Effects of nicotinamide riboside (vitamin B3) in patients with Ataxia Telangiectasia

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The purpose of this research is to investigate whether treatment with nicotinamide riboside (vitamine B3) has a positive effect on the disease course of patients with ataxia telangiectasia.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Immunodeficiency syndromes
Study type	Interventional

Summary

ID

NL-OMON48100

Source
ToetsingOnline

Brief title
Vitamine B3 in A-T

Condition

- Immunodeficiency syndromes
- Movement disorders (incl parkinsonism)
- Lower respiratory tract disorders (excl obstruction and infection)

Synonym
A-T, Louis Bar syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum
Source(s) of monetary or material Support: A-T childrens project;Twan foundation

Intervention

Keyword: Ataxia Telangiectasia, ATM, Nicotinamide riboside

Outcome measures

Primary outcome

Primary Objective:

To assess changes in motor performance (ataxia and dysarthria) during the

treatment period with NR.

Secondary outcome

Secondary objectives:

- To assess any changes in quality of life of A-T patients during the treatment

period with NR.

If changes occur in primary objectives (ataxia or dysarthria), it is

conceivable that this affects

quality of life.

- To assess any changes in laboratory parameters that are known to be abnormal

in most patients with A-T during the treatment period with NR. These values are

an objective measurement to investigate the effect of NR in patients with A-T.

Study description

Background summary

Ataxia Telangiectasia (A-T) is an autosomal recessively inherited neurodegenerative disorder that also affects the immune and respiratory system. Therapy for A-T is restricted to symptomatic treatment including rehabilitation care, combined with infection prevention and treatment, and screening for pulmonary dysfunction and malignancies.
Nicotinamide adenine dinucleotide (NAD+) is a molecule that plays a pivotal

role in many cell processes such as energy metabolism, cell signaling , and DNA repair. Studies have showed that NAD+ deficiency plays a role in disease mechanisms underlying DNA repair disorders such as Ataxia-Telangiectasia. NAD+ is available in food, but can also be synthesized in the body from its precursors nicotinamide, nicotinic acid, and nicotinamide riboside (NR), as a group called *vitamin B3*.
Treatment of experimental A-T animal models with nicotinamide riboside leads to an important decrease of their neurological symptoms, increases their survival, and improves biochemical parameters.
The aim of this study is to investigate whether treatment with NR during a period of six months indeed has a positive effect on the disease course of patients with A-T.

Study objective

The purpose of this research is to investigate whether treatment with nicotinamide riboside (vitamine B3) has a positive effect on the disease course of patients with ataxia telangiectasia.

Study design

This is a single center, explorative, interventional, open-label proof of concept study in an (outpatient) hospital setting
Included patients will start treatment with NR during four consecutive months. During the study period, included patients will visit the outpatient clinic at least 4 times. During these visits the clinical measures, questionnaires and laboratory tests will be performed.

Intervention

Patients will be treated with nicotinamide riboside, 25mg/kg/day divided over two or three gifts, with a maximum dose of 900mg/day, during four consecutive months.

Study burden and risks

The use of NR is considered safe. First of all, NR is FDA-approved.
In addition, dosage of NR is based on aforementioned clinical studies. At the same time, we will determine laboratory measurements during the study period to overcome adverse effects of using NR.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

-A-T patients who visit our outpatient clinic
-Genetically confirmed diagnosis of A-T by the identification of pathogenic mutations of the ATM gene.
-Age * 2 years or older and bodyweight * 12 Kg
-Informed consent

Exclusion criteria

-Additional medical condition or illness that impair the patient*s ability to participate in the study (e.g. actual treatment of a malignancy, active infection, poorly controlled diabetes mellitus, hypertension, organ failure, clinically significant hematological or biochemical abnormalities different from the usual abnormalities in A-T)

-Elevated serum transaminases (> 2 times upper limit of normal)
-Participation in another interventional study at start of the study or during the study
-pregnancy/ breastfeeding

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	18-03-2019
Enrollment:	25
Type:	Actual

Ethics review

Approved WMO

Date:	19-02-2019
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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
CCMO	NL68197.091.18