Clinical Trial Protocol
Iranian Registry of Clinical Trials

25 Nov 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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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
City
Mashhad
Province
Razavi Khorasan
Postal code
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
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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

Persons

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
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Person responsible for updating data

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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