INTRODUCTION

Age-related macular degeneration (AMD) is the leading cause of blindness in people ≥ 65 in the developed world and affects millions of patients [1]. There are currently no FDA-approved therapies beyond supplementation. In dry AMD, the retinal pigment epithelial (RPE) cells are damaged leading to atrophy and eventual cell death, which results in photoreceptor degeneration and loss of vision [2]. In wet AMD, subretinal neovascularization (NV) occurs due to the growth of pathological new vessels, which causes macular edema and retinal detachment [3]. A variety of visual defects can result, including progressing to legal blindness and losing independence [4]. Despite the significant impact of AMD, currently no approved therapies are available for the subretinal NV.[5]

OBJECTIVES

- To assess the safety and tolerability of cell implantation in patients with advanced dry AMD
- To evaluate the efficacy of subretinal transplantation of hESC cells in patients with advanced dry AMD
- To assess the long-term safety and tolerability of cell implantation

METHODS

- Patients (n=25) received subretinal transplantation of hESC cells (n=9), subretinal implantation of a pigment cell implant (n=2), or a combination of both (n=14)
- Follow-up visits were scheduled at 1, 3, 6, 12, and 24 months

RESULTS

- No significant adverse events were observed
- Improvement in visual acuity, OCT, and ICG imaging

SYSTEMIC AND OCULAR SAFETY AND TOLERABILITY OBSERVATIONS IN SUBJECTS TREATED (N=25)

- No systemic adverse events were reported
- Ocular adverse events included vision loss, dry eye, and photophobia

CONCLUSIONS

- Subretinal transplantation of hESC cells is feasible with no significant adverse events
- Improvement in visual acuity and OCT imaging suggests potential benefit

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Figure 16. Asymmetrical, reduced growth of GA in the treated area.

Figure 17. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 8. Comparison of QFAF transplanted and nontreated areas in patients with advanced dry AMD

Figure 9. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 10. Subretinal cell implantation was reported in one subject 2 weeks after surgery. Although no change of surgical procedure, transplanted and nontreated areas were significantly different

Figure 11. Comparison of QFAF transplanted and nontreated areas in patients with advanced dry AMD

Figure 12. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 13. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 14. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 15. Subject 8, Cohort 3, Selected images of drusen reduction

Figure 1. Integration and function of transplanted hESC-RPE in rat. High magnification fluorescence micrographs of hESC-RPE (orange) transplanted into the subretinal area of a 250 minipig post-death eye (Fig. 1A). The transplanted cells (n=25) were labeled with Oregon Green 488 (blue) and human RPE were labeled with Texas Red 590 (red) and DAPI (green). The panel shows the therapeutic potential of hESC-RPE cells transplanted into the subretinal area of a 250 minipig post-death eye. The transplanted cells are present in the subretinal area and the immunohistochemical staining is maintained for at least 12 weeks. The figure shows the integration and function of transplanted hESC-RPE in rat.