# Predictive Relationship of OCT Characteristics for Efficacy in an Intravitreal Injection of Allogeneic Human Retinal Progenitor Cells (hRPC) for the Treatment of Retinitis Pigmentosa

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## Introduction

- Ellipsoid zone (EZ) loss and the thinning of the outer retina are potential structural surrogates for disease severity in retinitis pigmentosa (RP).
- A Phase 2b trial was conducted to evaluate the intravitreal injection of allogeneic hRPC for treatment of RP.

## Purpose

To examine the baseline Spectral-domain optical coherence tomography (SD-OCT) imaging characteristics to identify objective predictors of efficacy in a more responsive target population in a phase 2b trial of intravitreal injection of allogeneic hRPC for the treatment of RP.

## Methods

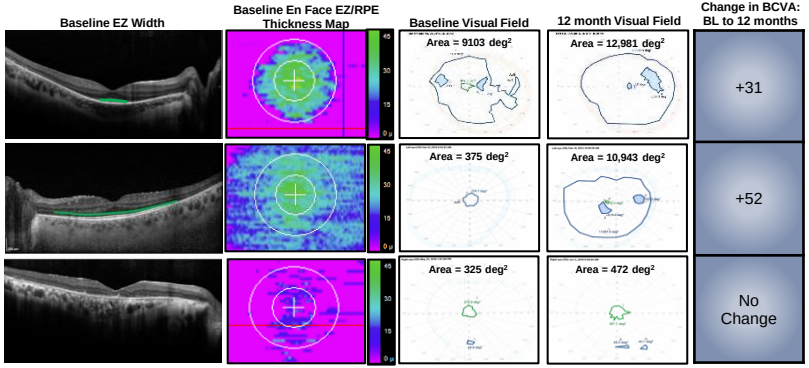
- In a post hoc analysis, a target subgroup of patients with less measurement variability was identified.
- Heidelberg 97 line SD-OCT (n=29; sham, n=10, low dose, n=9, high dose, n=10) were analyzed.
- Automated segmentation with manual correction was performed using a customized software platform
- OCT and EZ metrics were calculated including: mean central foveal thickness (CFT), EZ-RPE subfield or mid-subfield volume and thickness.
- Correlational analysis was performed between each OCT parameter and change in each Phase 2b trial endpoint from baseline to 12 months (Best Corrected Visual Acuity, Contrast Sensitivity, Kinetic Visual Fields, Low Luminance Mobility Test, and a VFQ).

## Results

| Change in Endpoint | EZ Thickness (Mid Subfield) Sham *(n=10)* | EZ Thickness (Mid Subfield) 6.0 x $10^6$ jCell *(n=10)* | Mean Central Foveal Thickness 6.0 x $10^6$ jCell *(n=10)* |
|---|---|---|---|
| BCVA | 0.32 | 0.60 | 0.88* |
| Visual Field | -0.10 | 0.65* | 0.78* |
| Contrast Sensitivity | 0.32 | 0.81* | 0.79* |
| Critical Illumination Level | 0.10 | 0.45 | 0.72* |
| VFQ | 0.12 | 0.75* | 0.82* |

**Table 1 Endpoint Change Correlations** **Significant value, p<0.05*

- 29 eyes from 29 subjects with advanced RP (mean BCVA 1.02 logMAR or 20/200; mean age 46)
- Moderate to strong correlations in all trial endpoints in the high dose (single injection of 6 x $10^6$ cells) target group were seen in mean central foveal thickness and mid-subfield mean EZ thickness
- Greater values in each parameter corresponded to greater improvement in endpoints
- The low dose (3 x $10^6$ cells) did not demonstrate a significant relationship




**Examples of Baseline EZ Metrics Corresponding to Visual Field/BCVA Changes at 12mo**

## Conclusion

- These results suggest in the high dose treatment group a correlation between outcomes and OCT baseline parameters of mid-subfield mean EZ thickness and mean central foveal thickness
- As there are a small number of subjects in the target population subset and given the post hoc nature of the analyses, these results should be viewed cautiously
- A certain minimum EZ or mean CFT thickness may facilitate selection of a population with the greatest chance for response in a Phase 3 study from the neurotrophic effects of hRPCs in RP patients

## References

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## Financial Disclosures

Disclosures:
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