Conditions (Specific Gene if Applicable) Achromatopsia (CNGA3, CNGB3)	Study Type Phases Interventions Interventional Phase 1 Phase 2 Gene Therapy: AAV-CNGB3 or AAV-CNGA3		Gender Title All Long-Term Follow-Up Gene Therapy Study for Achromatopsia CNGB3 and CNGA3	Acronym Status Start Date First Posted Last Update Primary Completion URL Locations Active, not recruiting 66/29/17 69/12/17 01/14/22 01/15/26 01/15/26 https://ClinicalTrials.gov/show/NCT03278873 Relion Eye Center, Ann Arbor, Michigan, United States Moorfields Eye Hospital NHS Foundation Trust, London, United Kingdom							
Achromatopsia (CNGA3)	Interventional Phase 1 Phase 2 Gene Therapy: AGTC-402	4 Years and older	All Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGAS Achromatopsia		Recru	ruiting 05/0	01/17 10/17/16 04/06/21	09/01/21	1 09/	9/01/25 https://ClinicalTrials.gov/show/NCT02935517	University of California, 3 in manages, an manages, an manages, an manages, an manages, an manages, an manages, and manage
							., ,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,	,			University of California, Sun Francisco, California, United States (Putrenterian Associates, Gainerwille, Florida, United States (Bascon Palmer Eye Institute, Mismi, Florida, United States (Panger Center for Inherited Refines) Dissease, The Chicago Lightonus for Popel Who Are Bill not O'visually Imp. Chicago, Illinois, United States (Bascon Davis en Infilmany, Boston, Massachusetts, United States) (Stotion Children's Hospital, Boston, Massachusetts, United States) (Columbia College of Physicians and Surgeons, New York, New York, United States) (Duke Eye Center, Duke University Medical Center, Durham, North Carolina, United States) (Lonival Estates) (Celevated Clinic, Cole Heye Festitute, Centerian, Explement (Pestitute, Centerian, United States) (Pestitute, Centerian, United States
Achromatopsia (CNGB3) Aicardī Syndrome	Interventional Phase 1 Phase 2 Gene Therapy: rAAV2tYF-PR1.7-hCNGB3 Observational	4 Years and older All Ages	All Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGB3 Achromatopsia All Study of Selected X-Linked Disorders: Alcardi Syndrome				/01/16 11/09/15 04/06/21 /01/02 06/13/08 03/05/21	09/01/21 01/01/25	1 09/	9/01/25 https://ClinicalTrials.gov/show/NCT02599922	Baylor College of Medicine, Houston, Texas, United States
Aicardi Syndrome Bardet-Biedl Syndrome	Observational Observational	All Ages All Ages	All Brain Development Research Program All Clinical Registry Investigating Bardet-Biedl Syndrome	CRIBBS	Recru	ruiting 08/0	01/02 00/25/00 05/05/21 01/03 03/21/06 06/10/21 01/14 12/31/14 03/02/22	01/01/25 12/01/30	5 01/	1/01/25 https://ClinicalTrials.gov/show/NCT00305305	University of California, San francisco, California, United States Marshfield Clinic Research Foundation, Marshfield, Wisconsin, United States
Best disease Autosomal Dominant Vitreoretinalchoroidopathy Chediak-Higashi Syndrome	Observational Observational	5 Years and older 1 Month to 70 Years	All Stem Cell Models of Best Disease and Other Retinal Degenerative Diseases. All Study of Chediak-Higashi Syndrome				/01/14 06/13/14 01/06/22 /10/02 06/19/00 03/02/22	12/01/23			National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesda, Maryland, United States
Chronic Progressive External Ophthalmoplegia Kearns-Sayre CLN3-related Disorders	Observational Interventional Not Applicable Device: OrCam MyEye 2	6 Years to 18 Years	All Genomic Profiling of Mitochondrial Disease - Imaging Analysis for Precise Mitochondrial Medicine All Optic-to-Audio Device in a Pediatric Cohort With CLN3-related Conditions or Low Vision		Recru	ruiting 09/:	/01/22 08/19/21 01/31/22 /30/21 07/23/21 10/07/21	12/01/23 01/20/25	5 01/		National Institutes of Health Clinical Center, Bethesda, Maryland, United States
Congenital cranial dysinnervation disorders Genetic Eye Diseases Genetic Eye Diseases	Observational Observational Observational	All Ages All Ages 1 Year and older	All Genetic Studies of Strabismus, Congenital Cranial Dysinnervation Disorders (CCDDs), and Their Associated Anomalies All Genetics of Inherited Eye Disease All Whole Exome and Whole Genome Sequencing for Genotyping of Inherited and Congenital Eye Conditions			ruiting 06/2	/01/04 02/23/17 09/10/21 /22/15 06/15/15 11/24/21 /05/14 03/04/14 03/09/22	01/01/27 09/01/23 08/05/25	3 09/	9/01/23 https://ClinicalTrials.gov/show/NCT02471287	Boston Children's Mospital, Boston, Massachusetts, United States National institutes of Health Clinical Centre, 9000 Rockville Pike, Bethesda, Manyland, United States National institutes of Health Clinical Centre, Bethesda, Manyland, United States National Institutes of Health Clinical Centre, Bethesda, Manyland, United States
Hermansky-Pudlak Syndrome	Observational	1 Month and older	All Clinical and Basic Investigations into Hermansky-Pudlak Syndrome				06/95 11/04/99 03/02/22	00/03/23	5 00,		National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesda, Maryland, United States University of California, Jon Biogo, La Jolina, United States (Juvessity of California, Los Angeles, California, United States (Juvessity of California, Los Angeles, California, United States (Juvessity of the Pulpida, Lahore, Paksitan (Juvessity of the Philippines, Manila, Philippines (The National United States) (Page 2014) (Page
Inherited Eye Diseases Inherited Eye Diseases	Observational Observational	4 Years and older All Ages	All Clinical and Molecular Studies in Families With Inherited Eye Disease All Inherited Retinal Degenerative Disease Registry	MRTR			/04/16 05/13/16 03/02/22 /01/14 05/06/15 05/04/21	01/01/32 06/01/37	2 01/ 7 06/	1/01/32 https://ClinicalTrials.gov/show/NCT02771236 https://ClinicalTrials.gov/show/NCT02435940	Academy of Medical Sciences of Ukraine, Odessa, Ukraine Foundation Fighting Blindness, Columbia, Maryland, United States
Inherited Eye Diseases	Observational	2 Years and older	All Repository for Inherited Eye Diseases		Active, not	ot recruiting 09/2	/20/06 09/21/06 03/07/22			https://ClinicalTrials.gov/show/NCT00378742	University of California, San Francisco, San Francisco, California, United States National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesda, Manyland, United States University of Iowa, Iowa City, Iowa, United States Schele Eye Institute, University of Pennsylvania, Philadelphia, Pennsylvania, United States Ghent University Hospital and Ghent University, Ghent, Belgium
Leber Congenital Amaurosis, Type 10 (CEP290)	Interventional Phase 1 Phase 2 Drug: QR-110	6 Years and older	All Extension Study to Study PQ-110-001 (NCT03140969)	INSIGHT	Active, not	ot recruiting 05/:	/13/19 04/12/19 02/09/22	05/01/23	3 05/	https://ClinicalTrials.gov/show/NCT03913130	University of Miami - Bascom Palmer Eye Institute, Miami, Florida, United States University of Iowa, Iowa City, Iowa, United States Columbia University Medical Center, New York, United States Eye Eye Institute - Oregon Nealth & Science University, Portland, Oregon, United States University of Pennsylvania - Center for Advanced Retential & Ocular Therapeutics, Philadelphia, Pennsylvania, United States Baylor College of Medicine, Pouston, Posso, University of States Paulo - Hospital States Paulo - University Paulo - Hospital States Paulo - University Paulo - Hospital States Paulo - (University - Paulo - (Univer
Leber Congenital Amaurosis, Type 10 (CEP290)	Interventional Phase 2 Phase 3 Drug: Sepofarsen	8 Years and older	All A Study to Evaluate Efficacy, Safety, Tolerability and Exposure After a Repeat-dose of Sepofarsen (QR-110) in LCA10	ILLUMINATI	TE Active, not	ot recruiting 04/0	/04/19 04/12/19 01/11/21	12/01/21	1 12/	2/01/21 https://ClinicalTrials.gov/show/NCT03913143	Kingdom
Leber Congenital Amaurosis, Type 10 (CEP290)	Interventional Phase 1 Phase 2 Drug: EDIT-101	3 Years and older	All Single Ascending Dose Study in Participants With LCA10		Recru	ruiting 09/2	/26/19 03/13/19 10/18/21	03/22/24	4 03/	3/22/24 https://ClinicalTrials.gov/show/NCT03872479	Bascom Palmer Eye Institute, Miami, Florida, United States Massachusetts Eye and Ear Infirmary, Boston, Massachusetts, United States Mx. Kellogg Eye Center - University of Michigan, Ann Arbor, Michigan, United States Mx. Kellogg Eye Center - University of Michigan, Ann Arbor, Michigan, United States Mx. Kellogg Eye Center - University of Michigan, Ann Arbor, Michigan, United States Mx. Kellogg Eye Center - University of Michigan, Ann Arbor, Michigan, United States Mx. Kellogg Eye Center - University of Ey
Leber Congenital Amaurosis, Type 10 (CEP290) Leber Congenital Amaurosis, Type 2 (RPE65)	Observational Interventional Phase 1 Phase 2 Gene Therapy: AAV2-hRPE65v2	3 Years to 99 Years 8 Years and older	All Natural History Study of CEP290-Related Retinal Degeneration All Phase 1 Follow-on Study of AAV2-hRPE65v2 Vector in Subjects With Leber Congenital Amaurosis (LCA) 2		Recru Active, not	ruiting 12/2 ot recruiting 11/0	/17/17 01/10/18 02/15/22 /01/10 09/24/10 03/12/21	03/31/22 03/01/30		3/31/22 https://ClinicalTrials.gov/show/NCT03396042 5/01/30 https://ClinicalTrials.gov/show/NCT01208389	
Leber Hereditary Optic Neuropathy	Interventional Phase 3 Gene Therapy: GS010	15 Years and older	All RESCUE and REVERSE Long-term Follow-up	RESCUE/REVE	ERSE Active, not	ot recruiting 01/0	/09/18 01/23/18 02/04/21	07/01/22	2 08/	8/01/22 https://ClinicalTrials.gov/show/NCT03406104	Domeny cyc Center UCLV, Psabaeria, Cantorna, United Sattes Jermory University nospital, Autuma, Georgia, United Sattes Jermory Canter Sattes Jermory University nospital, Autuma, Georgia, United Sattes Jermory Canter Sattes Jermory University Nospital London, Greater London, United Singdom France JLMU Klinikum der Universit\u00e4art M\u00e4Kinchen / Friedrich-Baur-Institut, Munich, Germany (Ospedale Bellaria, Bologna, Italy Moorfields Eye Hospital, London, Greater London, United Kingdom
											Doheny Spe Center UCLA Pasadena, Pasadena, California, United States University of Colorado Health Eye Center, Auron, Colorado, United States (Isrnory Healthcare - The Emory Clinic, Kalanta, Georgia, United States) (Massachusetts Fyear data in riflorman), seboto, Massachusetts, United States) (Jain School of Medicine at Mount Strain, New York, New York, Berthal States) (Will Special States) (W
Leber Hereditary Optic Neuropathy Leber Hereditary Optic Neuropathy	Interventional Phase 3 Gene Therapy: GS010 Interventional Phase 1 Gene Therapy: scAV2-P1ND4v2		All Efficacy & Safety Study of Bilateral IVT Injection of GS010 in LHON Subjects Due to the ND4 Mutation for up to 1 Year All Safety Study of an Adeno-associated Virus Vector for Gene Therapy of Leber's Hereditary Optic Neuropathy	REFLECT LHON			/12/18 09/26/17 02/08/21 /14/14 06/11/14 07/06/21	06/30/24 03/01/23		5/30/24 https://ClinicalTrials.gov/show/NCT03293524 8/01/23 https://ClinicalTrials.gov/show/NCT02161380	
Microphthalmia Anophthalmia Coloboma	Observational	1 Year and older	All Pathogenesis and Genetics of Microphthalmia, Anophthalmia and Uveal Coloboma (MAC)		Recru	ruiting 01/i	/08/13 01/29/13 03/03/22	12/27/27	7 12/	//27/27 https://ClinicalTrials.gov/show/NCT01778543	National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesids, Maryland, United States (Children's Hospital Stanford University, Palo Alto, California, United States (LUCS Medical Center-Mount Zion, San Francisco, California, United States) (LUCS Medical Center-Amount Zion, San Francisco, California, United States) (Children's Neutropial Colorado, Aurora, Colorado, United States) (Children's Neutropial Colorado, United States) (Children's Neutropial Colorado, United States) (Lindier's Neutropial Colorado, United States) (Inition's Neutropial Colorado, United States) (Inition's Neutropial Colorado), United States) (Inition's Neutropial (Inition's Neutropial (Inition's Neutropial (Inition's Neutropial (Inition's Neutropial Medical Center, Circinvasia), United States) (Inition's Neutropial (Inition's Neutropial (Inition's Neutropial (Inition's Neutropial Medical Center, Circinvasia), United States) (Inition's Neutropial Medical Center, Circinvasia), United States) (Inition's Neutropial (Inition's Neutropia
Neurofibromatosis Type 1	Interventional Phase 1 Phase 2 Drug: Selumetinib	3 Years to 21 Years	All Selumetinib in Treating Young Patients With Recurrent or Refractory Low Grade Glioma		Active, not	ot recruiting 04/:	/19/10 03/18/10 10/21/21	12/01/25	5	https://ClinicalTrials.gov/show/NCT01089101	Pennsylvania, United States [Saint Jude Children's Research Hospital, Memphis, Tennessee, United States Baylor College of Medicine/Dan L Duncan Comprehensive Cancer Center, Houston, Texas, United States States Texas Children's Hospital, Houston, Texas, United States
Neurofibromatosis Type 1 Optic atrophy (FDXR) Optic atrophy (SLC25A46)	Observational Observational Observational	All Ages 2 Years to 65 Years 2 Years to 65 Years	All Natural History Study of Patients With Neurofibromatosis Type I All Natural History Study of FDXR Mutation-related Mitochondriopathy All Natural History Study of SCLCSA4G Mutation-related Mitochondriopathy			ruiting 11/0	/25/08 06/18/09 12/23/21 /03/20 10/09/20 03/08/22 /03/20 10/20/20 03/08/22	01/01/24 01/01/24		3/01/24 https://ClinicalTrials.gov/show/NCT04580979 https://ClinicalTrials.gov/show/NCT04594590	National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesda, Maryland, United States UBMD Pediatrics, Buffalo, New York, United States UBMD Pediatrics, Buffalo, New York, United States
Retinitis Pigmentosa	Observational	12 Years and older	All Retinitis Pigmentosa Clinical Measures and Repeatability Testing of Potential Outcome Measures		Recru	ruiting 09/2	/27/19 02/19/19 12/16/21	10/30/22	2 10/	0/30/22 https://ClinicalTrials.gov/show/NCT03845218	National Institutes of Health Clinical Center, Bethesda, Maryland, United States Shiley Eye Institute - UCSD, La Jolia, California, United States Emory Eye Centre, Atlanta, Georgia, United States Massachusetts Eye and Ear Infirmary, Boston, Massachusetts, United States Kellogg Eye Center, Ann
Retinitis Pigmentosa (RPGR) Retinitis Pigmentosa	Observational Observational		All Natural History Study of Patients With X-linked Retinal Dystrophy Associated With Mutations in Retinits Pigmentosa GTPase Regulator (RPGR) All Retinal Imaging in Patients With Inherited Retinal Degenerations		Recru Recru		/19/17 11/21/17 02/15/21 /01/05 11/16/05 09/29/21	12/30/22 10/01/23		1/01/24 https://ClinicalTrials.gov/show/NCT00254605	Abox, Michigan, United States (IPMC Eye Centre, Pittsburgh, Pennsylvania, United States) (The Hospital for Sick Children, Toronto, Canada Mondriels Eye Hospital, London, United Kingdom Desartment of Ophthalmology Retelland Expenseration (Sinci, UCK) 5-sa Francisco, California, United States States States States (Missing Mondriels Eye Hospital) (London, United Kingdom Desarration (London)
Retinitis Pigmentosa (CNGB1) Retinitis Pigmentosa (Syndromic)	Observational Observational	All Ages All Ages	All CNGB1 and Allied Disorders All UAB HRFD Core Center: Core A: The Hepato/Renal Fibrocystic Diseases Translational Resource				/14/19 11/20/20 11/01/21 /01/11 07/26/11 02/21/21	03/01/23 09/01/22			Tuebinger, Germany Moorfields Eye Hospital MHS Foundation Trust, London, United Kingdom Children's National Health System, Washinghon, District of Choumble, United States (Dumble, United States) (University of Iowa Hospitals & Clinics, Iowa City, Iowa, United States) Children's Hospital of Los Angeles, Los Angeles, California, United States (Bascom Palmer Eye Institute, Milam, Florida, United States) (University of Iowa Hospitals & Clinics, Iowa City, Iowa, United States) States (Massachusts Eye and Ear Institute, Boston, Massachusts, Junied States) (Exloging Eye Center, Ann Arbor, Michigan, United States) (Encionati Eye Institute, Cincinnati, Chib., United States) States) (Massachuster) (States) (Sta
Retinitis Pigmentosa Leber Congenital Amaurosis (RPE65) Retinitis Pigmentosa Leber Congenital Amaurosis, Type 2 (RPE6:	Observational Gene Therapy: AAV2-hRPE65v2 interventional Phase 3 Gene Therapy: AAV2-hRPE65v2		All A Patient Registry Study for Patients Treated With Voretigene Neparvovec in US All Safety and Efficacy Study in Subjects With Leber Congenital Amaurosis				/10/19 07/24/18 10/25/21 /01/12 10/22/09 12/13/21	06/01/25 07/01/15		5/01/25 https://ClinicalTrials.gov/show/NCT03597399	Portland, Oregon, United States Children's Hospital of Philadelphia (CHOP), Philadelphia, Pennsylvania, United States Schele Eye Institute, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States University of Iowa, Lowa City, Lowa, United States Children's Hospital of Philadelphia, Philadelphia, Philadelphia, Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Philadelphia, Phil
Retinitis Pigmentosa Leber Congenital Amaurosis, Type 2 (RPE6: Retinitis Pigmentosa Stargardt's Disease		8 Years and older 5 Years and older	All Phase I Trial of Gene Vector to Patients With Retinal Disease Due to RPE65 Mutations All Rod and Cone Mediated Function in Retinal Disease	LCA	Active, not	t recruiting 07/0	/01/12 10/22/09 12/13/21 /01/07 06/01/07 12/14/21 /24/16 12/01/15 03/03/22	06/01/26	6 06/	5/01/26 https://ClinicalTrials.gov/show/NCT00481546	University of Lowa, Liv, Lowa, University Charlest Prospect of Principles, Principles, Principles, Principles, University of Lowards, University of Pennsylvania, United States Shards Children's Ropotal, Liversity of Principle, Carleston, University of Pennsylvania, Philadelphia, Pennsylvania, United States National Institutes of Health Clinical Center, 9000 Rockville Pike, Bethesda, Maryland, United States
											Research Site, Phoenix, Arizona, United States [Research Site, Los Angeles, California, United States [Research Site, Paina, United States [Research Site, Main, Horida, United States [Research Site, Main, United States [Research Site, Main, United States [Research Site, Online Or Sites (States [Research Site, Online States [Research S
Retinitis Pigmentosa, X-Linked	Observational	7 Years and older	All Natural History of the Progression of X-Linked Retinitis Pigmentosa	XOLARIS	Active, not	ot recruiting 09/2	/29/17 06/14/21 01/12/22	12/21/22	2 12/	2/21/22 https://ClinicalTrials.gov/show/NCT04926129	States Research Site, Medison, Wisconsin, United States Research Site, Month/Roll, Quebec, Canada Research Site, Vancouver, Canada Research Site, Heisinki, Finland Research Site, Montpellier, France Research Site, Sundampton Research Site, Montpellier, France Research Site, Montpellier, France Research Site, Montpellier, France Research Site, Sundampton Research Site, Montpellier, France Research Site, Sundampton Research Site, Southampton, United Kingdom Research Site, Southampton, United Kingdom Research Site, Montpellier, Site Southampton, United Kingdom Research Site, Southampton, United Kingdom Rese
Retinitis Pigmentosa, X-Linked	Interventional Phase 1 Phase 2 Biological: 4D-125 IVT Injection	12 Years and older	Male 4D-125 in Patients With X-Linked Retinitis Pigmentosa (XLRP)		Reco	ruiting 06/9	/09/20 08/18/20 03/04/22	04/01/26	5 09/	9/01/26 https://ClinicalTrials.gov/show/NCT04517149	University of Colorado, Aurora, Colorado, United States University of Michigan Kellogg Eye Center, Ann Arbor, Michigan, United States Duke University Eye Center/Dept. of Ophthalmology, Durham, North Carolina, United States Casey Eye Institute, Oregon Health and Science University, Portland, Oregon, United States Retina Foundation of the Southwest, Dallas, Texas, United States University of Utah John A. Moran Eye Center-Salt Jake (Thy Utah I Indied States)
Retinitis Pigmentosa, X-Linked	Observational	6 Years and older	Male Clinical Evaluation of Patients With X-linked Retinitis Pigmentosa (XLRP)		Recru	ruiting 12/0	01/17 10/19/17 01/14/21	12/01/21		2/01/21 https://ClinicalTrials.gov/show/NCT03314207	Duke Eye Center, Duke University Medical Center, Durham, North Carolina, United States Casey Eye Institute, Oregon Health and Sciences University, Portland, Oregon, United States Retina Foundation of the Southwest, Dallas, Texas, United States
Retinitis Pigmentosa, X-Linked (RPGR)	Interventional Phase 3 Gene Therapy: AAV5-RPGR	3 Years and older	All Follow-up Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene		Recru	ruiting 03/:	/16/21 03/11/21 09/30/21	07/05/23	3 07/	7/05/27 https://ClinicalTrials.gov/show/NCT04794101	Children's Hospital Los Angeles, Los Angeles, California, United States There Retinal Associates, Gainesville, Florida, United States The Emory Eye Centre, Atlanta, Georgia, United States Boston, Massachusetts, United States University of Michigan, Ann Arbor, Michigan, United States Retinal Consultants of Houston, Bellaier, Texas, United States States Asron Los Naselle, California, United States Wiltreo Retinal Associates, Gainesville, Florida, United States The Emory Eve Centre, Atlanta, Georgia, United States Massachusetts Eve and Ear, Centre for Associated Canada
Retinitis Pigmentosa, X-Linked (RPGR)	Interventional Phase 3 Gene Therapy: AAV5-RPGR	3 Years and older	All Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene		Recru	ruiting 03/	/16/21 12/17/20 09/27/21	07/05/22	2 07/	7/05/22 https://ClinicalTrials.gov/show/NCT04671433	Aaron Los Nagele, Los Angeles, Calatorna, United States (Wiredo Herinal Associates, Gaineswile, Horiza), United States) [In et mony Eye Lentre, Atlanta, Georgia, United States (Massachusetts eye and Earl, Lentre for Clinical Research Operations, Boston, Massachusetts, United States (University of Michigan, Ann Arbor, Michigan, United States) [Retinal Consultants of Houston, Bellaire, Texas, United States (University of Forida, Jacksonville, Florida, United States) Boston (Massachusetts, United States) [Columbia University, New York, New York, United States (States) [Outed States)] [Outed States (States)] [Outed States (States)] [Outed States) [Outed States (States)] [Outed States) [Outed States] [Outed States) [Outed States] [Outed States) [Outed States) [Outed States) [Outed States] [Outed States) [Outed States] [Outed States] [Outed States) [Outed States] [Outed
Retinitis Pigmentosa, X-Linked (RPGR)	Interventional Phase 1 Phase 2 Gene Therapy: rAAV2tYF-GRK1-RPGR	6 Years to 50 Years	Male Safety and Efficacy of rAAVZtYF-GRK1-RPGR in Subjects With X-linked Retinitis Pigmentosa Caused by RPGR Mutations		Recru		/16/18 10/20/17 07/15/21	08/01/22			Carolina, United States [Cincimati Eye Institute, Cincinnati, Ohio, United States Casey Eye Institute, Oregon Health and Science University, Portland, Oregon, United States Retina Foundation of the Southwest, Dallas, Texas, United States
Retinoblastoma Retinoblastoma	Observational Interventional Phase 1 Biological: 2nd generation 4-1BBζ B7H3-EGFRt-DHFR		All RB Liquid Biopsy Biorepository All B7H3 CAR T Cell Immunotherapy for Recurrent/Refractory Solid Tumors in Children and Young Adults	rbliqbx	Recru Recru		/01/17 07/13/21 07/13/21 /13/20 07/23/20 04/19/21			2/31/25 https://ClinicalTrials.gov/show/NCT04959097 2/01/40 https://ClinicalTrials.gov/show/NCT04483778	Children's Hospital Los Angeles, Los Angeles, California, United States Seattle Children's Hospital, Seattle, Washington, United States Lurie Children's Hospital, Chicago, Illinois, United States (University of Minnesoa, Minnesoa, Minnesota, United States (Washington School of Medicine Lurie Children's Hospital, Chicago, Illinois, United States (Washington School of Medicine
											at St. Louis, Smits Louis, Missouri, United States Grininati Children's Hospital Medical Center, Cincinnati, Children's Hosp
Retinoblastoma Retinoblastoma	Observational Interventional Phase 1 Biological: 2nd generation 4-1BBZ B7H3-EGFRt-DHFR		All Studying Health Outcomes After Treatment in Patients With Retinoblastoma All EGFR806 CAR T Cell Immunotherapy for Recurrent/Refractory Solid Tumors in Children and Young Adults	RIVERBOAT	Recru	ruiting 06/:	/24/19 05/01/19 04/21/21 /18/19 08/07/18 04/19/21	01/01/25 06/01/23	3 06/	5/01/38 https://ClinicalTrials.gov/show/NCT03618381	Milwaukee, Wisconsin, United States The Hospital for Sick Children, Toronto, Canada Seattle, Children's Hospital, Seattle, Washington, United States
Retinoblastoma Retinoblastoma Retinoblastoma	Interventional Phase 1 Drug: Metronomic Cyclophosphamide Interventional Phase 2 Radiation: 1311-3F8 Observational	All Ages 8 Years to 80 Years	All Anti-Angiogenic Therapy Post Transplant (ASCR) for Pediatric Solid Tumors All Indine I 131 Monoclonal Antibody 378 in Treating Patients With Central Nervous System Cancer or Leptomeningeal Cancer All Retinoblastoms Biomarker Study	ASCR	Active, not	ot recruiting 01/0	/26/12 08/09/12 09/23/21 /01/06 03/09/07 11/01/21 /17/93 06/21/06 12/16/21	11/23/22 01/01/23		1/01/23 https://ClinicalTrials.gov/show/NCT00445965	Washington University School of Medicine, Saint Louis, Missouri, United States Mational Cancer Institute (CL) 2000 Rockville Washington University States National Cancer Institute (NCI), 2000 Rockville Pike, Betheside, Manyland, United States
Retinoschisis, X-Linked	Observational		All Clinical and Genetic Studies of X-Linked Juvenile Retinoschisis				/19/03 02/17/03 02/25/22			https://ClinicalTrials.gov/show/NCT00055029	National Institutes of Health Clinical Center, Bethesda, Maryland, United States University of California San Francisco, Dept. of Ophthalmology, San Francisco, California, United States University of Miami - Miller School of Medicine Bascom Palmer Eye Institute, Miami, Florida, United
			Male Safety and Efficacy of rAAV-hRS1 in Patients With X-linked Retinoschisis (XLRS)					04/01/19			States The Wilmer Eye Institute, Johns Hopkins Hospital, Ballimore, Manyland, United States Massachusetts Eye and Ear Infilmany, Boston, Massachusetts, United States University of Michigan Kellogga Eye Center, Ann Arbor, Michigan, United States Duke Eye Center, Duke University Medical Center, Duham, North Carolina, United States Casey Eye Institute, Oregon Health and Sciences University, Portland, Oregon, United States Retina Foundation of the Southwest, Dallas, Texas, United States Baylor College of Medicine, Alkek Eye Center, Houston, Texas, United States States Retina Foundation of the Southwest, Dallas, Texas, United States Baylor College of Medicine, Alkek Eye Center, Houston, Texas, United States States Retina Foundation of the Southwest, Dallas, Texas, United States Sta
Retinoschisis, X-Linked Sjogren-Larsson Syndrome Smith-Lemli-Opitz Syndrome Cone-Rod Dystrophy	Interventional Phase 1 Phase 2 Gene Therapy: rAAV2tYF-CB-hRS1 Observational Interventional Phase 2 Drug: Antioxidants Drug: Cholesterol	All Ages up to 65 Years	Maile Safety and Efficacy of rAM-rNs.i in Patients With x-linked Retinoscribis (RLNS) All Slogren-Larsson Syndrome: Natural History, Clinical Variation and Evaluation of Biochemical Markers All Cholesterol and Antioxidant Treatment in Patients With Smith-Lemil-Ogitz Syndrome (\$LOS)	SLS	Active, not	ot recruiting 04/0	/01/15 04/15/15 07/14/20 /01/13 10/30/13 04/30/21 /01/08 01/23/13 01/20/22	04/01/19 08/31/21 12/01/25	1 08/	3/31/21 https://ClinicalTrials.gov/show/NCT01971957	States prettar aroundation or the Southwest, Johans, Lexas, United States Baylor Conege or Neutronic, Autex Eye Certifier, Mouston, 1 exas), United States University of Pedraska Medical Center, Omaha, Nebraska, United States University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania, United States Children's Hospital Colorado, Aurora, Colorado, United States
Stargardt Disease	Interventional Phase 1 Phase 2 Follow up for SAR422459 in prior study TDU13583	6 Years and older	All Phase I/II Follow-up Study of SAR422459 in Patients With Stargardt's Macular Degeneration		Active, not	ot recruiting 12/	/14/12 11/29/12 10/20/21	08/01/34	4 08/	3/01/34 https://ClinicalTrials.gov/show/NCT01736592	Investigational Site Number 840001, Portland, Oregon, United States Investigational Site Number 250001, Paris, France Retina-Vitreous Associates Medical Group, Beverly Hills, California, United States UCSF Dept. of Ophthalmology, San Francisco, California, United States Emory University, Atlanta, Georgia, United States The
											Wilmer fee Institute Johns Holphiss University, Baltimore, Maryland, United States [University of Michigan Kellog grey Center, Ann Arbon, Michigan, United States [Johns of the States [Johns of Land Johns of Land States [Johns of Land States [
											Germany (Inviersitätes-Augenkink Bonn, Bonn, Germany /ADU Universitá della Campania Luigi Yanvitelli, Napiles, Campania, Italy (Universitätes-Augenkink Bonn, Bonn, Germany /ADU Universitá della Campania Luigi Yanvitelli, Napiles, Campania, Italy) (Endicità Catolica del Sacro Curivesita della Signa (Studi dal Milano, Milan, Chambart, Mary) (Loro Culsitàca ADU Carega, Florence, Lauto, Italy) (Endoud University Medical Center, Nijmegen, Gelderfand, Netherfandsi (Pretoria Sey Institute, Pretoria, Gautern, South Africa (Fundacion Jimenez Data University Hospital, Madrid, Spain) (Oxford Eye Hospital, Oxford Coffordshire, Universit Mospital, Madrid, Spain) (Oxford Coffordshire, University) (Oxford Coffordshire, University) (Oxford Coffordshire, University) (Oxford, Oxfordshire, University) (Oxford, Oxfordshire, University) (Oxfordshire, University) (Oxfordshi
Stargardt Disease (ABCA4)	Interventional Phase 3 Drug: Emixustat		All Safety and Efficacy of Emixustat in Stargardt Disease	SeaSTAR	Active, not		/07/18 12/11/18 09/05/21			5/01/22 https://ClinicalTrials.gov/show/NCT03772665	University of California Los Angeles - Jules Stein Eye Institute, Los Angeles, California, United States Vitreoretinal Associates, Gainesville, Florida, United States University of Maimi - Bascom Palmer Eye Institute, Maimi, Florida, United States Johns Hopkins - Wilmer Eye Institute, Baltimore, Maryland, United States Columbia University Medical Center - Hariness Eye Institute, New York, New York, United States University of
Stargardt Disease (ABCA4) Stargardt Disease Retinitis Pigmentosa Choroideremia	Interventional Phase 2 Drug: ALK-001 Observational	7 Years and older	All Phase 2 Tolerability and Effects of ALK-001 on Stargardt Disease All High Resolution Retinal Imaging	TEASE AOSLO	Recru	ruiting 05/0	/01/15 03/30/15 03/03/22 /01/13 05/31/13 01/28/22	12/01/24	4 12/	2/01/25 https://ClinicalTrials.gov/show/NCT01866371	Utah - Moran Eye Institute, Salf Lake City, Utah, United States Medical College of Wisconsin - Eye Institute, Milwaukee, Wisconsin, United States University of Pennsylvania, Philadelphia, Pennsylvania, United States
Stargardt Disease and other ABCA4 disorders Stargardt Disease and other ABCA4-related disorders Stargardt-Like Macular Dystrophy (ELOVL4)	Interventional Phase 1 Phase 2 Drug: Metformin hydrochloride Observational Observational	12 Years and older 10 Years and older	All Oral Metformin for Treatment of ABCAA Betinopathy All Natural History of Eye Diseases Related to ABCA4 Mutations All Stargard-tike Macular Dystrophy (STDG3) Secondary to Mutations in ELOVL4		Active, not Recru	ot recruiting 10/0 ruiting 03/1	/23/20 09/11/20 02/16/22 /09/12 11/29/12 12/16/21 /14/22 10/19/20 03/09/22	07/16/25	5 07/	https://ClinicalTrials.gov/show/NCT01736293 7/16/25 https://ClinicalTrials.gov/show/NCT04591483	National Institutes of Health Clinical Center, Betheda, Maryland, United States National Institutes of Health Clinical Center, 9000 Rockville Pike, Betheda, Maryland, United States National Institutes of Health Clinical Center, Bethedd, Maryland, United States
Strabismus	Observational Whole genome sequencing	All Ages	All Genetic Studies of Strabismus and Associated Disorders		Recru	ruiting 09/0	/03/21 02/25/21 09/05/21	12/01/26	5 12/	2/01/26 https://ClinicalTrials.gov/show/NCT04770519	Boston Children's Nospital, Boston, Massachusetts, United States University of California, San Francisco, California, United States University of Kentucky, Advanced Eye Care, Lexington, Kentucky, United States The Johns Hopkins Wilmer Eye Institute, Baltimore, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Takbingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Takbingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Germany Haddassah Medical Center, Jerusalem, Israel Radboud University, Nijmegen, Manyland, United States Nospital for Sick Children, Toronco, Ontario, Canada University of Tubingen, Germany Haddassah Medical Center, Jerusalem, Manyland,
Usher Syndrome, Type 1F (PCDH15) Usher Syndrome, Type 2A (USH2A)	Observational Interventional Phase 2 Phase 3 Drug: QR-421a	12 Years and older	All Rate of Progression of PCDH15-Related Retinal Degeneration in Usher Syndrome 1F All 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	RUSH1F CELESTE	Recru	ruiting 12/:	/08/21 02/21/21 01/13/22 /15/21 01/04/22 02/24/22	04/01/25 06/01/25	5 06/	5/01/25 https://ClinicalTrials.gov/show/NCT05176717	Netherlands University Hospital Basel, Basel, Switzerland Return Switzerland Return Foundation of the Southwest, Dallas, Texas, United States University to Wisconsin - Madison, Misconsin, United States Moorfields Eye Hospital, London, United Kingdom
Usher Syndrome, Type 2A (USH2A)	Interventional Phase 2 Phase 3 Drug: QR-421a	12 Years and older	All Efficacy Safety and Tolerability of QR-421a in Subjects With RP Due to Mutations in Exon 13 of the USH2A Gene With Advanced Vision Loss	SIRIUS			/08/21 12/15/21 02/24/22	12/01/24	1 12/	2/01/24 https://ClinicalTrials.gov/show/NCT05158296	Retina Foundation of the Southwest, Dallas, Fexas, United States University of Wisconsin - Madison, Misconsin, United States Monorfields Eye Hospital, London, United Kingdom University of California, San Francisco, Sal Francisco, California, United States Winter-eitral Associates, Galierwille, Florida, United States Winter-eitral St
											States (Mssachusetts Eye and Ear, Boston, Massachusetts, United States (Jellong Eye Center, University of Michigan, Ann Arbon, Michigan, United States) (Duke University Eye Center, Dunham, North Carolina, United States) (BUSU Casey Eye Institute, Portland, Oregon, United States) (Retina Foundation of the Southwest, Dallas, Texas, United States) (Busic Eye Psyliciations and Surgeons, Houston, Texas, United States) (Moran Eye Center, University of Utah, Salt Lake City, Utah, United States) (Medical College of Wisconsin, Milwaukee, Wisconsin, United States) (Hospital for Sack Children, Toronto, Canada) (Enter hospital ler National Center hospital Ce
Usher Syndrome, Type 2A (USH2A)	Observational		All Rate of Progression in USH2A-related Retinal Degeneration	RUSH2A	Active, not	ot recruiting 08/	/11/17 05/09/17 04/20/21	01/01/23		1/01/23 https://ClinicalTrials.gov/show/NCT03146078	d'Ophtalmologie des Quinze-Vingts, Paris, France University of Tubingen, TĀXbingen, Germany Radboud University, Nijmegen, Netherlands Moorfields Eye Hostpital, London, United Kingdom
Wolfram Syndrome Wolfram Syndrome Wolfram Syndrome	Observational Observational Interventional Phase 1 Phase 2 Drug: Dantrolene sodium	All Ages All Ages 5 Years to 60 Years	All I-Tracking Neurodegeneration in Early Wolfram Syndrome All Wolfram Syndrome International Registry and Clinical Study All A Clinical Trial of Dantrolines Sodium in Pediatric and Adult Patients With Wolfram Syndrome	I-TRACK	Recru	ruiting 07/0	'10/18 05/15/19 06/04/21 '01/11 07/22/16 04/06/21 '01/17 07/12/16 02/18/22	12/01/25	5 12/	2/01/25 https://ClinicalTrials.gov/show/NCT02841553	Washington University School of Medicine, Saint Louis, Missourt, United States Washington University School of Medicine, Saint Louis, Missourt, United States Washington University School of Medicine, Saint Louis, Missourt, United States
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