MM958: Statistical Modelling and Analysis – Scientific Summary

University of Strathclyde

Chapter 1 – Background & Introduction

Myopia effects nearly half of all young adults in the US and Europe, particularly young populations. (Karpecki, 2024) Recent literature has suggested that tropical atropine may slow myopia progression, though dosing and validity remains debated. This scientific report investigated the efficacy of different atropine concentrations (0.01% and 0.02%) compared to placebo in managing myopia progression. The research aimed to evaluate changes in Spherical Equivalent Refraction (SER) over a 36-month period across treatment groups, whilst accounting for potential clinical centre effects.

Chapter 2 – Materials and Methods

The study employed a repeated measures design incorporating both between-subject (treatment) and within-subject (time) factors. A Repeated Measures Design is a research design that involves testing the same subjects under two or more conditions to analyse if there is a statistically significant difference among groups by partitioning variability into SS subjects and SS residual. (ScienceDirect, 2024)

The investigation included 180 patients distributed across four clinical centres. Patients received either 0.01% atropine, 0.02% atropine, or placebo, with SER measurements taken at baseline, 12, 24, and 36 months.

Statistical analysis was conducted using R version 4.40 with various analytical packages including “lme4” and “nlme”. A linear mixed-effects model was implemented to account for the hierarchical data structure, with treatment and time as fixed effects, and patient and clinical centre as random effects.

Model assumptions were assessed through residual diagnostics and normality plots. The significance level was set at 0.05 for all statistical tests. Treatment effects were evaluated using planned contrasts, specifically comparing the average atropine effect against placebo.

Chapter 3 – Results

The analysis revealed significant main effects for both treatment ($F(2,177) = 113.33$, $p < 0.001$) and time ($F(1,539) = 53.90$, $p < 0.001$), along with a significant treatment-by-time interaction ($F(6,531) = 42.98$, $p < 0.001$). The variance component analysis indicated that individual patient differences accounted for 37.54% of total variance, suggesting substantial between-patient variability in treatment response.

The planned contrast analysis demonstrated significantly better outcomes for atropine treatments compared to placebo ($t(174) = 13.969$, $p < 0.001$), with a positive estimate of 1.0 (SE = 0.0716). The 0.01% atropine group showed the most favourable outcomes, maintaining better SER values throughout the study period compared to both 0.02% atropine and placebo groups.

Model diagnostics confirmed that assumptions of linearity and homoscedasticity were adequately met, with residual plots showing appropriate random scatter around zero. The normal Q-Q plot demonstrated reasonable normality, with only minor deviations at the extreme tails.

Chapter 4 – Discussion and Conclusion

The findings provided strong evidence for the effectiveness of atropine in slowing myopia progression, with the 0.01% concentration demonstrating particularly promising results. The significant treatment-by-time interaction suggested that treatment effects varied over the study period, with initial improvements followed by gradual decline, though remaining superior to placebo.

Several limitations warrant consideration. The study's duration of 36 months, whilst substantial, may not capture very long-term effects. Additionally, the observed centre-level variation, though minimal, suggests potential differences in treatment administration or measurement practices across clinical sites.

In conclusion, this investigation provided robust statistical evidence supporting the use of atropine, particularly at 0.01% concentration, for myopia management.

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References

Karpecki, P. (2024, October 26). *Myopia: A Growing Concern for Young Patients*. Retrieved from Rendia: https://rendia.com/resources/insights/myopia-growing-concern-young-patients/

ScienceDirect. (2024, October 26). *Repeasted Measures Design*. Retrieved from ScienceDirect: https://www.sciencedirect.com/topics/social-sciences/repeated-measures-design