Clinical Trial Protocol
Iranian Registry of Clinical Trials

10 Jun 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 inTehran. Based on CNS involvement,one or much more injections will be planned by intrathecal and/or intravenous rout. Injectable mesenchymal stem cell will be prepared in clean room by Sinacell company, 3-4 million cell 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 24 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 inject to leukodystrophy, ataxic, myopathic, neuropathic patients by peripheral and/ or intrathecal routs.

Main outcome variables

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

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

Bita 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

Ir.sbmu.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 Assesment 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 much more injections will be planned by intrathecal and/or intravenous rout based on CNS involvement . Injectable mesenchymal stem cell will be prepared in clean room by Sinacell company, 3-4 million cell 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 24 48 hours for intrathecal injection. Patients will undergo a clinical and laboratory evaluation for a period of 2 years at defined intervals.
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.
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1666659534
Phone
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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
Street address
No.133,9th Boustan Ave.,Pasdaran Ave.
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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
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
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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