

Clinical Trial Protocol

Iranian Registry of Clinical Trials

11 Sep 2026

Evaluation of safety and efficacy of Allogenic Mesenchymal Stem Cell transplantation (core matrix extracted) in Late-Onset Neurometabolic disorders: an open label study

Protocol summary

Study aim

Effective treatment finding for some untreated neurometabolic disorders

Design

Community based and pragmatic, non-randomized single group, clinical trial without blinded postoperative care and outcome assessment

Settings and conduct

Patients that selected for this clinical trial, will be admitted to Labafinejad or Farmanieh Hospital in Tehran. Based on CNS involvement, one or more injections will be planned by intrathecal and/or intravenous route. Injectable mesenchymal stem cell will be prepared in clean room by Sinacell company, 3-4 million cells per kilogram more than 90% viability in 20-50 milliliter injectable human albumin as preservative in each intravenous injection and one million per kilogram in 5-10 milliliter in each intrathecal injection. Interval of these injections is between two days to two weeks. The hospitalization time will be about 6 hours for intravenous injection and 24 to 48 hours for intrathecal injection. Patients will undergo a clinical and laboratory evaluation for a period of 2 years at defined intervals.

Participants/Inclusion and exclusion criteria

Inclusion Criteria: Patients with genetic proven late onset neurometabolic disorders which have developed symptoms but have not yet reached end stage phase. Exclusion criteria: Patients who are susceptible to malignancy, patients with chronic infectious diseases such as AIDS, hepatitis, syphilis and HTLV, pregnancy, vulnerable ages and conditions.

Intervention groups

Wharton jelly derived allogenic mesenchymal stem cells in suitable albumin solution will be injected to leukodystrophy, ataxic, myopathic, neuropathic patients by peripheral and/or intrathecal routes.

Main outcome variables

Qualitative evaluation of ataxia by SARA Questionnaire, Qualitative evaluation of mental and physical functions by MSFC Questionnaire, Chance of Diabetes Mellitus occurrence, Chance of heart Failure occurrence.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20180228038899N1**

Registration date: **2018-07-16, 1397/04/25**

Registration timing: **registered_while_recruiting**

Last update: **2018-07-16, 1397/04/25**

Update count: **0**

Registration date

2018-07-16, 1397/04/25

Registrant information

Name

Bitia Shalbafan

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 21 2271 8565

Email address

shalbafan.b@gmail.com

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2017-02-21, 1395/12/03

Expected recruitment end date

2020-09-21, 1399/06/31

Actual recruitment start date

empty

Actual recruitment end date
empty

Trial completion date
empty

Scientific title
Evaluation of safety and efficacy of Allogenic Mesenchymal Stem Cell transplantation (core matrix extracted) in Late-Onset Neurometabolic disorders: an open label study

Public title
Effect of Allogenic Stem Cell in Neurometabolic disorders

Purpose
Treatment

Inclusion/Exclusion criteria
Inclusion criteria:
The definitive diagnosis of neurometabolic diseases leads to ataxia, leukodystrophy, Myopathy or Neuropathy based on laboratory findings of molecular genetics Age between 18-40 years old Written consent of the patient and parents to attend the study Normal routine biochemistry, hematology and negative serology and virology tests Negative Tumor survey includes abdominal and pelvic ultrasonography and prostatic and breast and thyroid ,and occult blood in the stool test
Exclusion criteria:
A pregnant woman (positive pregnancy test) or a nursing or illness who is planning a pregnancy during the study A disease that is in addition to the involvement of a nervous system with another serious illness, such as hemodynamic disorders, homeostasis disorders, diabetes, cardiovascular / pulmonary disease, etc. Having a serious psychiatric illness or having a history of suicide Treatment with cytotoxic drugs within a month before starting the study The presence of any suspected malignancy mass Serum creatinine more than 1.7 Rised liver enzyme tests more than three times White blood cell count lower than 3000 Positive response to each of the serum tests of HTLV1,2 Ab, HIV1,2Ab, HBcAb, HBsAg, HCVAb

Age
From **18 years** old to **40 years** old

Gender
Both

Phase
2

Groups that have been masked
No information

Sample size
Target sample size: **25**

Randomization (investigator's opinion)
N/A

Randomization description

Blinding (investigator's opinion)
Not blinded

Blinding description

Placebo
Not used

Assignment
Single

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee

Ethics committee of Shahid Beheshti University of Medical Sciences

Street address

Research Management office, 6th floor, building No.2, Shahid Beheshti Medical University, Arabi Ave., Daneshjoo Blvd., Velenjak Town

City

Tehran

Province

Tehran

Postal code

1983963113

Approval date

2017-02-21, 1395/12/03

Ethics committee reference number

lr.sbm.u.rec.1395.82

Health conditions studied

1

Description of health condition studied

Progressive Ataxia

ICD-10 code

G11

ICD-10 code description

Hereditary ataxia

2

Description of health condition studied

Myopathy

ICD-10 code

M62.5

ICD-10 code description

Muscle wasting and atrophy, not elsewhere classified, unspecified site

3

Description of health condition studied

Neuropathy

ICD-10 code

G60

ICD-10 code description

Hereditary and idiopathic neuropathy

4

Description of health condition studied

Leukodystrophy

ICD-10 code

E75.2

ICD-10 code description

Other sphingolipidosis

Primary outcomes**1****Description**

Ataxia in Ataxic patients

Timepoint

At first, 1,3,6,12,18,24 months after intervention

Method of measurement

Scale of Assessment and Rating of Ataxia in Ataxic patients

2**Description**

Functional Capacity in paretic patients with or without ataxia and mental problems

Timepoint

At first, 1,3,6,12,18,24 months after intervention

Method of measurement

Multiple Sclerosis Functional Capacity scoring

3**Description**

Blood Sugar assessment in patients with Friedreich Ataxia diagnosis

Timepoint

At first, 1,3,6,12,18,24 months after intervention

Method of measurement

Laboratory measurements of Fasting Blood Sugar and Hemoglobin A1C

4**Description**

Heart failure assessment in patients with Friedreich Ataxia diagnosis

Timepoint

At first, 1,3,6,12,18,24 months after intervention

Method of measurement

Ejection Fraction by Echocardiography

Secondary outcomes

empty

Intervention groups**1****Description**

Intervention group: After patient selection, one or more injections will be planned by intrathecal and/or intravenous route based on CNS involvement. Injectable mesenchymal stem cell will be prepared in clean room by Sinacell company, 3-4 million cells per kilogram more than 90% viability in 20-50 milliliter injectable human

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Category

Treatment - Drugs

Recruitment centers**1****Recruitment center****Name of recruitment center**

Labafinejad Hospital

Full name of responsible person

Bita Shalbafan

Street address

No. 133, 9th Boustan Ave., Pasdaran Ave.

City

Tehran

Province

Tehran

Postal code

1666659534

Phone

+98 21 2278 3140

Email

shalbafan.b@gmail.com

Sponsors / Funding sources**1****Sponsor****Name of organization / entity**

Pasture Institute of Iran

Full name of responsible person

Dr. Mohsen Asouri

Street address

North Institute, Pastor Institute of Iran, Km 5 Babol old road,

City

Amol

Province

Mazandaran

Postal code

4619332976

Phone

+98 11 4319 8074

Fax

+98 11 4319 8651

Email

mohsen.asouri@yahoo.com

Grant name**Grant code / Reference number****Is the source of funding the same sponsor organization/entity?**

Yes

Title of funding source

Pasture Institute of Iran

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**

Other

Person responsible for general inquiries**Contact****Name of organization / entity**

Iran Social Security Organization

Full name of responsible person

Bita Shalbafan

Position

consultant

Latest degree

Specialist

Other areas of specialty/work

Neurology

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Person responsible for scientific inquiries**Contact****Name of organization / entity**

iran social security Organization

Full name of responsible person

Bita Shalbafan

Position

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Latest degree

Specialist

Other areas of specialty/work

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Person responsible for updating data**Contact****Name of organization / entity**

Iran Social Security Organization

Full name of responsible person

Bita Shalbafan

Position

consultant

Latest degree

Specialist

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

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

Not applicable

Data Dictionary

Not applicable

Title and more details about the data/document

After keeping samples privacy , we will share their demographic data and all of findings.

When the data will become available and for how long

We will publish our findings as an article after 6 months.

To whom data/document is available

Somebody are interesting to research in the field of Stem cell.

Under which criteria data/document could be used

For researchers in the field of stem cells, statistical analyzes can be done.

From where data/document is obtainable

1-Dr. Bita Shalbafan +989161119656

shalbafan.b@gmail.com 2-Dr. Mandana Mohyeddin
Bonab +989122024962 mohyeddin@sina.tums.ac.ir

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

Six months after the final article is published, the

applicants will submit their application by email or telephone and receive data within a maximum of one month by email.

Comments

no