Cis Beta Carotene for Congenital Stationary Night Blindness

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Background

Fundus albipunctatus is a retinal dystrophy caused by a mutation in the gene encoding 11-*cis*-retinol dehydrogenase which delays the recovery of rod photoreceptor cells from light stimulation leading to night blindness. A recent study of a mouse model of fundus albipunctatus treated with 9-*cis*-retinal showed improvement in visual function and structure.

Methods

Seven patients with fundus albipunctatus were given a daily food supplement of four capsules containing high dose 9-*cis* β-carotene for 90 days. The subjects were tested before and after treatment by visual field and electroretinogram in both eyes. This nonrandomized prospective phase I study was registered at www.clinicaltrials.gov (NCT00478530).

Results

All patients showed significant improvements in peripheral visual field (mean deviation improved from -4.77 ± 2.0 to -3.28 ± 2.28, $P = 0.009$, *t*-test).and highly significant improvement in rod recovery rates measured electroretinographically (Maximal scotopic b-wave amplitude responses, improved from 197 ± 49 μV to 292 ± 48 μV, $P < 0.001$, *t*-test). No complications or side effects were observed.

Conclusion

Oral treatment with 9-*cis* β-carotene led to reversal of a human retinal dystrophy. This potential therapy is readily available and should be evaluated in retinal dystrophies of similar mechanisms such as various types of retinitis pigmentosa.