

Choroba	Gen	Identyfikacja	Rekrutacja	Tytuł	Badany lek	Sponsor	Lokalizacje	Kontakt
Retinitis pigmentosa (zwyrodnienie barwnikowe siatkówki)	MERTK	NCT01482195	tak	Trial of Ocular Subretinal Injection of a Recombinant Adeno-Associated Virus (rAAV2-VMD2-hMERTK) Gene Vector to	rAAV2-VMD2-hMERTK	Fowzan Alkuraya	Arabia Saudyjska	falkuraya@kfshrc.edu.sa
Zespół Ushera	MYO7A	NCT01505062	tak	Study of UshStat in Patients With Retinitis Pigmentosa Associated With	EIAV-CMV-MYO7A (UshStat)	Sanofi	Portland, Oregon; Iowa City; Houston, Texas;	Contact-Us@sanofi.com
Choroba Stargardta	ABCA4	NCT01367444	tak	Phase I/II Study of SAR422459 in Patients With Stargardt's Macular	EIAV-ABCA4 (SAR422459)	Sanofi	Oregon, USA; Paryż, Francja	Contact-Us@sanofi.com
Choroideremia	CHM	NCT02407678	tak	REP1 Gene Replacement Therapy for Choroideremia	AAV-REP1	University of Oxford	London; Oxford - Wielka Brytania	trials@eye.ox.ac.uk
Choroideremia	CHM	NCT03496012	tak	Efficacy and Safety of AAV2-RPE1 for the Treatment of Choroideremia	AAV2-RPE1	Nightstar Therapeutics	Los Angeles, Miami, Baltimore, New York, Portland, Dallas, Madison – USA; Québec, Vancouver – Kanada; Helsinki – Finlandia; Bonn, Tübingen – Niemcy, Nijmegen – Holandia; Manchester, Oxford – Wielka Brytania	researchenquiries@nightstartx.com
Wysiękowe zwyrodnienie plamki żółtej	fragment przeciwciała anty-VEGF	NCT03066258	tak	RGX-314 Gene Therapy for Neovascular AMD Trial	AAV-VEGF (RGX-314)	Regenxbio Inc.	Santa Barbara, Baltimore, Boston, Pennsylvania, Memphis,	patientadvocacy@regenxbio.com
Achromatopsia	CNGB3	NCT02599922	tak	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGB3	rAAV2tYF-PR1.7-hCNGB3	Applied Genetic Technologies Corp	Gainesville, Miami, Chicago, Portland,	advocacy@agtc.com
Achromatopsja	CNGB3	NCT03001310	tak	Gene Therapy for Achromatopsia (CNGB3)	AAV-CNGB3	MeiraGTx UK II Ltd	Londyn - Wielka Brytania	ocularinfo@meiragtx.com
Achromatopsja	CNGA3	NCT02935517	tak	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGA3	AGTC-402	Applied Genetic Technologies Corp	Gainesville, Miami, Boston, Portland,	advocacy@agtc.com
X-linked retinoschisis	RS1	NCT02317887	tak	Study of RS1 Ocular Gene Transfer for X-linked Retinoschisis	AAV RS1	National Eye Institute (NEI)	Bethesda, USA	turriffa@mail.nih.gov, ps261o@nih.gov
X-linked retinoschisis	RS1	NCT02416622	tak	Safety and Efficacy of rAAV-hRS1 in Patients With X-linked Retinoschisis	rAAV2tYF-CB-hRS1	Applied Genetic Technologies Corp	Boston, Miami, San Francisco, Baltimore,	advocacy@agtc.com
retinitis pigmentosa	PDE6B	NCT03328130	tak	Safety and Efficacy Study in Patients With Retinitis Pigmentosa Due to	AAV2/5-hPDE6B	Horama S.A.	Nantes - Francja	pierre.lebranchu@chu-nantes.fr
wrodzona ślepotą Lebera (LCA)	CEP290	NCT03140969	już nie	Study to Evaluate QR-110 in Subjects With Leber's Congenital Amaurosis (LCA) Due to the c.2991+1655A>G	QR-110	ProQR Therapeutics	-	-
wrodzona ślepotą Lebera (LCA)	RPE65	NCT02781480	tak	Clinical Trial of Gene Therapy for the Treatment of Leber Congenital Amaurosis (LCA) (OPTIRPE65)	AAV RPE65	MeiraGTx UK II Ltd	Londyn - Wielka Brytania	julie.bakobaki@meiragtx.com; praseeda.thaikalloor@meiragtx.com; michel.michaelides@ucl.ac.uk
wrodzony zanik nerwu wzrokowego Lebera (LHON)	G11778A mutacja	NCT02161380	tak	Safety Study of an Adeno-associated Virus Vector for Gene Therapy of	scAAV2-P1ND4v2	National Eye Institute (NEI)	Miami, USA	kguy@miami.edu; jgonzalez@med.miami.edu
retinitis pigmentosa sprzężona z chr. X	RPGR	NCT03252847	tak	Gene Therapy for X-linked Retinitis Pigmentosa (XLRP) Retinitis Pigmentosa GTPase Regulator (RPGR)	AAV-RPGR	MeiraGTx UK II Ltd Syne Qua Non Limited EMAS Pharma	Londyn - Wielka Brytania	julie.bakobaki@meiragtx.com; praseeda.thaikalloor@meiragtx.com; michel.michaelides@ucl.ac.uk
retinitis pigmentosa sprzężona z chr. X	RPGR-ORF15	NCT03316560	TAK	Safety and Efficacy of rAAV2tYF-GRK1-RPGR in Subjects With X-linked Retinitis	rAAV2tYF-GRK1-RPGR	Applied Genetic Technologies Corp	Durham, Portland, Pennsylvania, Dallas,	advocacy@agtc.com
retinitis pigmentosa (optogenetyka - genowoniezależna)	ChrimsonR	NCT03326336	jeszcze nie	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in	GS030-DP	GenSight Biologics	-	-
retinitis pigmentosa (optogenetyka - genowoniezależna)	ChR-2	NCT02556736	rekrutuje	RST-001 Phase I/II Trial for Advanced Retinitis Pigmentosa	RST-001	Allergan	Cincinnati, Dallas - USA	IR-CTRegistration@Allergan.com; mkingdon@cincinnatieye.com; dbirch@retinafoundation.org