

**A bioengineered retinal pigment epithelial monolayer for advanced, dry age-related macular degeneration.**

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**Public Summary:**

Retinal pigment epithelium (RPE) dysfunction and loss are a hallmark of non-neovascular age-related macular degeneration (NNAMD). Without the RPE, a majority of overlying photoreceptors ultimately degenerate, leading to severe, progressive vision loss. Clinical and histological studies suggest that RPE replacement strategies may delay disease progression or restore vision. A prospective, interventional, U.S. Food and Drug Administration-cleared, phase 1/2a study is being conducted to assess the safety and efficacy of a composite subretinal implant in subjects with advanced NNAMD. The composite implant, termed the California Project to Cure Blindness-Retinal Pigment Epithelium 1 (CPCB-RPE1), consists of a polarized monolayer of human embryonic stem cell-derived RPE (hESC-RPE) on an ultrathin, synthetic parylene substrate designed to mimic Bruch's membrane. We report an interim analysis of the phase 1 cohort consisting of five subjects. Four of five subjects enrolled in the study successfully received the composite implant. In all implanted subjects, optical coherence tomography imaging showed changes consistent with hESC-RPE and host photoreceptor integration. None of the implanted eyes showed progression of vision loss, one eye improved by 17 letters and two eyes demonstrated improved fixation. The concurrent structural and functional findings suggest that CPCB-RPE1 may improve visual function, at least in the short term, in some patients with severe vision loss from advanced NNAMD.

**Scientific Abstract:**

Retinal pigment epithelium (RPE) dysfunction and loss are a hallmark of non-neovascular age-related macular degeneration (NNAMD). Without the RPE, a majority of overlying photoreceptors ultimately degenerate, leading to severe, progressive vision loss. Clinical and histological studies suggest that RPE replacement strategies may delay disease progression or restore vision. A prospective, interventional, U.S. Food and Drug Administration-cleared, phase 1/2a study is being conducted to assess the safety and efficacy of a composite subretinal implant in subjects with advanced NNAMD. The composite implant, termed the California Project to Cure Blindness-Retinal Pigment Epithelium 1 (CPCB-RPE1), consists of a polarized monolayer of human embryonic stem cell-derived RPE (hESC-RPE) on an ultrathin, synthetic parylene substrate designed to mimic Bruch's membrane. We report an interim analysis of the phase 1 cohort consisting of five subjects. Four of five subjects enrolled in the study successfully received the composite implant. In all implanted subjects, optical coherence tomography imaging showed changes consistent with hESC-RPE and host photoreceptor integration. None of the implanted eyes showed progression of vision loss, one eye improved by 17 letters and two eyes demonstrated improved fixation. The concurrent structural and functional findings suggest that CPCB-RPE1 may improve visual function, at least in the short term, in some patients with severe vision loss from advanced NNAMD.

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