

Clinical Trial Protocol

Iranian Registry of Clinical Trials

12 Sep 2026

The effects of trehalose on the improvement of clinical symptoms in patients with Ataxia Telangiectasia

Protocol summary

Study aim

The effects of trehalose on the improvement of clinical symptoms in patients with Ataxia Telangiectasia

Design

The current study is a single-arm, open-label pilot study. A total of 10 subjects are enrolled between Jun.21.2024 and Oct.23.2025.

Settings and conduct

Patients with Ataxia Telangiectasia who referring to Neurology Clinic of the Ghaem Hospital are enrolled in the study.

Participants/Inclusion and exclusion criteria

Inclusion criteria: Having a definitive diagnosis of Ataxia Telangiectasia with clinical signs, having a stable treatment. Exclusion criteria: Having chronic diarrhea, visual loss, malignancies, insulin-dependent diabetes mellitus, Having severe vision or hearing impairment, Having arthritis or other musculoskeletal disorders.

Intervention groups

Subjects in the intervention group receive Trehalose (10 gr TID) orally for 8 weeks (n=10).

Main outcome variables

Scale for Assessment and Rating of Ataxia (SARA) score; Spinocerebellar Ataxia Functional Index (SCAFI)

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20210413050958N8**

Registration date: **2024-05-20, 1403/02/31**

Registration timing: **prospective**

Last update: **2024-05-20, 1403/02/31**

Update count: **0**

Registration date

2024-05-20, 1403/02/31

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

2024-06-21, 1403/04/01

Expected recruitment end date

2025-10-23, 1404/08/01

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

The effects of trehalose on the improvement of clinical symptoms in patients with Ataxia Telangiectasia

Public title

Effect of trehalose on treatment of Ataxia Telangiectasia

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

Signed informed consent form by the subjects or their parents after explaining the study objectives by the research team Patients with a definitive diagnosis of Ataxia Telangiectasia Having clinical signs If the patient is taking any medication he/she should maintain a constant dose and not change his/her treatment during

the study period If the patient is receiving concomitant speech therapy or physiotherapy he/she has been on a stable duration and type of therapy for at least 4 weeks before visit 1 and throughout the duration of the study

Exclusion criteria:
not having informed consent to participate in the study
Asymptomatic patients Patient who have clinical signs of Ataxia Telangiectasia, but do not have a confirmed genetic test for Ataxia Telangiectasia

Age
No age limit

Gender
Both

Phase
2

Groups that have been masked
No information

Sample size
Target sample size: **10**

Randomization (investigator's opinion)
N/A

Randomization description

Blinding (investigator's opinion)
Not blinded

Blinding description

Placebo
Not used

Assignment
Single

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Research Ethics Committees of Imam Reza Hospital
Educational, Research and Treatment Center,
Mashhad

Street address

Research Council, Ghoreishi bildings, Daneshgah Ave.

City

Mashhad

Province

Razavi Khorasan

Postal code

99199-91766

Approval date

2024-04-15, 1403/01/27

Ethics committee reference number

IR.MUMS.IRH.REC.1403.026

Health conditions studied

1

Description of health condition studied

Ataxia Telangiectasia

ICD-10 code

G11.3

ICD-10 code description

Cerebellar ataxia with defective DNA repair

Primary outcomes

1

Description

Movement signs

Timepoint

Before the intervention and 8 weeks after taking
Trehalose

Method of measurement

Using the Scale for Assessment and Rating of Ataxia
(SARA) score and Spinocerebellar Ataxia Functional Index
(SCAFI)

Secondary outcomes

1

Description

The quality of life

Timepoint

Before the intervention and 8 weeks after taking
trehalose

Method of measurement

Using PedsQL questionnaire

2

Description

Cell blood count

Timepoint

Before the intervention and 8 weeks after taking
trehalose

Method of measurement

Sysmex Cell Counter

3

Description

Lactate dehydrogenase

Timepoint

Before the intervention and 8 weeks after taking
trehalose

Method of measurement

Auto analyzer instrument

4

Description

Aspartate aminotransferase

Timepoint

Before the intervention and 8 weeks after taking
trehalose

Method of measurement

Auto analyzer instrument

5

Description

Alanine aminotransferase

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

6

Description

Urea

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

7

Description

Creatinine

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

8

Description

Alkaline phosphatase

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

9

Description

Na

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

10

Description

K

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

11

Description

Total bilirubin

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

Auto analyzer instrument

12

Description

Food recall

Timepoint

Before the intervention and 8 weeks after taking trehalose

Method of measurement

24-hour food recall tool

Intervention groups

1

Description

Intervention group: In treatment group (n=10), Trehalose powder is taken orally (10 gr TID) in subjects with Ataxia Telangiectasia for 8 weeks. Supplements are provided by Shaanxi Fruiterco Biotechnology Co., Ltd.

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Ghaem Hospital

Full name of responsible person

Maryam Saberi-Karimian

Street address

Ahmadabad Ave.

City

Mashhad

Province

Razavi Khorasan

Postal code

99199-91766

Phone

+98 51 3840 0001

Email

maryamsabery2012@gmail.com

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Dr. Mohsen Tafaghodi

Street address

Research Council, Ghoreishi bildings, Daneshgah Ave.

City

Mashhad

Province

Razavi Khorasan

Postal code

99199-91766

Phone

+98 51 3845 2472

Email

tafaghodim@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

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

Person responsible for scientific inquiries

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Maryam Saberi-Karimian

Position

Associate professor

Latest degree

Ph.D.

Other areas of specialty/work

Biochemistry

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

Person responsible for updating data

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Maryam Saberi-Karimian

Position

Associate professor

Latest degree

Ph.D.

Other areas of specialty/work

Biochemistry

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

Sharing plan

Deidentified Individual Participant Data Set (IPD)

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

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
Following a reasonable request, deidentified data will be shared.
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