Appendix XVII: Summary of Preliminary Data Analysis Corneal Trial

Primary Endpoint

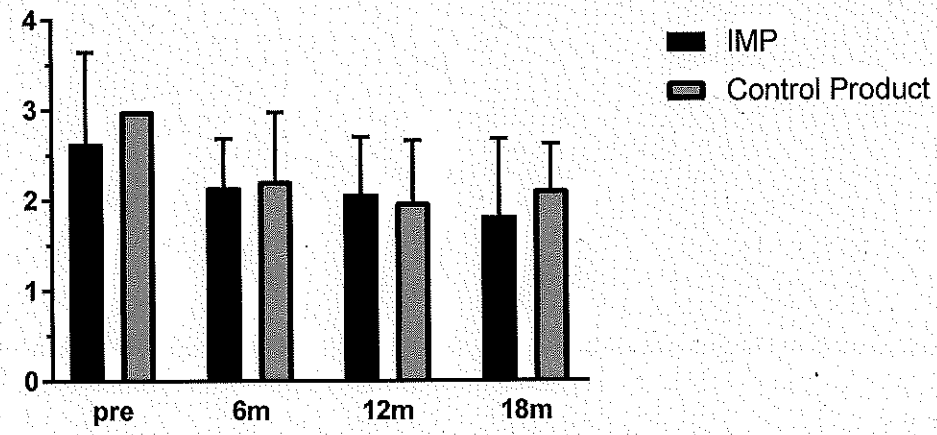
Visual acuity assessments generate the following LogMAR scores. In this analysis, "Perceives Light" was given a score of 4, this is not included in standard LogMAR scales.

	LogMAR
Perceives Light	4
Hand Movement	3
Counts Fingers	2
u/a 1/60	1.78
u/a 2/60	1.48
u/a 3/60	1.3
u/a 4/60	1.18
u/a 5/60	1.08
u/a 6/24	0.6

Visual acuity was compared between groups receiving product or control product, and also compared between patients with Aniridia or other diseases irrespective of the presence of cells in the product.

Comparisons were made including patients who received other medical interventions that could have improved sight, and without.

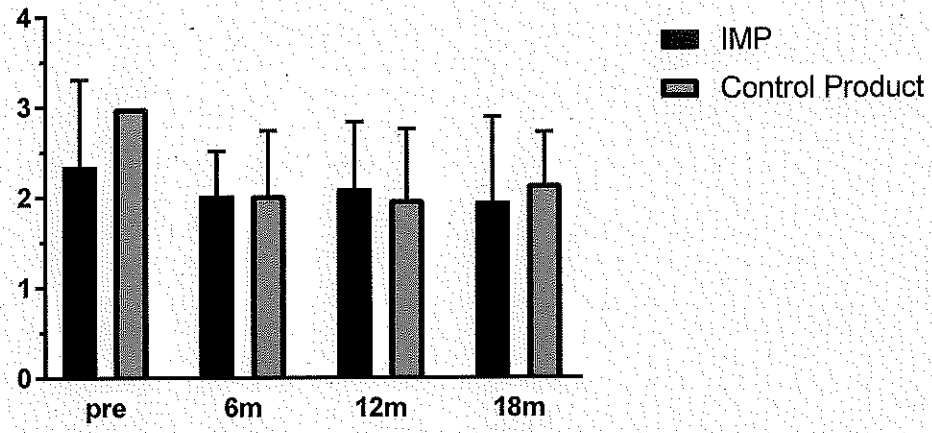
Visual Acuity – treated versus control, all diseases, all patients



Mean LogMAR scores +/- SD (lower=better). "Pre" control product group all scored 3 for LogMAR, hence no variance. IMP and control groups had similar levels of visual acuity at the beginning (no significant difference). There was no significant difference in LogMAR score between groups at any time point. The overall trend was downwards, and 5/8 in the IMP and 4/5 in the control groups had lower LogMAR scores at the end of the trial. The changes in LogMAR scores from trial entry to the

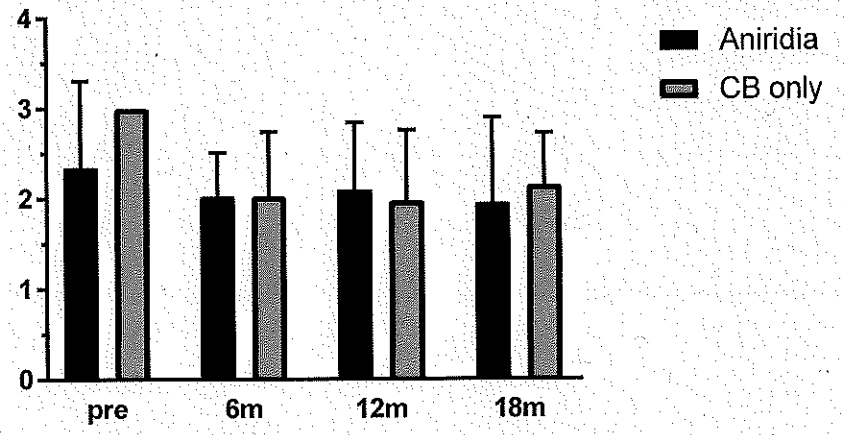
18 month time point were not significantly different in or between the IMP or control groups.

Visual Acuity – treated versus control, patients with cataract operations removed from data set from time operations carried out.



Removing data from patients who received cataract operations did not affect the overall results, if anything the results in the IMP and control groups converged further (there were 2 cataract operations in the IMP and 1 in the control groups). In the IMP groups 3 patients had improved vision, 2 no change and 1 deteriorated at 18 months. In the control product group, 3 improved and one was no change at 18 months.

Visual Acuity Scores Aniridia versus Chemical Burns



There was no significant difference in mean visual acuity scores between the 2 disease groups (grouped by disease, irrespective of treatment).. Aniridia n=5, CB n=6. The data was skewed in that 4/5 Aniridia received IMP, and 2/6 CB received IMP.

Removing patients from the analysis who received a cataract operation did not change these results.

Secondary Endpoint – Ocular Surface Scores

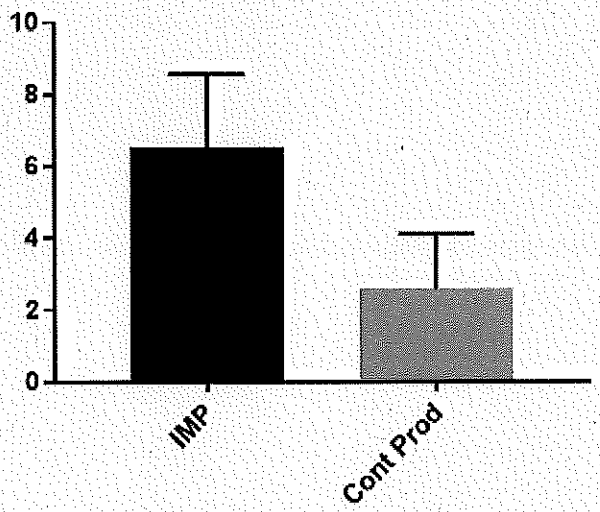
Ocular surface scores were generated by examination of 5 different parameters:

corneal epithelium
conjuctivilisation
corneal neovascularisation
corneal opacification
conjunctival hyperemia

Each was scored 0-3 with 0 representing a normal eye. These scores were combined to give a score out of 15 for each time point in the trial where 0= a normal eye, and 15 = the most damaged eye. The starting combined ocular scores in the treated (10.55) and control groups (11.1) were not significantly different. Statistical analysis was carried out using unpaired t-tests and one way Anova.

Comparison Treatment vs. Control Groups – 18 months

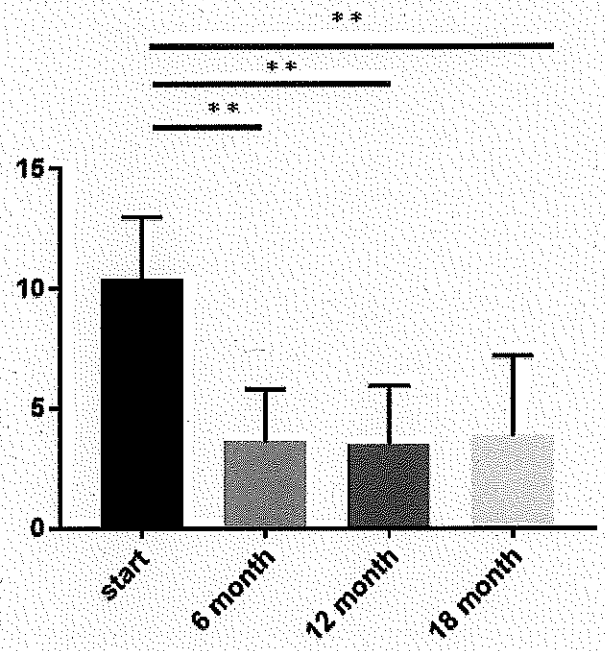
8 patients in the treatment group and 5 patients in the control group had ocular surface scores which were evaluated throughout, and at conclusion of the trial. All patients showed lower (improved) ocular surface scores at the conclusion of the trial. However, patients who received the stem cell product showed a significantly higher mean improvement in combined ocular surface scores than those who received the control (p=0.0040, unpaired t-test).



Graph shows change in OSS score from pre-trial values (i.e. magnitude of reduction in score). IMP – sd = 2.07, CV= 31.8%. Control Product sd= 1.42, CV= 57.7%

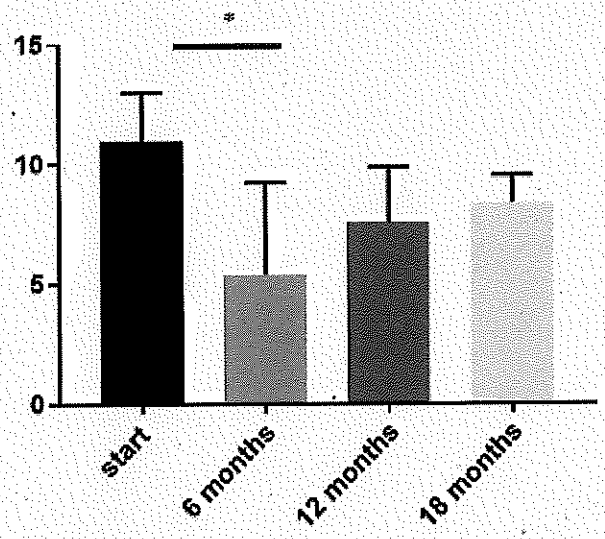
Comparison IMP vs. Control Groups – Throughout trial

IMP



Mean OSS scores +/-sd. The treated group showed a significant and sustained reduction (improvement) in combined ocular surface scoring, which reached statistical significance at 6 months and was maintained until 18 months p=0.0043, p=0.0018, p=0.0002 respectively. One-way Anova Tukey's Multiple Comparisons test.

Control Product



Mean OSS scores +/-sd In the control group there was also an initial, significant improvement in scores (p=0.0138), but the effect was not sustained, and the improvement in scores returned to not being statistically significant by 12 months, sustained at 18 months (p= 0.1848 and p=0.390 respectively). One-way Anova, Tukey's Multiple Comparisons test.