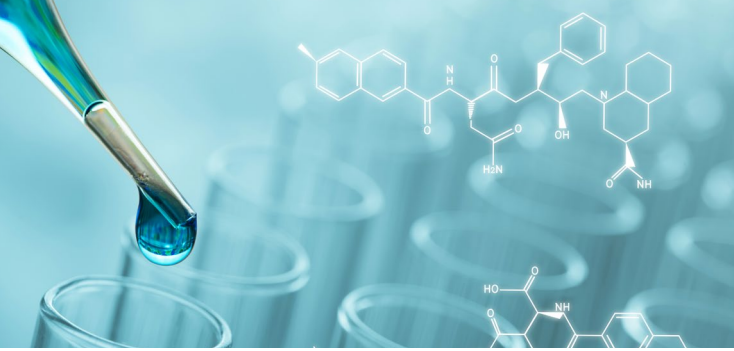
Northern Sydney
Local Health District

NSW GOVERNMENT

Does your child with NF1 have muscle weakness and fatigue?

If so, learn about this study and find out if your child can participate

This project has been approved by the Northern Sydney Local Health District Ethics Committee – 2024/ETH00028



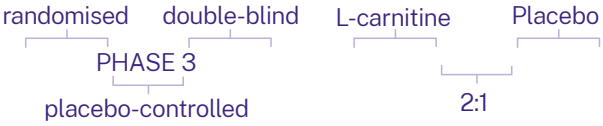
What is the L-carnitine study?

The L-carnitine study for children with neurofibromatosis 1 is a clinical research study designed to find out whether L-carnitine can improve bodily functions and treat muscle fatigue and weakness

More about the study

L-carnitine is an over-the-counter nutraceutical supplement taken to improve fatty acid oxidation and energy production. This supplement is commonly used to improve weight loss and muscle performance in the general population. L-carnitine supplementation is also used to treat primary carnitine deficiency and other metabolic myopathies.

Eligible patients will enter an approximately 1-week, but no more than 2-week, screening period that includes a baseline assessment of self-reported muscle weakness, safety laboratory tests and physical examination. Following screening period, approximately 30-40 eligible patients will be randomized 2:1 to receive L-carnitine or placebo in a 12-week Treatment/Supplementation Period.



Frequently asked questions

What is the study medicine?

L-carnitine is an over-the-counter nutraceutical supplement taken to improve fatty acid oxidation and energy production.

Who can join the study?

Children aged 8-12 years with a diagnosis of NF1.

What will happen?

Participants will take an oral tablet of L-carnitine and will have their muscle strength monitored over the course of the study using wearable device similar to a Fitbit, physical assessments, and examinations done by a doctor or a study nurse and by answering questionnaires.

Why is it important?

We hope to find a way to help children with NF1 improve their muscle strength, which can make a big difference in their daily lives.

Where will it take place?

The study will be conducted at Royal North Shore Hospital.

What can study participants expect?

Participation in this research is voluntary. If your child does not wish to take part, they do not have to. They will receive the best possible care whether they take part or not.

Screening
(About 2 weeks)

Day 1 to Week 12
(12 weeks | 3 month)

- In this period, your child will be randomly assigned to either the L-carnitine or placebo and will commence on treatment.
- This period is 12 weeks long. During this time, your child will have face-to-face and telephone consultations with the study doctor and will be asked to complete questionnaires and measures to assess muscle strength.

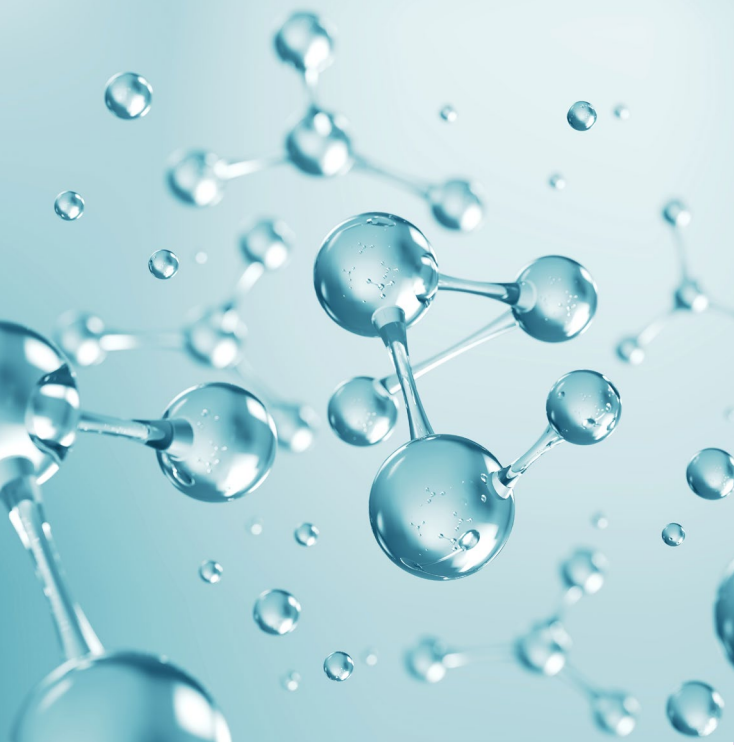
Washout Period
(1 week)

- There will be a 1-week washout period, and at this time your child is given a choice whether to participate in the crossover study.

Week 13 to Week 25
(12 weeks | Crossover Study)

- This period is a 12-week long treatment extension wherein your child will be given L-carnitine treatment. Similarly, they will have face-to-face and telephone consultations with the study doctor and will be asked to complete questionnaires and measures to assess muscle strength.

Follow up



What is a clinical research study?

Clinical research studies help scientists and doctors find out whether a medical strategy, drug, or device is safe and works in humans. Before a new drug or device can be prescribed for a specific condition, it must go through several rounds of clinical research.

Participation in a clinical research study is the participant's decision, and participants may stop at any time.

If you or your child experience distress, you can call at any time

Lifeline Australia

131 114

https://www.lifeline.org.au

NF1 Support Groups

Children's Tumor Foundation, US

697 Third Avenue, Suite 418, New York, NY 10017

1800-323-7938 or +12127470004

https://www.ctf.org/contact/

Children's Tumour Foundation Australia

info@ctf.org.au

02 9713 6111

https://www.ctf.org.au

To register your interest in this study or for more information, scan the QR code below or contact

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JUL24/V1

JOB ID 67239-1