

Table S1: Clinical trials categorized based on specific retinal diseases as reported in clinicaltrials.gov

NCT Number	Study Title	Acronym	Study Status	Conditions	Interventions	Phases	Start Date	Completion Date
1	Usher Syndrome NCT02065011 A Study to Determine the Long-Term Safety, Tolerability and Biological Activity of SAR421869 in Patients With Usher Syndrome Type 1B		ACTIVE_NOT_RECRUITING	Usher's Syndrome	DRUG: Blood draw for the laboratory assessment	PHASE1 PHASE2	12-09-2013	13-06-2031
2	Usher Syndrome and RP NCT05176717 Study to Evaluate the Efficacy Safety and Tolerability of QR-421a in Subjects With RP Due to Mutations in Exon 13 of the USH2A Gene With Early to Moderate Vision Loss (Celeste)		TERMINATED	Retinitis Pigmentosa Usher Syndrome Type 2 Deaf Blind Retinal Disease Eye Diseases Eye Diseases, Hereditary Eye Disorders Congenital Vision Disorders	DRUG: QR-421a OTHER: Sham-procedure	PHASE2 PHASE3	15-12-2021	02-08-2022
3	NCT05085964 An Open-Label Extension Study to Evaluate Safety & Tolerability of QR-421a in Subjects With Retinitis Pigmentosa	HELIA	TERMINATED	Retinitis Pigmentosa Usher Syndrome Type 2	DRUG: RNA antisense oligonucleotide for intravitreal injection	PHASE2	16-09-2021	18-10-2022
4	NCT03780257 Study to Evaluate Safety and Tolerability of QR-421a in Subjects With RP Due to Mutations in Exon 13 of the USH2A Gene	Stellar	COMPLETED	Retinitis Pigmentosa Usher Syndrome Type 2 Deaf Blind Retinal Disease Eye Diseases Eye Diseases, Hereditary Eye Disorders Congenital Vision Disorders	DRUG: QR-421a OTHER: Sham-procedure (dose cohort 1&2 only)	PHASE1 PHASE2	06-03-2019	14-10-2021
5	NCT05158296 Study to Evaluate the Efficacy Safety and Tolerability of Ulteversen in Subjects With RP Due to Mutations in Exon 13 of the USH2A Gene (Sirius)		ACTIVE_NOT_RECRUITING	Retinitis Pigmentosa Usher Syndrome Type 2 Deaf Blind Retinal Disease Eye Diseases Eye Diseases, Hereditary Eye Disorders Congenital Vision Disorders	DRUG: Ulteversen OTHER: Sham-procedure	PHASE2 PHASE3	08-12-2021	2024-12
6	NCT01505062 Study of SAR421869 in Participants With Retinitis Pigmentosa Associated With Usher Syndrome Type 1B		TERMINATED	Usher Syndrome Retinitis Pigmentosa	DRUG: SAR421869	PHASE1 PHASE2	26-03-2012	16-08-2019
1	RP NCT04945772 Efficacy and Safety of MCO-010 Optogenetic Therapy in Adults With Retinitis Pigmentosa [RESTORE]	RESTORE	ACTIVE_NOT_RECRUITING	Retinitis Pigmentosa Retinitis Retinal Diseases Eye Diseases Eye Diseases, Hereditary Retinal Dystrophies Retinal Degeneration	BIOLOGICAL: Gene Therapy Product: MCO-010 PROCEDURE: Sham Injection	PHASE2	13-07-2021	01-03-2024
2	NCT04611503 PDE6A Gene Therapy for Retinitis Pigmentosa	Pigment	ACTIVE_NOT_RECRUITING	Retinitis Pigmentosa	DRUG: subretinal injection of rAAV.hPDE6A	PHASE1 PHASE2	24-09-2019	2027-07

Contid...

3	NCT05203939	Study to Assess the Safety and Efficacy of OC400 for Retinitis Pigmentosa and Leber Congenital Amaurosis		RECRUITING	Retinitis Pigmentosa Leber Congenital Amaurosis	DRUG: OC400 Low Dose DRUG: OC400 Med Dose DRUG: OC400 High Dose	PHASE1 PHASE2	24-01-2022	2024-02
4	NCT05805007	Safety and Tolerability Study of Gene Editing Drug ZVS203e in Participants With Retinitis Pigmentosa		NOT_YET_RECRUITING	Retinitis Pigmentosa	DRUG: ZVS203e	EARLY_PHASE1	2023-06	2026-04
5	NCT03252847	Gene Therapy for X-linked Retinitis Pigmentosa (XLRP) - Retinitis Pigmentosa GTPase Regulator (RPGR)		COMPLETED	X-Linked Retinitis Pigmentosa	GENETIC: AAV2/J5-RPGR	PHASE1 PHASE2	14-07-2017	18-11-2021
6	NCT02416622	Safety and Efficacy of rAAV-hRS1 in Patients With X-linked Retinoschisis (XLRs)		COMPLETED	X-linked Retinoschisis	BIOLOGICAL: rAAV21YF-CB-hRS1	PHASE1 PHASE2	2015-05	09-05-2023
7	NCT03328130	Safety and Efficacy Study in Patients With Retinitis Pigmentosa Due to Mutations in PDE6B Gene		RECRUITING	Retinitis Pigmentosa	BIOLOGICAL: AAV2/J5-hPDE6B	PHASE1 PHASE2	06-11-2017	2029-12
8	NCT04278131	BS01 in Patients With Retinitis Pigmentosa		RECRUITING	Retinitis Pigmentosa	DRUG: BS01	PHASE1 PHASE2	06-02-2020	30-12-2029
9	NCT05921162	A Long-Term Follow-Up Study in Subjects Who Received VMCO-1 Administered Via Intravitreal Injection	EXTEND	ENROLLING_BY_INVITATION	Retinitis Pigmentosa Retinal Disease Retinal Degeneration	BIOLOGICAL: Gene Therapy product:VMCO-1		14-07-2023	2024-12
10	NCT05748873	Promising Rod-cone Dystrophy Gene therapy	PRODIGY	RECRUITING	Retinitis Pigmentosa	DRUG: SPVN06	PHASE1 PHASE2	12-04-2023	2029-03
11	NCT04312672	Long Term Follow-Up Gene Therapy Study for XLRP RPGR		RECRUITING	X-Linked Retinitis Pigmentosa	BIOLOGICAL: AAV-RPGR		18-02-2019	2023-06
12	NCT03316560	Safety and Efficacy of rAAV21YF-GRK1-RPGR in Subjects With X-linked Retinitis Pigmentosa Caused by RPGR Mutations		RECRUITING	X-Linked Retinitis Pigmentosa	BIOLOGICAL: rAAV21YF-GRK1-RPGR	PHASE1 PHASE2	16-04-2018	2026-08
13	NCT05874310	Gene Therapy for Subjects With RPGR Mutation-associated X-linked Retinitis Pigmentosa		RECRUITING	X-Linked Retinitis Pigmentosa	GENETIC: FT-002	EARLY_PHASE1	01-02-2023	01-11-2027
14	NCT04919473	Dose-Escalation Study to Evaluate the Safety and Tolerability of Intravitreal VMCO-1 in Patients With Advanced Retinitis Pigmentosa		COMPLETED	Retinitis Pigmentosa Retinal Diseases Retinal Degeneration	BIOLOGICAL: Gene Therapy product:VMCO-1	PHASE1 PHASE2	23-10-2019	31-10-2020
15	NCT04850118	A Clinical Trial Evaluating the Safety and Efficacy of a Single Subretinal Injection of AGTC-501 in Participants With X-linked Retinitis Pigmentosa Caused by RPGR Mutations		NOT_YET_RECRUITING	X-Linked Retinitis Pigmentosa	BIOLOGICAL: rAAV21YF-GRK1-hRPGRco	PHASE2 PHASE3	2021-08	2029-03

Contd...

16	NCT03326336	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects With Retinitis Pigmentosa	PIONEER	RECRUITING	Non-syndromic Retinitis Pigmentosa	COMBINATION_PRODUCT: Gene therapy: GS030-DP AND Medical device: GS030-MD	PHASE1 PHASE2	26-09-2018	2025-12
17	NCT03116113	A Clinical Trial of Retinal Gene Therapy for X-linked Retinitis Pigmentosa Using B1B112	XIRIUS	COMPLETED	X-Linked Retinitis Pigmentosa	BIOLOGICAL: B1B112	PHASE1 PHASE2	16-03-2017	18-11-2020
18	NCT02556736	RST-001 Phase I/II Trial for Advanced Retinitis Pigmentosa		ACTIVE_NOT_RECRUITING	Advanced Retinitis Pigmentosa	DRUG: RST-001	PHASE1 PHASE2	14-12-2015	02-09-2024
19	NCT04794101	Follow-up Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene		RECRUITING	X-Linked Retinitis Pigmentosa	BIOLOGICAL: Genetic: AAV5-RPGR 4e11 BIOLOGICAL: Genetic: AAV5-RPGR 2e11	PHASE3	16-03-2021	2029-05
20	NCT03374657	A First-in-human, Proof of Concept Study of CPK850 in Patients With RLPB1 Retinitis Pigmentosa		ACTIVE_NOT_RECRUITING	Retinitis Pigmentosa	BIOLOGICAL: CPK850	PHASE1 PHASE2	22-08-2018	11-05-2026
21	NCT01482195	Trial of Subretinal Injection of (rAAV2-VMD2-hMERTK)		COMPLETED	Retinal Disease Retinitis Pigmentosa	BIOLOGICAL: Subretinal administration of rAAV2-VMD2-hMERTK recombinant Adeno-Associated Virus	PHASE1	2011-08	2019-08
22	NCT05926583	A Study of AAV5-hRkP.RPGR for the Treatment of Japanese Participants With X-linked Retinitis Pigmentosa		RECRUITING	X-Linked Retinitis Pigmentosa	GENETIC: AAV5-hRkP.RPGR GENE TIC: AAV5-hRkP.RPGR	PHASE3	21-08-2023	10-05-2029
	RP/LCA								
23	NCT05858983	Gene Therapy in Subjects With Biallelic RPE65 Mutation-associated Retinal Dystrophy		RECRUITING	Biallelic RPE65 Mutation-associated Retinal Dystrophy	GENETIC: FT-001 Low Dose GENETIC: FT-001 Mid Dose GENETIC: FT-001 High Dose	PHASE1 PHASE2	30-11-2022	30-11-2029
24	NCT03602820	Long-term Follow-up Study in Subjects Who Received Voretigene Neparvovec-rzy1 (AAV2-hRPE65v2)		ACTIVE_NOT_RECRUITING	Inherited Retinal Dystrophy Due to RPE65 Mutations	BIOLOGICAL: AAV2-hRPE65v2		2015-06	2030-06
25	NCT04516369	Study of Efficacy and Safety of Voretigene Neparvovec in Japanese Patients With Biallelic RPE65 Mutation-associated Retinal Dystrophy		ACTIVE_NOT_RECRUITING	Biallelic RPE65 Mutation-associated Retinal Dystrophy	GENETIC: voretigene neparvovec	PHASE3	24-11-2020	31-05-2026
26	NCT03597399	A Patient Registry Study for Patients Treated With Voretigene Neparvovec in US		ACTIVE_NOT_RECRUITING	Confirmed Biallelic RPE65 Mutation-associated Retinal Dystrophy	BIOLOGICAL: AAV2-hRPE65v2, voretigene neparvovec-rzy1		10-01-2019	2025-06
	LCA								

Contd...

27	NCT03913130	Extension Study to Study PQ-110-001 (NCT03140969)	INSIGHT	TERMINATED	Leber Congenital Amaurosis 10 Blindness Leber Congenital Amaurosis Vision Disorders Sensation Disorders Neurologic Manifestations Eye Diseases Eye Diseases, Hereditary Eye Disorders Congenital Retinal Disease	DRUG: QR-110	PHASE1 PHASE2	13-05-2019	03-10-2022
28	NCT02946879	Long-Term Follow-Up Gene Therapy Study for Leber Congenital Amaurosis OPTIRPE65 (Retinal Dystrophy Associated With Defects in RPE65)		COMPLETED	Leber Congenital Amaurosis (LCA) Eye Diseases Eye Diseases, Hereditary Retinal Diseases	BIOLOGICAL: AAV OPTIRPE65		2016-11	16-06-2023
29	NCT03872479	Single Ascending Dose Study in Participants With LCA10		ACTIVE_NOT_RECRUITING	Leber Congenital Amaurosis 10 Inherited Retinal Dystrophies Eye Diseases, Hereditary Retinal Disease Retinal Degeneration Vision Disorders Eye Disorders Congenital	DRUG: EDIT-101	PHASE1 PHASE2	26-09-2019	23-05-2025
30	NCT03913143	A Study to Evaluate Efficacy, Safety, Tolerability and Exposure After a Repeat-dose of Sepofarsen (QR-110) in LCA10 (ILLUMINATE)	ILLUMINATE	ACTIVE_NOT_RECRUITING	Leber Congenital Amaurosis 10 Blindness Leber Congenital Amaurosis Vision Disorders Sensation Disorders Neurologic Manifestations Eye Diseases Eye Diseases, Hereditary Eye Disorders Congenital Retinal Disease	DRUG: sepfarsen OTHER: Sham	PHASE2 PHASE3	04-04-2019	2023-03
31	NCT00643747	Safety Study of RPE65 Gene Therapy to Treat Leber Congenital Amaurosis		COMPLETED	Retinal Degeneration	BIOLOGICAL: tgAAAG76 (rAAV 2/2.hrPE65p.hrPE65)	PHASE1 PHASE2	2007-01	2014-12
32	NCT01208389	Phase 1 Follow-on Study of AAV2-hRPE65v2 Vector in Subjects With Leber Congenital Amaurosis (LCA) 2		ACTIVE_NOT_RECRUITING	Leber Congenital Amaurosis	BIOLOGICAL: voretigene neparvovect-ryyl	PHASE1 PHASE2	2010-11	2030-06
33	NCT00481546	Phase I Trial of Gene Vector to Patients With Retinal Disease Due to RPE65 Mutations	LCA	ACTIVE_NOT_RECRUITING	Amaurosis of Leber Retinal Diseases	GENETIC: rAAV2-CBSB-hRPE65	PHASE1	2007-07	2026-06
34	NCT05906953	A Safety and Efficacy Study of HG004 in Subjects With Leber Congenital Amaurosis		NOT_YET_RECRUITING	Leber Congenital Amaurosis	DRUG: HG004	PHASE1 PHASE2	2023-09	2025-12
35	NCT00999609	Safety and Efficacy Study in Subjects With Leber Congenital Amaurosis		ACTIVE_NOT_RECRUITING	Inherited Retinal Dystrophy Due to RPE65 Mutations Leber Congenital Amaurosis	BIOLOGICAL: AAV2-hRPE65v2,voretigene neparvovect-ryyl	PHASE3	2012-10	2029-07

Contd...

36	NCT05616793	Safety and Tolerability Subretinal OPX-001 for LCA5-Associated Inherited Retinal Degeneration (LCA5-IRD)		RECRUITING	LCA5	BIOLOGICAL: AAV8.hLCA5	PHASE1 PHASE2	15-06-2023	30-09-2027
37	NCT04855045	An Open-label, Dose Escalation and Double-masked, Randomized, Controlled Trial Evaluating Safety and Tolerability of Sepofarsen in Children (<8 Years of Age) With LCA10 Caused by Mutations in the CEP290 Gene.	BRIGHTEN	RECRUITING	Leber Congenital Amaurosis 10 Blindness Leber Congenital Amaurosis Vision Disorders Sensation Disorders Neurologic Manifestations Eye Diseases Eye Disorders Congenital Retinal Disease Retinal Degeneration Retinal Dystrophies	DRUG: sepofarsen	PHASE2 PHASE3	23-03-2021	2023-12
38	NCT00821340	Clinical Trial of Gene Therapy for Leber Congenital Amaurosis Caused by RPE65 Mutations		COMPLETED	Leber Congenital Amaurosis	GENETIC: rAAV2-hRPE65	PHASE1	01-02-2009	01-01-2017
39	NCT00749957	Phase 1/2 Safety and Efficacy Study of AAV-RPE65 Vector to Treat Leber Congenital Amaurosis		COMPLETED	Leber Congenital Amaurosis	BIOLOGICAL: rAAV2-CB-hRPE65	PHASE1 PHASE2	17-06-2009	22-09-2017
40	NCT03140969	Study to Evaluate QR-110 in Leber's Congenital Amaurosis (LCA) Due to the c.2991+1655A>G Mutation (p.Cys998X) in the CEP290 Gene		COMPLETED	Leber's Congenital Amaurosis	DRUG: QR-110	PHASE1 PHASE2	16-10-2017	02-10-2019
41	NCT00516477	Safety Study in Subjects With Leber Congenital Amaurosis		COMPLETED	Leber Congenital Amaurosis	BIOLOGICAL: voretigene neparvovec-rzyl	PHASE1	2007-09	20-03-2018
42	NCT02781480	Clinical Trial of Gene Therapy for the Treatment of Leber Congenital Amaurosis (LCA)	OPTIRPE65	COMPLETED	Leber Congenital Amaurosis	BIOLOGICAL: AAV RPE65	PHASE1 PHASE2	2016-04	2018-12
43	NCT01496040	Clinical Gene Therapy Protocol for the Treatment of Retinal Dystrophy Caused by Defects in RPE65	RPE65	COMPLETED	Leber Congenital Amaurosis	DRUG: rAAV2/4-hRPE65	PHASE1 PHASE2	2011-09	2014-08
44	nAMD/Wet AMD NCT05986864	Phase I/II Study to Evaluate the Safety, Preliminary Efficacy, Immunogenicity, and Pharmacokinetics of SKG0106 Intraocular Solution in Patients With Neovascular Age-related Macular Degeneration (nAMD)		NOT_YET_RECRUITING	Neovascular Age-related Macular Degeneration	GENETIC: SKG0106	PHASE1 PHASE2	30-10-2023	30-09-2029
45	NCT03585556	AAVCAGsCD59 for the Treatment of Wet AMD		COMPLETED	Wet Age-related Macular Degeneration	DRUG: Intravitreal anti-VEGF BIOLOGICAL: Intravitreal AAVCAGsCD59 DRUG: Oral prednisolone	PHASE1	13-09-2018	18-01-2022

46	NCT04704921	Pivotal 1 Study of RGX-314 Gene Therapy in Participants With nAMD	ATMOSPHERE	RECRUITING	AMD nAMD Wet Age-related Macular Degeneration wAMD Wet AMD CNV	GENETIC: RGX-314 GENETIC: RGX-314 BIOLOGICAL: Ranibizumab (LUCENTIS®)	PHASE2 PHASE3	29-12-2020	2026-05
47	NCT04645212	Long-term Study of ADVM-022 in Neovascular (Wet) AMD [OPTIC-EXT]		ACTIVE_NOT_RECRUITING	Wet Age-related Macular Degeneration Neovascular Age-related Macular Degeneration	BIOLOGICAL: ADVM-022		14-12-2020	2025-06
48	NCT05536973	Safety and Efficacy of ADVM-022 in Treatment-Experienced Patients With Neovascular Age-related Macular Degeneration [LUNA]		RECRUITING	Neovascular Age-related Macular Degeneration	GENETIC: ADVM-022 GENETIC: ADVM-022	PHASE2	23-08-2022	2024-02
49	NCT04514653	RGX-314 Gene Therapy Administered in the Suprachoroidal Space for Participants With Neovascular Age-Related Macular Degeneration (nAMD)	AAVIATE	RECRUITING	Neovascular Age-Related Macular Degeneration (nAMD)	GENETIC: RGX-314 Dose 1 GENETIC: RGX-314 Dose 2 BIOLOGICAL: Ranibizumab GENETIC: RGX-314 Dose 3 DRUG: Local steroid DRUG: Topical steroid	PHASE2	25-08-2020	2024-01
50	NCT05099094	VEGFA-targeting Gene Therapy to Treat Retinal and Choroidal Neovascularization Diseases		RECRUITING	Neovascular Age-related Macular Degeneration Diabetic Macular Edema Retinal Vein Occlusion	GENETIC: BD311	EARLY_PHASE1	25-11-2021	2023-09
51	NCT06031727	CRISPR/Cas13-mediated RNA Targeting Therapy for the Treatment of nAMD Investigator-Initiated Trial (SIGHT-I)		NOT_YET_RECRUITING	Neovascular Age-related Macular Degeneration(nAMD)	GENETIC: HG202	EARLY_PHASE1	30-09-2023	27-06-2025
52	NCT01494805	Safety and Efficacy Study of rAAV.sFlt-1 in Patients With Exudative Age-Related Macular Degeneration	AMD	COMPLETED	Macular Degeneration Age-related Maculopathies Age-related Maculopathy Maculopathies, Age-related Retinal Degeneration Retinal Neovascularization Eye Diseases	BIOLOGICAL: rAAV.sFlt-1 BIOLOGICAL: rAAV.sFlt-1 OTHER: Control (ranibizumab alone)	PHASE1 PHASE2	2011-12	2017-08
53	NCT05903794	A Study of EXG102-031 in Patients With wAMD (Everest)		RECRUITING	Neovascular (Wet) Age-related Macular Degeneration (nAMD)	BIOLOGICAL: EXG102-031	PHASE1	24-07-2023	31-12-2025
54	NCT05407636	Pivotal 2 Study of RGX-314 Gene Therapy in Participants With nAMD	ASCENT	RECRUITING	AMD nAMD Wet Age-related Macular Degeneration wAMD WetAMD CNV	GENETIC: RGX-314 Dose 1 GENETIC: RGX-314 Dose 2 BIOLOGICAL: Aflibercept (EYLEA®)	PHASE3	28-12-2021	2025-12

Contd...

55	NCT01024998	Safety and Tolerability Study of AAV2-sFLT01 in Patients With Neovascular Age-Related Macular Degeneration (AMD)		COMPLETED	Macular Degeneration Age-Related Maculopathies Age-Related Maculopathy Maculopathies, Age-Related Maculopathy, Age-Related Retinal Degeneration Retinal Neovascularization Gene Therapy Therapy, Gene Eye Diseases	BIOLOGICAL: AAV2-sFLT01 BIOLOGICAL: AAV2-sFLT01 BIOLOGICAL: AAV2-sFLT01 BIOLOGICAL: AAV2-sFLT01	PHASE1	11-01-2010	2018-07
56	NCT05611424	Gene Therapy for Wet AMD		NOT_YET_RECRUITING	Neovascular Age-related Macular Degeneration	GENETIC: FT-003	PHASE1	01-05-2023	30-12-2027
57	NCT05197270	4D-150 in Patients With Neovascular (Wet) Age-Related Macular Degeneration		RECRUITING	Neovascular (Wet) Age-Related Macular Degeneration	BIOLOGICAL: 4D-150 IVT BIOLOGICAL: Aflibercept IVT	PHASE1 PHASE2	09-12-2021	2025-11
58	NCT03999801	Long-term Follow-Up Study of RGX-314 and Fellow Eye Substudy	RGX-314 SRLTFU	ENROLLING_BY_INVITATION	Neovascular Age-related Macular Degeneration Wet Macular Degeneration	GENETIC: RGX-314	PHASE2	31-05-2019	2028-12
59	NCT03748784	ADVM-022 Intravitreal Gene Therapy for Wet AMD	OPTIC	COMPLETED	Wet Age-related Macular Degeneration Neovascular Age-related Macular Degeneration	BIOLOGICAL: ADVM-022	PHASE1	14-11-2018	22-06-2022
60	NCT03066258	Safety and Tolerability of RGX-314 (Investigational Product) Gene Therapy for Neovascular AMD Trial		COMPLETED	Neovascular Age-related Macular Degeneration Wet Age-related Macular Degeneration	GENETIC: RGX-314	PHASE1 PHASE2	29-03-2017	17-06-2021
61	NCT05657301	Safety and Tolerability of KH631 Gene Therapy in Participants With Neovascular Age-related Macular Degeneration		RECRUITING	Age-Related Macular Degeneration	DRUG: KH631	PHASE1	2023-08	2027-09
62	NCT04832724	RGX-314 Gene Therapy Pharmacodynamic Study for Neovascular Age-related Macular Degeneration (nAMD)		ACTIVE_NOT_RECRUITING	Neovascular Age-related Macular Degeneration Wet Macular Degeneration Wet Age-related Macular Degeneration	GENETIC: RGX-314	PHASE2	22-02-2021	18-03-2024
63	NCT06022744	An Exploratory Clinical Trial Evaluating LX109 Gene Therapy in Patients With nAMD		NOT_YET_RECRUITING	To Evaluate the Safety and Tolerability of Intravitreal Injection of LX109 in Patients With nAMD	DRUG: LX109	NA	01-09-2023	30-09-2027
64	NCT02671539	THOR - Tubingen Choroideremia Gene Therapy Trial	THOR	COMPLETED	Choroideremia	GENETIC: rAAV2.REP1	PHASE2	2016-01	2018-02
65	NCT03507686	A Safety Study of Retinal Gene Therapy for Choroideremia With Administration of BiIB111	GEMINI	COMPLETED	Choroideremia	DRUG: BiIB111	PHASE2	29-11-2017	29-06-2022

Contd...

66	NCT02341807	Safety and Dose Escalation Study of AAV2-hCHM in Subjects With CHM (Choroideremia) Gene Mutations		COMPLETED	Choroideremia CHM (Choroideremia) Gene Mutations	BIOLOGICAL: AAV2-hCHM	PHASE1 PHASE2	2015-01	12-10-2022
67	NCT03496012	Efficacy and Safety of BIIB111 for the Treatment of Choroideremia	STAR	COMPLETED	Choroideremia	GENETIC: BIIB111	PHASE3	11-12-2017	01-12-2020
68	NCT03584165	Long-term Safety and Efficacy Follow-up of BIIB111 for the Treatment of Choroideremia and BIIB112 for the Treatment of X-Linked Retinitis Pigmentosa	SOLSTICE	ENROLLING_BY_INVITATION	Choroideremia X-Linked Retinitis Pigmentosa	GENETIC: BIIB111 GENETIC: BIIB112	PHASE3	04-06-2018	04-06-2026
69	NCT02077361	An Open Label Clinical Trial of Retinal Gene Therapy for Choroideremia		COMPLETED	Choroideremia	GENETIC: rAAV2.REP1 vector	PHASE1 PHASE2	2015-04	16-05-2022
70	NCT02407678	REP1 Gene Replacement Therapy for Choroideremia	REGENERATE	COMPLETED	Choroideremia	GENETIC: AAV-mediated REP1 gene replacement	PHASE2	16-08-2016	23-07-2021
71	NCT01461213	Gene Therapy for Blindness Caused by Choroideremia		COMPLETED	Choroideremia	DRUG: rAAV2.REP1	PHASE1 PHASE2	2011-10	2017-10
72	NCT02553135	Choroideremia Gene Therapy Clinical Trial		COMPLETED	Choroideremia	BIOLOGICAL: Injection of AAV2-REP1 (10e11 vg)	PHASE2	2015-09	2018-02
73	NCT05417126	Safety and Effects of a Single Intravitreal Injection of vMCO-010 Optogenetic Therapy in Subjects With Stargardt Disease	STARLIGHT	ACTIVE_NOT_RECRUITING	Stargardt Disease	BIOLOGICAL: Gene Therapy-vMCO-010	PHASE2	05-07-2022	2023-10
74	NCT02599922	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNG3A3 Achromatopsia (A Clarity Clinical Trial)		ACTIVE_NOT_RECRUITING	Achromatopsia	BIOLOGICAL: rAAV2YF-PR1.7-hCNGB3	PHASE1 PHASE2	11-04-2016	2026-07
75	NCT02610582	Safety and Efficacy of rAAV.hCNGA3 Gene Therapy in Patients With CNGA3-linked Achromatopsia	Colourbridge	RECRUITING	Achromatopsia	DRUG: rAAV.hCNGA3	PHASE1 PHASE2	2015-11	2027-04
76	NCT03001310	Gene Therapy for Achromatopsia (CNGB3)	CNGB3	COMPLETED	Achromatopsia	BIOLOGICAL: AAV - CNGB3	PHASE1 PHASE2	16-01-2017	25-10-2019
77	NCT03758404	Gene Therapy for Achromatopsia (CNGA3)	CNGA3	COMPLETED	Achromatopsia	BIOLOGICAL: adeno-associated virus vector AAV- CNGA3	PHASE1 PHASE2	12-08-2019	10-06-2021
78	NCT02935517	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGA3 Achromatopsia (A Clarity Clinical Trial)		ACTIVE_NOT_RECRUITING	Achromatopsia	BIOLOGICAL: AGTC-402	PHASE1 PHASE2	03-08-2017	2026-08
79	NCT03278873	Long-Term Follow-Up Gene Therapy Study for Achromatopsia CNGB3 and CNGA3		ACTIVE_NOT_RECRUITING	Achromatopsia	BIOLOGICAL: either AAV - CNGB3 or AAV - CNGA3	PHASE1 PHASE2	29-06-2017	15-01-2026

Contid...

93	NCT05607810	Long-Term Follow-up Study of ADVM-022 in DME (INFINITY-EXT)		ENROLLING_BY_INVITATION	Diabetic Macular Edema Diabetic Retinopathy	GENETIC: ADVM-022	10-08-2022	2026-03
94	NCT05930561	4D-150 in Patients With Diabetic Macular Edema		RECRUITING	Diabetic Macular Edema Diabetic Retinopathy	BIOLOGICAL: 4D-150 IVT BIOLOGICAL: Aflibercept IVT	09-08-2023	01-07-2028
95	NCT04418427	ADVM-022 Intravitreal Gene Therapy for DME	INFINITY	COMPLETED	Diabetic Macular Edema Diabetic Retinopathy	BIOLOGICAL: 6E11 vg/eye of ADVM-022 BIOLOGICAL: 2E11 vg/eye of ADVM-022 BIOLOGICAL: Aflibercept	28-05-2020	22-11-2022
96	NCT04418427	ADVM-022 Intravitreal Gene Therapy for DME	INFINITY	COMPLETED	Diabetic Macular Edema Diabetic Retinopathy	BIOLOGICAL: 6E11 vg/eye of ADVM-022 BIOLOGICAL : 2E11 vg/eye of ADVM-022 BIOLOGICAL : Aflibercept	28-05-2020	22-11-2022
97	NCT05930561	4D-150 in Patients With Diabetic Macular Edema		RECRUITING	Diabetic Macular Edema Diabetic Retinopathy	BIOLOGICAL: 4D-150 IVT BIOLOGICAL: Aflibercept IVT	09-08-2023	01-07-2028
98	NCT05607810	Long-Term Follow-up Study of ADVM-022 in DME (INFINITY-EXT)		ENROLLING_BY_INVITATION	Diabetic Macular Edema Diabetic Retinopathy	GENETIC: ADVM-022	10-08-2022	2026-03
	DR							
99	NCT04567550	RGX-314 Gene Therapy Administered in the Suprachoroidal Space for Participants With Diabetic Retinopathy (DR) Without Center Involved-Diabetic Macular Edema (CI-DME)	ALTIITUDE	RECRUITING	Diabetic Retinopathy (DR)	GENETIC: RGX-314 Dose 1 GENETIC: RGX-314 Dose 2 GENETIC: RGX-314 Dose 3 DRUG: Topical Steroid	20-11-2020	2024-10
100	NCT04567550	RGX-314 Gene Therapy Administered in the Suprachoroidal Space for Participants With Diabetic Retinopathy (DR) Without Center Involved-Diabetic Macular Edema (CI-DME)	ALTIITUDE	RECRUITING	Diabetic Retinopathy (DR)	GENETIC: RGX-314 Dose 1 GENETIC: RGX-314 Dose 2 GENETIC: RGX-314 Dose 3 DRUG: Topical Steroid	20-11-2020	2024-10
	Retinal degeneration							
101	NCT00643747	Safety Study of RPE65 Gene Therapy to Treat Leber Congenital Amaurosis		COMPLETED	Retinal Degeneration	BIOLOGICAL: tgaAG76 (rAAV 2/2.hRPE65p.hRPE65)	2007-01	2014-12
	Retinoblastoma							

Contid...

102	NCT03284268	Evaluate Safety and the Oncolytic Adenovirus VCN-01 Activity in Patients With Refractory Retinoblastoma	RTB	RECRUITING	Retinoblastoma, Recurrent	GENETIC: VCN-01	NA	06-09-2017	30-06-2022
	Bietti's Crystalline Dystrophy								
103	NCT04722107	Safety Study of rAAV2/8-hCYP4V2 in Patients With Bietti's Crystalline Dystrophy (BCD)		RECRUITING	Bietti's Crystalline Dystrophy	DRUG: rAAV2/8-hCYP4V2	EARLY_PHASE1	21-04-2021	29-04-2024
104	NCT05399069	Safety and Tolerability of VGR-R01 in Patients With Bietti Crystalline Dystrophy		RECRUITING	Bietti Crystalline Dystrophy	DRUG: VGR-R01	EARLY_PHASE1	15-09-2022	2024-05
105	NCT05832684	Safety and Efficacy of ZVS101e in Patients With Bietti's Crystalline Dystrophy		RECRUITING	Bietti's Crystalline Dystrophy	DRUG: ZVS101e	PHASE1 PHASE2	20-02-2023	2028-12
106	NCT05694598	Safety and Tolerability of VGR-R01 for Patients With Bietti Crystalline Dystrophy		NOT_YET_RECRUITING	Bietti Crystalline Dystrophy	GENETIC: VGR-R01	PHASE1	01-03-2023	01-09-2025
	Dry AMD								
107	NCT05984927	NG101 AAV Gene Therapy in Subjects With Wet Age-Related Macular Degeneration		RECRUITING	Age-Related Macular Degeneration	GENETIC: NG101 AAV gene therapy	PHASE1 PHASE2	2023-09	2030-01
108	NCT05672121	Safety and Tolerability of KH631 Gene Therapy in Subjects With Neovascular Age-related Macular Degeneration (nAMD)		NOT_YET_RECRUITING	Age-Related Macular Degeneration	DRUG: KH631	PHASE1 PHASE2	2023-01	2026-12
109	NCT05481827	ORACLE: A Long-term Follow-up Study to Evaluate the Safety and Durability of GT005 in a Gyroscope-sponsored Antecedent Study		ENROLLING_BY_INVITATION	Age Related Macular Degeneration (AMD)	GENETIC: GT005		12-07-2022	16-09-2028
110	NCT03144999	Treatment of Advanced Dry Age Related Macular Degeneration With AAVCAGsCD59		COMPLETED	Dry Age-related Macular Degeneration	BIOLOGICAL: AAVCAGsCD59	PHASE1	29-03-2017	09-12-2019