

# 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)

##### Phone

+98 51 3764 3808

##### Email address

saberikm@mums.ac.ir

##### 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](http://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

#### **City**

Mashhad

**Province**  
Razavi Khorasan  
**Postal code**  
99199-91766  
**Phone**  
+98 51 3840 0001  
**Email**  
saberikm@mums.ac.ir

**Street address**  
Research Council, Ghoreishi bildings, Daneshgah Ave.  
**City**  
Mashhad  
**Province**  
Razavi Khorasan  
**Postal code**  
99199-91766  
**Phone**  
+98 51 3882 3260  
**Email**  
saberikm@mums.ac.ir

## Sponsors / Funding sources

### 1

#### Sponsor

**Name of organization / entity**  
Mashhad University of Medical Sciences  
**Full name of responsible person**  
Majid Ghayour-Mobarhan  
**Street address**  
Research Council, Ghoreishi bildings, Daneshgah Ave.  
**City**  
Mashhad  
**Province**  
Razavi Khorasan  
**Postal code**  
99199-91766  
**Phone**  
+98 51 3845 2472  
**Email**  
ghayourm@mums.ac.ir  
**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

## Person responsible for scientific 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  
**Street address**  
Research Council, Ghoreishi bildings, Daneshgah Ave.  
**City**  
Mashhad  
**Province**  
Razavi Khorasan  
**Postal code**  
99199-91766  
**Phone**  
+98 915 528 2874  
**Email**  
saberikm@mums.ac.ir

## Person responsible for updating data

#### 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  
**Street address**  
Research Council, Ghoreishi bildings, Daneshgah Ave.  
**City**  
Mashhad  
**Province**  
Razavi Khorasan  
**Postal code**  
99199-91766  
**Phone**

+98 51 3882 3260

**Email**

saberikm@mums.ac.ir

## 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**