

Clinical Trial Protocol

Iranian Registry of Clinical Trials

17 Sep 2026

The effectiveness of allogeneic Adipose derived mesenchymal stem cells on the phenotypic changes of Werdnig Hoffman patients

Protocol summary

Summary

1) Objectives: Currently there is no treatment for Werdnig Hoffman patients. Stem cell transplantation is a strategy that is actively ongoing in research and development to protect neurons. Non-neural cells could be used to prevent the death of motor neurons. Some studies on hematopoietic stem cells, microglia cells and mesenchymal stem cells (MSC) by injection into Cerebro-Spinal Fluid (CSF) have considered effective in protecting motor neurons. So perhaps the only way for such patients depends on using stem cells that could deliver trophic factors to the dying cells. 2) Design: In this study we will discuss a non-randomize trial for the efficiency of MSCs on the possible phenotypic changes in Werdnig Hoffman patients. 3) Setting & Conduct: Processing of adipose tissue and isolation of MSCs under GMP condition, Proliferation of MSCs, Conducting quality control tests, Performing electromyogram (EMG) before any injection, The first injection of MSCs via intrathecal route at the dose of 1 million cells, The second injection after 2 weeks at the dose of 3 million cells, The third injection after 4 weeks at the dose of 5 million cells, Sampling of CSF to detect the injected cells, EMG again after the last intervention; 4) Participant including major eligibility criteria: Inclusion criteria: Age under 12 month; Weak muscle tone; Patient sit is not fully nerve guided; Existence of home senses; Normal brain function; Exclusion criteria: Age up to 12 month; Other types of spinal muscular atrophies; Patient with sensory loss; Brain abnormalities; 5) Intervention: Overall 10 patients will be enrolled, of which 5 patients will be injected with MSCs; Allogeneic adipose derived MSCs will be injected (3 times) intrathecally, Sampling from CSF to detect the injected cells; 6) Main outcome measures: Improve in disease phenotype, Probable increased longevity.

General information

Acronym

IRCT registration information

IRCT registration number: **IRCT2015073023417N1**

Registration date: **2015-09-12, 1394/06/21**

Registration timing: **prospective**

Last update:

Update count: **0**

Registration date

2015-09-12, 1394/06/21

Registrant information

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Amir Ali Hamidieh

Name of organization / entity

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Recruitment status

Recruitment complete

Funding source

Vice Chancellor for Research, Tehran University of Medical Sciences ; Iranian council for development of Stem cell sciences & Technologies, Vice presidency for Science and Technology

Expected recruitment start date

2015-09-23, 1394/07/01

Expected recruitment end date

2016-05-21, 1395/03/01

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

The effectiveness of allogeneic Adipose derived mesenchymal stem cells on the phenotypic changes of Werdnig Hoffman patients

Public title

The efficiency of Cell therapy for Werdnig Hoffman patients

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria: Age under 12 month; Weak muscle tone; Weakness in mobility; Patients sitting without full conduction of nerve; Existence of home senses; Normal Brain function; Exclusion criteria: Age beyond 12 month; Brain abnormality; Loss of sensory functions; Malignancies.

Age

From **6 months** old to **1 year** old

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

Parallel

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Ethics committee of Tehran University of Medical Sciences

Street address

6th Floor, Central Organization of University, Qods Ave, Keshavarz Blvd, Tel. +982188987381

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Tehran

Postal code

Approval date

2015-08-17, 1394/05/26

Ethics committee reference number

IR.TUMS.REC.1394.607

Health conditions studied

1

Description of health condition studied

Werdnig Hoffman

ICD-10 code

G12

ICD-10 code description

Infantile spinal muscular atrophy, type I [Werdnig-Hoffman]

Primary outcomes

1

Description

Muscle Tone

Timepoint

Before intervention, 3 month after final intervention

Method of measurement

Electromyogram (EMG)

Secondary outcomes

1

Description

Change in patients muscle tone

Timepoint

Immediately after intervention

Method of measurement

Electromyogram

2

Description

Existence of injected cells

Timepoint

After final intervention

Method of measurement

Spinal cord fluid sampling

3

Description

Sensitivity or infection

Timepoint

After intervention

Method of measurement

Direct observation

4

Description

Patients Longevity

Timepoint

After the final intervention

Method of measurement

Direct observation

Intervention groups

1

Description

In the intervention group that comprises of 5 patients: After genetical confirmation of disease and the electromyogram test, there will be intrathecal injections of mesenchymal stem cells at 3 doses after 2 weeks interval. Dose escalation will be 2, 5 and 10 million allogeneic adipose derived mesenchymal stem cells in order. After the last injection of stem cells there will be an electromyogram confirmation test.

Category

Treatment - Other

2

Description

In the control group that comprises of 5 patients: There will be no injection. The only intervention will be the electromyogram tests at the same time with intervention group.

Category

Other

Recruitment centers

1

Recruitment center

Name of recruitment center

Children Medical Center

Full name of responsible person

Mahmoudreza Ashrafi

Street address

No 62, Neurology department, Children Medical Center, Near Imam Khomeini Hospital, Dr Gharib Ave, Keshavarz Blvd

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Vice chancellor for research, Tehran University of Medical Sciences

Full name of responsible person

Dr. Masoud Younesian

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Grant name

Grant code / Reference number

28524

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

Yes

Title of funding source

Vice chancellor for research, Tehran University of Medical Sciences

Proportion provided by this source

Public or private sector

empty

Domestic or foreign origin

empty

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

empty

2

Sponsor

Name of organization / entity

Vice Presidency for Science and Technology

Full name of responsible person

Dr. Ramin Heshmat

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Ladan Alley, Sheikh Bahayi Shomali Ave, Mollasadra Ave

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Grant name

Grant code / Reference number

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

Yes

Title of funding source

Vice Presidency for Science and Technology

Proportion provided by this source

Public or private sector

empty

Domestic or foreign origin

empty

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

empty

Person responsible for general inquiries

Contact

Name of organization / entity

School of Advanced Technologies in Medicine, Tehran University of Medical Sciences

Full name of responsible person

Rashin Mohseni

Position

PhD candidate

Other areas of specialty/work

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Person responsible for scientific inquiries

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Sharing plan

Deidentified Individual Participant Data Set (IPD)

empty

Study Protocol

empty

Statistical Analysis Plan

empty

Informed Consent Form

empty

Clinical Study Report

empty

Analytic Code

empty

Data Dictionary

empty