World first research to test vitamin's curing powers

Imagine if a treatment or, better still, a cure could be found for Charcot Marie Tooth (CMT) disease?

About 10,000 Australians and New Zealanders are estimated to have the debilitating inherited nerve disorder which causes progressive lower limb muscle weakness and produces painful foot deformities. There are more than 50 types of the disease. It's the most common nerve disorder in children and during adolescence typically produces the very high-arched foot deformity.

Although apparent to podiatrists who often are the first to diagnose the disease in patients, it can remain undiagnosed and is often dismissed as clumsiness, especially in children.

Now, in a world first, Sydney podiatrist and National Health and Medical Research Council (NHMRC) research fellow, Dr Josh Burns will lead a double-blind, randomised and placebo-controlled clinical research trial to determine if high dosage vitamin C is an effective treatment for children with CMT.

The trial, part of a four-year research program, has earned Dr Burns and his clinical research team at the Institute for Neuromuscular Research in The Children's Hospital at Westmead an $8000 Australian Podiatry Education and Research Foundation (APERF) grant. This will complement his Australian Clinical Research Fellowship for the four-year study.

Dr Burns says the study will either prove or dismiss the preventative and curative effects of high doses of Vitamin C on CMT disease.

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"Vitamin C is currently the most exciting area of research for patients with CMT disease. There are four other research groups in the world conducting similar studies, but their participants are adults. CMT disease progresses rapidly in childhood between the ages of five and ten, and that's the time when we need to address it. There is a chance that if we apply a proven treatment at this time we could not only reverse damage, but we may be able to prevent it happening in the first place."

The study follows a recent landmark study in France published in Nature Medicine which shows that vitamin C improves the strength, balance, and locomotion by increasing peripheral nerve function in a mouse model of CMT.[1]

"A major advance towards a cure for CMT disease may have been identified recently in this mouse model of CMT. In the French study of 42 mice with CMT that's close to the human version, the placebo group showed no improvements, but those treated with high doses of vitamin C, a popular antioxidant and promoter of nerve regeneration were stronger, ran faster and lived longer. In these animals, vitamin C slowed progression of locomotion difficulties, restored muscle strength and partially corrected the genetic abnormality causing CMT."

Earlier evidence, that vitamin C could play a role in CMT disease, was shown in a case study of a young healthy male where experimentally-induced vitamin C deficiency caused progressive lower limb sensory loss, muscle weakness, absent tendon reflexes and walking difficulties after three months. This nerve damage was then reversed after vitamin C repletion for four months.[2]

The clinical research team at the Institute for Neuromuscular Research which will conduct the vitamin C trials, (l-r) Prof Robert Ouvrier, Dr Josh Burns and Dr Monique Ryan.

Dr Burns is undertaking the study with the head of the Institute for Neuromuscular Research at The Children's Hospital at Westmead, Professor Robert Ouvrier and paediatric neurologist from Melbourne's Royal Children's Hospital, Dr Monique Ryan.

The study will compare two groups of 30 children, aged from two to 16 years, over 12 months. One group will be given daily high dose oral vitamin C supplements and the other an identical tasting inert placebo.

Doses will be 22 to 44 times the regular daily intake of vitamin C but will remain within safe levels recommended by the American Food and Nutrition Board of Medicine.

Children intolerant to vitamin C, or who have another significant disease or injury which could affect the muscle strength of the feet, will not be eligible to take part in the study.

At the beginning and again at the end of the 12 months, the children will be tested for: nerve function; foot and ankle muscle strength; gross/fine motor co-ordination; quality of life; and running speed and endurance. Safety will be monitored by monthly phone calls, blood tests and an external monitoring safety committee

Dr Burns said the study has taken two years to develop and refine but says the design is scientifically robust.

"The world is abuzz with this work. We have a leading professor of paediatric neurology at The Children's Hospital at Westmead involved, which is why we are able to conduct such a large trial of kids with CMT. It really is exciting to conduct such a robust piece of science. The whole concept seems so simple, but if we don't do the right study to see if it works, we will never know."

Recruitment of participants is expected to begin later this month (February). ●

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References:

1. Passage et al, Nature Medicine 2004; 10:396-401.
2. Hood J. Femoral neuropathy in scurvy. New England Journal of Medicine 1969; 281:1292-1293.