

Supplemental Table S1. Registered clinical trials of pharmaceutical therapies for Stargardt disease. Information is available online at www.clinicaltrials.gov/ <https://www.clinicaltrialsregister.eu/> and <http://www.anzctr.org.au/Default.aspx>. Accessed on 16-Jun-2021.

Phase/ Recruitment Status	Sponsor	Evaluation	Intervention	Administration	Updated	Identifier
Phase 2- Completed	Kubota Vision Inc.	Pharmacodynamics, safety and tolerability of Emixustat in subjects with macular atrophy secondary to STGD1	Emixustat (inhibitor of retinoid cycle isomerase RPE65)	Oral	19-May-2021	NCT03033108
Phase 3- Active	Kubota Vision Inc.	Safety and efficacy of Emixustat compared to placebo in subjects with STGD1	Emixustat (inhibitor of retinoid cycle isomerase RPE65)	Oral	31-Aug-2020	NCT03772665
Phase 2- Recruiting	IVERIC bio, Inc.	Safety and efficacy of Zimura compared to sham in subjects with STGD1	Zimura (Complement C5 inhibitor)	Intravitreal	03-May-2021	NCT03364153
N.A.- Completed	Ophthalmos Research and Education Institute	Therapeutic potential of Omega-3 Fatty Acids Supplementation in subjects with STGD1	Madeos (Omega-3 Fatty Acids)	Oral	23-Feb-2021	NCT03297515
Phase 2- Recruiting	Alkeus Pharmaceuticals, Inc.	Long-term safety, tolerability, pharmacokinetics and effects of ALK-001 in subjects with STGD1	ALK-001 (chemical modified vitamin A)	Oral	05-May-2020	NCT02402660
Phase 2- Enrolling by invitation	Alkeus Pharmaceuticals, Inc.	Long-term safety, pharmacokinetics and effects of ALK-001 (C20-D3-retinyl acetate) on the STGD1 progression	ALK-001 (chemical modified vitamin A)	Oral	27-Jan-2020	NCT04239625
Phase 1- Completed	University of Utah	Inhibition effect of dark adaptation in subjects with STGD1	4-methylpyrazole (alcohol dehydrogenase inhibitor)	Intravenous	05-Aug-2020	NCT00346853
Phase 1- Completed	National Eye Institute	Effect of DHA on macular function in subjects with STGD1	Docosahexaenoic Acid (DHA, omega-3 fatty acid)	Oral	02-Jul-2017	NCT00060749
Phase 1/2- Unknown	Catholic University of the Sacred Heart	Short-term Saffron on retinal function in subjects with STGD1	Saffron (antioxidant supplementation)	Oral	19-Jul-2017	NCT01278277
Phase 2- Completed	Stargazer Pharmaceuticals, Inc	Safety, pharmacokinetics and pharmacodynamics of STG-001 in subjects with STGD1	Drug STG-001 (inhibitor of RPE4)	Oral	27-Apr-2021	NCT04489511
Phase 2-Active	Katairo GmbH	Safety and efficacy of soraprazan in subjects with STGD1	Soraprazan (H ⁺ , K ⁺ -ATPase inhibitor)	Oral	14-May-2019	2018-001496-20
Phase 2- Completed	Stargazer Pharmaceuticals Inc.	Safety, pharmacokinetics and pharmacodynamics of STG-001 in subjects with STGD1	STG-001	Oral	27-Apr-2021	NCT04489511
Phase 1- Active	RBP4 Pty Ltd	Safety and tolerability of LBS-008 in healthy adults	LBS-008	Oral	20-Jan_2020	ACTRN 12618001823268
Phase 1/2- Recruiting	National Eye Institute	Safety and possible efficacy of metformin to slow the rate of STGD1 retinopathy	Metformin	Oral	04-May-2021	NCT04545736

Supplemental Table S2. Registered clinical trials of gene therapies for Stargardt disease. Information is available online at www.clinicaltrials.gov. Accessed on 16-Jun-2021.

Phase/ Recruitment Status	Sponsor	Evaluation	Intervention	Route of administration	Updated	Identifier
Phase 1/2- Terminated	Sanofi	Safety and tolerability of ascending doses of SAR422459 and its biological activity	SAR422459	Subretinal	05-Jun-2020	NCT01367444
Phase 1/2- Active, not recruiting	Sanofi	Long-term safety and tolerability of SAR422459 and its biological activity	SAR422459	Subretinal	11-Aug-2020	NCT01736592

Supplemental Table S3. Registered clinical trials of cell-based therapies for Stargardt disease. Information is available online at www.clinicaltrials.gov.

Accessed on 16-Jun-2021.

Phase/ Recruitment Status	Sponsor	Evaluation	Study type/Intervention	Route of administration	Updated	Identifier
Phase 1/2- Completed	Astellas Institute for Regenerative Medicine	Safety and tolerability of MA09-hRPE cellular therapy	human ESCs-derived RPE (MA09-hRPE) cells	Subretinal	23-Feb-2017	NCT01345006
Phase 1/2- Completed	Astellas Institute for Regenerative Medicine	Long-term safety and tolerability of MA09-hRPE cellular therapy	Observational study of human ESCs-derived RPE (MA09-hRPE) cells	Subretinal	13-Nov-2020	NCT02445612
Phase 1/2- Completed	Astellas Institute for Regenerative Medicine	Safety, tolerability and potential efficacy endpoints of RPE cellular therapy	human ESCs-derived RPE(MA09-hRPE) cells, five cohorts with 4 doses of cells	Subretinal	23-Feb-2017	NCT01469832
Phase 1/2- Completed	Astellas Institute for Regenerative Medicine	Long-term (1-5 years) safety and tolerability of MA09-hRPE cellular therapy	Observational study of human ESCs-derived RPE (MA09-hRPE) cells	Subretinal	10-Dec-2020	NCT02941991
Phase 1/2- Unknown	Southwest Hospital, China	Safety and therapeutic effect of human embryo stem cell derived RPE therapy	human ESCs-derived RPE cells	Subretinal	31-Jan-2018	NCT02749734
Phase 1- Enrolling	University of California, Davis	Safety and feasibility of CD34+ stem cell therapy	autologous adult CD34+ bone marrow stem cells	Intravitreal	28-Apr-2021	NCT01736059
Phase I- Unknown	CHABiotech Co., Ltd	Safety and tolerability RPE cellular therapy	human ESCs-derived RPE(MA09-hRPE) cells	Subretinal	18-Feb-2015	NCT01625559
Phase 1/2- Completed	Federal University of São Paulo	Safety of MA09-hRPE cellular therapy	human ESCs-derived RPE in suspension/seeded onto a polymeric	Subretinal	22-Jul-2020	NCT02903576
N.A.- Recruiting	MD Stem Cells	Efficacy of autologous bone marrow derived stem cell therapy	autologous bone marrow-derived stem cells	retrobulbar, subtenon, intravitreal, intraocular, subretinal, intravenous	24-Feb-2021	NCT03011541
N.A.-Enrolling by invitation	MD Stem Cells	Efficacy of autologous bone marrow derived stem cell therapy	autologous bone marrow-derived stem cells	retrobulbar, subtenon, intravitreal, intraocular, subretinal, intravenous	23-Oct-2019	NCT01920867
Phase 1- Enrolling by invitation	Pomeranian Medical University Szczecin	Safety and effectiveness of autologous bone marrow-derived stem/progenitor cell therapy	autologous bone marrow-derived stem/progenitor cells	intravitreal	17-Dec-2018	NCT03772938