Clinical Trial Protocol
Iranian Registry of Clinical Trials

12 Nov 2026

The effectivness 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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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 effectivness 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 beyound 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
City
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
Street address
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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
Street address
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

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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