

Clinical Trial Protocol

Iranian Registry of Clinical Trials

18 Jul 2026

Safety and Efficacy of Bone marrow derived-mesenchymal stem cells in comparison with their exosomes in Amyotrophic Lateral Sclerosis: A randomized controlled phase 1 study

Protocol summary

Study aim

The aim of this research is to evaluate the safety and clinical efficacy of bone marrow-derived mesenchymal stem cells (BMMSCs) and exosomes in ALS.

Design

This phase 1 clinical trial has a control group, with parallel groups, one-arm blinded, randomized, on 5 patients. A computerized randomization program is used for randomization.

Settings and conduct

The location of the project will be Imam Reza Hospital in Mashhad. After the inclusion visit, patients are followed up for 6 months before the first visit to evaluate the progress of their disease. In a therapeutic visit, patients are transplanted by intrathecal injection of MSCs or exosomes, or a combination. After transplantation, patients are examined monthly and evaluated for ALSFRS-R scoring and FVC for the entire follow-up period after the last injection. Follow-up visits include observation of side effects, complete neurological evaluation, ALS score, and FVC testing. Collection and analysis of study data will be collected and analyzed by a research assistant who is blinded to allocation and intervention.

Participants/Inclusion and exclusion criteria

Inclusion criteria: definitive diagnosis of ALS, duration of symptoms less than 36 months, progressive muscle weakness, forced vital capacity (FVC) more than 60%, ability to provide informed consent, no use of riluzole, negative pregnancy test, ability to perform Thoracolumbar laminectomy or laminoplasty, and tolerance of immunosuppressive regimen. Exclusion criteria: use of invasive breathing equipment, diagnosis of other significant diseases, appearance of known immunodeficiency syndrome, unstable psychiatric diseases, and any condition in the lower limb.

Intervention groups

1. Injection of BMMSCs, 2. Exosome, 3. Injection of BMMSCs + exosome, and 4. Routine treatments.

Main outcome variables

The main outcome variable will be included the individual's score on the ALS-FRS-R and FVC.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20200904048610N1**

Registration date: **2023-05-09, 1402/02/19**

Registration timing: **prospective**

Last update: **2023-05-09, 1402/02/19**

Update count: **0**

Registration date

2023-05-09, 1402/02/19

Registrant information

Name

Javad Momeni

Name of organization / entity

Country

Iran (Islamic Republic of)

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+98 51 3884 9152

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Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2023-06-22, 1402/04/01

Expected recruitment end date

2023-10-22, 1402/07/30

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Safety and Efficacy of Bone marrow derived-mesenchymal stem cells in comparison with their exosomes in Amyotrophic Lateral Sclerosis: A randomized controlled phase 1 study

Public title

Effects of cell therapy and exosome in the treatment of amyotrophic lateral sclerosis

Purpose

Treatment

Inclusion/Exclusion criteria**Inclusion criteria:**

Definitive diagnosis of ALS (according to laboratory findings or El Escorial criteria) Duration of symptoms less than 36 months Progressive muscle weakness in the lower limb, based on EMG in the lower limb bilaterally Mandatory vital capacity more than 60% of the predicted normal in lying on the back Age 18 and above Ability to provide informed consent The person can be geographically available and refer for the required visits. Being accompanied by a caregiver and performing the care needed by him during the study Failure to use riluzole or a fixed dose in the previous 30 days or more In women of reproductive age, having a negative pregnancy test before stem cell injection Medically able to perform thoracolumbar laminectomy or laminoplasty as determined by the surgeon. Medically able to tolerate an immunosuppressive regimen.

Exclusion criteria:

Use of aggressive breathing equipment Diagnosis of other significant or unstable medical and clinical conditions that can interfere with the study. The occurrence of any of the following situations: Alcohol consumption with illegal and non-prescription drugs, Appearance of known immunodeficiency syndrome, Unstable medical conditions, Occurrence of unstable psychiatric diseases including psychosis, or major depression People of reproductive age who are not interested in birth control. Receiving any other investigational device or drug Any condition in the lower limb that prevents the testing of muscle strength Any condition that the specialist feels can cause difficulty in performing injections and follow-ups Any condition or subset of ALS that the researcher feels could interfere with the study or final interpretation.

Age

From **18 years** old

Gender

Both

Phase

1

Groups that have been masked

- Outcome assessor
- Data analyser

- Data and Safety Monitoring Board

Sample size

Target sample size: **5**

Randomization (investigator's opinion)

Randomized

Randomization description

Patients who meet the inclusion criteria are randomly assigned to treatment and control groups after signing the informed consent form. Random allocation will be done by a person outside the study using a computerized randomization program and random blocks of four people.

Blinding (investigator's opinion)

Single blinded

Blinding description

Random allocation will be done by an individual outside the study. This randomization technique will be used to ensure that principal investigators are blinded to random allocation. According to the study design, the researcher who implements the cell or exosome injection intervention will be aware of the groups. However, unblinded clinical teams administering the cells or exosome (anaesthesiologist or other teams) will not have contact with participants before or after administration. Also, the data collection and analysis of the study will be done by a research assistant who is blinded to the allocation and intervention.

Placebo

Not used

Assignment

Parallel

Other design features**Secondary Ids**

empty

Ethics committees**1****Ethics committee****Name of ethics committee**

Research Ethics Committees of Mashhad University of Medical Sciences

Street address

Qurshi Building, Daneshgah Street

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Razavi Khorasan

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9177899191

Approval date

2023-03-11, 1401/12/20

Ethics committee reference number

IR.MUMS.REC.1401.404

Health conditions studied

1

Description of health condition studied

Amyotrophic Lateral Sclerosis

ICD-10 code

G12.21

ICD-10 code description

Amyotrophic lateral sclerosis

Primary outcomes

1

Description

Score in revised ALS functional rating scale (ALSFRS-R)

Timepoint

1, 3 and 6 months and 1 year

Method of measurement

revised ALS functional rating scale (ALSFRS-R)

2

Description

The patient's score is Forced Vital Capacity (FVC)

Timepoint

1, 3 and 6 months and 1 year

Method of measurement

Spirometry

Secondary outcomes

empty

Intervention groups

1

Description

Intervention group: They receive MSCs derived from bone marrow.

Category

Treatment - Other

2

Description

Intervention group: They receive exosomes.

Category

Treatment - Other

3

Description

Intervention group: They receive bone marrow-derived mesenchymal stem cells along with exosomes.

Category

Treatment - Other

4

Description

Control group: Receives routine drug therapy.

Category

N/A

Recruitment centers

1

Recruitment center

Name of recruitment center

Imam Reza Research and Medical Training Center

Full name of responsible person

Sajad Sahab Negah

Street address

Imam Reza Hospital Square

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

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Grant name

Grant code / Reference number

Is the source of funding the same sponsor organization/entity?

No

Title of funding source

Patient-centered

Proportion provided by this source

100

Public or private sector

Private

Domestic or foreign origin

Domestic

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

Person responsible for general inquiries

Contact

Name of organization / entity

Mashhad University of Medical Sciences

Full name of responsible person

Sajad Sahab Negah

Position

Associate professor

Latest degree

Ph.D.

Other areas of specialty/work

Neuroscience

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Position

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Latest degree

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Other areas of specialty/work

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Sharing plan

Deidentified Individual Participant Data Set (IPD)

Yes - There is a plan to make this available

Study Protocol

Undecided - It is not yet known if there will be 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

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

Clinical Study Report

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

Analytic Code

Undecided - It is not yet known if there will be 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

Not applicable

When the data will become available and for how long

Not applicable

To whom data/document is available

Not applicable

Under which criteria data/document could be used

Not applicable

From where data/document is obtainable

Not applicable

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

Not applicable

Comments