnessing Endogenous Anti-Inflammatory Signaling for Improved Pain Management. Frontiers in Immunology, 2019, 10, 3009.	2.2	109
217	Gene therapy for visual loss: Opportunities and concerns. Progress in Retinal and Eye Research, 2019, 68, 31-53.	7.3	78
218	Patient-derived induced pluripotent stem cells for modelling genetic retinal dystrophies. Progress in Retinal and Eye Research, 2019, 68, 54-66.	7. 3	37
219	Optogenetic approaches to vision restoration. Experimental Eye Research, 2019, 178, 15-26.	1.2	77
220	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.	1.8	52
221	Preclinical Evaluation of ADVM-022, a Novel Gene Therapy Approach to Treating Wet Age-Related Macular Degeneration. Molecular Therapy, 2019, 27, 118-129.	3.7	83
222	Commentary on Randomization: The forgotten component of the randomized clinical trial. Statistics in Medicine, 2019, 38, 14-16.	0.8	3
223	AAV-Mediated Progranulin Delivery to a Mouse Model of Progranulin Deficiency Causes T Cell-Mediated Toxicity. Molecular Therapy, 2019, 27, 465-478.	3.7	41
224	Effect of an intravitreal antisense oligonucleotide on vision in Leber congenital amaurosis due to a photoreceptor cilium defect. Nature Medicine, 2019, 25, 225-228.	15.2	177
225	Night-vision aid using see-through display for patients with retinitis pigmentosa. Japanese Journal of Ophthalmology, 2019, 63, 181-185.	0.9	10
226	Pharmaceutical Development of AAV-Based Gene Therapy Products for the Eye. Pharmaceutical Research, 2019, 36, 29.	1.7	146

#	ARTICLE	IF	CITATIONS
227	Attenuation of Inherited and Acquired Retinal Degeneration Progression with Gene-based Techniques. Molecular Diagnosis and Therapy, 2019, 23, 113-120.	1.6	6
228	Entering the Modern Era of Gene Therapy. Annual Review of Medicine, 2019, 70, 273-288.	5.0	311
229	Overexpressing kringle 1 domain of hepatocyte growth factor with adeno-associated virus inhibits the pathological retinal neovascularization in an oxygen-induced retinopathy mouse model. Biochemical and Biophysical Research Communications, 2019, 508, 130-137.	1.0	7
230	Adeno-Associated Viral Gene Therapy for Inherited Retinal Disease. Pharmaceutical Research, 2019, 36, 34.	1.7	43
231	Biological Products: Cellular Therapy and FDA Approved Products. Stem Cell Reviews and Reports, 2019, 15, 166-175.	5.6	102
232	Differentially Branched Ester Amine Quadpolymers with Amphiphilic and pH-Sensitive Properties for Efficient Plasmid DNA Delivery. Molecular Pharmaceutics, 2019, 16, 655-668.	2.3	27
233	Top Five Gene Therapy Stories of 2019. Human Gene Therapy, 2019, 30, 1-2.	1.4	2
234	Gene therapy and the adenoâ€associated virus in the treatment of genetic and acquired ophthalmic diseases in humans: Trials, future directions and safety considerations. Clinical and Experimental Ophthalmology, 2019, 47, 521-536.	1.3	47
235	Gene Therapy for Neurologic Disease: A Neurosurgical Review. World Neurosurgery, 2019, 121, 261-273.	0.7	11
236	Cost Utility of Voretigene Neparvovec for Biallelic RPE65-Mediated Inherited Retinal Disease. Value in Health, 2019, 22, 161-167.	0.1	46
237	An AAV Dual Vector Strategy Ameliorates the Stargardt Phenotype in Adult <i>Abca4^{â~lâ~}</i> lbance Adult <i>Abca4^{accepted by Ameliorates the Stargardt Phenotype in Adult<ia>Adult (1900-1900) 100 (</ia>}</i>	1.4	72
238	Retinal Degeneration. Methods in Molecular Biology, 2019, , .	0.4	5
239	Optimized Subretinal Injection Technique for Gene Therapy Approaches. Methods in Molecular Biology, 2019, 1834, 405-412.	0.4	7
240	Choroideremia Gene Therapy Phase 2 Clinical Trial: 24-Month Results. American Journal of Ophthalmology, 2019, 197, 65-73.	1.7	119
241	Non-Viral Delivery To Enable Genome Editing. Trends in Biotechnology, 2019, 37, 281-293.	4.9	86
242	Nanoengineered biomaterials for retinal repair. , 2019, , 215-264.		5
243	Prevalence and long-term monitoring of humoral immunity against adeno-associated virus in Duchenne Muscular Dystrophy patients. Cellular Immunology, 2019, 342, 103780.	1.4	33
244	CRISPR/Cas9 for cancer research and therapy. Seminars in Cancer Biology, 2019, 55, 106-119.	4.3	206

#	Article	IF	Citations
245	CHANGES IN RETINAL SENSITIVITY AFTER GENE THERAPY IN CHOROIDEREMIA. Retina, 2020, 40, 160-168.	1.0	47
246	Cancerâ€Targeting Nanoparticles for Combinatorial Nucleic Acid Delivery. Advanced Materials, 2020, 32, e1901081.	11.1	146
247	Statement of the DOG, the RG, and the BVA on the therapeutic use of voretigene neparvovec (Luxturnaâ,,¢) in ophthalmology. English version. Ophthalmologe, 2020, 117, 16-24.	0.4	8
248	Delivering cellular and gene therapies to patients: solutions for realizing the potential of the next generation of medicine. Gene Therapy, 2020, 27, 537-544.	2.3	55
249	Genetic testing of various eye disorders. , 2020, , 239-258.		0
250	Production and use of adeno-associated virus vectors as tools for cancer immunotherapy. Methods in Enzymology, 2020, 635, 185-203.	0.4	3
251	Non-viral Gene Therapy for Stargardt Disease with ECO/pRHO-ABCA4 Self-Assembled Nanoparticles. Molecular Therapy, 2020, 28, 293-303.	3.7	32
252	Gene Replacement Therapy: A Primer for the Health-system Pharmacist. Journal of Pharmacy Practice, 2020, 33, 846-855.	0.5	28
253	Current Advances in Ophthalmic Technology. Current Practices in Ophthalmology, 2020, , .	0.1	0
254	Neuroprotection in Glaucoma: Towards Clinical Trials and Precision Medicine. Current Eye Research, 2020, 45, 327-338.	0.7	41
255	Gene therapy for inherited retinal diseases. , 2020, , 279-295.		3
256	Healthcare recommendations for Joubert syndrome. American Journal of Medical Genetics, Part A, 2020, 182, 229-249.	0.7	66
257	Long-Term Structural Outcomes of Late-Stage RPE65 Gene Therapy. Molecular Therapy, 2020, 28, 266-278.	3.7	56
258	AAV Vector Immunogenicity in Humans: A Long Journey to Successful Gene Transfer. Molecular Therapy, 2020, 28, 723-746.	3.7	363
259	Chemical modification of the adeno-associated virus capsid to improve gene delivery. Chemical Science, 2020, 11, 1122-1131.	3.7	40
260	The approved gene therapy drugs worldwide: from 1998 to 2019. Biotechnology Advances, 2020, 40, 107502.	6.0	216
261	Inclusion of PF68 Surfactant Improves Stability of rAAV Titer when Passed through a Surgical Device Used in Retinal Gene Therapy. Molecular Therapy - Methods and Clinical Development, 2020, 17, 99-106.	1.8	12
262	Application of Genetic Engineering in Biotherapeutics Development. Journal of Pharmaceutical Innovation, 2020, 15, 232-254.	1.1	4

#	Article	IF	CITATIONS
263	Clinical-grade production and safe delivery of human ESC derived RPE sheets in primates and rodents. Biomaterials, 2020, 230, 119603.	5.7	21
265	Bioengineering strategies for gene delivery. , 2020, , 107-148.		4
266	Genome Editing in Patient iPSCs Corrects the Most Prevalent USH2A Mutations and Reveals Intriguing Mutant mRNA Expression Profiles. Molecular Therapy - Methods and Clinical Development, 2020, 17, 156-173.	1.8	56
267	Adeno-Associated Viral Vectors in Neuroscience Research. Molecular Therapy - Methods and Clinical Development, 2020, 17, 69-82.	1.8	76
268	GPR108 Is a Highly Conserved AAV Entry Factor. Molecular Therapy, 2020, 28, 367-381.	3.7	77
269	Progress in treating inherited retinal diseases: Early subretinal gene therapy clinical trials and candidates for future initiatives. Progress in Retinal and Eye Research, 2020, 77, 100827.	7.3	133
270	RNA-based therapies in animal models of Leber congenital amaurosis causing blindness. Precision Clinical Medicine, 2020, 3, 113-126.	1.3	1
271	Plant Viruses and Bacteriophage-Based Reagents for Diagnosis and Therapy. Annual Review of Virology, 2020, 7, 559-587.	3.0	25
272	Mechanisms of Photoreceptor Death in Retinitis Pigmentosa. Genes, 2020, 11, 1120.	1.0	110
273	Sensing through Non-Sensing Ocular Ion Channels. International Journal of Molecular Sciences, 2020, 21, 6925.	1.8	11
274	Synthetically Engineered Adeno-Associated Virus for Efficient, Safe, and Versatile Gene Therapy Applications. ACS Nano, 2020, 14, 14262-14283.	7.3	33
275	Association of a Novel Intronic Variant in <i>RPGR</i> With Hypomorphic Phenotype of X-Linked Retinitis Pigmentosa. JAMA Ophthalmology, 2020, 138, 1151.	1.4	9
276	Immune responses to retinal gene therapy using adeno-associated viral vectors – Implications for treatment success and safety. Progress in Retinal and Eye Research, 2021, 83, 100915.	7.3	105
277	Gene therapy and gene correction: targets, progress, and challenges for treating human diseases. Gene Therapy, 2022, 29, 3-12.	2.3	53
278	Noise-induced hearing loss and its prevention: current issues in mammalian hearing. Current Opinion in Physiology, 2020, 18, 32-36.	0.9	5
279	Biosafety Practices for In Vivo Viral-Mediated Gene Therapy in the Health Care Setting. Applied Biosafety, 2020, 25, 194-200.	0.2	3
280	Gene therapy: a double-edged sword with great powers. Molecular and Cellular Biochemistry, 2020, 474, 73-81.	1.4	44
281	Assessment of AAV Dual Vector Safety in the <i>Abca4^{â°'/â°'}</i> Mouse Model of Stargardt Disease. Translational Vision Science and Technology, 2020, 9, 20.	1.1	10

#	Article	IF	CITATIONS
282	Content generation for patient-reported outcome measures for retinal degeneration therapeutic trials. Ophthalmic Genetics, 2020, 41, 315-324.	0.5	11
283	SUMOylation Targets Adeno-associated Virus Capsids but Mainly Restricts Transduction by Cellular Mechanisms. Journal of Virology, 2020, 94, .	1.5	7
284	Immunomodulatory Effects of Hydroxychloroquine and Chloroquine in Viral Infections and Their Potential Application in Retinal Gene Therapy. International Journal of Molecular Sciences, 2020, 21, 4972.	1.8	24
285	Targeting of the NRL Pathway as a Therapeutic Strategy to Treat Retinitis Pigmentosa. Journal of Clinical Medicine, 2020, 9, 2224.	1.0	17
287	Emerging Gene Therapy Approaches Under Clinical Investigation for Retinal Degenerative Diseases. Topics in Medicinal Chemistry, 2020, , 257-272.	0.4	0
288	Whole exome sequencing and homozygosity mapping reveals genetic defects in consanguineous Iranian families with inherited retinal dystrophies. Scientific Reports, 2020, 10, 19413.	1.6	9
289	Optogenetic Gene Therapy for the Degenerate Retina: Recent Advances. Frontiers in Neuroscience, 2020, 14, 570909.	1.4	34
290	Genome Editing for CNS Disorders. Frontiers in Neuroscience, 2020, 14, 579062.	1.4	18
291	Biocompatibility of a Conjugated Polymer Retinal Prosthesis in the Domestic Pig. Frontiers in Bioengineering and Biotechnology, 2020, 8, 579141.	2.0	10
292	Neurons under genetic control: What are the next steps towards the treatment of movement disorders?. Computational and Structural Biotechnology Journal, 2020, 18, 3577-3589.	1.9	2
293	Turning genes into medicinesâ€"what have we learned from gene therapy drug development in the past decade?. Nature Communications, 2020, 11, 5821.	5.8	18
294	<p>Voretigene Neparvovec in Retinal Diseases: A Review of the Current Clinical Evidence</p> . Clinical Ophthalmology, 2020, Volume 14, 3855-3869.	0.9	31
295	Adeno-associated virus characterization for cargo discrimination through nanopore responsiveness. Nanoscale, 2020, 12, 23721-23731.	2.8	18
296	EYS is a major gene involved in retinitis pigmentosa in Japan: genetic landscapes revealed by stepwise genetic screening. Scientific Reports, 2020, 10, 20770.	1.6	17
297	Natural history and clinical biomarkers of progression in Xâ€linked retinitis pigmentosa: a systematic review. Acta Ophthalmologica, 2021, 99, 499-510.	0.6	6
298	The cGMP system in normal and degenerating mouse neuroretina: New proteins with cGMP interaction potential identified by a proteomics approach. Journal of Neurochemistry, 2020, 157, 2173-2186.	2.1	9
299	Translational Read-Through Therapy of RPGR Nonsense Mutations. International Journal of Molecular Sciences, 2020, 21, 8418.	1.8	15
300	Of rAAV and Men: From Genetic Neuromuscular Disorder Efficacy and Toxicity Preclinical Studies to Clinical Trials and Back. Journal of Personalized Medicine, 2020, 10, 258.	1.1	17

#	Article	IF	CITATIONS
301	An evidence map of randomised controlled trials evaluating genetic therapies. BMJ Evidence-Based Medicine, 2021, 26, 194-194.	1.7	4
302	Validation of a Vision-Guided Mobility Assessment for <i>RPE65</i> -Associated Retinal Dystrophy. Translational Vision Science and Technology, 2020, 9, 5.	1.1	18
303	AAV Vectored Immunoprophylaxis for Filovirus Infections. Tropical Medicine and Infectious Disease, 2020, 5, 169.	0.9	11
304	Promises and pitfalls of evaluating photoreceptor-based retinal disease with adaptive optics scanning light ophthalmoscopy (AOSLO). Progress in Retinal and Eye Research, 2021, 83, 100920.	7.3	29
305	Overcoming the delivery problem for therapeutic genome editing: Current status and perspective of non-viral methods. Biomaterials, 2020, 258, 120282.	5.7	58
306	Mechanism for enhanced transduction of hematopoietic cells by recombinant adenoâ€associated virus serotype 6 vectors. FASEB Journal, 2020, 34, 12379-12391.	0.2	3
307	Targeting the photoreceptor cilium for the treatment of retinal diseases. Acta Pharmacologica Sinica, 2020, 41, 1410-1415.	2.8	13
308	Antisense oligonucleotide therapeutics in clinical trials for the treatment of inherited retinal diseases. Expert Opinion on Investigational Drugs, 2020, 29, 1163-1170.	1.9	44
309	The Evolution of Gene Therapy in the Treatment of Metabolic Liver Diseases. Genes, 2020, 11, 915.	1.0	3
310	Micro-dystrophin AAV Vectors Made by Transient Transfection and Herpesvirus System Are Equally Potent in Treating mdx Mouse Muscle Disease. Molecular Therapy - Methods and Clinical Development, 2020, 18, 664-678.	1.8	10
311	Inhibitors of metalloprotease, Î ³ -sectretase, protein kinase C and Rho kinase inhibit wild-type adenoviral replication. PLoS ONE, 2020, 15, e0236175.	1.1	1
312	Advancing Clinical Trials for Inherited Retinal Diseases: Recommendations from the Second Monaciano Symposium. Translational Vision Science and Technology, 2020, 9, 2.	1.1	56
313	Development of New Strategies Using Extracellular Vesicles Loaded with Exogenous Nucleic Acid. Pharmaceutics, 2020, 12, 705.	2.0	34
314	Will Airway Gene Therapy for Cystic Fibrosis Improve Lung Function? New Imaging Technologies Can Help Us Find Out. Human Gene Therapy, 2020, 31, 973-984.	1.4	5
315	Treatment-Emergent Adverse Events in Gene Therapy Trials for Inherited Retinal Diseases: A Narrative Review. Ophthalmology and Therapy, 2020, 9, 709-724.	1.0	20
316	Modeling and Rescue of RP2 Retinitis Pigmentosa Using iPSC-Derived Retinal Organoids. Stem Cell Reports, 2020, 15, 67-79.	2.3	109
317	Nanomedicine Revisited: Next Generation Therapies for Brain Cancer. Advanced Therapeutics, 2020, 3, 2000118.	1.6	14
318	Human iPSC Modeling Reveals Mutation-Specific Responses to Gene Therapy in a Genotypically Diverse Dominant Maculopathy. American Journal of Human Genetics, 2020, 107, 278-292.	2.6	35

#	Article	IF	CITATIONS
320	Gene Therapy in Rare Respiratory Diseases: What Have We Learned So Far?. Journal of Clinical Medicine, 2020, 9, 2577.	1.0	15
321	Sight of Action: the Rationale and Evolution of Gene Therapy Approaches to the Treatment of Retinal Diseases. Current Ophthalmology Reports, 2020, 8, 267-280.	0.5	0
322	Focused Update on AAV-Based Gene Therapy Clinical Trials for Inherited Retinal Degeneration. BioDrugs, 2020, 34, 763-781.	2.2	26
323	USH2A-retinopathy: From genetics to therapeutics. Experimental Eye Research, 2020, 201, 108330.	1.2	53
324	Novel 199 base pair NEFH promoter drives expression in retinal ganglion cells. Scientific Reports, 2020, 10, 16515.	1.6	7
325	Limited time window for retinal gene therapy in a preclinical model of ciliopathy. Human Molecular Genetics, 2020, 29, 2337-2352.	1.4	11
326	LONGITUDINAL STUDY OF RPE65-ASSOCIATED INHERITED RETINAL DEGENERATIONS. Retina, 2020, 40, 1812-1828.	1.0	12
327	Voretigene Neparvovec for Treating Inherited Retinal Dystrophies Caused by RPE65 Gene Mutations: An Evidence Review Group Perspective of a NICE Highly Specialised Technology Appraisal. Pharmacoeconomics, 2020, 38, 1309-1318.	1.7	8
329	Clinical Protocols for the Evaluation of Rod Function. Ophthalmologica, 2021, 244, 396-407.	1.0	11
330	Retinal Tropism and Transduction of Adeno-Associated Virus Varies by Serotype and Route of Delivery (Intravitreal, Subretinal, or Suprachoroidal) in Rats. Human Gene Therapy, 2020, 31, 1288-1299.	1.4	28
331	Human Organoids for the Study of Retinal Development and Disease. Annual Review of Vision Science, 2020, 6, 91-114.	2.3	38
332	Localized Photoreceptor Ablation Using Femtosecond Pulses Focused With Adaptive Optics. Translational Vision Science and Technology, 2020, 9, 16.	1.1	8
333	Adeno-Associated Virus Vector Mobilization, Risk Versus Reality. Human Gene Therapy, 2020, 31, 1054-1067.	1.4	7
334	Molecular diagnostic challenges for nonâ€retinal developmental eye disorders in the United Kingdom. American Journal of Medical Genetics, Part C: Seminars in Medical Genetics, 2020, 184, 578-589.	0.7	38
335	Translating CRISPR-Cas Therapeutics: Approaches and Challenges. CRISPR Journal, 2020, 3, 253-275.	1.4	19
336	New Directions in Pulmonary Gene Therapy. Human Gene Therapy, 2020, 31, 921-939.	1.4	10
337	Ophthalmic genetics in South America. American Journal of Medical Genetics, Part C: Seminars in Medical Genetics, 2020, 184, 753-761.	0.7	5
338	The new landscape of retinal gene therapy. American Journal of Medical Genetics, Part C: Seminars in Medical Genetics, 2020, 184, 846-859.	0.7	16

#	Article	IF	CITATIONS
339	Novel Therapeutic Approaches for the Treatment of Retinal Degenerative Diseases: Focus on CRISPR/Cas-Based Gene Editing. Frontiers in Neuroscience, 2020, 14, 838.	1.4	12
340	Genetic testing for inherited retinal degenerations: Triumphs and tribulations. American Journal of Medical Genetics, Part C: Seminars in Medical Genetics, 2020, 184, 571-577.	0.7	10
341	Cost Effectiveness of Voretigene Neparvovec for RPE65-Mediated Inherited Retinal Degeneration in Germany. Translational Vision Science and Technology, 2020, 9, 17.	1.1	16
342	Enhanced genome editing to ameliorate a genetic metabolic liver disease through co-delivery of adeno-associated virus receptor. Science China Life Sciences, 2022, 65, 718-730.	2.3	16
343	Advances in the tools and techniques of vitreoretinal surgery. Expert Review of Ophthalmology, 2020, 15, 331-345.	0.3	1
344	Opportunities and limitations of genetically modified nonhuman primate models for neuroscience research. Proceedings of the National Academy of Sciences of the United States of America, 2020, 117, 24022-24031.	3.3	64
345	Promoter Orientation within an AAV-CRISPR Vector Affects Cas9 Expression and Gene Editing Efficiency. CRISPR Journal, 2020, 3, 276-283.	1.4	8
346	COG5 variants lead to complex early onset retinal degeneration, upregulation of PERK and DNA damage. Scientific Reports, 2020, 10, 21269.	1.6	1
347	Two Decades of Global Progress in Authorized Advanced Therapy Medicinal Products: An Emerging Revolution in Therapeutic Strategies. Frontiers in Cell and Developmental Biology, 2020, 8, 547653.	1.8	44
348	The RUSH2A Study: Best-Corrected Visual Acuity, Full-Field Electroretinography Amplitudes, and Full-Field Stimulus Thresholds at Baseline. Translational Vision Science and Technology, 2020, 9, 9.	1.1	31
349	<p>Voretigene Neparvovec and Gene Therapy for Leber's Congenital Amaurosis: Review of Evidence to Date</p> . The Application of Clinical Genetics, 2020, Volume 13, 179-208.	1.4	16
350	Bilateral visual improvement with unilateral gene therapy injection for Leber hereditary optic neuropathy. Science Translational Medicine, 2020, 12, .	5.8	128
351	A comparison of AAV-vector production methods for gene therapy and preclinical assessment. Scientific Reports, 2020, 10, 21532.	1.6	16
352	Sourcing Photoreceptor-like Cells for Treating Vision Loss. New England Journal of Medicine, 2020, 383, 1888-1890.	13.9	0
353	PKM2 ablation enhanced retinal function and survival in a preclinical model of retinitis pigmentosa. Mammalian Genome, 2020, 31, 77-85.	1.0	9
354	Safety and Vision Outcomes of Subretinal Gene Therapy Targeting Cone Photoreceptors in Achromatopsia. JAMA Ophthalmology, 2020, 138, 643.	1.4	100
355	Precision Therapy for Inherited Retinal Disease. Clinics in Laboratory Medicine, 2020, 40, 189-204.	0.7	3
356	Genetic Basis of Inherited Retinal Disease in a Molecularly Characterized Cohort of More Than 3000 Families from the United Kingdom. Ophthalmology, 2020, 127, 1384-1394.	2.5	131

#	Article	IF	CITATIONS
357	A Small-Molecule-Responsive Riboswitch Enables Conditional Induction of Viral Vector-Mediated Gene Expression in Mice. ACS Synthetic Biology, 2020, 9, 1292-1305.	1.9	33
358	Treatment Potential for <i>LCA5</i> -Associated Leber Congenital Amaurosis., 2020, 61, 30.		11
359	A review of emerging physical transfection methods for CRISPR/Cas9-mediated gene editing. Theranostics, 2020, 10, 5532-5549.	4.6	96
360	Homozygous familial hypercholesterolemia: what treatments are on the horizon?. Current Opinion in Lipidology, 2020, 31, 119-124.	1.2	13
361	Tunneling Nanotubes and the Eye: Intercellular Communication and Implications for Ocular Health and Disease. BioMed Research International, 2020, 2020, 1-15.	0.9	19
362	Volume and Infusion Rate Dynamics of Intraparenchymal Central Nervous System Infusion in a Large Animal Model. Human Gene Therapy, 2020, 31, 617-625.	1.4	5
363	Intraoperative optical coherence tomographic findings in patients undergoing subretinal gene therapy surgery. International Journal of Retina and Vitreous, 2020, 6, 13.	0.9	22
364	Restoring vision to the blind. Science, 2020, 368, 827-828.	6.0	4
365	Comprehensive genomic diagnosis of inherited retinal and optical nerve disorders reveals hidden syndromes and personalized therapeutic options. Acta Ophthalmologica, 2020, 98, e1034-e1048.	0.6	8
366	Corneal Regeneration. Methods in Molecular Biology, 2020, , .	0.4	2
367	Surgical Techniques for the Subretinal Delivery of Pediatric Gene Therapy. Ophthalmology Retina, 2020, 4, 644-645.	1.2	8
368	Targeted Treatments for Inherited Neuromuscular Diseases of Childhood. Seminars in Neurology, 2020, 40, 335-341.	0.5	3
369	Strategies to Modulate MicroRNA Functions for the Treatment of Cancer or Organ Injury. Pharmacological Reviews, 2020, 72, 639-667.	7.1	45
370	Next generation sequencing using phenotype-based panels for genetic testing in inherited retinal diseases. Ophthalmic Genetics, 2020, 41, 331-337.	0.5	16
371	Future Vision 2020 and Beyond—5 Critical Trends in Eye Research. Asia-Pacific Journal of Ophthalmology, 2020, 9, 180-185.	1.3	6
372	Voretigene Neparvovec: A Review in RPE65 Mutation-Associated Inherited Retinal Dystrophy. Molecular Diagnosis and Therapy, 2020, 24, 487-495.	1.6	15
373	Fetal gene therapy and pharmacotherapy to treat congenital hearing loss and vestibular dysfunction. Hearing Research, 2020, 394, 107931.	0.9	16
374	Voretigene neparvovec-rzyl for treatment of <i>RPE65</i> -mediated inherited retinal diseases: a model for ocular gene therapy development. Expert Opinion on Biological Therapy, 2020, 20, 565-578.	1.4	35

#	Article	IF	CITATIONS
375	Phenotypic expansion of autosomal dominant retinitis pigmentosa associated with the D477G mutation in <i>RPE65</i> . Journal of Physical Education and Sports Management, 2020, 6, a004952.	0.5	11
376	Genetic testing for inherited ocular conditions in a developing country. Ophthalmic Genetics, 2020, 41, 36-40.	0.5	7
377	Macular Fold Complicating a Subretinal Injection of Voretigene Neparvovec. Ophthalmology Retina, 2020, 4, 456-458.	1.2	8
378	Emerging approaches for restoration of hearing and vision. Physiological Reviews, 2020, 100, 1467-1525.	13.1	45
379	Congenital blindness and autism spectrum disorder (ASD): diagnostic challenges and intervention options. BMJ Case Reports, 2020, 13, e232981.	0.2	1
380	Disease mechanisms and gene therapy for Usher syndrome. Hearing Research, 2020, 394, 107932.	0.9	50
381	Lessons Learned About Autonomous Al: Finding a Safe, Efficacious, and Ethical Path Through the Development Process. American Journal of Ophthalmology, 2020, 214, 134-142.	1.7	72
382	Intraocular Lens Dislocation into the Anterior Chamber because of Repeated Eye-Poking in a Patient with Leber's Congenital Amaurosis. Case Reports in Ophthalmology, 2020, 11, 48-53.	0.3	3
383	Metabolic and Redox Signaling of the Nucleoredoxin-Like-1 Gene for the Treatment of Genetic Retinal Diseases. International Journal of Molecular Sciences, 2020, 21, 1625.	1.8	20
384	Niosome-Based Approach for In Situ Gene Delivery to Retina and Brain Cortex as Immune-Privileged Tissues. Pharmaceutics, 2020, 12, 198.	2.0	34
385	Silence superoxide dismutase 1 (SOD1): a promising therapeutic target for amyotrophic lateral sclerosis (ALS). Expert Opinion on Therapeutic Targets, 2020, 24, 295-310.	1.5	49
386	Shihu Yeguang Pill protects against bright light-induced photoreceptor degeneration in part through suppressing photoreceptor apoptosis. Biomedicine and Pharmacotherapy, 2020, 126, 110050.	2.5	6
387	Drug Tissue Distribution of TUDCA From a Biodegradable Suprachoroidal Implant versus Intravitreal or Systemic Delivery in the Pig Model. Translational Vision Science and Technology, 2020, 9, 11.	1.1	6
389	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
390	In vivo biodistribution analysis of transmission competent and defective RNA virus-based episomal vector. Scientific Reports, 2020, 10, 5890.	1.6	7
391	Latest Developed Strategies to Minimize the Off-Target Effects in CRISPR-Cas-Mediated Genome Editing. Cells, 2020, 9, 1608.	1.8	257
392	Short-Wavelength and Near-Infrared Autofluorescence in Patients with Deficiencies of the Visual Cycle and Phototransduction. Scientific Reports, 2020, 10, 8998.	1.6	9
393	A Review of Gene, Drug and Cell-Based Therapies for Usher Syndrome. Frontiers in Cellular Neuroscience, 2020, 14, 183.	1.8	18

#	Article	IF	CITATIONS
394	Nucleic acid therapeutics in neurodevelopmental disease. Current Opinion in Genetics and Development, 2020, 65, 112-116.	1.5	7
395	Ready for Repair? Gene Editing Enters the Clinic for the Treatment of Human Disease. Molecular Therapy - Methods and Clinical Development, 2020, 18, 532-557.	1.8	67
396	Improved cell-specificity of adeno-associated viral vectors for medullary thyroid carcinoma using calcitonin gene regulatory elements. PLoS ONE, 2020, 15, e0228005.	1.1	2
397	RPGR-Associated Dystrophies: Clinical, Genetic, and Histopathological Features. International Journal of Molecular Sciences, 2020, 21, 835.	1.8	23
398	Gene therapy for neurodegenerative disorders: advances, insights and prospects. Acta Pharmaceutica Sinica B, 2020, 10, 1347-1359.	5.7	94
399	Clinical development on the frontier: gene therapy for duchenne muscular dystrophy. Expert Opinion on Biological Therapy, 2020, 20, 263-274.	1.4	49
400	Tetramer-Based Enrichment of Preexisting Anti-AAV8 CD8+ T Cells in Human Donors Allows the Detection of a TEMRA Subpopulation. Frontiers in Immunology, 2019, 10, 3110.	2,2	15
401	Patient-reported outcome measures in inherited retinal degeneration gene therapy trials. Ophthalmic Genetics, 2020, 41, 1-6.	0.5	16
402	The special considerations of gene therapy for mitochondrial diseases. Npj Genomic Medicine, 2020, 5, 7.	1.7	35
403	Disease asymmetry and hyperautofluorescent ring shape in retinitis pigmentosa patients. Scientific Reports, 2020, 10, 3364.	1.6	9
404	The effect of human gene therapy for RPE65-associated Leber's congenital amaurosis on visual function: a systematic review and meta-analysis. Orphanet Journal of Rare Diseases, 2020, 15, 49.	1,2	46
405	Initial results from a first-in-human gene therapy trial on X-linked retinitis pigmentosa caused by mutations in RPGR. Nature Medicine, 2020, 26, 354-359.	15.2	208
406	MicroRNAâ€based recombinant AAV vector assembly improves efficiency of suicide gene transfer in a murine model of lymphoma. Cancer Medicine, 2020, 9, 3188-3201.	1.3	4
407	Reimbursement and Payment Models for Therapies With Transformative and Curative Intent., 2020,, 705-745.		1
408	An Economic Evaluation of Voretigene Neparvovec for the Treatment of Biallelic RPE65-Mediated Inherited Retinal Dystrophies in the UK. Advances in Therapy, 2020, 37, 1233-1247.	1.3	25
409	Suprachoroidal and Subretinal Injections of AAV Using Transscleral Microneedles for Retinal Gene Delivery in Nonhuman Primates. Molecular Therapy - Methods and Clinical Development, 2020, 16, 179-191.	1.8	73
410	Late presentation of RPE65 retinopathy in three siblings. Documenta Ophthalmologica, 2020, 140, 289-297.	1.0	10
411	Cytosine and adenine base editing of the brain, liver, retina, heart and skeletal muscle of mice via adeno-associated viruses. Nature Biomedical Engineering, 2020, 4, 97-110.	11.6	293

#	Article	IF	CITATIONS
412	An Annotated Journey through Modern Visual Neuroscience. Journal of Neuroscience, 2020, 40, 44-53.	1.7	6
413	RNA Editing as a Therapeutic Approach for Retinal Gene Therapy Requiring Long Coding Sequences. International Journal of Molecular Sciences, 2020, 21, 777.	1.8	46
414	Gene Therapy for Glaucoma by Ciliary Body Aquaporin 1 Disruption Using CRISPR-Cas9. Molecular Therapy, 2020, 28, 820-829.	3.7	52
415	RPE65 and retinal dystrophy: Report of new and recurrent mutations. Journal of Gene Medicine, 2020, 22, e3154.	1.4	7
416	Retinal Structure in <i>RPE65</i> -Associated Retinal Dystrophy., 2020, 61, 47.		27
417	Use of Virtual Reality Simulation to Identify Vision-Related Disability in Patients With Glaucoma. JAMA Ophthalmology, 2020, 138, 490.	1.4	24
419	The biomedical and bioengineering potential of protein nanocompartments. Biotechnology Advances, 2020, 41, 107547.	6.0	25
420	Optogenetic restoration of retinal ganglion cell activity in the living primate. Nature Communications, 2020, $11,1703$.	5.8	50
421	Monitoring progression of retinitis pigmentosa: current recommendations and recent advances. Expert Opinion on Orphan Drugs, 2020, 8, 67-78.	0.5	28
422	Adeno-Associated Virus and Hematopoietic Stem Cells: The Potential of Adeno-Associated Virus Hematopoietic Stem Cells in Genetic Medicines. Human Gene Therapy, 2020, 31, 542-552.	1.4	6
423	Suprachoroidal Delivery of Viral and Nonviral Gene Therapy for Retinal Diseases. Journal of Ocular Pharmacology and Therapeutics, 2020, 36, 384-392.	0.6	40
424	Genotypes and Phenotypes: A Search for Influential Genes in Diabetic Retinopathy. International Journal of Molecular Sciences, 2020, 21, 2712.	1.8	20
425	In Vivo CRISPR/Cas9-Mediated Genome Editing Mitigates Photoreceptor Degeneration in a Mouse Model of X-Linked Retinitis Pigmentosa., 2020, 61, 31.		27
426	Comparative Analysis of Functional and Structural Decline in Retinitis Pigmentosas. International Journal of Molecular Sciences, 2020, 21, 2730.	1.8	5
427	A novel homozygous c.67C>T variant in retinol binding protein 4 (RBP4) associated with retinitis pigmentosa and childhood acne vulgaris. Ophthalmic Genetics, 2020, 41, 288-292.	0.5	7
428	Neurotrophin gene therapy to promote survival of spiral ganglion neurons after deafness. Hearing Research, 2020, 394, 107955.	0.9	21
429	Correcting visual loss by genetics and prosthetics. Current Opinion in Physiology, 2020, 16, 1-7.	0.9	3
430	Gene Therapy Clinical Trials. , 2020, , 285-301.		3

#	Article	IF	CITATIONS
431	Genome and base editing for genetic hearing loss. Hearing Research, 2020, 394, 107958.	0.9	18
432	Perspectives on Gene Therapy: Choroideremia Represents a Challenging Model for the Treatment of Other Inherited Retinal Degenerations. Translational Vision Science and Technology, 2020, 9, 17.	1.1	11
433	Nr2e3 is a genetic modifier that rescues retinal degeneration and promotes homeostasis in multiple models of retinitis pigmentosa. Gene Therapy, 2021, 28, 223-241.	2.3	39
434	Retinal gene therapy: an eye-opener of the 21st century. Gene Therapy, 2021, 28, 209-216.	2.3	21
435	A primer to gene therapy: Progress, prospects, and problems. Journal of Inherited Metabolic Disease, 2021, 44, 54-71.	1.7	9
436	Moving towards clinical trials for mitochondrial diseases. Journal of Inherited Metabolic Disease, 2021, 44, 22-41.	1.7	45
437	The functional characteristics of optogenetic gene therapy for vision restoration. Cellular and Molecular Life Sciences, 2021, 78, 1597-1613.	2.4	8
438	The road to restore vision with photoreceptor regeneration. Experimental Eye Research, 2021, 202, 108283.	1.2	21
439	The X-linked retinopathies: Physiological insights, pathogenic mechanisms, phenotypic features and novel therapies. Progress in Retinal and Eye Research, 2021, 82, 100898.	7.3	65
440	Pulmonary gene delivery—Realities and possibilities. Experimental Biology and Medicine, 2021, 246, 260-274.	1.1	12
441	Treatments for inherited retinal degenerations are coming to Canada: brief update on a new standard of care for inherited retinal degenerations. Canadian Journal of Ophthalmology, 2021, 56, e34-e35.	0.4	2
442	Pathways and disease-causing alterations in visual chromophore production for vertebrate vision. Journal of Biological Chemistry, 2021, 296, 100072.	1.6	27
443	Regenerative medicine: Current and future hypothetical research directions. Research in Veterinary Science, 2021, 135, 555-556.	0.9	1
444	Molecular diagnosis based on comprehensive genetic testing in 800 Chinese families with nonâ€syndromic inherited retinal dystrophies. Clinical and Experimental Ophthalmology, 2021, 49, 46-59.	1.3	30
445	Current Clinical Applications of InÂVivo Gene Therapy with AAVs. Molecular Therapy, 2021, 29, 464-488.	3.7	380
446	Neuroplasticity of the visual cortex: in sickness and in health. Experimental Neurology, 2021, 335, 113515.	2.0	31
447	The Michigan Retinal Degeneration Questionnaire: A Patient-Reported Outcome Instrument for Inherited Retinal Degenerations. American Journal of Ophthalmology, 2021, 222, 60-68.	1.7	28
448	A NOVEL CASE SERIES OF NMNAT1-ASSOCIATED EARLY-ONSET RETINAL DYSTROPHY: EXTENDING THE PHENOTYPIC SPECTRUM. Retinal Cases and Brief Reports, 2021, 15, 139-144.	0.3	6

#	Article	IF	CITATIONS
449	A Comprehensive Study of the Retinal Phenotype of Rpe65-Deficient Dogs. Cells, 2021, 10, 115.	1.8	2
451	Retinal Surgical Techniques for Gene Therapy. , 2021, , 389-395.		0
452	Viral Vector Technologies and Strategies: Improving on Nature. International Ophthalmology Clinics, 2021, 61, 59-89.	0.3	2
453	CRISPR genome engineering for retinal diseases. Progress in Molecular Biology and Translational Science, 2021, 182, 29-79.	0.9	13
454	Präentionsfalle und Selbstschreck. Karger Kompass Ophthalmologie, 2021, 7, 112-112.	0.0	0
455	Genetic Disease and Therapy. Annual Review of Pathology: Mechanisms of Disease, 2021, 16, 145-166.	9.6	21
456	Spatial and temporal resolution of the photoreceptors rescue dynamics after treatment with voretigene neparvovec. British Journal of Ophthalmology, 2022, 106, 831-838.	2.1	26
457	Gene Therapy for Retinal Diseases. Essentials in Ophthalmology, 2021, , 435-454.	0.0	0
458	The role of small molecules in cell and gene therapy. RSC Medicinal Chemistry, 2021, 12, 330-352.	1.7	3
459	Rewriting CFTR to cure cystic fibrosis. Progress in Molecular Biology and Translational Science, 2021, 182, 185-224.	0.9	8
460	Novel therapeutics in nystagmus: what has the genetics taught us so far?. Therapeutic Advances in Rare Disease, 2021, 2, 263300402199871.	0.3	1
461	Rämliche und zeitliche Auflösung der Wiederherstellungsdynamik der Photorezeptoren nach Behandlung mit Voretigen Neparvovec. Karger Kompass Ophthalmologie, 2021, 7, 154-163.	0.0	0
462	Using the RETeval Device in Healthy Children to Establish Normative Electroretinogram Values. Journal of Pediatric Ophthalmology and Strabismus, 2021, 58, 17-22.	0.3	5
463	Gene therapy in retinal diseases: A review. Indian Journal of Ophthalmology, 2021, 69, 2257.	0.5	22
465	Translation of gene therapies. , 2021, , 683-697.		0
466	Attenuation of Antiviral Immune Response Caused by Perturbation of TRIM25-Mediated RIG-I Activation under Simulated Microgravity. Cell Reports, 2021, 34, 108600.	2.9	11
467	Administration of Ocular Gene Therapy. International Ophthalmology Clinics, 2021, 61, 131-149.	0.3	0
468	Targeted delivery of therapeutic agents to the heart. Nature Reviews Cardiology, 2021, 18, 389-399.	6.1	51

#	Article	IF	CITATIONS
469	Retina., 2021,, 89-114.		0
470	Update on Gene Therapy Clinical Trials for Choroideremia and Potential Experimental Therapies. Medicina (Lithuania), 2021, 57, 64.	0.8	9
471	DNA- and RNA-based Gene Therapies in Ophthalmology. International Ophthalmology Clinics, 2021, 61, 3-16.	0.3	3
472	Value-based Medicine and Gene Therapy. International Ophthalmology Clinics, 2021, 61, 195-215.	0.3	0
473	Inherited Retinal Degenerations in the Pediatric Population. , 2021, , 183-209.		0
475	Gene Therapy for Inherited Bleeding Disorders. Seminars in Thrombosis and Hemostasis, 2021, 47, 161-173.	1.5	11
476	Engineering adeno-associated viral vectors to evade innate immune and inflammatory responses. Science Translational Medicine, 2021, 13, .	5.8	99
477	Methodological Challenges in the Economic Evaluation of a Gene Therapy for RPE65-Mediated Inherited Retinal Disease: The Value of Vision. Pharmacoeconomics, 2021, 39, 383-397.	1.7	11
478	Viral vector platforms within the gene therapy landscape. Signal Transduction and Targeted Therapy, 2021, 6, 53.	7.1	514
479	Frustration and Direct-Coupling Analyses to Predict Formation and Function of Adeno-Associated Virus. Biophysical Journal, 2021, 120, 489-503.	0.2	3
480	Predicting visual acuity in Bietti crystalline dystrophy: evaluation of image parameters. BMC Ophthalmology, 2021, 21, 68.	0.6	6
481	AAV-Mediated CRISPRi and RNAi Based Gene Silencing in Mouse Hippocampal Neurons. Cells, 2021, 10, 324.	1.8	5
482	Future Prospects of Gene Therapy for Friedreich's Ataxia. International Journal of Molecular Sciences, 2021, 22, 1815.	1.8	25
483	Metabolic Syndromes as Important Comorbidities in Patients of Inherited Retinal Degenerations: Experiences from the Nationwide Health Database and a Large Hospital-Based Cohort. International Journal of Environmental Research and Public Health, 2021, 18, 2065.	1.2	1
484	Gene editing technology: Towards precision medicine in inherited retinal diseases. Seminars in Ophthalmology, 2021, 36, 176-184.	0.8	1
485	Enhancement of liver-directed transgene expression at initial and repeat doses of AAV vectors admixed with ImmTOR nanoparticles. Science Advances, 2021, 7, .	4.7	28
486	Deep diversification of an AAV capsid protein by machine learning. Nature Biotechnology, 2021, 39, 691-696.	9.4	165
487	Clinical Perspective: Treating RPE65-Associated Retinal Dystrophy. Molecular Therapy, 2021, 29, 442-463.	3.7	92

#	Article	IF	CITATIONS
488	Whole Locus Sequencing Identifies a Prevalent Founder Deep Intronic RPGRIP1 Pathologic Variant in the French Leber Congenital Amaurosis Cohort. Genes, 2021, 12, 287.	1.0	3
489	Stargardt disease masquerades. Current Opinion in Ophthalmology, 2021, 32, 214-224.	1.3	5
490	Long-Term Liver Expression of an Apolipoprotein A-I Mimetic Peptide Attenuates Interferon-Alpha-Induced Inflammation and Promotes Antiviral Activity. Frontiers in Immunology, 2020, 11, 620283.	2.2	2
491	Influence of Systematic Gaze Patterns in Navigation and Search Tasks with Simulated Retinitis Pigmentosa. Brain Sciences, 2021, 11, 223.	1.1	5
492	Genetic characteristics and epidemiology of inherited retinal degeneration in Taiwan. Npj Genomic Medicine, 2021, 6, 16.	1.7	36
493	Clinical Perspectives and Trends: Microperimetry as a Trial Endpoint in Retinal Disease. Ophthalmologica, 2021, 244, 418-450.	1.0	22
494	Surgical Aspects in Gene Therapy for Inherited Retinal Diseases. Klinische Monatsblatter Fur Augenheilkunde, 2021, 238, 267-271.	0.3	1
495	Quantitative assessment of visual pathway function in blind retinitis pigmentosa patients. Clinical Neurophysiology, 2021, 132, 392-403.	0.7	10
496	Strategies for Treating Inherited Retinal Degeneration With Large Genes That Are Not Amenable to Adeno-Associated Virus–Based Gene Replacement Therapy. JAMA Ophthalmology, 2021, 139, 328.	1.4	0
498	Gene Therapy to the Retina and the Cochlea. Frontiers in Neuroscience, 2021, 15, 652215.	1.4	13
499	Advanced Therapy Medicinal Products for the Eye: Definitions and Regulatory Framework. Pharmaceutics, 2021, 13, 347.	2.0	14
500	Gene Therapy for Inherited Retinal Disorders: Update on Clinical Trials. Klinische Monatsblatter Fur Augenheilkunde, 2021, 238, 272-281.	0.3	9
502	Panelâ€based genetic testing for inherited retinal disease screening 176 genes. Molecular Genetics & Cenomic Medicine, 2021, 9, e1663.	0.6	12
503	Genome-Editing Strategies for Treating Human Retinal Degenerations. Human Gene Therapy, 2021, 32, 247-259.	1.4	23
504	Recent Advances in Preclinical Research Using PAMAM Dendrimers for Cancer Gene Therapy. International Journal of Molecular Sciences, 2021, 22, 2912.	1.8	54
505	Eliminating mesothelioma by AAV-vectored, PD1-based vaccination in the tumor microenvironment. Molecular Therapy - Oncolytics, 2021, 20, 373-386.	2.0	10
506	Decoding gene therapy: Current impact and future considerations for health-system and specialty pharmacy practice. American Journal of Health-System Pharmacy, 2021, 78, 953-961.	0.5	1
507	The complement system in age-related macular degeneration. Cellular and Molecular Life Sciences, 2021, 78, 4487-4505.	2.4	96

#	ARTICLE	IF	Citations
508	Analysis of Pathogenic Variants Correctable With CRISPR Base Editing Among Patients With Recessive Inherited Retinal Degeneration. JAMA Ophthalmology, 2021, 139, 319.	1.4	26
509	Leber congenital amaurosis/early-onset severe retinal dystrophy: current management and clinical trials. British Journal of Ophthalmology, 2022, 106, 445-451.	2.1	35
510	Neurodegeneration, Neuroprotection and Regeneration in the Zebrafish Retina. Cells, 2021, 10, 633.	1.8	21
511	Clinical ophtalmoâ€pharmacology. Looking ahead. British Journal of Clinical Pharmacology, 2021, 87, 3483-3484.	1.1	O
512	Adeno-associated virus capsid assembly is divergent and stochastic. Nature Communications, 2021, 12, 1642.	5.8	99
514	A Virtual Reality Orientation and Mobility Test for Inherited Retinal Degenerations: Testing a Proof-of-Concept After Gene Therapy. Clinical Ophthalmology, 2021, Volume 15, 939-952.	0.9	13
515	The architectural design of CD8+ T cell responses in acute and chronic infection: Parallel structures with divergent fates. Journal of Experimental Medicine, 2021 , 218 , .	4.2	41
516	Gene therapy for inherited retinal diseases: progress and possibilities. Australasian journal of optometry, The, 2021, 104, 444-454.	0.6	53
517	Receptor-ligand supplementation via a self-cleaving 2A peptide–based gene therapy promotes CNS axonal transport with functional recovery. Science Advances, 2021, 7, .	4.7	17
518	Association of No-Cost Genetic Testing Program Implementation and Patient Characteristics With Access to Genetic Testing for Inherited Retinal Degenerations. JAMA Ophthalmology, 2021, 139, 449.	1.4	6
519	Intravitreal quantum dots for retinitis pigmentosa: a first-in-human safety study. Nanomedicine, 2021, 16, 617-626.	1.7	3
520	Treatment and prevention of lipoprotein(a)-mediated cardiovascular disease: the emerging potential of RNA interference therapeutics. Cardiovascular Research, 2022, 118, 1218-1231.	1.8	30
521	Humoral immune responses to <scp>AAV</scp> gene therapy in the ocular compartment. Biological Reviews, 2021, 96, 1616-1644.	4.7	20
522	CRISPR Cas9 based genome editing in inherited retinal dystrophies. Ophthalmic Genetics, 2021, 42, 365-374.	0.5	5
524	An Update on Gene Therapy for Inherited Retinal Dystrophy: Experience in Leber Congenital Amaurosis Clinical Trials. International Journal of Molecular Sciences, 2021, 22, 4534.	1.8	45
525	Perifoveal Chorioretinal Atrophy after Subretinal Voretigene Neparvovec-rzyl for RPE65-Mediated Leber Congenital Amaurosis. Ophthalmology Retina, 2022, 6, 58-64.	1.2	68
526	Suppression-Replacement <i>KCNQ1</i> Gene Therapy for Type 1 Long QT Syndrome. Circulation, 2021, 143, 1411-1425.	1.6	39
528	Immunomodulation in Administration of rAAV: Preclinical and Clinical Adjuvant Pharmacotherapies. Frontiers in Immunology, 2021, 12, 658038.	2.2	31

#	Article	IF	CITATIONS
529	On the Corner of Models and Cure: Gene Editing in Cystic Fibrosis. Frontiers in Pharmacology, 2021, 12, 662110.	1.6	16
530	Genetic Basis of Type IV Collagen Disorders of the Kidney. Clinical Journal of the American Society of Nephrology: CJASN, 2021, 16, 1101-1109.	2.2	29
531	Target 5000: a standardized all-Ireland pathway for the diagnosis and management of inherited retinal degenerations. Orphanet Journal of Rare Diseases, 2021, 16, 200.	1.2	10
532	Development and validation of a visual field cluster in retinitis pigmentosa. Scientific Reports, 2021, 11, 9671.	1.6	3
533	Efficacy and Safety of Intravitreal Gene Therapy for Leber Hereditary Optic Neuropathy Treated within 6 Months of Disease Onset. Ophthalmology, 2021, 128, 649-660.	2.5	87
535	Challenging Safety and Efficacy of Retinal Gene Therapies by Retinogenesis. International Journal of Molecular Sciences, 2021, 22, 5767.	1.8	4
536	Partial recovery of visual function in a blind patient after optogenetic therapy. Nature Medicine, 2021, 27, 1223-1229.	15.2	335
537	Genetics and therapy for pediatric eye diseases. EBioMedicine, 2021, 67, 103360.	2.7	7
538	Choriocapillaris flow loss in center-involving retinitis pigmentosa: a quantitative optical coherence tomography angiography study using a novel classification system. Graefe's Archive for Clinical and Experimental Ophthalmology, 2021, 259, 3235-3242.	1.0	2
540	CRISPR-Based Genome Editing as a New Therapeutic Tool in Retinal Diseases. Molecular Biotechnology, 2021, 63, 768-779.	1.3	9
541	A Systematic Review and Meta-Analyses of Interventional Clinical Trial Studies for Gene Therapies for the Inherited Retinal Degenerations (IRDs). Biomolecules, 2021, 11, 760.	1.8	16
542	The current landscape of nucleic acid therapeutics. Nature Nanotechnology, 2021, 16, 630-643.	15.6	578
543	Cell-Based Delivery Approaches for DNA-Binding Domains into the Central Nervous System. Current Neuropharmacology, 2021, 19, .	1.4	1
544	Novel disease-causing variant in <i>RDH12</i> presenting with autosomal dominant retinitis pigmentosa. British Journal of Ophthalmology, 2022, 106, 1274-1281.	2.1	7
545	Inherited Retinal Degenerations in Portugal: Addressing the Unmet Needs. Acta Medica Portuguesa, 2021, 34, 332-334.	0.2	2
546	Antisense Oligonucleotide Therapy for Ophthalmic Conditions. Seminars in Ophthalmology, 2021, 36, 1-6.	0.8	7
547	Emerging biological therapies for the treatment of age-related macular degeneration. Expert Opinion on Emerging Drugs, 2021, 26, 193-207.	1.0	6
548	Neuroprotective Effect of siRNA Entrapped in Hyaluronic Acid-Coated Lipoplexes by Intravitreal Administration. Pharmaceutics, 2021, 13, 845.	2.0	7

#	ARTICLE	IF	CITATIONS
549	Alginate hydrogel polymers enable efficient delivery of a vascular-targeted AAV vector into aortic tissue. Molecular Therapy - Methods and Clinical Development, 2021, 21, 83-93.	1.8	12
550	Perspectives of people with inherited retinal diseases on ocular gene therapy in Australia: protocol for a national survey. BMJ Open, 2021, 11, e048361.	0.8	8
551	Modeling Cone/Cone–Rod Dystrophy Pathology by AAV-Mediated Overexpression of Mutant CRX Protein in the Mouse Retina. Translational Vision Science and Technology, 2021, 10, 25.	1.1	0
553	ON-bipolar cell gene expression during retinal degeneration: Implications for optogenetic visual restoration. Experimental Eye Research, 2021, 207, 108553.	1.2	15
554	Human gene therapy: A scientometric analysis. Biomedicine and Pharmacotherapy, 2021, 138, 111510.	2.5	7
555	RPE65-associated inherited retinal diseases: consensus recommendations for eligibility to gene therapy. Orphanet Journal of Rare Diseases, 2021, 16, 257.	1.2	24
556	Microfluidic processing of stem cells for autologous cell replacement. Stem Cells Translational Medicine, 2021, 10, 1384-1393.	1.6	6
557	Microscope-Integrated OCT-Guided Volumetric Measurements of Subretinal Blebs Created by a Suprachoroidal Approach. Translational Vision Science and Technology, 2021, 10, 24.	1.1	3
558	Adeno-Associated Virus Vector for Central Nervous System Gene Therapy. Trends in Molecular Medicine, 2021, 27, 524-537.	3.5	33
559	Improved CRISPR genome editing using small highly active and specific engineered RNA-guided nucleases. Nature Communications, 2021, 12, 4219.	5.8	29
560	Functional Dynamics of Deafferented Early Visual Cortex in Glaucoma. Frontiers in Neuroscience, 2021, 15, 653632.	1.4	3
561	Optimized Adeno-Associated Virus Vectors for Efficient Transduction of Human Retinal Organoids. Human Gene Therapy, 2021, 32, 694-706.	1.4	22
562	CRB1-Associated Retinal Dystrophies: A Prospective Natural History Study in Anticipation of Future Clinical Trials. American Journal of Ophthalmology, 2022, 234, 37-48.	1.7	17
563	Genetic counseling practice for inherited eye diseases in an Israeli medical center during the COVID‶9 pandemic. Journal of Genetic Counseling, 2021, 30, 969-973.	0.9	3
564	The electroretinogram in the genomics era: outer retinal disorders. Eye, 2021, 35, 2406-2418.	1.1	11
565	Genetics in Drug Discovery. Trends in Genetics, 2021, 37, 603-605.	2.9	0
566	Manufacturing Challenges and Rational Formulation Development for AAV Viral Vectors. Journal of Pharmaceutical Sciences, 2021, 110, 2609-2624.	1.6	103
567	Gene therapy for Fabry disease: Progress, challenges, and outlooks on gene-editing. Molecular Genetics and Metabolism, 2021, 134, 117-131.	0.5	13

#	Article	IF	CITATIONS
568	Genetics, pathogenesis and therapeutic developments for Usher syndrome type 2. Human Genetics, 2022, 141, 737-758.	1.8	19
570	Functions and Diseases of the Retinal Pigment Epithelium. Frontiers in Pharmacology, 2021, 12, 727870.	1.6	75
572	The Lratâ^'/â^' Rat: CRISPR/Cas9 Construction and Phenotyping of a New Animal Model for Retinitis Pigmentosa. International Journal of Molecular Sciences, 2021, 22, 7234.	1.8	6
574	Deep phenotyping of the Cdhr1 mouse validates its use in pre-clinical studies for human CDHR1-associated retinal degeneration. Experimental Eye Research, 2021, 208, 108603.	1.2	4
575	High-Throughput Sequencing to Identify Mutations Associated with Retinal Dystrophies. Genes, 2021, 12, 1269.	1.0	3
576	Augmentation of CD47/SIRPα signaling protects cones in genetic models of retinal degeneration. JCI Insight, 2021, 6, .	2.3	7
577	Leber's Congenital Amaurosis: Current Concepts of Genotype-Phenotype Correlations. Genes, 2021, 12, 1261.	1.0	23
578	Antisense oligonucleotide-based treatment of retinitis pigmentosa caused by USH2A exon 13 mutations. Molecular Therapy, 2021, 29, 2441-2455.	3.7	7 5
579	Cell Ferroptosis: New Mechanism and New Hope for Retinitis Pigmentosa. Cells, 2021, 10, 2153.	1.8	10
580	Gene therapy reforms photoreceptor structure and restores vision in NPHP5-associated Leber congenital amaurosis. Molecular Therapy, 2021, 29, 2456-2468.	3.7	18
581	Pluripotent stem cell therapy for retinal diseases. Annals of Translational Medicine, 2021, 9, 1279-1279.	0.7	12
582	Cortical visual mapping following ocular gene augmentation therapy for achromatopsia. Journal of Neuroscience, 2021, 41, JN-RM-3222-20.	1.7	7
583	Clinical Presentation and Demographic Distribution of Retinitis Pigmentosa in India and Implications for Potential Treatments: Electronic Medical Records Driven Big Data Analytics: Report I. Seminars in Ophthalmology, 2022, 37, 284-290.	0.8	3
584	The Ocular Gene Delivery Landscape. Biomolecules, 2021, 11, 1135.	1.8	11
585	Gene therapy for inherited retinal diseases. Annals of Translational Medicine, 2021, 9, 1278-1278.	0.7	36
586	Mirtron-mediated RNA knockdown/replacement therapy for the treatment of dominant retinitis pigmentosa. Nature Communications, 2021, 12, 4934.	5.8	18
587	Size Exclusion Chromatography with Dual Wavelength Detection as a Sensitive and Accurate Method for Determining the Empty and Full Capsids of Recombinant Adeno-Associated Viral Vectors. Human Gene Therapy, 2022, 33, 202-212.	1.4	17
588	Therapy Approaches for Stargardt Disease. Biomolecules, 2021, 11, 1179.	1.8	26

#	Article	IF	CITATIONS
589	Long-Term, Noninvasive <i>In Vivo</i> Tracking of Progenitor Cells Using Multimodality Photoacoustic, Optical Coherence Tomography, and Fluorescence Imaging. ACS Nano, 2021, 15, 13289-13306.	7.3	17
590	Zebrafish as a Model to Evaluate a CRISPR/Cas9-Based Exon Excision Approach as a Future Treatment Option for EYS-Associated Retinitis Pigmentosa. International Journal of Molecular Sciences, 2021, 22, 9154.	1.8	6
591	Application of prime editing to the correction of mutations and phenotypes in adult mice with liver and eye diseases. Nature Biomedical Engineering, 2022, 6, 181-194.	11.6	92
592	Adeno-Associated Virus Vector Gene Delivery Elevates Factor I Levels and Downregulates the Complement Alternative Pathway <i>In Vivo</i> . Human Gene Therapy, 2021, 32, 1370-1381.	1.4	7
593	Advantages of ocular regeneration research. Annals of Translational Medicine, 2021, 9, 1269-1269.	0.7	0
594	cGMP-grade human iPSC-derived retinal photoreceptor precursor cells rescue cone photoreceptor damage in non-human primates. Stem Cell Research and Therapy, 2021, 12, 464.	2.4	18
595	An Optimized Treatment Protocol for Subretinal Injections Limits Intravitreal Vector Distribution. Ophthalmology Science, 2021, 1, 100050.	1.0	5
596	Cloud-based genomics pipelines for ophthalmology: reviewed from research to clinical practice. Modeling and Artificial Intelligence in Ophthalmology, 2021, 3, 101-140.	0.1	1
597	Prospects for the diagnosis and gene therapy of inherited retinal dystrophies caused by biallelic mutations in the RPE65 gene. Rossiiskii Oftal'mologicheskii Zhurnal, 2021, 14, 78-82.	0.1	2
598	A Second-Generation (44-Channel) Suprachoroidal Retinal Prosthesis: Interim Clinical Trial Results. Translational Vision Science and Technology, 2021, 10, 12.	1.1	28
599	Safety and Tolerability of the Adeno-Associated Virus Vector, AAV6.2FF, Expressing a Monoclonal Antibody in Murine and Ovine Animal Models. Biomedicines, 2021, 9, 1186.	1.4	7
600	Recent advances in regenerative medicine strategies for cancer treatment. Biomedicine and Pharmacotherapy, 2021, 141, 111875.	2.5	38
601	Treatment Options in Congenital Disorders of Glycosylation. Frontiers in Genetics, 2021, 12, 735348.	1.1	13
602	Mfsd2a overexpression alleviates vascular dysfunction in diabetic retinopathy. Pharmacological Research, 2021, 171, 105755.	3.1	8
603	The development and improvement of ribonucleic acid therapy strategies. Molecular Therapy - Nucleic Acids, 2021, 26, 997-1013.	2.3	11
604	Precision Medicine Trials in Retinal Degenerations. Annual Review of Vision Science, 2021, 7, 851-865.	2.3	6
605	Injection pressure levels for creating blebs during subretinal gene therapy. Gene Therapy, 2022, 29, 601-607.	2.3	4
606	Assessing the Accuracy, Quality, and Readability of Patient Accessible Online Resources Regarding Ocular Gene Therapy and Voretigene Neparvovec. Clinical Ophthalmology, 2021, Volume 15, 3849-3857.	0.9	1

#	Article	IF	CITATIONS
607	Voretigene Neparvovec Gene Therapy in Clinical Practice: Treatment of the First Two Italian Pediatric Patients. Translational Vision Science and Technology, 2021, 10, 11.	1.1	15
608	Nucleic acid delivery for therapeutic applications. Advanced Drug Delivery Reviews, 2021, 178, 113834.	6.6	122
610	Characterizing the cellular immune response to subretinal AAV gene therapy in the murine retina. Molecular Therapy - Methods and Clinical Development, 2021, 22, 52-65.	1.8	16
611	A Stem Cell Journey in Ophthalmology: From the Bench to the Clinic. Stem Cells Translational Medicine, 2021, 10, 1581-1587.	1.6	6
612	Advances in base editing with an emphasis on an AAV-based strategy. Methods, 2021, 194, 56-64.	1.9	1
613	Durability of Voretigene Neparvovec for Biallelic RPE65-Mediated Inherited Retinal Disease. Ophthalmology, 2021, 128, 1460-1468.	2.5	82
614	Technological advancements to study cellular signaling pathways in inherited retinal degenerative diseases. Current Opinion in Pharmacology, 2021, 60, 102-110.	1.7	2
615	Gene therapy strategies for rare monogenic disorders with nuclear or mitochondrial gene mutations. Biomaterials, 2021, 277, 121108.	5.7	6
616	Chimeric Helper-Dependent Adenoviruses Transduce Retinal Ganglion Cells and Mý ller Cells in Human Retinal Explants. Journal of Ocular Pharmacology and Therapeutics, 2021, 37, 575-579.	0.6	5
617	Biodistribution of intravitreal lenadogene nolparvovec gene therapy in nonhuman primates. Molecular Therapy - Methods and Clinical Development, 2021, 23, 307-318.	1.8	20
618	Assessing Photoreceptor Status in Retinal Dystrophies: From High-Resolution Imaging to Functional Vision. American Journal of Ophthalmology, 2021, 230, 12-47.	1.7	19
619	Genome editing in large animal models. Molecular Therapy, 2021, 29, 3140-3152.	3.7	18
620	Comparative structural, biophysical, and receptor binding study of true type and wild type AAV2. Journal of Structural Biology, 2021, 213, 107795.	1.3	3
621	Pediatric Bone Marrow Transplantation. Organ and Tissue Transplantation, 2021, , 577-616.	0.0	0
622	Creating an Ocular Biofactory: Surgical Approaches in Gene Therapy for Acquired Retinal Diseases. Asia-Pacific Journal of Ophthalmology, 2021, 10, 5-11.	1.3	10
623	RPE65 Mutation-associated Inherited Retinal Disease and Gene Therapies. International Ophthalmology Clinics, 2021, 61, 125-132.	0.3	1
624	Suprachoroidal Delivery of Subretinal Gene and Cell Therapy. , 2021, , 141-153.		1
625	Genes and Gene Therapy in Inherited Retinal Disease. International Ophthalmology Clinics, 2021, 61, 3-45.	0.3	2

#	Article	IF	Citations
626	The Complex Clinical and Genetic Landscape of Hereditary Peripheral Neuropathy. Annual Review of Pathology: Mechanisms of Disease, 2021, 16, 487-509.	9.6	2
627	A Newly Developed Web-Based Resource on Genetic Eye Disorders for Users With Visual Impairment (Gene.Vision): Usability Study. Journal of Medical Internet Research, 2021, 23, e19151.	2.1	2
628	Oblique injection depth correction by a two parallel OCT sensor guided handheld SMART injector. Biomedical Optics Express, 2021, 12, 926.	1.5	3
629	Inhibition of GABA _A -ï•receptors induces retina regeneration in zebrafish. Neural Regeneration Research, 2021, 16, 367.	1.6	12
630	From Transcriptomics to Treatment in Inherited Optic Neuropathies. Genes, 2021, 12, 147.	1.0	7
631	Hints for Gentle Submacular Injection in Non-Human Primates Based on Intraoperative OCT Guidance. Translational Vision Science and Technology, 2021, 10, 10.	1.1	10
632	Endpoints for Measuring Efficacy in Clinical Trials for Inherited Retinal Disease. International Ophthalmology Clinics, 2021, 61, 63-78.	0.3	0
633	Choosing Outcome Measures and Assessing Efficacy of Therapeutic Interventions in Inherited Retinal Diseases: The Importance of Natural History Studies. International Ophthalmology Clinics, 2021, 61, 47-61.	0.3	1
634	Preparation and Administration of Adeno-associated Virus Vectors for Corneal Gene Delivery. Methods in Molecular Biology, 2020, 2145, 77-102.	0.4	5
635	Identification and Analysis of Genes Associated with Inherited Retinal Diseases. Methods in Molecular Biology, 2019, 1834, 3-27.	0.4	12
636	RNA-Based Therapeutic Strategies for Inherited Retinal Dystrophies. Advances in Experimental Medicine and Biology, 2019, 1185, 71-77.	0.8	8
637	Emerging Concepts for RNA Therapeutics for Inherited Retinal Disease. Advances in Experimental Medicine and Biology, 2019, 1185, 85-89.	0.8	4
638	Functional Assessment of Vision Restoration. Advances in Experimental Medicine and Biology, 2019, 1185, 145-149.	0.8	5
639	A Mini Review: Moving iPSC-Derived Retinal Subtypes Forward for Clinical Applications for Retinal Degenerative Diseases. Advances in Experimental Medicine and Biology, 2019, 1185, 557-561.	0.8	2
640	Principles of Clinical Trials: Bias and Precision Control. , 2020, , 1-27.		2
641	Gene therapy and gene editing., 2020,, 463-477.		2
642	BMP gene delivery for skeletal tissue regeneration. Bone, 2020, 137, 115449.	1.4	13
643	FDA advisers back gene therapy for rare form of blindness. Nature, 2017, 550, 314-314.	13.7	29

#	Article	IF	Citations
644	Photoreceptor metabolic reprogramming: current understanding and therapeutic implications. Communications Biology, 2021, 4, 245.	2.0	33
645	Effects of deficiency in the RLBP1-encoded visual cycle protein CRALBP on visual dysfunction in humans and mice. Journal of Biological Chemistry, 2020, 295, 6767-6780.	1.6	24
646	Surgical Observations from the First 120 Cases of Subretinal Gene Therapy for Inherited Retinal Degenerations Retina, 2020, Publish Ahead of Print, .	1.0	4
656	Congenital stationary night blindness: an update and review of the disease spectrum in Saudi Arabia. Acta Ophthalmologica, 2021, 99, 581-591.	0.6	9
657	Gene therapy and genome surgery in the retina. Journal of Clinical Investigation, 2018, 128, 2177-2188.	3.9	111
658	Exposure to wild-type AAV drives distinct capsid immunity profiles in humans. Journal of Clinical Investigation, 2018, 128, 5267-5279.	3.9	76
659	Microglia modulation by TGF- \hat{l}^21 protects cones in mouse models of retinal degeneration. Journal of Clinical Investigation, 2020, 130, 4360-4369.	3.9	45
660	<p>Gene, Cell and Antibody-Based Therapies for the Treatment of Age-Related Macular Degeneration</p> . Biologics: Targets and Therapy, 2020, Volume 14, 83-94.	3.0	17
661	Pharmacological Adjuncts to Vitrectomy Surgery. Current Pharmaceutical Design, 2019, 24, 4843-4852.	0.9	2
662	A Review of Gene Therapy Delivery Systems for Intervertebral Disc Degeneration. Current Pharmaceutical Biotechnology, 2020, 21, 194-205.	0.9	24
663	Expression and Purification of Adeno-associated Virus Virus-like Particles in a Baculovirus System and AAVR Ectodomain Constructs in E. coli. Bio-protocol, 2020, 10, e3513.	0.2	9
664	Nanoscale Therapeutic System: Safety Assessment Features. Safety and Risk of Pharmacotherapy, 2019, 7, 127-138.	0.1	5
665	Peripheral Neuropathic Pain: From Experimental Models to Potential Therapeutic Targets in Dorsal Root Ganglion Neurons. Cells, 2020, 9, 2725.	1.8	21
666	Gene Therapy for Progressive Familial Intrahepatic Cholestasis: Current Progress and Future Prospects. International Journal of Molecular Sciences, 2021, 22, 273.	1.8	12
667	The potential of small molecule brain-derived neurotrophic factor: mimetics to treat inherited retinal degeneration. Neural Regeneration Research, 2019, 14, 85.	1.6	2
668	Precision medicine and clinical ophthalmology. Indian Journal of Ophthalmology, 2018, 66, 1389.	0.5	4
669	Structure of the gene therapy vector, adeno-associated virus with its cell receptor, AAVR. ELife, 2019, 8, .	2.8	60
670	KIT ligand protects against both light-induced and genetic photoreceptor degeneration. ELife, 2020, 9, .	2.8	13

#	Article	IF	CITATIONS
671	Evaluation of AAV-DJ vector for retinal gene therapy. PeerJ, 2019, 7, e6317.	0.9	33
672	Accuracy of a deep convolutional neural network in detection of retinitis pigmentosa on ultrawide-field images. PeerJ, 2019, 7, e6900.	0.9	30
673	The detection of trans gene fragments of hEPO in gene doping model mice by Taqman qPCR assay. PeerJ, 2020, 8, e8595.	0.9	10
674	Autonomous Artificial Intelligence Safety and Trust. , 2021, , 55-67.		O
675	Molecular Therapy for Choroideremia: Pre-clinical and Clinical Progress to Date. Molecular Diagnosis and Therapy, 2021, 25, 661-675.	1.6	1
676	Retinal pigment epithelium 65ÂkDa protein (RPE65): An update. Progress in Retinal and Eye Research, 2022, 88, 101013.	7.3	36
677	Gene replacement therapy restores <i>RCBTB1</i> expression and cilium length in patientâ€derived retinal pigment epithelium. Journal of Cellular and Molecular Medicine, 2021, 25, 10020-10027.	1.6	3
678	CRISPR-derived genome editing therapies: Progress from bench to bedside. Molecular Therapy, 2021, 29, 3125-3139.	3.7	14
680	Regenerative Medicine and Angiogenesis; Focused on Cardiovascular Disease. Advanced Pharmaceutical Bulletin, 2021, , .	0.6	2
681	Macrophage Depletion via Clodronate Pretreatment Reduces Transgene Expression from AAV Vectors In Vivo. Viruses, 2021, 13, 2002.	1.5	4
682	Allele-specific gene editing to rescue dominant CRX-associated LCA7 phenotypes in a retinal organoid model. Stem Cell Reports, 2021, 16, 2690-2702.	2.3	28
683	Prenatal Gene Therapy for Metabolic Disorders. Clinical Obstetrics and Gynecology, 2021, 64, 904-916.	0.6	1
684	Mathematics and modeling., 0,, 5-1-5-67.		0
690	Four technologies that could transform the treatment of blindness. Nature, 2019, , .	13.7	8
692	Newer Techniques in Vision Restoration and Rehabilitation. Current Practices in Ophthalmology, 2020, , 133-151.	0.1	1
693	Newer Technologies for Ocular Drug Development and Deployment. Current Practices in Ophthalmology, 2020, , 125-131.	0.1	0
694	Technology in the Making and the Future of Ophthalmology. Current Practices in Ophthalmology, 2020, , 153-160.	0.1	0
698	Qualitative Interviews to Better Understand the Patient Experience and Evaluate Patient-Reported Outcomes (PRO) in RLBP1 Retinitis Pigmentosa (RLBP1 RP). Advances in Therapy, 2020, 37, 2884-2901.	1.3	7

#	Article	IF	CITATIONS
700	Utility of a Genetic Screening Panel in Patients With Suspected Inherited Retinal Dystrophies. Ophthalmic Surgery Lasers and Imaging Retina, 2020, 51, 338-345.	0.4	2
701	Genetic engineering approaches to the development of modern therapeutics. Meditsinskii Akademicheskii Zhurnal, 2020, 20, 49-60.	0.2	0
702	Progress in Gene Editing Tools and Their Potential for Correcting Mutations Underlying Hearing and Vision Loss. Frontiers in Genome Editing, 2021, 3, 737632.	2.7	13
703	Voretigene Neparvovec challenges in clinical practice. Journal Francais D'Ophtalmologie, 2021, 44, 1481-1483.	0.2	0
704	The Next Generation of Molecular and Cellular Therapeutics for Inherited Retinal Disease. International Journal of Molecular Sciences, 2021, 22, 11542.	1.8	7
705	The 2021 National Eye Institute Strategic Plan: Fostering Collaboration in Vision Research and Clinical Care. Optometry and Vision Science, 2021, 98, 1228-1230.	0.6	3
706	The 2021 National Eye Institute Strategic Plan: Driving Innovation in Eye and Vision Research. , 2021, 62, 2.		4
707	Lab-Scale Production of Recombinant Adeno-Associated Viruses (AAV) for Expression of Optogenetic Elements. Methods in Molecular Biology, 2020, 2173, 83-100.	0.4	3
708	Gene Therapy for Monogenic Inherited Disorders: Opportunities and Challenges. Deutsches Ärzteblatt International, 2020, 117, 878-885.	0.6	8
710	Retinal Bioengineering. , 2020, , 581-637.		0
711	Pediatric Bone Marrow Transplantation. Organ and Tissue Transplantation, 2020, , 1-41.	0.0	0
712	Neue Arzneimittel 2019. , 2020, , 43-150.		2
713	Refractive Errors in Childhood. , 2020, , 1-29.		0
714	Retinal Gene Therapy. , 2020, , 487-515.		0
715	Visual Impairment in Infants and Young Children. , 2020, , 1-24.		0
716	Sodium Fluorescein as an Optical Label to Evaluate Subretinal Injection. Retina, 2023, 43, 158-161.	1.0	3
718	The 2021 National Eye Institute Strategic Plan: Eliminating Vision Loss and Improving Quality of Life. Ophthalmology, 2022, 129, 12-14.	2.5	6
720	Genome Surgery and Gene Therapy in Retinal Disorders. Yale Journal of Biology and Medicine, 2017, 90, 523-532.	0.2	9

#	Article	IF	CITATIONS
721	Early onset flecked retinal dystrophy associated with new compound heterozygous variants. Molecular Vision, 2018, 24, 286-296.	1.1	15
722	Seven novel variants expand the spectrum of related Leber congenital amaurosis in the Chinese population. Molecular Vision, 2019, 25, 204-214.	1.1	10
724	Management and treatment of inherited retinal dystrophies. Taiwan Journal of Ophthalmology, 2021, 11, 205-206.	0.3	0
725	Gene Therapy, Diet and Drug Approaches to Treating Inherited Retinal Disease. , 2021, , .		0
726	Expansion of methods of gene editing therapy and analysis of safety and efficacy., 2022, , 155-179.		0
727	Risk Mitigation of Immunogenicity: A Key to Personalized Retinal Gene Therapy. International Journal of Molecular Sciences, 2021, 22, 12818.	1.8	3
728	Methodological Characteristics of Clinical Trials Supporting the Marketing Authorisation of Advanced Therapies in the European Union. Frontiers in Pharmacology, 2021, 12, 773712.	1.6	9
729	Association between the number of visual fields and the accuracy of future prediction in eyes with retinitis pigmentosa. BMJ Open Ophthalmology, 2021, 6, e000900.	0.8	0
730	Prime Editing for Inherited Retinal Diseases. Frontiers in Genome Editing, 2021, 3, 775330.	2.7	17
731	The safety and efficacy of gene therapy treatment for monogenic retinal and optic nerve diseases: A systematic review. Genetics in Medicine, 2022, 24, 521-534.	1.1	20
732	Clinical Features and Natural History in a Cohort of Chinese Patients with RPE65-Associated Inherited Retinal Dystrophy. Journal of Clinical Medicine, 2021, 10, 5229.	1.0	1
733	Detecting Progression of Retinitis Pigmentosa Using the Binomial Pointwise Linear Regression Method. Translational Vision Science and Technology, 2021, 10, 15.	1.1	2
734	First-in-Human Robot-Assisted Subretinal Drug Delivery Under Local Anesthesia. American Journal of Ophthalmology, 2022, 237, 104-113.	1.7	21
735	Care Pathway of RPE65-Related Inherited Retinal Disorders from Early Symptoms to Genetic Counseling: A Multicenter Narrative Medicine Project in Italy. Clinical Ophthalmology, 2021, Volume 15, 4591-4605.	0.9	4
736	Translation of New and Emerging Therapies for Genetic Cardiomyopathies. JACC Basic To Translational Science, 2022, 7, 70-83.	1.9	20
737	Current landscape of clinical development and approval of advanced therapies. Molecular Therapy - Methods and Clinical Development, 2021, 23, 606-618.	1.8	21
738	Latest Advances in Gene Therapy in Management of Cystic Fibrosis Lung Disease, Literature Review. Journal of Biochemical Technology, 2021, 12, 67-70.	0.1	0
739	Knowledge of genetic eye diseases and genetic services and attitudes toward genetic testing and gene therapy. Taiwan Journal of Ophthalmology, 2021, 11, 372.	0.3	6

#	Article	IF	CITATIONS
740	Delivery of nVEGFi using AAV8 for the treatment of neovascular age-related macular degeneration. Molecular Therapy - Methods and Clinical Development, 2022, 24, 210-221.	1.8	5
741	Real-world outcomes of voretigene neparvovec treatment in pediatric patients with RPE65-associated Leber congenital amaurosis. Graefe's Archive for Clinical and Experimental Ophthalmology, 2022, 260, 1543-1550.	1.0	27
742	Review of gene therapies for age-related macular degeneration. Eye, 2022, 36, 303-311.	1.1	38
743	Stem cell transplantation as a progressing treatment for retinitis pigmentosa. Cell and Tissue Research, 2022, 387, 177-205.	1.5	5
744	Retinal degeneration in humanized mice expressing mutant rhodopsin under the control of the endogenous murine promoter. Experimental Eye Research, 2022, 215, 108893.	1,2	2
745	Application of an organotypic ocular perfusion model to assess intravitreal drug distribution in human and animal eyes. Journal of the Royal Society Interface, 2022, 19, 20210734.	1.5	3
746	Gene therapy and treatment trials. , 2022, , 63-66.		0
747	Bioengineering strategies for restoring vision. Nature Biomedical Engineering, 2023, 7, 387-404.	11.6	30
748	Drug delivery systems for RNA therapeutics. Nature Reviews Genetics, 2022, 23, 265-280.	7.7	417
749	Genetic disorders causing non-syndromic retinopathy. , 2022, , 161-265.		0
750	Neurofibromin and suppression of tumorigenesis: beyond the GAP. Oncogene, 2022, 41, 1235-1251.	2.6	13
751	Transforming Glia to Neurons Effectively Treats Temporal Lobe Seizures. Epilepsy Currents, 2022, 22, 130-131.	0.4	1
752	Gene-Based Therapeutics for Inherited Retinal Diseases. Frontiers in Genetics, 2021, 12, 794805.	1.1	24
753	The Role of Vitamin A in Retinal Diseases. International Journal of Molecular Sciences, 2022, 23, 1014.	1.8	30
754	Toward Gene Transfer Nanoparticles as Therapeutics. Advanced Healthcare Materials, 2022, 11, e2102145.	3.9	17
7 55	An in vivo Cell-Based Delivery Platform for Zinc Finger Artificial Transcription Factors in Pre-clinical Animal Models. Frontiers in Molecular Neuroscience, 2021, 14, 789913.	1.4	2
756	Epidemiology of Mutations in the 65-kDa Retinal Pigment Epithelium (RPE65) Gene-Mediated Inherited Retinal Dystrophies: A Systematic Literature Review. Advances in Therapy, 2022, 39, 1179-1198.	1.3	15
757	Vision at the limits: Absolute threshold, visual function, and outcomes in clinical trials. Survey of Ophthalmology, 2022, 67, 1270-1286.	1.7	6

#	Article	IF	Citations
758	Circumventing the packaging limit of AAV-mediated gene replacement therapy for neurological disorders. Expert Opinion on Biological Therapy, 2022, 22, 1163-1176.	1.4	19
7 59	Adeno-Associated Virus Delivery Limitations for Neurological Indications. Human Gene Therapy, 2022, 33, 1-7.	1.4	6
760	Loss-of-function mutations in the co-chaperone protein BAG5 cause dilated cardiomyopathy requiring heart transplantation. Science Translational Medicine, 2022, 14, eabf3274.	5.8	16
761	In vivo Gene Therapy to the Liver and Nervous System: Promises and Challenges. Frontiers in Medicine, 2021, 8, 774618.	1.2	3
762	Development and ex-vivo validation of 36G polyimide cannulas integrating a guiding miniaturized OCT probe for robotic assisted subretinal injections. Biomedical Optics Express, 2022, 13, 850.	1.5	2
764	Economic outcomes of centralized procurements of gene therapy for patients with orphan diseases: inherited retinal dystrophy. Farmakoekonomika, 2022, 14, 451-461.	0.4	1
765	Constructing and evaluating a validity argument for a performance outcome measure for clinical trials: An example using the Multi-luminance Mobility Test. Clinical Trials, 2022, , 174077452110736.	0.7	1
766	Gene Therapy for Rare Neurological Disorders. Clinical Pharmacology and Therapeutics, 2022, 111, 743-757.	2.3	7
767	RPE based gene and cell therapy for inherited retinal diseases: A review. Experimental Eye Research, 2022, 217, 108961.	1.2	4
769	Pharmacological Aspects of Clinically Approved Gene Therapy Drugs and Products., 2022,,.		0
772	GUCY2D-Related Retinal Dystrophy with Autosomal Dominant Inheritanceâ€"A Multicenter Case Series and Review of Reported Data. Genes, 2022, 13, 313.	1.0	4
773	Advances in Ophthalmic Optogenetics: Approaches and Applications. Biomolecules, 2022, 12, 269.	1.8	15
774	Programmed Cell Death and Autophagy in an in vitro Model of Spontaneous Neuroretinal Degeneration. Frontiers in Neuroanatomy, 2022, 16, 812487.	0.9	1
775	<i>RPE65</i> -Associated Retinopathies in the Italian Population: A Longitudinal Natural History Study., 2022, 63, 13.		11
776	Immune Responses to Adeno-Associated Virus-Mediated CRISPR Therapy. Human Gene Therapy, 2021, 32, 1430-1432.	1.4	1
777	Comparative Natural History of Visual Function From Patients With Biallelic Variants in <i>BBS1</i> and <i>BBS10</i> ., 2021, 62, 26.		11
778	Adeno-associated Viral Vectors in the Retina: Delivering Gene Therapy to the Right Destination. International Ophthalmology Clinics, 2022, 62, 215-229.	0.3	0
779	Current applications and future perspective of CRISPR/Cas9 gene editing in cancer. Molecular Cancer, 2022, 21, 57.	7.9	85

#	ARTICLE	IF	CITATIONS
780	Cone photoreceptor preservation with laser photobiomodulation in murine and human retinal dystrophy. Clinical and Translational Medicine, 2022, 12, e673.	1.7	2
781	Deciphering the Retinal Epigenome during Development, Disease and Reprogramming: Advancements, Challenges and Perspectives. Cells, 2022, 11, 806.	1.8	3
782	Patient-derived cellular models of primary ciliopathies. Journal of Medical Genetics, 2022, , jmedgenet-2021-108315.	1.5	5
783	Late-stage rescue of visually guided behavior in the context of a significantly remodeled retinitis pigmentosa mouse model. Cellular and Molecular Life Sciences, 2022, 79, 148.	2.4	3
784	Response to "Comment on the Article: Subretinal Bleb of Voretigene Neparvovec― Asia-Pacific Journal of Ophthalmology, 2023, 12, 338-339.	1.3	0
785	Early disruption of photoreceptor cell architecture and loss of vision in a humanized pig model of usher syndromes. EMBO Molecular Medicine, 2022, 14, e14817.	3.3	14
787	Cellular stress signaling and the unfolded protein response in retinal degeneration: mechanisms and therapeutic implications. Molecular Neurodegeneration, 2022, 17, 25.	4.4	26
788	Viral Vectors in Gene Therapy and Clinical Applications. , 0, , .		0
789	Conversion of the Liver into a Biofactory for DNasel Using Adeno-Associated Virus Vector Gene Transfer Reduces Neutrophil Extracellular Traps in a Model of Systemic Lupus Erythematosus. Human Gene Therapy, 2022, 33, 560-571.	1.4	1
7 90	Benefits and Shortcomings of Laboratory Model Systems in the Development of Genetic Therapies. Klinische Monatsblatter Fur Augenheilkunde, 2022, 239, 263-269.	0.3	O
791	Targeting ON-bipolar cells by AAV gene therapy stably reverses <i>LRIT3</i> -congenital stationary night blindness. Proceedings of the National Academy of Sciences of the United States of America, 2022, 119, e2117038119.	3.3	14
792	The Learning Curve of Murine Subretinal Injection Among Clinically Trained Ophthalmic Surgeons. Translational Vision Science and Technology, 2022, 11, 13.	1.1	3
793	Nanoscale delivery platforms for RNA therapeutics: Challenges and the current state of the art. Med, 2022, 3, 167-187.	2.2	7
794	A decision making algorithm for inherited retinal dystrophies, caused by biallelic mutations in the RPE65 gene, in the clinical practice of an ophthalmologist. Rossiiskii Oftal'mologicheskii Zhurnal, 2022, 15, 113-116.	0.1	0
795	AAV-PHP.eB transduces both the inner and outer retina with high efficacy in mice. Molecular Therapy - Methods and Clinical Development, 2022, 25, 236-249.	1.8	12
796	The genetic and phenotypic landscapes of Usher syndrome: from disease mechanisms to a new classification. Human Genetics, 2022, 141, 709-735.	1.8	33
798	AAV Deployment of Enhancer-Based Expression Constructs In Vivo in Mouse Brain. Journal of Visualized Experiments, 2022, , .	0.2	1
799	Biosafety assessment of delivery systems for clinical nucleic acid therapeutics. Biosafety and Health, 2022, 4, 105-117.	1,2	15

#	Article	IF	Citations
800	Visual Acuity, Retinal Morphology, and Patients' Perceptions after Voretigene Neparovec-rzyl Therapy for RPE65-Associated Retinal Disease. Ophthalmology Retina, 2022, 6, 273-283.	1.2	22
801	A systematic comparison of optogenetic approaches to visual restoration. Molecular Therapy - Methods and Clinical Development, 2022, 25, 111-123.	1.8	13
802	Why medicines work. , 2022, 238, 108175.		1
803	Two-step versus 1-step subretinal injection to compare subretinal drug delivery: a randomised study protocol. BMJ Open, 2021, 11, e049976.	0.8	0
804	Precision Medicine through Next-Generation Sequencing in Inherited Eye Diseases in a Korean Cohort. Genes, 2022, 13, 27.	1.0	11
806	Novel anion exchange membrane chromatography method for the separation of empty and full adenoâ€associated virus. Biotechnology Journal, 2022, 17, e2100219.	1.8	9
807	Potential Applications for Targeted GeneÂTherapy to Protect Against Anthracycline Cardiotoxicity. JACC: CardioOncology, 2021, 3, 650-662.	1.7	9
808	Membranous Bubbles: High-Purity and High-Titer Exosomes as the Potential Solution for Adeno-Associated Viruses to Evade Neutralization?. Human Gene Therapy, 2021, 32, 1427-1429.	1.4	0
810	Gene-Based Therapeutics for Acquired Retinal Disease: Opportunities and Progress. Frontiers in Genetics, 2021, 12, 795010.	1.1	13
811	Gene editing and its applications in biomedicine. Science China Life Sciences, 2022, 65, 660-700.	2.3	20
812	Adeno-Associated Virus Toolkit to Target Diverse Brain Cells. Annual Review of Neuroscience, 2022, 45, 447-469.	5.0	44
813	Subretinal injection in mice to study retinal physiology and disease. Nature Protocols, 2022, 17, 1468-1485.	5 . 5	1
814	Insulin receptor activation by proinsulin preserves synapses and vision in retinitis pigmentosa. Cell Death and Disease, 2022, 13, 383.	2.7	4
815	The first gene therapy for <i>RPE65</i> biallelic dystrophy with voretigene neparvovec-rzyl in Brazil. Ophthalmic Genetics, 2022, 43, 550-554.	0.5	7
820	New frontiers of retinal therapeutic intervention: a critical analysis of novel approaches. Annals of Medicine, 2022, 54, 1067-1080.	1.5	8
821	Management and treatment of inherited retinal dystrophies. Taiwan Journal of Ophthalmology, 2021, 11, 205.	0.3	1
822	Visual Impairment in Infants and Young Children. , 2022, , 6691-6714.		0
823	Refractive Errors in Childhood. , 2022, , 6545-6572.		0

#	Article	IF	CITATIONS
824	The State of Patient-Reported Outcome Measures for Pediatric Patients with Inherited Retinal Disease. Ophthalmology and Therapy, 2022, 11, 1031-1046.	1.0	3
825	Enhanced cGMP Interactor Rap Guanine Exchange Factor 4 (EPAC2) Expression and Activity in Degenerating Photoreceptors: A Neuroprotective Response?. International Journal of Molecular Sciences, 2022, 23, 4619.	1.8	3
826	Inherited Retinal Dystrophy and Quality of Life Questionnaire: a Scoping Review. SN Comprehensive Clinical Medicine, 2022, 4, .	0.3	0
827	<i>ABCA4</i> c.859-25A>G, a Frequent Palestinian Founder Mutation Affecting the Intron 7 Branchpoint, Is Associated With Early-Onset Stargardt Disease., 2022, 63, 20.		3
828	Quantitative single-cell transcriptome-based ranking of engineered AAVs in human retinal explants. Molecular Therapy - Methods and Clinical Development, 2022, 25, 476-489.	1.8	5
829	Retinal Organoids over the Decade., 0, , .		1
830	RDH12 retinopathy: clinical features, biology, genetics and future directions. Ophthalmic Genetics, 2022, 43, 301-306.	0.5	4
831	Metabolism Dysregulation in Retinal Diseases and Related Therapies. Antioxidants, 2022, 11, 942.	2.2	9
832	Retinitis pigmentosa 2 pathogenic mutants degrade through BAG6/HUWE1 complex. Experimental Eye Research, 2022, 220, 109110.	1.2	0
833	BioAdhere: tailor-made bioadhesives for epiretinal visual prostheses. Biomaterials Science, 2022, 10, 3282-3295.	2.6	2
834	Targeting the HDAC6â€Cilium Axis Ameliorates the Pathological Changes Associated with Retinopathy of Prematurity. Advanced Science, 2022, 9, .	5.6	14
835	Gene Therapy for Acquired and Genetic Cholestasis. Biomedicines, 2022, 10, 1238.	1.4	3
836	Restoration of Vision and Retinal Responses After Adeno-Associated Virus–Mediated Optogenetic Therapy in Blind Dogs. Translational Vision Science and Technology, 2022, 11, 24.	1.1	6
838	Targeted Therapeutics for Rare Disorders. , 2024, , 249-271.		1
839	Structural basis for the neurotropic AAV9 and the engineered AAVPHP.eB recognition with cellular receptors. Molecular Therapy - Methods and Clinical Development, 2022, 26, 52-60.	1.8	17
840	Adenoviral vectors for cardiovascular gene therapy applications: a clinical and industry perspective. Journal of Molecular Medicine, 2022, 100, 875-901.	1.7	8
841	Beyond Genetics: The Role of Metabolism in Photoreceptor Survival, Development and Repair. Frontiers in Cell and Developmental Biology, 2022, 10, .	1.8	2
842	Intranasal application of adeno-associated viruses: a systematic review. Translational Research, 2022, 248, 87-110.	2.2	3

#	Article	IF	CITATIONS
843	Ocular Gene Therapy: A Literature Review with Special Focus on Immune and Inflammatory Responses. Clinical Ophthalmology, 0, Volume 16, 1753-1771.	0.9	21
844	AAV-vector based gene therapy for mitochondrial disease: progress and future perspectives. Orphanet Journal of Rare Diseases, 2022, 17, .	1.2	9
845	Genetic therapeutic advancements for Dravet Syndrome. Epilepsy and Behavior, 2022, 132, 108741.	0.9	10
847	Short-Term Outcomes of the First in Vivo Gene Therapy for RPE65-Mediated Retinitis Pigmentosa. Yonsei Medical Journal, 2022, 63, 701.	0.9	8
848	Long-term correction of hemophilia B through CRISPR/Cas9 induced homology-independent targeted integration. Journal of Genetics and Genomics, 2022, 49, 1114-1126.	1.7	6
849	Gene Therapy for Mitochondrial Diseases: Current Status and Future Perspective. Pharmaceutics, 2022, 14, 1287.	2.0	22
850	Emerging therapeutic potential of adeno-associated virus-mediated gene therapy in liver fibrosis. Molecular Therapy - Methods and Clinical Development, 2022, 26, 191-206.	1.8	8
851	Adeno-Associated Virus Serotype 2–hCHM Subretinal Delivery to the Macula in Choroideremia. Ophthalmology, 2022, 129, 1177-1191.	2.5	11
852	The retinal pigmentation pathway in human albinism: Not so black and white. Progress in Retinal and Eye Research, 2022, 91, 101091.	7.3	21
853	Clinical exome sequencing for inherited retinal degenerations at a tertiary care center. Scientific Reports, 2022, 12, .	1.6	5
854	Retinal Tissue Engineering: Regenerative and Drug Delivery Approaches. Current Stem Cell Research and Therapy, 2023, 18, 608-640.	0.6	1
855	Regenerative and restorative medicine for eye disease. Nature Medicine, 2022, 28, 1149-1156.	15.2	34
856	Predicting potentially pathogenic effects of <i>h</i> RPE65 missense mutations: a computational strategy based on molecular dynamics simulations. Journal of Enzyme Inhibition and Medicinal Chemistry, 2022, 37, 1765-1772.	2.5	7
857	Opportunities and challenges of gene therapy for retinitis pigmentosa. Scientia Sinica Vitae, 2022, 52, 1015-1022.	0.1	1
858	Newer therapeutic options for inherited retinal diseases: Gene and cell replacement therapy. Indian Journal of Ophthalmology, 2022, 70, 2316.	0.5	5
859	Systemically targeted cancer immunotherapy and gene delivery using transmorphic particles. EMBO Molecular Medicine, 2022, 14, .	3.3	12
860	Gene Therapy in Orthopaedics: Progress and Challenges in Pre-Clinical Development and Translation. Frontiers in Bioengineering and Biotechnology, 0, 10 , .	2.0	9
861	Inflammation after Voretigene Neparvovec Administration in Patients with RPE65-Related Retinal Dystrophy. Ophthalmology, 2022, 129, 1287-1293.	2.5	17

#	Article	IF	CITATIONS
862	Optogenetics for visual restoration: From proof of principle to translational challenges. Progress in Retinal and Eye Research, 2022, 91, 101089.	7.3	15
863	The Progression of Treatment for Refractory Hypercholesterolemia: Focus on the Prospect of Gene Therapy. Frontiers in Genetics, 0, 13, .	1.1	2
864	Cost-effectiveness of voretigene neparvovec in the treatment of patients with inherited retinal disease with RPE65 mutation in Switzerland. BMC Health Services Research, 2022, 22, .	0.9	2
865	Intravitreal Delivery of rAAV2tYF-CB-hRS1 Vector for Gene Augmentation Therapy in Patients with X-Linked Retinoschisis. Ophthalmology Retina, 2022, 6, 1130-1144.	1.2	18
866	What â€~translating science' can learn from â€~translating languages'. Drug Discovery Today, 2022, , .	3.2	0
867	Subretinal deposits in young patients treated with voretigene neparvovec-rzyl for RPE65-mediated retinal dystrophy. British Journal of Ophthalmology, 2023, 107, 299-301.	2.1	9
868	Cardiac-specific overexpression of Ndufs1 ameliorates cardiac dysfunction after myocardial infarction by alleviating mitochondrial dysfunction and apoptosis. Experimental and Molecular Medicine, 2022, 54, 946-960.	3.2	18
869	Current landscape of geneâ€editing technology in biomedicine: Applications, advantages, challenges, and perspectives. MedComm, 2022, 3, .	3.1	2
870	Therapeutic inÂvivo delivery of gene editing agents. Cell, 2022, 185, 2806-2827.	13.5	131
871	ICG-mediated photodisruption of the inner limiting membrane enhances retinal drug delivery. Journal of Controlled Release, 2022, 349, 315-326.	4.8	11
872	Gene therapy clinical trials, where do we go? An overview. Biomedicine and Pharmacotherapy, 2022, 153, 113324.	2.5	68
873	Allele-Specific Inactivation of an Autosomal Dominant Epidermolysis Bullosa Simplex Mutation Using CRISPR-Cas9. CRISPR Journal, 2022, 5, 586-597.	1.4	4
874	CRISPR DNA Base Editing Strategies for Treating Retinitis Pigmentosa Caused by Mutations in Rhodopsin. Genes, 2022, 13, 1327.	1.0	5
875	Inherited retinal dystrophy: first results of RPE65 gene replacement therapy in Russia. Vestnik Oftalmologii, 2022, 138, 49.	0.1	0
876	Efficient in vivo base editing via single adeno-associated viruses with size-optimized genomes encoding compact adenine base editors. Nature Biomedical Engineering, 2022, 6, 1272-1283.	11.6	70
877	Clinical and Genetic Analysis of <i>RDH12</i> -Associated Retinopathy in 27 Chinese Families: A Hypomorphic Allele Leads to Cone-Rod Dystrophy., 2022, 63, 24.		8
878	Frequency of <i>RPE65</i> Gene Mutation in Patients with Hereditary Retinal Dystrophy. Türk Oftalmoloji Dergisi, 2022, 52, 270-275.	0.4	1
879	Approved gene therapies in Australia: coming to a store near you. Internal Medicine Journal, 2022, 52, 1313-1321.	0.5	3

#	Article	IF	Citations
881	Utility of No-Charge Panel Genetic Testing for Inherited Retinal Diseases in a Real-World Clinical Setting. Journal of Vitreoretinal Diseases, 2022, 6, 351-357.	0.2	1
882	Multiplex viral tropism assay in complex cell populations with single-cell resolution. Gene Therapy, 2022, 29, 555-565.	2.3	1
883	Effective gene therapy of Stargardt disease with PEG-ECO/pGRK1-ABCA4-S/MAR nanoparticles. Molecular Therapy - Nucleic Acids, 2022, 29, 823-835.	2.3	13
884	Harnessing nucleic acid technologies for human health on earth and in space. Life Sciences in Space Research, 2022, 35, 113-126.	1.2	2
885	Subretinal Injection Techniques for Retinal Disease: A Review. Journal of Clinical Medicine, 2022, 11, 4717.	1.0	18
886	Immunogenicity assessment of AAV-based gene therapies: An IQ consortium industry white paper. Molecular Therapy - Methods and Clinical Development, 2022, 26, 471-494.	1.8	20
887	Compound dominant-null heterozygosity in a family with RP1-related retinal dystrophy. American Journal of Ophthalmology Case Reports, 2022, , 101698.	0.4	0
888	Inherited retinal dystrophies. , 2022, , 357-366.		0
889	Principles of Clinical Trials: Bias and Precision Control. , 2022, , 739-765.		0
890	Optogenetic approaches to gene therapy for vision restoration in retinal degenerative diseases. , 2022, , 581-606.		0
891	Microbiological Nanotechnology. Micro/Nano Technologies, 2022, , 1-29.	0.1	0
892	Inherited retinal dystrophy: first results of RPE65 gene replacement therapy in Russia. Vestnik Oftalmologii, 2022, 138, 48.	0.1	2
893	Emerging Gene Manipulation Strategies for the Treatment of Monogenic Eye Disease. Asia-Pacific Journal of Ophthalmology, 2022, 11, 380-391.	1.3	3
894	Pathogenesis and Treatment of Usher Syndrome Type IIA. Asia-Pacific Journal of Ophthalmology, 2022, 11, 369-379.	1.3	5
895	High Brightness, Highly Directional Organic Lightâ€Emitting Diodes as Light Sources for Future Lightâ€Amplifying Prosthetics in the Optogenetic Management of Vision Loss. Advanced Optical Materials, 2023, 11, .	3.6	5
896	MERTK missense variants in three patients with retinitis pigmentosa. Ophthalmic Genetics, 0, , 1-9.	0.5	1
897	Structure-Function Relationship in Patients with Retinitis Pigmentosa and Hyperautofluorescent Rings. Journal of Clinical Medicine, 2022, 11, 5137.	1.0	0
898	Retinoic acid delays initial photoreceptor differentiation and results in a highly structured mature retinal organoid. Stem Cell Research and Therapy, 2022, 13, .	2.4	10

#	Article	IF	Citations
899	Ocular Gene Therapy: A Literature Review With Focus on Current Clinical Trials. Cureus, 2022, , .	0.2	7
900	Retinal gene therapy in RPE-65 gene mediated inherited retinal dystrophy. Eye, 2023, 37, 1874-1877.	1.1	4
901	Immune Responses to Gene Editing by Viral and Non-Viral Delivery Vectors Used in Retinal Gene Therapy. Pharmaceutics, 2022, 14, 1973.	2.0	13
902	Ocular Drug Delivery: Advancements and Innovations. Pharmaceutics, 2022, 14, 1931.	2.0	15
903	Narrative medicine to investigate the quality of life and emotional impact of inherited retinal disorders through the perspectives of patients, caregivers and clinicians: an Italian multicentre project. BMJ Open, 2022, 12, e061080.	0.8	2
904	Gene Therapy for Inherited Retinal Disease: Long-Term Durability of Effect. Ophthalmic Research, 2023, 66, 179-196.	1.0	12
905	Therapeutic Gene Editing in Inherited Retinal Disorders. Cold Spring Harbor Perspectives in Medicine, 0, , a041292.	2.9	2
906	Advancements in ocular gene therapy delivery: vectors and subretinal, intravitreal, and suprachoroidal techniques. Expert Opinion on Biological Therapy, 2022, 22, 1193-1208.	1.4	7
907	Ocular barriers as a double-edged sword: preventing and facilitating drug delivery to the retina. Drug Delivery and Translational Research, 2023, 13, 547-567.	3.0	6
909	Therapy with voretigene neparvovec. How to measure success?. Progress in Retinal and Eye Research, 2023, 92, 101115.	7.3	15
910	Current AAV-mediated gene therapy in sensorineural hearing loss. Fundamental Research, 2022, , .	1.6	5
911	Retinal Pigment Epithelium Atrophy After Subretinal Voretigene Neparvovec-rzyl for RPE65-Related Disease: A 6-Month Follow-Up. Retina, 2022, 42, e55-e56.	1.0	3
912	Trial by "Firsts― Clinical Trial Design and Regulatory Considerations in the Development and Approval of the First AAV Gene Therapy Product in the United States. Cold Spring Harbor Perspectives in Medicine, 0, , a041312.	2.9	2
914	Lessons Learned from the Development of the First FDA-Approved Gene Therapy Drug, Voretigene Neparvovec-rzyl. Cold Spring Harbor Perspectives in Medicine, 2023, 13, a041307.	2.9	6
915	Precision genome editing in the eye. Proceedings of the National Academy of Sciences of the United States of America, 2022, 119 , .	3.3	15
916	A versatile toolkit for overcoming AAV immunity. Frontiers in Immunology, 0, 13, .	2.2	12
917	Medical treatment of patients with hypertrophic cardiomyopathy: An overview of current and emerging therapy. Archives of Cardiovascular Diseases, 2022, 115, 529-537.	0.7	11
919	Disease progression of retinitis pigmentosa caused by <i>PRPF31</i> variants in a Nordic population: a retrospective study with up to 36 years follow-up. Ophthalmic Genetics, 2023, 44, 139-146.	0.5	4

#	Article	IF	CITATIONS
920	Gene Therapy Cargoes Based on Viral Vector Delivery. Current Gene Therapy, 2023, 23, 111-134.	0.9	5
921	Multidisciplinary team directed analysis of whole genome sequencing reveals pathogenic non-coding variants in molecularly undiagnosed inherited retinal dystrophies. Human Molecular Genetics, 2023, 32, 595-607.	1.4	5
922	Non-Viral Delivery of CRISPR/Cas Cargo to the Retina Using Nanoparticles: Current Possibilities, Challenges, and Limitations. Pharmaceutics, 2022, 14, 1842.	2.0	15
923	Gene therapy in hereditary retinal dystrophy. Tzu Chi Medical Journal, 2022, 34, 367.	0.4	2
924	Conducting gene therapy clinical trials based on natural history studies for inherited retinal diseases in Chinese population. Advances in Ophthalmology Practice and Research, 2023, 3, 6-8.	0.3	1
925	Episomes and Transposases—Utilities to Maintain Transgene Expression from Nonviral Vectors. Genes, 2022, 13, 1872.	1.0	0
926	Ageâ€related macular degeneration: A disease of extracellular complement amplification. Immunological Reviews, 2023, 313, 279-297.	2.8	9
927	Base and Prime Editing in the Retina—From Preclinical Research toward Human Clinical Trials. International Journal of Molecular Sciences, 2022, 23, 12375.	1.8	4
928	Advances in Vitreoretinal Surgery. Journal of Clinical Medicine, 2022, 11, 6428.	1.0	8
929	Genetically modified organisms: adapting regulatory frameworks for evolving genome editing technologies. Biological Research, 2022, 55, .	1.5	10
930	In vivo application of base and prime editing to treat inherited retinal diseases. Progress in Retinal and Eye Research, 2023, 94, 101132.	7.3	3
931	Genetic characterization of 1,210 Japanese pedigrees with inherited retinal diseases by wholeâ€exome sequencing. Human Mutation, 0, , .	1.1	9
932	Gene therapy for primary mitochondrial diseases: experimental advances and clinical challenges. Nature Reviews Neurology, 2022, 18, 689-698.	4.9	18
933	Visual function and retinal changes after voretigene neparvovec treatment in children with biallelic RPE65-related inherited retinal dystrophy. Scientific Reports, 2022, 12, .	1.6	10
934	The gap between development and manufacturing in gene therapy: strategic options for overcoming traps. Drug Discovery Today, 2022, , 103429.	3.2	1
935	Prostaglandin-based rAAV-mediated glaucoma gene therapy in Brown Norway rats. Communications Biology, 2022, 5, .	2.0	5
936	Catabolism of the Lipofuscin Cycloretinal by MsP1. Biochemistry, 2022, 61, 2560-2567.	1.2	0
937	Minigene-Based Splice Assays Reveal the Effect of Non-Canonical Splice Site Variants in USH2A. International Journal of Molecular Sciences, 2022, 23, 13343.	1.8	2

#	Article	IF	CITATIONS
938	Identification of a novel large multigene deletion and a frameshift indel in <i>PDE6B</i> as the underlying cause of early onset recessive rod-cone degeneration. Journal of Physical Education and Sports Management, 0, , mcs.a006247.	0.5	0
940	X-Linked Retinitis Pigmentosa Gene Therapy: Preclinical Aspects. Ophthalmology and Therapy, 2023, 12, 7-34.	1.0	4
941	Molecular background of Leber congenital amaurosis in a Polish cohort of patients—novel variants discovered by NGS. Journal of Applied Genetics, 2023, 64, 89-104.	1.0	1
942	Pathogenic gene variants identified in patients with retinitis pigmentosa at the referral center clinic of the University of Minnesota (UMN). Ophthalmic Genetics, 2023, 44, 414-416.	0.5	2
943	Mobility Testing and Other Performance-Based Assessments of Functional Vision in Patients with Inherited Retinal Disease. Cold Spring Harbor Perspectives in Medicine, 0, , a041299.	2.9	0
944	Optogenetic restoration of high sensitivity vision with bReaChES, a red-shifted channelrhodopsin. Scientific Reports, 2022, 12, .	1.6	1
945	Randomized trial of bilateral gene therapy injection for m.11778G>A <i>MT-ND4</i> Leber optic neuropathy. Brain, 2023, 146, 1328-1341.	3.7	20
947	Glaucomatous optic neuropathy: Mitochondrial dynamics, dysfunction and protection in retinal ganglion cells. Progress in Retinal and Eye Research, 2023, 95, 101136.	7.3	24
948	Potential CRISPR Base Editing Therapeutic Options in a Sorsby Fundus Dystrophy Patient. Genes, 2022, 13, 2103.	1.0	2
949	Application of multicolour reflectance imaging for the characterisation of inherited retinal disorders. European Journal of Ophthalmology, 0, , 112067212211388.	0.7	0
950	The future of non-viral gene delivery for the treatment of inherited retinal diseases. Molecular Therapy - Nucleic Acids, 2022, 30, 354.	2.3	2
951	Research trends in the field of retinitis pigmentosa from 2002 to 2021: a 20Âyears bibliometric analysis. International Ophthalmology, 2023, 43, 1825-1833.	0.6	1
952	A sensitive and drug tolerant assay for detecting anti-AAV9 antibodies using affinity capture elution. Journal of Immunological Methods, 2023, 512, 113397.	0.6	3
953	Intravitreal gene therapy preserves retinal function in a canine model of CLN2 neuronal ceroid lipofuscinosis. Experimental Eye Research, 2023, 226, 109344.	1.2	2
954	Visual function restoration in a mouse model of Leber congenital amaurosis via therapeutic base editing. Molecular Therapy - Nucleic Acids, 2023, 31, 16-27.	2.3	14
955	Gene Therapy in Combination with Nitrogen Scavenger Pretreatment Corrects Biochemical and Behavioral Abnormalities of Infant Citrullinemia Type 1 Mice. International Journal of Molecular Sciences, 2022, 23, 14940.	1.8	1
956	Systemic AAV9.BVES delivery ameliorates muscular dystrophy in a mouse model of LGMDR25. Molecular Therapy, 2022, , .	3.7	1
957	In Vivo Hematopoietic Stem Cell Genome Editing: Perspectives and Limitations. Genes, 2022, 13, 2222.	1.0	6

#	Article	IF	CITATIONS
960	Szemészeti javallat alapján végzett génterápiás kezelés RPE65 biallelikus génmutáció okozta ör ideghártya-dystrophiában Orvosi Hetilap, 2022, 163, 1923-1931.	öklődő	0
961	Recent Advances in Lipid Nanoparticles for Delivery of mRNA. Pharmaceutics, 2022, 14, 2682.	2.0	22
962	Safety of Lenadogene Nolparvovec Gene Therapy Over 5 Years in 189 Patients With Leber Hereditary Optic Neuropathy. American Journal of Ophthalmology, 2023, 249, 108-125.	1.7	4
963	Potential therapeutic strategies for photoreceptor degeneration: the path to restore vision. Journal of Translational Medicine, 2022, 20, .	1.8	5
964	Neuroplasticity of the Lateral Geniculate Nucleus in Response to Retinal Gene Therapy in a Group of Patients with RPE65 Mutations. Eye and Brain, 0, Volume 14, 137-147.	3.8	1
965	Cellular and subcellular optogenetic approaches towards neuroprotection and vision restoration. Progress in Retinal and Eye Research, 2022, , 101153.	7.3	1
966	Progressive accumulation of cytoplasmic aggregates in PRPF31 retinal pigment epithelium cells interferes with cell survival. Clinical and Translational Discovery, 2022, 2, .	0.2	0
967	AAV2/4-RS1 gene therapy in the retinoschisin knockout mouse model of X-linked retinoschisis. PLoS ONE, 2022, 17, e0276298.	1.1	6
969	Longitudinal analysis of health care costs in patients with childhood onset inherited retinal dystrophies compared to healthy controls. BMC Ophthalmology, 2022, 22, .	0.6	2
970	Nanoparticles-mediated CRISPR-Cas9 gene therapy in inherited retinal diseases: applications, challenges, and emerging opportunities. Journal of Nanobiotechnology, 2022, 20, .	4.2	10
972	Towards a Smart Bionic Eye: Al-powered artificial vision for the treatment of incurable blindness. Journal of Neural Engineering, 2022, 19, 063001.	1.8	11
973	Subretinal gene therapy delays vision loss in a Bardet-Biedl Syndrome type 10 mouse model. Molecular Therapy - Nucleic Acids, 2023, 31, 164-181.	2.3	8
974	Multi-luminance Mobility Testing Endpoint. Methods in Molecular Biology, 2023, , 175-179.	0.4	O
975	Evading and overcoming AAV neutralization in gene therapy. Trends in Biotechnology, 2023, 41, 836-845.	4.9	16
976	Perceptions of airway gene therapy for cystic fibrosis. Expert Opinion on Biological Therapy, 2023, 23, 103-113.	1.4	3
977	Endpoints for clinical trials in ophthalmology. Progress in Retinal and Eye Research, 2023, 97, 101160.	7.3	10
978	Inherited retinal disorders: a genotype–phenotype correlation in an Indian cohort and the importance of genetic testing and genetic counselling. Graefe's Archive for Clinical and Experimental Ophthalmology, 2023, 261, 2003-2017.	1.0	1
979	Current Management of Inherited Retinal Degeneration Patients in Europe: Results of a 2-Year Follow-Up Multinational Survey by the European Vision Institute Clinical Research Network – EVICR.net. Ophthalmic Research, 2023, , 550-568.	1.0	3

#	Article	IF	CITATIONS
980	The influence of stimulating electrode conditions on electrically evoked potentials and resistance in suprachoroidal transretinal stimulation. Japanese Journal of Ophthalmology, 0, , .	0.9	0
981	The Role of Striatal Cav1.3 Calcium Channels in Therapeutics for Parkinson's Disease. Handbook of Experimental Pharmacology, 2023, , .	0.9	1
982	Improving adeno-associated viral (AAV) vector-mediated transgene expression in retinal ganglion cells: comparison of five promoters. Gene Therapy, 2023, 30, 503-519.	2.3	14
983	Peptide-guided lipid nanoparticles deliver mRNA to the neural retina of rodents and nonhuman primates. Science Advances, 2023, 9, .	4.7	52
984	Correlation between the Serum Concentration of Vitamin A and Disease Severity in Patients Carrying p.G90D in RHO, the Most Frequent Gene Associated with Dominant Retinitis Pigmentosa: Implications for Therapy with Vitamin A. International Journal of Molecular Sciences, 2023, 24, 780.	1.8	2
985	Microbiological Nanotechnology. Micro/Nano Technologies, 2023, , 525-553.	0.1	0
986	Pluripotent stem cell-derived retinal organoid/cells for retinal regeneration therapies: A review. Regenerative Therapy, 2023, 22, 59-67.	1.4	9
987	Gene Therapy with Voretigene Neparvovec Improves Vision and Partially Restores Electrophysiological Function in Pre-School Children with Leber Congenital Amaurosis. Biomedicines, 2023, 11, 103.	1.4	8
988	High-Level rAAV Vector Production by rAdV-Mediated Amplification of Small Amounts of Input Vector. Viruses, 2023, 15, 64.	1.5	2
989	Type I Interferon Signaling Is Critical During the Innate Immune Response to HSV-1 Retinal Infection. , 2022, 63, 28.		4
990	Secreted phosphoprotein 1 slows neurodegeneration and rescues visual function in mouse models of aging and glaucoma. Cell Reports, 2022, 41, 111880 .	2.9	12
991	Intravenous AAV9 administration results in safe and widespread distribution of transgene in the brain of mini-pig. Frontiers in Cell and Developmental Biology, $0,10,10$	1.8	2
992	The Importance of Natural History Studies in Inherited Retinal Diseases. Cold Spring Harbor Perspectives in Medicine, 0, , a041297.	2.9	0
993	Therapeutic landscape for inherited ocular diseases: Current and emerging therapies. Singapore Medical Journal, 2023, 64, 17.	0.3	6
994	A New Classification for Retinitis Pigmentosa Including Multifocal Electroretinography to Evaluate the Disease Severity. Open Journal of Ophthalmology, 2023, 13, 37-47.	0.1	0
995	Modulating the activity of human nociceptors with a SCN10A promoter-specific viral vector tool. Neurobiology of Pain (Cambridge, Mass), 2023, 13, 100120.	1.0	2
996	From genetic variation to precision medicine. , 2023, 1, .		2
997	Genomics in Treatment Development. Advances in Neurobiology, 2023, , 363-385.	1.3	0

#	Article	IF	CITATIONS
998	Extra-viral DNA in adeno-associated viral vector preparations induces TLR9-dependent innate immune responses in human plasmacytoid dendritic cells. Scientific Reports, 2023, 13, .	1.6	4
999	In Vivo Efficacy and Safety Evaluations of Therapeutic Splicing Correction Using U1 snRNA in the Mouse Retina. Cells, 2023, 12, 955.	1.8	1
1000	Genome editing, a superior therapy for inherited retinal diseases. Vision Research, 2023, 206, 108192.	0.7	10
1001	Static Perimetry in the Rate of Progression in USH2A-related Retinal Degeneration (RUSH2A) Study: Assessment Through 2 Years. American Journal of Ophthalmology, 2023, 250, 103-110.	1.7	1
1002	Immunosuppression reduces rAAV2.5T neutralizing antibodies that limit efficacy following repeat dosing to ferret lungs. Molecular Therapy - Methods and Clinical Development, 2023, 29, 70-80.	1.8	5
1003	î²-cyclodextrin based nano gene delivery using pharmaceutical applications to treat Wolfram syndrome. Therapeutic Delivery, 2022, 13, 449-462.	1.2	2
1008	Cost-effective sequence analysis of 113 genes in $1,192$ probands with retinitis pigmentosa and Leber congenital amaurosis. Frontiers in Cell and Developmental Biology, $0,11,.$	1.8	4
1011	Genetic Modifiers of Non-Penetrance and RNA Expression Levels in PRPF31-Associated Retinitis Pigmentosa in a Danish Cohort. Genes, 2023, 14, 435.	1.0	1
1012	Vision rescue via unconstrained in vivo prime editing in degenerating neural retinas. Journal of Experimental Medicine, 2023, 220, .	4.2	24
1013	The first genetic landscape of inherited retinal dystrophies in Portuguese patients identifies recurrent homozygous mutations as a frequent cause of pathogenesis. , 2023, 2, .		4
1014	Roles of biomaterials in modulating the innate immune response in ocular therapy. Frontiers in Drug Delivery, 0, 3, .	0.4	0
1015	The Degree of Adeno-Associated Virus-Induced Retinal Inflammation Varies Based on Serotype and Route of Delivery: Intravitreal, Subretinal, or Suprachoroidal. Human Gene Therapy, 2023, 34, 530-539.	1.4	5
1016	Immunogenicity of CRISPR therapeutics $\hat{a} \in \text{``Critical considerations for clinical translation.}$ Frontiers in Bioengineering and Biotechnology, 0, 11, .	2.0	9
1018	Retinitis Pigmentosa: Novel Therapeutic Targets and Drug Development. Pharmaceutics, 2023, 15, 685.	2.0	18
1019	Full-field Scotopic Threshold Improvement after Voretigene Neparvovec-rzyl Treatment Correlates with Chorioretinal Atrophy. Ophthalmology, 2023, 130, 764-770.	2.5	13
1021	A Summary on Tuberculosis Vaccine Developmentâ€"Where to Go?. Journal of Personalized Medicine, 2023, 13, 408.	1.1	8
1022	Targeting the liver to treat the eye. EMBO Molecular Medicine, 2023, 15, .	3.3	2
1023	Characterizing the genotypic spectrum of retinitis pigmentosa in East Asian populations: a systematic review. Ophthalmic Genetics, 2023, 44, 109-118.	0.5	1

#	Article	IF	CITATIONS
1024	Various AAV Serotypes and Their Applications in Gene Therapy: An Overview. Cells, 2023, 12, 785.	1.8	35
1025	Polyunsaturated Lipids in the Light-Exposed and Prooxidant Retinal Environment. Antioxidants, 2023, 12, 617.	2.2	1
1026	The evolution of comprehensive genetic analysis in neurology: Implications for precision medicine. Journal of the Neurological Sciences, 2023, 447, 120609.	0.3	3
1027	Therapeutic applications of <scp>CRISPR</scp> /Cas9 gene editing technology for the treatment of ocular diseases. FEBS Journal, 2023, 290, 5248-5269.	2.2	1
1028	Current and future concepts for the generation and application of genetically engineered CAR-T and TCR-T cells. Frontiers in Immunology, $0,14,.$	2.2	13
1029	Current Management of Patients with RPE65 Mutation Associated Inherited Retinal Degenerations in Europe: Results of a 2-Year Follow-Up Multinational Survey. Ophthalmic Research, 2023, , 727-748.	1.0	1
1030	Comment on "Retinitis Pigmentosa and Molar Tooth Sign Caused by Novel AHI1 Compound Heterozygote Pathogenic Variants: A Case Report― , 2023, 2, 1-2.		0
1031	Viral Vectors in Gene Therapy: Where Do We Stand in 2023?. Viruses, 2023, 15, 698.	1.5	20
1033	Assessment of Pre-Clinical Liver Models Based on Their Ability to Predict the Liver-Tropism of Adeno-Associated Virus Vectors. Human Gene Therapy, 2023, 34, 273-288.	1.4	8
1034	The Potential Revolution of Cancer Treatment with CRISPR Technology. Cancers, 2023, 15, 1813.	1.7	7
1035	Voretigene Neparvovec for the Treatment of RPE65-associated Retinal Dystrophy: Consensus and Recommendations from the Korea RPE65-IRD Consensus Paper Committee. Korean Journal of Ophthalmology: KJO, 2023, 37, 166-186.	0.5	1
1036	Biallelic CLCN2 mutations cause retinal degeneration by impairing retinal pigment epithelium phagocytosis and chloride channel function. Human Genetics, 2023, 142, 577-593.	1.8	4
1037	Pathogenic Variants in CEP290 or IQCB1 Cause Earlier-Onset Retinopathy in Senior-Loken Syndrome Compared to Those in INVS, NPHP3, or NPHP4. American Journal of Ophthalmology, 2023, 252, 188-204.	1.7	3
1038	Computer-Aided Directed Evolution Generates Novel AAV Variants with High Transduction Efficiency. Viruses, 2023, 15, 848.	1.5	1
1039	Treatments for diabetic retinopathy and diabetic macular edema in pipeline., 2024,, 215-263.		0
1040	Ocular stress enhances contralateral transfer of lenadogene nolparvovec gene therapy through astrocyte networks. Molecular Therapy, 2023, 31, 2005-2013.	3.7	2
1041	Early Alterations of RNA Binding Protein (RBP) Homeostasis and ER Stress-Mediated Autophagy Contributes to Progressive Retinal Degeneration in the rd10 Mouse Model of Retinitis Pigmentosa (RP). Cells, 2023, 12, 1094.	1.8	5
1042	Immunologic Ocular Disease. , 2023, , 943-958.		0

#	Article	IF	CITATIONS
1043	Improving cell and gene therapy safety and performance using next-generation Nanoplasmid vectors. Molecular Therapy - Nucleic Acids, 2023, 32, 494-503.	2.3	3
1044	Gene-agnostic approaches to treating inherited retinal degenerations. Frontiers in Cell and Developmental Biology, 0, 11 , .	1.8	3
1045	Development of a translatable gene augmentation therapy for CNGB1-retinitis pigmentosa. Molecular Therapy, 2023, 31, 2028-2041.	3.7	1
1046	Improved Rod Sensitivity as Assessed by Two-Color Dark-Adapted Perimetry in Patients With <i>RPE65</i> -Related Retinopathy Treated With Voretigene Neparvovec-rzyl. Translational Vision Science and Technology, 2023, 12, 17.	1.1	5
1047	CAR-Treg cell therapies and their future potential in treating ocular autoimmune conditions. Frontiers in Ophthalmology, 0, 3, .	0.2	0
1048	rAAV-PHP.B escapes the mouse eye and causes lethality whereas rAAV9 can transduce aniridic corneal limbal stem cells without lethality. Gene Therapy, 0, , .	2.3	0
1049	OPA1 Dominant Optic Atrophy: Pathogenesis and Therapeutic Targets. Journal of Neuro-Ophthalmology, 2023, 43, 464-474.	0.4	0
1050	Adaptive Optics Flood Illumination Ophthalmoscopy in Nonhuman Primates. Ophthalmology Science, 2023, 3, 100316.	1.0	0
1051	Stem cells and genetic engineering empower therapeutic development for blinding eye diseases. , 2023, , 139-170.		0
1081	Surgical Anatomy of Pediatric Eyes: Differences from Adults. , 2023, , 21-35.		0
1085	Definitive Treatments for Chronic Granulomatous Disease with a Focus on Gene Therapy., 2023,, 557-572.		0
1121	New Avenues of Delivery (Subretinal Gene Therapy, Port Delivery, Suprachoroidal). Current Practices in Ophthalmology, 2023, , 339-351.	0.1	0
1129	Drug delivery systems for CRISPR-based genome editors. Nature Reviews Drug Discovery, 2023, 22, 875-894.	21.5	9
1132	Commercialization of regenerative-medicine therapies. , 0, , .		1
1133	Genome editing in the treatment of ocular diseases. Experimental and Molecular Medicine, 2023, 55, 1678-1690.	3.2	3
1144	Bacterial therapies at the interface of synthetic biology and nanomedicine. , 2024, 2, 120-135.		6
1146	AAV-Based Strategies for Treatment of Retinal and Choroidal Vascular Diseases: Advances in Age-Related Macular Degeneration and Diabetic Retinopathy Therapies. BioDrugs, 0, , .	2.2	0
1158	Genetisch determinierte Netzhautdystrophien. Springer Reference Medizin, 2023, , 1-8.	0.0	0

CITATION REPORT

#	Article	IF	CITATIONS
1168	Looking to the Future: Drug Delivery and Targeting in the Prophylaxis and Therapy of Severe and Chronic Diseases. Handbook of Experimental Pharmacology, 2023, , .	0.9	0
1170	Evaluating the Quantity and Quality of Health Economic Literature in Blinding Childhood Disorders: A Systematic Literature Review. Pharmacoeconomics, 2024, 42, 275-299.	1.7	0
1211	Genetic and Genomic Results and Management. , 2024, , 93-110.		0
1224	Non-viral delivery of nucleic acid for treatment of rare diseases of the muscle. Journal of Biosciences, 2024, 49, .	0.5	1
1227	Molecular Genetic Therapies in the Muscular Dystrophies. Current Clinical Neurology, 2023, , 281-302.	0.1	0
1243	Fractal Phototherapy in Maximizing Retina and Brain Plasticity. Advances in Neurobiology, 2024, , 585-637.	1.3	0