

Clinical Trial Protocol

Iranian Registry of Clinical Trials

26 Jul 2026

Assessment of Efficacy of umbilical cord derived mesenchymal Stem Cells transplantation on movement improvement in patients with neuromuscular disorders

Protocol summary

Study aim

Efficacy of umbilical cord derived Mesenchymal Stem Cells transplantation on movement improvement in patients with neuromuscular disorders

Design

This study is phase 3 of clinical trial. In this study, there are four categories of inherited neuromuscular diseases. Each category includes 30 people of two 15 people of test and control. Blinding has not been done. Random allocation to two groups of test and control is using block randomization with four blocks. Sample size is 30 people in each category, with 4 categories, total number of patients is 60 people in control group and 60 people in test group.

Settings and conduct

The site of transplantation is in Farhikhtegan hospital. For each patient, four injections are given at the first injection, two weeks later, one month later, and again three months intervals. 3 million per kilogram body weight mesenchymal stem cell are transplanted into the patient's weak muscles under sedation conditions and under ultrasound guidance. After the cell injection, the patient is hospitalized and monitored for 24 hours.

Participants/Inclusion and exclusion criteria

Inclusion Criteria: Patients 4 to 35 years old affected with genetic neuromuscular disorders. Exclusion Criteria: cancer; chromosomal defects; Patients with HIV, HCV, HBV, HTLV1,2; Dissatisfaction for entering the study.

Intervention groups

120 patients with four inherited neuromuscular diseases entered in this study. These patients were classified into 4 groups of 30 people. Each group consists of two groups of 15 test and 15 control. In patients of test group, mesenchymal stem cells are injected in weak muscles. There is no intervention in the control group. After 9 months, the clinical sign and symptoms, including the distance of walking for 6 minutes, were compared

between the two groups.

Main outcome variables

Muscle strength enhancement with umbilical cord mesenchymal stem cell transplantation

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20200426047213N1**

Registration date: **2020-05-14, 1399/02/25**

Registration timing: **registered_while_recruiting**

Last update: **2020-05-14, 1399/02/25**

Update count: **0**

Registration date

2020-05-14, 1399/02/25

Registrant information

Name

MOHAMMADKAZEM BAKHSHANDEH

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 21 2640 0779

Email address

drbakhshandeh.com@gmail.com

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2019-10-23, 1398/08/01

Expected recruitment end date

2020-06-21, 1399/04/01

Actual recruitment start date

empty

Actual recruitment end date
empty

Trial completion date
empty

Scientific title
Assessment of Efficacy of umbilical cord derived mesenchymal Stem Cells transplantation on movement improvement in patients with neuromuscular disorders

Public title
Efficacy of umbilical cord Mesenchymal Stem Cells on patients with neuromuscular disorders

Purpose
Treatment

Inclusion/Exclusion criteria
Inclusion criteria:
Genetic confirmed patients with Dystrophinopathy(Duchenne muscular dystrophy, Becker muscular dystrophy) Genetic confirmed patients with Neuropathy(Spinal muscular atrophy, Charcot-Marie-Tooth) Genetic confirmed patients with other Muscular dystrophy(Limb girdle muscular dystrophy, Congenital muscular dystrophy) Genetic confirmed patients with other Myopathy (Congenital myopathy, Congenital myasthenic syndrome)
Exclusion criteria:
Patients with cancer Patients with chromosomal defects Patients with HIV, HCV, HBV, HTLV1,2 Dissatisfaction for entering the study

Age
From **4 years** old to **35 years** old

Gender
Both

Phase
3

Groups that have been masked
No information

Sample size
Target sample size: **120**

Randomization (investigator's opinion)
Randomized

Randomization description
In order to randomly assign to the two groups of test and control, the block random division method with four blocks was used. In this way, the letter A was assigned to the test group and the letter B to the control group. In four blocks with letters A and B, the six modes AAB, BBAA, ABAB, BABA, ABBA, BAAB were written on separate sheets and thrown inside a container and accidentally removed one of these sheets from the container. And the composition written on it was noted and again "that sheet was thrown into the container." Because the sample size in this study was 30 patients, this operation was repeated eight times, and each time the composition written on each sheet was written in the sequence of the composition written on the previous sheet. Then, one letter from one to thirty letters was assigned to each of the letters in order, and each letter was placed in an envelope and the number was written on the envelope. Each time the disease was selected,

one of the envelopes was opened in order of the number written on the envelope, and it was determined that the patient should be in the test or control group.

Blinding (investigator's opinion)
Not blinded

Blinding description
Placebo
Not used

Assignment
Parallel

Other design features
Patients are divided into 4 groups of 30 patients (15 for Test group and 15 for control patients).The first group of patients with Duchenne and Becker muscular dystrophy based on genetic testing, the second group of neuropathic patients such as Spinal Muscular Atrophy and Charcot-Marie-Thoth disease based on genetic testing, the third group of patients with other types of muscular dystrophy such as Limb girdle Muscular Dystrophy and Congenital muscular dystrophy Based on genetic testing, the fourth group are other inherited myopathies such as congenital myopathy and congenital myasthenic syndrome.

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Research ethics committee of Tehran Islamic Azad university of Medical Sciences

Street address

Dr. MohammadKazem Bakhshandeh, Unit 6, Second floor, Mehr Afarin Doctors' Building, End of Morshedi Dead End, In Front of IranMehr Hospital, After Zafar St., Shariati St., Theran, Iran

City

Tehran

Province

Tehran

Postal code

1913776638

Approval date

2019-09-02, 1398/06/11

Ethics committee reference number

IR.IAU.TMU.REC.1398.104

Health conditions studied

1

Description of health condition studied

Dystrophinopathy(Duchenne muscular dystrophy, Becker muscular dystrophy)

ICD-10 code

G71.0

ICD-10 code description

Muscular dystrophy

2

Description of health condition studied

Spinal muscular atrophy

ICD-10 code

G12

ICD-10 code description

Spinal muscular atrophy and related syndromes

3

Description of health condition studied

other Myopathy (Congenital myopathy, Congenital myasthenic syndrome)

ICD-10 code

G71.2

ICD-10 code description

Congenital myopathies

4

Description of health condition studied

Charcot-Marie-Tooth)

ICD-10 code

G60.0

ICD-10 code description

Hereditary motor and sensory neuropathy

Primary outcomes

1

Description

Muscle power

Timepoint

Before study, 6 and 9 and 12 months after Mesenchymal stem cell transplantation

Method of measurement

Walking distance in 6 minutes and muscular force examination

Secondary outcomes

empty

Intervention groups

1

Description

Intervention group: In intervention group or test group the transplantation or injection of mesenchymal stem cell will be done. In this study the site of transplantation is in operation room of Farhikhtegan subspecialty hospital. Before the stem cell injection, the patient needs to be hospitalized for one day. For each patient, four injections are given at the first injection, two weeks later, one month later, and again three months intervals. 3 million per kilogram body weight mesenchymal stem cell are transplanted into the patient's weak muscles under sedation conditions and under ultrasound guidance. After

the umbilical cord mesenchymal cell injection, the patient is hospitalized and monitored for 24 hours.

Category

Treatment - Other

2

Description

Control group: For each patient in Test group, there is a patient with same genetic mutation in control group. There is no intervention in control group. After determined intervention the clinical feature of these two group will be compared.

Category

Treatment - Other

Recruitment centers

1

Recruitment center

Name of recruitment center

Farhikhtegan subspecialty hospital

Full name of responsible person

Mohammad Kazem Bakhshandeh

Street address

Dr. MohammadKazem Bakhshandeh, Unit 6, Second floor, Mehr Afarin Doctors' Building, End of Morshedi Dead End, In Front of IranMehr Hospital, After Zafar St., Shariati St., Theran, Iran

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Tehran Islamic Azad university of Medical Sciences

Full name of responsible person

Mohammad Kazem Bakhshandeh

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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

Tehran Islamic Azad university of Medical Sciences

Proportion provided by this source

20

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

Academic

Person responsible for general inquiries

Contact

Name of organization / entity

Tehran Islamic Azad university of Medical Sciences

Full name of responsible person

Mohammad Kazem Bakhshandeh

Position

Assistant professor

Latest degree

Subspecialist

Other areas of specialty/work

Pediatric neurologist, neuromuscular fellowship

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Person responsible for scientific inquiries

Contact

Name of organization / entity

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Full name of responsible person

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Position

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Latest degree

Subspecialist

Other areas of specialty/work

pediatric neurologist, neuromuscular fellowship

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Person responsible for updating data

Contact

Name of organization / entity

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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

Yes - There is a plan to make this available

Statistical Analysis Plan

Yes - There is 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

Yes - There is a plan to make this available

Data Dictionary

Yes - There is a plan to make this available

Title and more details about the data/document

After 12 months we will publish the analysis of datas

When the data will become available and for how long

After 12 months we will publish the analysis of datas

To whom data/document is available

All interested researchers

Under which criteria data/document could be used

After 12 months we will publish the analysis of datas

From where data/document is obtainable

Mohammadkazem Bakhshandeh

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

Official request from research department

Comments