

# Comparison of efficacy between intent-to-treat (ITT) and modified ITT populations of NVK002, a novel formulation of low-dose atropine, in treating myopia progression in children in the CHAMP clinical trial

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Background:

Using a modified intent-to-treat (mITT) analysis in subjects who were aged 6 to 10 years at baseline, the CHAMP Phase 3 clinical trial (NCT03350620) demonstrated the effectiveness of topical NVK002; a novel, preservative-free low-dose atropine formulation, in slowing myopia progression over 36 months. We showed that in this subgroup of children, NVK002 0.01%, was statistically significant compared to placebo in increasing the proportion of responders (<0.50D myopia

progression over 3 years) and slowing progression of both axial length (AL) and myopia (spherical equivalent refraction, SER). Here, we compare the efficacy in the entire intent to treat (ITT) set of subjects aged 3 to <17 years at baseline with the mITT population.

## Methods:

US and European subjects aged 3 to <17 years with an SER between  $-0.50$  D to  $-6.00$  D, were treated with once-daily topical placebo or NVK002 0.01% for 36 months. The efficacy endpoints analyzed were the proportion of responders and mean change from baseline in both SER and AL at Month 36 in both the mITT and ITT sets.

## Results:

In the ITT, 165 subjects were randomized to placebo and 164 subjects were randomized to NVK002 0.01%. In the mITT, 144 subjects were randomized to placebo and 133 subjects were randomized to NVK002 0.01%. At Month 36, NVK002 0.01% significantly increased the proportion of responders compared with placebo in both groups (mITT: 17.5% placebo responder vs. 28.5% 0.01% responder; odds ratio 3.9;  $p=0.031$ ; ITT: 21.3% placebo responder vs. 31.6% 0.01% responder; odds ratio 3.9;  $p=0.035$ ). NVK002 0.01% also significantly slowed mean SER progression (mITT: 0.24D least square mean difference [LSMD]; ITT: 0.25D LSMD) and axial elongation (mITT:  $-0.13$ mm LSMD; ITT:  $-0.13$ mm LSMD), with both endpoints at  $p<0.001$  for mITT and ITT.

## Conclusion:

Analyses of the mITT and ITT sets showed comparable percentages of responders to NVK002 0.01% treatment for the two groups. These results suggest that the inclusion of data from subjects of ages 3 to 17 years (ITT) rather than ages 6 to 10 years (mITT) may not alter the efficacy of NVK002 0.01% in the CHAMP trial, and NVK002 0.01% may be suitable to treat myopic children with a broad age range.

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