Clinical Trial Protocol
Iranian Registry of Clinical Trials

08 Jun 2026

The effects of N-Acetyl-L-Leucine on the improvement of symptoms in patients with Spinocerebellar ataxia

Protocol summary

Study aim
Effects of Acetyl Leucine on symptoms of Spinocerebellar ataxia

Design
The current study is a randomized, triple-blind, crossover clinical trial with parallel groups. A total of 4 subjects will be enrolled. The table of random numbers was used for randomization.

Settings and conduct
Patients with Spinocerebellar ataxia referring to Neurology Clinic of the Ghaem Hospital are enrolled in the study. All volunteers, care providers and statistician are blinded after assignment to intervention. The supplements containers were coded as A and B by a non-researcher person and remained confidential until data analysis. Placebo powders are similar to supplements regarding the weight and color.

Participants/Inclusion and exclusion criteria
Inclusion criteria:Age>6 years, Having a definitive diagnosis of Spinocerebellar ataxia with clinical signs, having a stable treatment, and not taken forbidden drugs. Exclusion criteria:Having diarrhea, visual loss, malignancies, diabetes mellitus, history of hypersensitivity to the N-Acetyl-Leucine, Having severe vision or hearing impairment, Having arthritis.

Intervention groups
Intervention group: The intervention group receive N-Acetyl-L-Leucine sachet (daily intake of 2-4 grams depending on their weight) for 4 weeks and then after a 4-weeks wash-out period, crossed over to the alternate regimen. The participants take one sachet every day, which was contained in an unlabeled bottle. Supplements are from Hubei (Shenzhen, China). Control group: Receiving a placebo sachet (daily consumption between 2-4 grams depending on the subject's weight) for 4 weeks and then after a 4-weeks wash-out period, crossed over to the alternate regimen. Participants take a placebo daily orally in an unlabeled bottle. The placebo is prepared by from faculty of pharmacy (Mashhad, Iran).

Main outcome variables
Scale for Assessment and Rating of Ataxia score

General information

Reason for update

Acronym

IRCT registration information
IRCT registration number: **IRCT20210413050958N7**
Registration date: **2023-05-26, 1402/03/05**
Registration timing: **prospective**

Last update: **2023-05-26, 1402/03/05**
Update count: **0**

Registration date
2023-05-26, 1402/03/05

Registrant information

Name
Maryam Saberi-Karimian

Name of organization / entity

Country
Iran (Islamic Republic of)

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Recruitment status
Recruitment complete

Funding source

Expected recruitment start date
2023-06-22, 1402/04/01

Expected recruitment end date
2023-11-22, 1402/09/01

Actual recruitment start date
empty

Actual recruitment end date
empty

Trial completion date
empty

Scientific title
The effects of N-Acetyl-L-Leucine on the improvement of symptoms in patients with Spinocerebellar ataxia

Public title
Effects of Acetyl-Leucine on Spinocerebellar ataxia

Purpose
Treatment

Inclusion/Exclusion criteria

Inclusion criteria:
Age above 6 years Patients with a definitive diagnosis of Spinocerebellar ataxia Having clinical signs If the patient is taking any medication, he/she should maintain a constant dose/not change his/her treatment during the study period. Have not taken any forbidden drugs including any variant of N-acetyl-DL-leucine, aminopyridines, Riluzole, gabapentin, Varenicline, Chlorzoxazone, sulfasalazine, Rosuvastatin at least 4 weeks before visit 1 and throughout the duration of the study Signed informed consent form by the subjects or their parents after explaining the study objectives by the research team Patient satisfaction

Exclusion criteria:
Having chronic diarrhea, visual loss, malignancies or insulin-dependent diabetes mellitus History of hypersensitivity to the N-Acetyl-Leucine Having severe vision or hearing impairment Having arthritis or other musculoskeletal disorders

Age
From **6 years** old

Gender
Both

Phase
1-2

Groups that have been masked

- Participant
- Care provider
- Investigator
- Data analyser

Sample size
Target sample size: **4**

Randomization (investigator's opinion)
Randomized

Randomization description
Subjects are divided into one of two intervention/control groups using the random numbers prepared by a methodologist using www.sealedenvelope.com, so that who enrolled the trial can select a sealed envelope with random allocation sequence to the intervention or control group. An expert outside the research team blinds the drugs. The executive team register the subjects and assign them to the intervention. All volunteers, the executive team, and the statistical analyst will be blinded by the interventions.

Blinding (investigator's opinion)
Triple blinded

Blinding description
All volunteers, the executive team, and the statistical analyst will be blinded by the interventions. So that, the supplements containers were coded as A and B by a non-researcher person and remained confidential until data analysis. The placebos powders are similar to the supplements regarding the weight and color.

Placebo
Used

Assignment
Crossover

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee
Ethics Committee of Mashhad University of Medical Sciences

Street address
Research Council, Ghoreishi bildings, Daneshgah Ave.

City
Mashhad

Province
Razavi Khorasan

Postal code
99199-91766

Approval date
2023-02-28, 1401/12/09

Ethics committee reference number
IR.MUMS.MEDICAL.REC.1402.038

Health conditions studied

1

Description of health condition studied
Spinocerebellar Ataxia

ICD-10 code
G11

ICD-10 code description
Hereditary ataxia

Primary outcomes

1

Description
Scale for Assessment and Rating of Ataxia (SARA) score

Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage

Method of measurement
Scale for Assessment and Rating of Ataxia (SARA) Questionnaire

2

Description
Spinocerebellar Ataxia Functional Index (SCAFI)
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Spinocerebellar Ataxia Functional 8-m walking time (SCAFI-8MWT) and Spinocerebellar Ataxia Functional 9-hole peg test (SCAFI-9HPT)

Secondary outcomes

1

Description
The quality of life
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Pediatric Quality of Life (PedsQL) questionnaire

2

Description
Cell blood counts
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Sysmex autoanalyser

3

Description
Aspartate amino transferase
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Autoanalyser

4

Description
Alanin amino transferase
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Autoanalyser

5

Description
Creatinine
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Autoanalyser

6

Description
Urea
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Autoanalyser

7

Description
Bilirubin
Timepoint
Before the intervention and 4 weeks after taking supplement or placebo in every study stage
Method of measurement
Autoanalyser

Intervention groups

1

Description
Intervention group: Subjects in the intervention group receive N-Acetyl-L-Leucine sachet (daily intake of 2-4 gr depending on the subjects' weight) for 4 weeks (n=2) and then after a 4-weeks wash-out period, they were crossed over to the alternate regimen. The participants take one sachet every day, which was contained in an unlabeled bottle. Supplements are from Hubei ipure Biotech co., ltd (Shenzhen, China).
Category
Treatment - Drugs

2

Description
Control group: The control group received a placebo sachet (daily consumption between 2 to 4 grams depending on the subject's weight) for 4 weeks (n=2) and then after a 4-weeks wash-out period, they were crossed over to the alternate regimen. Participants take a placebo every day orally in an unlabeled bottle. The placebo is prepared by from faculty of pharmacy (Mashhad, Iran) company.
Category
Placebo

Recruitment centers

1

Recruitment center
Name of recruitment center
Ghaem hospital
Full name of responsible person
Maryam Saberi-Karimian
Street address
Ahmadabad Street, Ghaem Hospital
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Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Mashhad University of Medical Sciences
Full name of responsible person
Majid Ghayour-Mobarhan
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Research Council, Ghoreishi bildings, Daneshgah Ave.
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Grant name
Grant code / Reference number
Is the source of funding the same sponsor organization/entity?
Yes
Title of funding source
Mashhad University of Medical Sciences
Proportion provided by this source
100
Public or private sector
Public
Domestic or foreign origin
Domestic
Category of foreign source of funding
empty
Country of origin
Type of organization providing the funding
Academic

Person responsible for general inquiries

Contact
Name of organization / entity
Mashhad University of Medical Sciences
Full name of responsible person
Maryam Saberi-Karimian
Position
Assistant professor
Latest degree
Ph.D.
Other areas of specialty/work
Biochemistry
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Person responsible for updating data

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Other areas of specialty/work
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Sharing plan

Deidentified Individual Participant Data Set (IPD)

No - There is not a plan to make this available

Justification/reason for indecision/not sharing IPD

Raw data will be shared upon a reasonable request from the corresponding author.

Study Protocol

Yes - There is a plan to make this available

Statistical Analysis Plan

No - There is not a plan to make this available

Informed Consent Form

Undecided - It is not yet known if there will be a plan to make this available

Clinical Study Report

Yes - There is a plan to make this available

Analytic Code

No - There is not a plan to make this available

Data Dictionary

No - There is not a plan to make this available

Title and more details about the data/document

There is no plan to share the data.

When the data will become available and for how long

After publication of paper(s) upon a reasonable request

To whom data/document is available

Study PI and executive team

Under which criteria data/document could be used

For reasonable research or clinical purpose

From where data/document is obtainable

Dr. Maryam Saberi-Karimian

What processes are involved for a request to access data/document

Direct e-mail

Comments