Abstract 81

A PHASE 1/2A STUDY USING AUTOLOGOUS INDUCED PLURIPOTENT STEM CELL DERIVED RETINAL PIGMENT EPITHELIUM FOR TREATMENT OF ADVANCED DRY AGE RELATED MACULAR DEGENERATION

Oral

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Purpose:

To report the successful subretinal surgical placement of an autologous induced pluripotent stem cell-derived retinal pigment epithelium (iPSC-RPE) implant in a subject with geographic atrophy (GA) secondary to advanced, dry age-related macular degeneration (AMD).

Methods:

A Phase 1/2a study assessing the safety and preliminary efficacy of subretinal, autologous iPSC-RPE implant for treatment of AMD is being conducted at the National Eye Institute. The study will enroll the worse-seeing eye of up to 20 subjects aged 55 years or older with GA and best corrected visual acuity of 20/80 or worse. Detailed inclusion criteria are online (NCT04339764). Each subject will receive one 2x4mm implant consisting of autologous iPSC-RPE monolayer on a biodegradable scaffold intended to straddle the border of GA. Surgery is performed using pars plana vitrectomy approach and a custom investigational injector.

Results:

An 89 year old male with severe, advanced, bilateral GA resulting from AMD underwent successful iPSC-RPE implant placement in their worse-seeing eye (BCVA 20/125). The surgery was performed as planned using a commercially available 23 gauge PPV system and intraoperative OCT (iOCT) to assist with targeted subretinal hydrodissection overlying GA. The implant was placed in the subretinal space straddling the border of GA. Sulfur hexafluoride gas tamponade was used. A single dose of intravenous Solumedrol was administered immediately before surgery without oral immunosuppressive medication. No serious adverse events have been reported resulting from the surgical procedure or implant placement.

Conclusions:

Targeted subretinal delivery of an autologous iPSC-RPE straddling the border of GA in subjects with severe, advanced dry age-related macular degeneration is feasible. Efficacy and safety of the surgery and implant will be assessed at one year in this ongoing study.