

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

10 Jun 2026

### Evaluation of safety and motivation status of patients with cerebellar ataxia after intrathecal treatment with mesenchymal stem cells

#### Protocol summary

##### Study aim

Evaluating the safety and efficacy of mesenchymal stem cell therapy in patients with cerebellar ataxia

##### Design

Clinical trial with a control group, with parallel groups, unblinded, randomized, phase 1 on 20 patients. The random number table of the software was used for randomization.

##### Settings and conduct

Ten patients with cerebellar ataxia receive 50-70 million autologous bone marrow-derived MSCs at Pasteur Hospital. In the control group, other common treatments are prescribed for patients.

##### Participants/Inclusion and exclusion criteria

inclusion criteria : people aged 10-65 years and with a definitive diagnosis of ataxia and no history of other mental illnesses. Exclusion criteria : pregnancy or a history of other serious mental and non-mental illnesses.

##### Intervention groups

Intervention group: 10 patients with cerebellar ataxia receiving mesenchymal stem cells Control group: 10 patients with cerebellar ataxia who will receive routine treatments.

##### Main outcome variables

\*-neurologic sign of patients including: Scale for the Assessment and Rating of Ataxia (SARA)-Quality of life-Depression-Beck Scale-Improve in international Co-Operative Ataxia Rating Scale-Balance Test-Tremor Rating Scale \*-Before the intervention, month 3 and month 6 after the intervention \*-Clinical examination and questionnaire

#### General information

##### Reason for update

##### Acronym

##### IRCT registration information

IRCT registration number: **IRCT20160809029275N3**

Registration date: **2022-08-31, 1401/06/09**

Registration timing: **retrospective**

Last update: **2022-08-31, 1401/06/09**

Update count: **0**

##### Registration date

2022-08-31, 1401/06/09

##### Registrant information

###### Name

**Name of organization / entity**

###### Country

Iran (Islamic Republic of)

###### Phone

+98 51376276015

###### Email address

tavakolaj@mums.ac.ir

##### Recruitment status

**Recruitment complete**

##### Funding source

##### Expected recruitment start date

2021-07-23, 1400/05/01

##### Expected recruitment end date

2022-07-23, 1401/05/01

##### Actual recruitment start date

2021-09-04, 1400/06/13

##### Actual recruitment end date

2022-06-30, 1401/04/09

##### Trial completion date

2022-06-30, 1401/04/09

##### Scientific title

Evaluation of safety and motivation status of patients with cerebellar ataxia after intrathecal treatment with mesenchymal stem cells

##### Public title

cerebellar ataxia treatment with mesenchymal stem cells

##### Purpose

Treatment

## Inclusion/Exclusion criteria

### Inclusion criteria:

Does not receive stem cells in the last 6 months. signing the written consent form by the participants. The age range of 10-65 years without limitation in sex. Definitive diagnosis of ataxia based on brain and cervical spinal cord MRI. Presence of cerebellar or brainstem atrophy and/or cervical spinal cord. Not having other mental illness such as schizophrenia etc.

### Exclusion criteria:

Positive pregnancy test. Heart, kidney and liver failure, epilepsy, lung disease, cardiac arrhythmia, diabetes, leukemia and other diseases of the central nervous system such as Parkinson's. If the total level of bilirubin is greater than 1.5 times the upper limit of its normal value. History of chronic or acute alcohol consumption The presence of evidence of seizures in the brain scan Any evidence of infection History of severe drug sensitivity or anaphylaxis to two or more foods or drugs Other organic brain diseases HIV positive and tumor markers positive Patients with severe psychosis, cognitive disorders and inability to understand or sign consent Other organic or systemic diseases Uncontrollable high blood pressure Participation in other clinical trials in the last three months The presence of thyroid disease Presence of evidence of encephalitis Presence of sarcoidosis and Behcet's disease Vitamin E deficiency Wilson's disease Increased blood ammonia Positive anti-GAD and anti-neuronal antibodies Lupus and Wegener's disease Middle ear dysfunction

### Age

From **10 years** old to **65 years** old

### Gender

Both

### Phase

1

### Groups that have been masked

*No information*

### Sample size

Target sample size: **20**

Actual sample size reached: **20**

### Randomization (investigator's opinion)

Randomized

### Randomization description

A simple randomization method using the random number table method was used to assign patients to groups. In this method, even numbers were placed in the intervention group and odd numbers were randomly placed in the control group.

### Blinding (investigator's opinion)

Not blinded

### Blinding description

### Placebo

Not used

### Assignment

Parallel

### 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

Mashhad, Daneshgah Street, Qurashi Building, Mashhad University of Medical Sciences

##### City

Mashhad

##### Province

Razavi Khorasan

##### Postal code

9137913316

##### Approval date

2022-07-02, 1401/04/11

##### Ethics committee reference number

IR.MUMS.REC.1401.128

## Health conditions studied

### 1

#### Description of health condition studied

Diagnosis Code G32.81: Cerebellar ataxia

#### ICD-10 code

G32.81

#### ICD-10 code description

Cerebellar ataxia in diseases classified elsewhere

## Primary outcomes

### 1

#### Description

neuromotor sign of patients including: Scale for the Assessment and Rating of Ataxia (SARA)-Quality of life-Depression-Beck Scale-Improve in international Co-Operative Ataxia Rating Scale-Balance Test-Tremor Rating Scale

#### Timepoint

Before the intervention, month 3 and month 6 after the intervention

#### Method of measurement

Clinical examination and questionnaire

### 2

#### Description

Blood markers including: • T4 and TSH • Alpha-fetoprotein • IgG and IgE • OGTT and FBS • ACE • Vitamin E • Electrophoresis of serum proteins • Chol, TG, LDL and HDL • Ceruloplasmin, Cu and 24-hour urine Cu for patients less than 40 years old • Anti-GAD, Anti-dsDNA, P-ANCA and C-ANCA • Vitamin B12 and folic acid

#### Timepoint

Before the intervention, month 3 and month 6 after the intervention

#### Method of measurement

Laboratory methods such as electrophoresis, VIDAS and ELISA

### 3

#### **Description**

Investigating the safety of stem cell treatment in patients participating in the study

#### **Timepoint**

24-48 hours after receiving injections and the third and sixth months

#### **Method of measurement**

Clinical examination of patients to check for fever, headache or any possible allergic reactions.

### **Secondary outcomes**

empty

### **Intervention groups**

#### 1

#### **Description**

Intervention group: Patients receiving 50-70 million autologous MSCs derived from bone marrow

#### **Category**

Treatment - Drugs

#### 2

#### **Description**

Control group: Control group: patients with cerebellar ataxia who will receive routine treatments including vitamin E, CoQ10, physical therapy and occupational therapy.

#### **Category**

Treatment - Drugs

### **Recruitment centers**

#### 1

#### **Recruitment center**

##### **Name of recruitment center**

Pasteur hospital

##### **Full name of responsible person**

Dr. Amir Reza Boroumand

##### **Street address**

Ahmad Abad St. Pasteur Av. Pasteur Hospital

##### **City**

Mashhad

##### **Province**

Razavi Khorasan

##### **Postal code**

09194216791

##### **Phone**

+98 51 3841 0245

##### **Email**

info@pastorno-hospital.com

##### **Web page address**

<http://pastorno-hospital.com/>

### **Sponsors / Funding sources**

#### 1

#### **Sponsor**

##### **Name of organization / entity**

Mashhad University of Medical Sciences

##### **Full name of responsible person**

Dr Majid Ghayour Mobarhan

##### **Street address**

Azadi SQ. Mashhad

##### **City**

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3316-913791

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##### **Email**

Ums@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**

Dr. Amir Reza Boroumand

##### **Position**

specialist

##### **Latest degree**

Subspecialist

##### **Other areas of specialty/work**

Neuroscience

##### **Street address**

Daneshgah st. Kafaei1 Av. Sadra building

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## Person responsible for scientific inquiries

**Contact**

**Name of organization / entity**

Mashhad University of Medical Sciences

**Full name of responsible person**

Dr. Seyyed Jalil tavakol afshari

**Position**

prof.

**Latest degree**

Ph.D.

**Other areas of specialty/work**

Immunology

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Bou Ali SQ, Bou Ali Research center

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## Person responsible for updating data

**Contact**

**Name of organization / entity**

Mashhad University of Medical Sciences

**Full name of responsible person**

Dr.Seyad Jalil Tavakol Afshari

**Position**

Professor

**Latest degree**

Ph.D.

**Other areas of specialty/work**

Immunology

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Tavakolaj@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**

there is no more information

**Study Protocol**

Yes - There is a plan to make this available

**Statistical Analysis Plan**

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

**Informed Consent Form**

Yes - There is 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**

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

**Title and more details about the data/document**

the project protocol will be reported after the end of the study

**When the data will become available and for how long**

Access time will be 6 moth after results publication

**To whom data/document is available**

All people without limitation

**Under which criteria data/document could be used**

any scientific usage

**From where data/document is obtainable**

Contact the project manager Dr. Tavakal Afshari with the following details. Address: Ferdowsi Square, Bo Ali Square, Mashhad, Bo Ali Research Institute, Department of Immunogenetics and Cell Culture Phone: 0517112674 tavakolaj@mums.ac.ir

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

Send a written request by email to the project manager, Dr. Tavakal Afshari, with the mentioned address and contact number

**Comments**