



Vitamin E and Coenzyme Q10 randomised controlled trial in Friedreich's ataxia

Dr JM Cooper, Professor AHV Schapira, Dr LVP Korlipava, Dr PE Hart, and Dr JL Bradley from the Royal Free Hospital and University College Medical School London, describe the results of their latest trial.

The team at the Royal Free Hospital in London has been involved in research in Friedreich's ataxia for a number of years. A few years ago we published the results of a pilot study in 10 patients with Friedreich's ataxia which produced encouraging results for the potential treatment of the condition with Vitamin E and CoenzymeQ10 (CoQ10) in relation to both ataxia and heart function. (1, 2) In order to determine whether this treatment could be recommended to patients with Friedreich's ataxia a larger trial was needed. We have just completed and published results from this larger study involving 50 patients. (3) This was a randomised double blind controlled trial which compared the effect of high dose* Vitamin E/CoQ10 with low dose* on the participants' ataxia, as measured using the International Cooperative Ataxia rating scale. The trial lasted 2 years.

Over the period of the trial there was no significant difference in the ataxia rating scale when comparing the two treatment groups. However, 49% of patients in the trial showed an improvement in the ataxia rating scale (responder group) when compared with data from patients who were not on the trial and therefore not taking the trial medication. We also found that pre-treatment, the levels of CoQ10 and Vitamin E in the patients' blood were significantly lower than normal and that these increased to normal levels after treatment with both low and high doses of Vitamin E and CoQ10. Interestingly, the 'responder group' (ie: the 49% of patients whose ataxia rating scale improved) had significantly lower pre-treatment levels of CoQ10 and Vitamin E compared with the 'non-responder' group.

Our conclusion from this study is that the levels of CoQ10 and Vitamin E in patients' blood are a predictor of whether they will respond to Vitamin E and CoQ10 therapy.** In other words, if the level of CoQ10 and vitamin E in the blood of a patient with Friedreich's ataxia is low they may respond positively to treatment with CoQ10 and Vitamin E at either low dose or high dose.

This research project was funded by Ataxia UK.

References

1. Lodi *et al.* *Ann Neurol* 2001 May;49(5):590-6.
2. Hart *et al.* *Arch Neurol* 2005 Apr;62(4):621-6.
3. Cooper *et al.* *European Journal of Neurology* 2008 15:1371-9.

*High dose used in the trial was: 2100 IU/day Vitamin E tablet and 600mg/day CoQ10 tablet for adults and for patients under 18 years of age it was 30 IU/kg/day Vitamin E and 9mg/kg/day CoQ10.

Low dose used in the trial was: 30mg CoQ10 tablet and a placebo tablet which contained Vitamin E (4IU/day) as a preservative.

** Testing for CoQ10 levels is not normally carried out in patients with Friedreich's ataxia. If required, testing for CoQ10 is routinely available at the Neurometabolic Unit, National Hospital for Neurology and Neurosurgery, London. Please contact Dr I.P Hargreaves for details (Tel: 0845 1555000 ext 723844 email: jain.hargreaves@uclh.nhs.uk).

Disclaimer

Readers should seek their own medical advice before taking any action based on this summary of the results of this trial. No person shall have any claim of any nature whatsoever arising out of or in connection with this publication against Ataxia UK, the Royal Free Hospital, the University College London Medical School or any of their officers or employees.

For more support or information please contact:

Ataxia UK, Lincoln House, Kennington Park, 1-3 Brixton Road, London SW9 6DE www.ataxia.org.uk

Helpline: 0845 644 0606 Tel: +44 (0)20 7582 1444 Fax: +44 (0)20 7582 9444

Email helpline@ataxia.org.uk

We have a number of other publications on the ataxias available free of charge. In addition we publish a quarterly magazine called *The Ataxian* containing articles on research, living with ataxia and other relevant information. Our website also contains news of research projects.

March 2009