Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
A Prospective, Multicenter, Open-Label, Single-Arm Study of the Safety and Tolerability of a Single, Intravitreal Injection of Human Retinal Progenitor Cells (jCell) in Adult Subjects with RP NCT02320812 jCyte, Inc.	Prospective, multicenter, open-label, single-arm study of safety and tolerability.	This study evaluates the safety and potential activity of a single dose of live human retinal progenitor cells (jCell) administered to adults with RP. Two different dose levels of cells will be assessed in each of two groups of patients.	June 2015 December 2016 n=28	Number of subjects with adverse events as a measure of safety and tolerability through 12 months.	Effect of treatment on ocular function through 12 months.	Clinical diagnosis of RP confirmed by electroretinogram (ERG) and willing to consent to mutation typing, if not already done, BCVA 20/63 or worse and no worse than HM (hand motions) Adequate organ function and negative infectious disease screen	Eye disease other than RP that impairs visual function Pseudo-RP, cancer-associated retinopathies
Feasibility and Safety of Adult Human Bone Marrow-Derived Mesenchymal Stem Cells by Intravitreal Injection in Patients With RP NCT01531348 Mahidol University	Single group assignment, open label, safety study.	The purpose of this study is to determine the feasibility and safety of adult human bone marrow-derived mesenchymal stem cells by intravitreal injection in patients with RP.	February 2012 Expected date of completion March 2014 Entry states “enrolling by invitation only” No results or publications n=10	Change from baseline in laser flare and cell measurements through 12 months.	Change from baseline in visual function tests through 12 months.	RP Central visual field less than or equal to 20 degrees Best corrected visual acuity less than 6/120 by Snellen visual acuity chart ERG nonrecordable or the amplitudes were less than 25% of normal	Other eye conditions that could mask the interpretation of the results

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
A Pilot Clinical Trial of the Feasibility and Safety of Intravitreal Autologous Adult Bone Marrow Stem Cells in Treating Eyes With Vision Loss from Retinopathy NCT01736059 University of California, Davis	Single group assignment, open label, safety/ efficacy study.	This pilot study is to determine whether it would be safe and feasible to inject CD34+ stem cells from bone marrow into the eye as treatment for patients who are irreversibly blind from various retinal conditions.	July 2012 December 2017 n=15	Incidence and severity of ocular adverse events through 6 months.	The number of stem cells isolated and injected into the study eye.	Visual acuity 20/100 to CF Duration of vision loss >3 months Vision loss from macular degeneration, RP, retinal vein occlusion or diabetic retinopathy No active eye or systemic disease No history of macular edema or retinal/choroidal neovascularization requiring treatment within 6 months No significant media opacity No coagulopathy or other hematologic abnormality No concurrent immunosuppressive therapy	Allergy to fluorescein dye Other concurrent retinal or optic nerve disease affecting vision
Bone Marrow Derived Stem Cell Ophthalmology Treatment Study NCT01920867 Retina Associates of South Florida	Non-randomized efficacy study with open label parallel assignment.	This study will evaluate the use of autologous bone marrow derived stem cells (BMSC) for the treatment of retinal and optic nerve damage or disease.	August 2013 August 2017 n=300	BCVA will be measured with Snellen Eye Chart and the ETDRS Chart when available at each post-procedure visit. Intervals at minimum will be first post-procedure day, then 3 months, 6 months and	Visual fields will be evaluated with automated perimetry during post-procedure visits as needed and specifically at 6 months and 12 months.	Have objective, documented damage to the retina or optic nerve unlikely to improve OR Have objective, documented damage to the retina or optic nerve that is progressive AND have less than or equal to 20/40 best corrected central visual acuity in one or both eyes AND/OR an abnormal visual field in one or both	Patients who are not capable of an adequate ophthalmologic examination or evaluation to document the pathology. Patients who are not capable or not willing to undergo follow up eye exams with the principle investigator or their ophthalmologist or optometrist as outlined in the protocol. Patients who may be at significant risk to general health or to the eyes and

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
				12 months post-procedure day. Recommended visit 1 month post-procedure day.		eyes. Be at least 3 months post-surgical treatment intended to treat any ophthalmologic disease and stable. If under current medical therapy (pharmacologic treatment) for a retinal or optic nerve disease be considered stable on that treatment and unlikely to have visual function improvement (for example, glaucoma with intraocular pressure stable on topical medications but visual field damage). Have the potential for improvement with BMSC treatment and be at minimal risk of any potential harm from the procedure.	visual function should they undergo the procedure.

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
First-in-human Phase I/IIa, Open-Label, Prospective Study of the Safety and Tolerability of Subretinally Transplanted Human Retinal Progenitor Cells (hRPC) in Patients With Retinitis Pigmentosa (RP) NCT02464436 ReNeuron Limited	First-in-human Phase I/IIa, Open-Label, Prospective Study of the Safety and Tolerability of Subretinally Transplanted Human Retinal Progenitor Cells (hRPC) in Patients With RP.	hRPC is a cell therapy for RP. This is a first-in-human, dose escalation study in which participants with RP will receive a single subretinal injection of hRPC cells in one eye to evaluate safety and tolerability. Participants will be followed for one year. Additional testing will seek to establish any preliminary efficacy from hRPC.	December 2015 September 2017 n=15	Safety as assessed by the absence of any grade 3 or greater AE considered "related" to hRPC at 6 months.	Visual acuity Visual field Transplant and host retina integrity and survival.	RP based upon one or more of the following: clinical features, electrophysiological measures and genetic testing, if available (genetic confirmation is not obligatory). BCVA of 20/200 or worse in the study eye (in patients with differing acuities between eyes, the worse eye will be enrolled as the study eye Medically able to undergo vitrectomy and subretinal injection, a surgery which may require general anesthesia. Good general health	The presence of ocular disease or ocular media opacity, which in the opinion of the investigator, precludes accurate evaluation during the study. Prior vitrectomy in the study eye Patients with a history of amblyopia will be excluded High myopia (>6 diopters) in the study eye Cataract surgery in the study within 3 months Participation within 6 months in any clinical trial involving a drug or device treatment No prior stem cell injections in any part of the body
Phase I Clinical Trial of Intravitreal Injection of Autologous Bone Marrow Stem Cells in Patients with Retinitis NCT02280135 Red de Terapia Celular	Prospective, single-center, randomized, parallel, double-blind, phase I placebo-controlled clinical trial.	The purpose of this study is to evaluate the safety of intravitreal injection of autologous bone marrow stem cells in patients with RP.	November 2014 August 2016 n=10	Rate of serious and non-serious adverse events related with the use of bone marrow mononuclear cells through 12 months	Quality of Life: Questionnaire VFQ-25 Visual acuity (VA): Test ETDRS Color Vision: Ishihara Color Test Contrast sensitivity: CSV-1000E Intraocular	RP bilateral diagnosis Visual acuity (measured with ETDRS) less than or equal to 20/70 and visual field below 30° central in both eyes.	Concurrence of any systemic or ocular disease that precludes or affects tracking study variables. Specifically retinal involvement with diabetes mellitus, glaucoma, macular degeneration or age Eye surgery in the previous 6 months

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
					pressure (IOP): measured in mm Hg with applanation tonometer Haag Streit AT 900, Examination of the anterior and posterior pole: Made with biomicroscopy (BMC) Width of retinal macula layer and nerve fiber: Measured with Optical Coherence Tomography Spectral domain (OCT) (Topcon 3D OCT-2000 Spectral Domain OCT) Visual field (VF) and macular sensitivity (The Humphrey perimeter), Study eye fundus: Made by Retinography and Angiography fluorescein Electrical retinal function:		

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
					electroretinogram (ERG) (altered/ unaltered) Visual evoked potentials with Pattern Reversal (VEP) (altered/ no altered).		
Autologous Bone Marrow-Derived CD34+, CD133+, and CD271+ Stem Cell Transplantation for RP NCT02709876 Stem Cells Arabia	Single arm, single center trial to evaluate the safety and efficacy of autologous purified populations of bone-marrow derived stem cells in patients with RP(BM-SCs) through a 48 month follow up period.	To assess the safety and efficacy of purified adult autologous bone marrow derived CD34+, CD133+, and CD271+ stem cells through a 48-month follow-up period. The combination of these three cell types was based on their diverse potentialities to differentiate into specific functional cell types to regenerate damaged retinal tissue, and the availability of clinical-grade purification system (CliniMACS) and microbeads to	April 2014 March 2019 n=50	ETDRS visual acuity change through 12 months	Quality of life Color vision Contrast sensitivity	RP Visual acuity (ETDRS) less than or equal to 20/70 and visual field below 30° central in both eyes BCVA less than 6/120 by Snellen visual acuity chart	Other eye conditions that could mask the interpretation of the results

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
		purify the target cell populations in clinically-approved methods.					
An Open Labeled Clinical Study to Evaluate the Safety and Efficacy OF Autologous Bone Marrow Derived Mono Nuclear Stem Cell (BMMNCs) in RP. NCT01914913 Chaitanya Hospital, Pune	Clinical Study to Evaluate Safety and Efficacy of BMMNC in RP.	To study the safety and efficacy of autologous bone marrow derived Mono Nuclear Stem Cell (BMMNCs) in patients with RP.	September 2014 November 2016 n=15	Visual Acuity through 12 months	NR	RP Willingness to undergo bone marrow and umbilical cord derived Mesenchymal stem cell transplantation	Complications of diabetic retinopathy
Modulating Ocular/Retinal Blood Flow and Visual Function in RP NCT02086890 Nova Southeastern University. Collaborator: National Eye Institute	Phase I and II triple-masked (patient, investigator, outcome assessor), randomized crossover study assessing the following procedures: electro-acupuncture, laser acupuncture, TES, sham electro-	To gain a better understanding of possible changes in ocular and retinal blood flow and measures of vision in patients with RP receiving 2 promising therapies, electro-acupuncture and TES	August 2014 June 2016 Ongoing but not recruiting subjects n=21	Significant changes from baseline in ocular and retinal blood flow through 12 weeks after intervention	Significant changes from baseline in dark adaptation function through 12 weeks after intervention using the AdaptDx by Maculogix Significant changes from baseline in multifocal electro-retinogram through 12 weeks after intervention initiation Significant	Diagnosis of RP BCVA better than 20/400 in at least 1 eye More than 20% loss of Goldmann visual field area (III4e test target) in at least 1 eye	Very severe vision losses in both eyes (e.g., hand motions or light perception only) with difficulty performing the proposed vision tests Vision loss due to ocular diseases other than RP, cystoid macular edema, or cataracts Previous acupuncture or TES treatment for RP

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
	acupuncture, sham laser acupuncture, sham TES				changes from baseline in Goldmann visual field area through 12 weeks after intervention initiation Significant changes from baseline in best-corrected ETDRS visual acuity through 12 weeks after intervention initiation Significant changes from baseline in contrast sensitivity through 12 weeks after intervention initiation OCT through 12 weeks after intervention initiation Changes in macular edema		

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
A Phase I/IIa, Open-Label, Single-Center, Prospective Study to Determine the Safety and Tolerability of Sub-retinal Transplantation of Human Embryonic Stem Cell Derived Retinal Pigmented Epithelial (MA09-hRPE) Cells in Patients with Advanced Dry Age-related Macular Degeneration (AMD) NCT01674829 CHABiotech Co., Ltd	Open-label single-group assignment study	To evaluate the safety and tolerability of MA09-hRPE cellular therapy in patients with advanced dry AMD To evaluate the safety of the surgical procedures when used to implant MA09-hRPE cells To assess the number of hRPE cells to be transplanted in future studies To evaluate on an exploratory basis potential efficacy endpoints to be used in future studies of MA09-hRPE cellular therapy.	September 2012 April 2016 Recruiting patients n=12	Safety of HeSC- derived RPE cells at 12 months, with none of the following: Any grade 2 (NCI grading system) or greater adverse events related to the cell product Any evidence that the cells are contaminated with an infectious agent Any evidence that the cells show tumorigenic potential	Change in the mean of BCVA Autofluorescence photography Reading speed Evidence of successful engraftment will include: Structural evidence (OCT imaging, FA, slit lamp examination with fundus photography) that cells have been implanted in the correct location, electro-retinographic evidence (mfERG) showing enhanced activity in the implant location	Clinical findings consistent with advanced dry AMD with evidence of 1 or more areas of >250 microns of GA involving the central fovea (GA defined as attenuation or loss of RPE as observed by biomicroscopy, OCT, and FA) No evidence of current or prior CNV in the treated eye The BCVA of the eye to receive the transplant will be no better than 20/400 BCVA of the eye that is NOT to receive the transplant will be no worse than 20/400 Electrophysiological findings consistent with advanced dry AMD	Presence of active or inactive CNV in the eye to be treated Presence or history of retinal dystrophy, RP, chorioretinitis, central serious choroidopathy, diabetic retinopathy, or other retinal vascular or degenerative disease other than AMD History of optic neuropathy Macular atrophy due to causes other than AMD Presence of glaucomatous optic neuropathy in the study eye Uncontrolled IOP, or use of 2 or more agents to control IOP Cataract of sufficient severity likely to necessitate surgical extraction within 1 year History of retinal detachment repair in the study eye Axial myopia of greater than -8 diopters Axial length greater than 28 mm Any other sight-threatening ocular disease Any history of retinal vascular disease (compromised blood-retinal barrier) Glaucoma, uveitis, or other intraocular inflammatory disease

Table C-33. Ongoing clinical trials of other treatments on the horizon (10 studies) (continued)

Clinicaltrials.gov Title Clinicaltrials.gov Identifier Sponsor	Study Design	Purpose	Start Date Expected Completion Date Estimated Enrollment	Primary Outcomes	Secondary Outcomes	Eye-specific Inclusion Criteria	Eye-specific Exclusion Criteria
							Significant lens opacities or other media opacity Ocular lens removal within previous 3 months Ocular surgery in the study eye in the previous 3 months

AMD=age-related macular degeneration; BCVA=best corrected visual acuity; CNV=choroidal neovascularization; DTL= Dawson-Trick-Litzkow; ETDRS=Early Treatment Diabetic Retinopathy Study (test); FA=fluorescein angiography; GA=geographic atrophy; HeSC=human embryonic stem cell; IOP=intraocular pressure; NR=not reported; OCT=optical coherence tomography; RP=retinitis pigmentosa; RPE=retinal pigment epithelium; TES=transcorneal electrical stimulation