The "Diet for Life" study.

No registrations found.

Ethical review	Not applicable
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON23483

Source
Nationaal Trial Register

Brief title
N/A

Health condition

1. PKU;

2. Phenylalanine;

3. Diet;

4. Neuropsychological function;

5. Wellbeing.

Sponsors and support

Primary sponsor: AMC
Source(s) of monetary or material Support: SHS international

Intervention

Outcome measures

Primary outcome

The neuropsychological deficit profile of adults with treated PKU.

1. What are the relative strengths and weaknesses in the neurocognitive functioning of adults with treated PKU?;

2. Does supplementation of Phe to plasma levels comparable to levels observed in adult patients who fully discontinued their diet, influence neurocognitive functioning, and/or other disease parameters?

Secondary outcome

The neurophysiological mechanisms responsible for neuropsychological dysfunction in adults with treated PKU.

1. Can abnormal functional networks be demonstrated in adults with treated PKU?;

2. Is network disruption more severe following the intervention-related increase in phenylalanine level?;

3. Is there a correlation between the nature and severity of neuropsychological dysfunction in adults with treated PKU and the nature and severity of network disruption in these subjects?

Study description

Background summary

Rationale:

Phenylketonuria (PKU; MIM 261600) is an autosomal recessive disorder of phenylalanine (Phe) metabolism. Untreated PKU results in severely retarded mental development and neurological abnormalities. Patients with PKU are treated with a Phe-restricted diet and supplementation of all amino acids except Phe. With the introduction of newborn screening and the early institution of the diet, mental retardation due to PKU has been nearly eliminated. At this moment, the most important issue in the treatment of PKU is whether the reduction of the Phe levels, by a strict and socially invalidating diet, is still relevant in adults.

Objectives:

To evaluate the effects of short term supplementation of Phe to levels comparable to levels observed in adult patients who fully discontinued their diet, on neuropsychological functions and wellbeing of adult patients with PKU.

Research questions:

1. What is the neuropsychological deficit profile of adults with treated PKU?;

2. Does supplementation of Phe to plasma levels comparable to levels observed in adult patients who fully discontinued their diet, influence neurocognitive functioning or wellbeing?;

3. Which neurophysiological mechanisms are responsible for neuropsychological dysfunction in adults with treated PKU? Can network disruption be demonstrated?;

4. Does supplementation of Phe to plasma levels comparable to levels observed in adult patients who fully discontinued their diet influence network disruption?

Study Design:

We aim to perform a double blind study with crossover design with repeated measures.

Procedure:

A total of 10-20 early and continuously treated patients with PKU will participate in the study. During two periods of four weeks each, an additional supplement of amino acids will be added to the diets of the subjects. During one of the 4 weeks periods the supplement will not contain any Phe or its metabolite tyrosine, but different amino acids. The other 4 weeks period the supplement will contain the amount of Phe that, added to the Phe intake from the patients' own diet, increases the daily Phe intake to a normal Phe intake for a healthy person of that age and sex. During each study period patients will undergo dietary evaluation, neuropsychological evaluation, evaluation of Phe levels, and a MEG scan. Also, patients and one significant other of each patient will complete a weekly questionnaire to evaluate their wellbeing. The MEG scan will be performed in the VUMC, and the protocol for that part of the study will be submitted to the medical ethical committee of the VUMC.

Study objective

A short term increase of phenylalanine levels does affect neuropsychological functions and well-being
in adults with phenylketonuria.

Intervention

Supplementation of phenylalanine (Phe) to levels that simulate full discontinuation of dietary

treatment. During two periods of four weeks each, an additional supplement of aminoacids will be added to the diets of the subjects, to be taken in 3 servings per day, together with breakfast lunch and dinner. During one of the 4 weeks periods the supplement will not contain any Phe or its metabolite tyrosine, but different aminoacids. The other 4 weeks period the supplement will contain the amount of Phe that, added to the Phe intake from the patients' own diet, increases the daily Phe intake to a normal Phe intake for a healthy person of that age and sex.

Contacts

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Eligibility criteria

Inclusion criteria

Patients with PKU aged 18 and older who have been detected by newborn screening and have been continuously treated with a protein restricted diet and supplementation of aminoacids.

Exclusion criteria

Poor dietary adherence with a mean phe value above 1100 umol/l in the year prior to the start of the study

Pregnancy or the wish to conceive within 3 months after the end of the study.

Study design

Design

Study type:	Interventional
Intervention model:	Crossover
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-01-2008
Enrollment:	20
Type:	Anticipated

Ethics review

Not applicable

Application type:	Not applicable

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL1024
NTR-old	NTR1056
Other	MEC : 07/262 07.17.1461,
ISRCTN	ISRCTN wordt niet meer aangevraagd

Study results