

# 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 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 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 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

Bitá 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 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.

**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**

Bitá 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**

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

Iran Social Security Organization

**Full name of responsible person**

Bitá Shalbafan

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**Latest degree**

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**Email**

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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. Bitá 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