Elamipretide Topical Ophthalmic Solution for the Treatment of LHON: Outcomes and Efficacy

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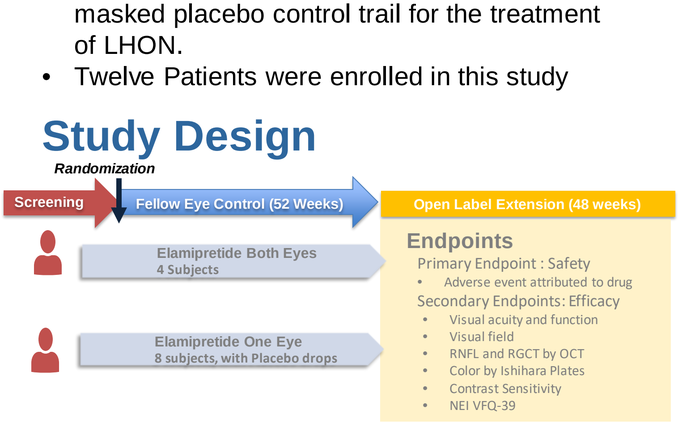
Background

- Elamipretide has been demonstrated to colocalize with cardiolipin in the inner membrane of mitochondria where it alters mitochondrial bioenergetics
- Topical administration of Elamipretide was trialed in a Phase IIB randomized double masked placebo control trail for the treatment of LHON.
- Twelve Patients were enrolled in this study

Study Design



Safety Metrics

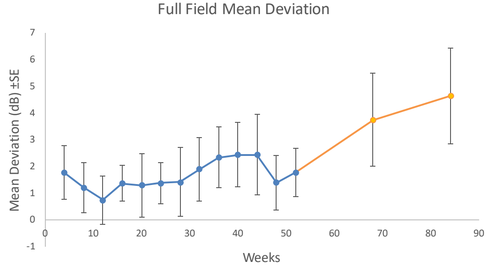
Adverse Events	Placebo (n=8)	Elamipretide (n=16)	Total (n= 24)
≥ one ocular AE	5 (63%)	11 (69%)	16 (67%)
Hyperemia	2 (25%)	2 (12.5%)	4 (16.7%)
Punctate keratitis	1 (12.5%)	3 (18.8%)	4 (16.7%)
Dry eye	0	3 (18.8%)	3 (12.5%)
Irritation	2 (25%)	4 (25%)	6 (25%)
Lacrimation	0	2 (12.5%)	2 (8.3%)

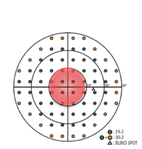
Outcome	Fellow Eye Control (52 wk Avg)				Open Label Extension	
	Placebo	Elamipretide	Effect	P-Value	Effect v Baseline	P-Value
BCVA	*1.3*	*1.9*	*0.6*	*0.43*	*7.1*	*0.051*
Automated Visual Field (MD)	*1.0*	*1.8*	*0.8*	*0.02*	*4.6*	*0.025*
Central Visual Field (MD)	*0*	*1.8*	*1.8*	*<0.001*	*5.2*	*0.054*
Color Discrimination (Plates)	*0.3*	*0.5*	*0.2*	*0.08*	*1.6*	*0.005*
Contrast Sensitivity	*0.09*	*0.035*	*-0.055*	*0.002*	*0.18*	*0.005*
RNFL Thickness	*-3.9*	*-2.0*	*1.9*	*0.43*		
RGCL Thickness	*-4.5*	*-3.0*	*1.5*	*0.12*		
Quality of Life (NEI VFQ-39)			*6.0*	*0.006*		

Best Corrected Visual Acuity

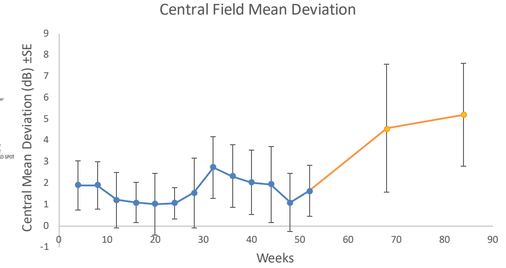
Contrast Sensitivity

Full Field Mean Deviation





Central Field Mean Deviation



Results

1. There was an improvement in
   - Mean Deviation
   - Color Discrimination
   - Contrast Sensitivity
   - VFQ-39
2. No serious adverse events were reported.

Discussion

- There is ongoing improvement in visual function in the continuing open label extension
- Patients describe a meaningful improvement in Quality of Life and visual function
- Adverse events were mild and occurred in a similar frequency between Elamipretide and placebo treated eyes
- Elamipretide topical solution was well tolerated

Conclusions

- Elamipretide topical solution was well tolerated
- The clinical significance of the observed improvements in visual function need to be further evaluated
- Data collection is ongoing

Acknowledgements

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