Clinical Trial Protocol
Iranian Registry of Clinical Trials

09 Jun 2026

Evaluation of the effectiveness of intrathecal injection of allogeneic umbilical cord derived mesenchymal stem cells in the phenotypic changes of Spinal Muscular Atrophy type I, II and III in comparison with control group, A Clinical trial phase I, II

Protocol summary

Study aim

Evaluation of the efficacy of intrathecal injection of umbilical cord derived mesenchymal stem cells in the phenotypic changes of SMA I, II and III

Design

Randomized double blind clinical trial phase 1-2 with control group .60 confirmed SMA patients (20 cases from each type) recruited in this study , After explanation about side effects and its probable effectiveness and taking of parent's informed consent, each type of disease will be divided accidentally in two group of 10 patients of intervention or control .

Settings and conduct

Clinical exams and baseline electrodiagnostic tests were done. Patients will be admitted in Children's Medical Center and after injection will be monitored for 24 hours. After taking of 2 ml fluid, cellular suspension will be injected. ŴWith simulation of intrathecal injection in control group, patients and clinical evaluators will not be aware about two groups. Clinical evaluations and electrodiagnostic tests will be continued for a year.

Participants/Inclusion and exclusion criteria

inclusion criteria :Genetically confirmed SMA patients and its type, Minimum age of 6 months and maximum age of 16 years, Ventilator independent, Absence of other organs disorders Exclusion criteria : Age under 6 months and over 16 years, Ventilator dependent at the beginning of intervention, Serious diseases of other organs

Intervention groups

In the intervention group 3 sessions of intrathecal injection of 20 millions mesemchymal cells will be done every 2 weeks. In the control group 3 sessions of intrathecal injection simulation without any injection will be done every 2 weeks.

Main outcome variables

Evaluation of effectiveness of mesenchymal stem cells in life expectancy and probable muscle strength of SMA type 1 .Evaluation of effectiveness of mesenchymal stem cells in muscle strength of SMA type 2 and 3.

General information

Reason for update

Acronym

MSCSMA

IRCT registration information

IRCT registration number: **IRCT20110628006907N15**
Registration date: **2020-03-17, 1398/12/27**
Registration timing: **prospective**

Last update: **2020-03-17, 1398/12/27**
Update count: **0**

Registration date

2020-03-17, 1398/12/27

Registrant information

Name

Mahmoudreza Ashrafi

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 21 6612 9252

Email address

ashrafim@tums.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2020-04-20, 1399/02/01

Expected recruitment end date
2021-04-20, 1400/01/31
Actual recruitment start date
empty
Actual recruitment end date
empty
Trial completion date
empty

Scientific title
Evaluation of the effectiveness of intrathecal injection of allogeneic umbilical cord derived mesenchymal stem cells in the phenotypic changes of Spinal Muscular Atrophy type I, II and III in comparison with control group, A Clinical trial phase I, II

Public title
Effects of umbilical cord derived mesenchymal stem cells injection in the treatment of Spinal Muscular Atrophy (SMA)
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Genetically confirmed deletion of SMN1 gene for the diagnosis of SMA disease, SMN2 gene copy number count for the diagnostic classification of disease type, Minimum age of 6 months in type I, Ventilator independent patients at the beginning of treatment in type I, Maximum age of 16 years in type II and III, Ventilator independent patients in type II and III, Absence of brain damage, Absence of liver disease, Absence of renal disease, Absence of hematological disease, Informed consent of patients and their parents
Exclusion criteria:
Acquired brain damage including hypoxia, Structural and functional brain disorders, Acute infections such as(HCV, HIV,HBV), Malignancies, Hemorrhagic diathesis, Severe anemia (Hb less than 8 gram/dl), Renal dysfunction, Hepatic dysfunction
Age
From **6 months** old to **16 years** old
Gender
Both

Phase
N/A
Groups that have been masked

- Participant
- Care provider
- Outcome assessor
- Data analyser

Sample size
Target sample size: **60**
Randomization (investigator's opinion)
Randomized
Randomization description
The patients are randomly allocated using a balanced block randomization technique, into two groups of intervention and control (30 cases of intervention with injection of umbilical cord derived Mesenchymal cells, 10 cases in each type of disease and 30 cases of control group without injection, 10 cases in each type of disease) . Randomization will be done by using of blocks of 6 and 9. All subjects randomly allocated with online randomization software to generate random-number sequences. Coordinator of randomization and clinical evaluators will not be aware of patients of each group .
Blinding (investigator's opinion)
Double blinded
Blinding description
This study is double blind therefore, in the control group after insertion of the needle into the skin with an appearance simulating lumbar puncture, needle will be removed without any injection. This technique will be repeated three times every two weeks, similar to intervention groups. Patients and their parents and clinical evaluators will not be aware of this subject. At the end of the study if safety and effectiveness of cell therapy will be proved in the intervention groups, for the ethical consideration stem cell injections will be performed for control groups .
Placebo
Used
Assignment
Parallel
Other design features
First study about intrathecal cell therapy in SMA disease in comparison with control group in IRAN - First study about cell therapy by using of mesenchymal stem cells in SMA disease in IRAN

Secondary Ids

empty

Ethics committees

1

Ethics committee
Name of ethics committee
Tehran University of Medical Sciences
Street address
No 226, Central organization of Tehran University of Medical Sciences, Ghods Street, Keshavarz Blvd
City
Tehran
Province
Tehran
Postal code
1417653761
Approval date
2020-02-04, 1398/11/15
Ethics committee reference number
IR.TUMS.VCR.REC.1398.899

Health conditions studied

1

Description of health condition studied
Spinal Muscular Atrophy Type 1-2 3
ICD-10 code

G12

ICD-10 code description

Spinal muscular atrophy and related syndromes

Primary outcomes

1

Description

Evaluation of effectiveness of umbilical cord derived mesenchymal stem cells in increasing life expectancy of SMA type 1

Timepoint

Before intervention and then every 2 months till 1 year

Method of measurement

Questionnaire and clinical exams

2

Description

Evaluation of effectiveness of umbilical cord derived mesenchymal stem cells in increasing muscle strength of SMA type 1

Timepoint

Before intervention and then every 2 months till 1 year

Method of measurement

Hammersmith Infant Neurological Examinations (HINE) ,Children's Hospital of Philadelphia Questionnaire (CHOP intent)

3

Description

Evaluation of effectiveness of umbilical cord derived mesenchymal stem cells in increasing muscle strength of SMA type 2

Timepoint

Before intervention and then every 4 months till 1 year

Method of measurement

Expanded Hammersmith Functional Motor Scale(HFMSE) WHO Motor Mile stones (WMM) questionnaire , Upper limb Module Scale (ULMS)

4

Description

Evaluation of effectiveness of umbilical cord derived mesenchymal stem cells in increasing muscle strength of SMA type 3

Timepoint

Before intervention and then every 4 months till 1 year

Method of measurement

Expanded Hammersmith Functional Motor Scale(HFMSE) WHO Motor Mile stones (WMM) questionnaire, Upper limb Module Scale (ULMS) questionnaire, 6 Minute Walk Test (6MWT)

Secondary outcomes

1

Description

Improvement of Electrodiagnosic evaluative indexes of SMA patients

Timepoint

Before intervention , 2 months after last injection and then every 4 months till 1 year of first injection

Method of measurement

Motor unit number estimation (CMAP scan)

2

Description

Number of participants experiencing adverse effects and serious adverse effects

Timepoint

First 24 hours after injection and then any time if occurred

Method of measurement

Questionnaire , parents report and periodic planned clinical evaluations

Intervention groups

1

Description

Intervention group 1: Type 1 SMA patients that will receive three intrathecal injections of mesenchymal stem cells derived from umbilical cord, prepared by Royan stem cell Technology Company and Cell Thec Pharmed company. Intrathecal injections will be done under anesthesia via lumbar puncture. After taking 2 milliliters of cerebrospinal fluid, 2 milliliters suspension that contains 20 million stem cells will be injected with a syringe. The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Treatment - Drugs

2

Description

Intervention group 2 : Type 2 SMA patients that will receive three intrathecal injections of mesenchymal stem cells derived from umbilical cord, prepared by Royan stem cell Technology Company and Cell Thec Pharmed company. Intrathecal injections will be done under anesthesia via lumbar puncture. After taking 2 milliliters of cerebrospinal fluid, 2 milliliters suspension that contains 20 million stem cells will be injected with a syringe. The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Treatment - Drugs

3

Description

Intervention group 3 : Type 3 SMA patients that will receive three intrathecal injections of mesenchymal stem

cells, derived from umbilical cord prepared by Royan stem cell Technology Company and Cell Thec Pharmed company. Intrathecal injections will be done under anesthesia via lumbar puncture. After taking 2 milliliters of cerebrospinal fluid, 2 milliliters suspension that contains 20 million stem cells will be injected with a syringe. The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Treatment - Drugs

4

Description

Control group 1:Type 1 SMA patients that after insertion of needle into the skin, without entrance to cerebrospinal fluid space, needle will be removed without any injection . In this group only intrathecal injection will be simulated, without patients or their parents awareness . The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Placebo

5

Description

Control group 2: Type 2 SMA patients that after insertion of needle into the skin, without entrance to cerebrospinal fluid space, needle will be removed without any injection . In this group only intrathecal injection will be simulated, without patients or their parents awareness . The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Placebo

6

Description

Control group 3:Type 3 SMA patients that after insertion of needle into the skin, without entrance to cerebrospinal fluid space, needle will be removed without any injection . In this group only intrathecal injection will be simulated, without patients or their parents awareness . The patient will be admitted for one day of probable adverse reactions monitoring . One year followup and evaluations with regular and similar rehabilitative therapy will be done .

Category

Placebo

Recruitment centers

1

Recruitment center

Name of recruitment center

Children's Medical Center Hospital

Full name of responsible person

Reza Shervin Badv

Street address

Children's Medical Center Hospital, No 62, Gharib Street, end of keshavarz Blvd

City

Tehran

Province

Tehran

Postal code

1419733151

Phone

+98 21 6692 9234

Fax

+98 21 6612 9252

Email

drbadv@gmail.com

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Tehran University of Medical Sciences

Full name of responsible person

Mohammad Ali Sahraeian

Street address

No 226, Central organization of Tehran University of Medical Sciences, Ghods Street, Keshavarz Blvd

City

Tehran

Province

Tehran

Postal code

1417653761

Phone

+98 21 8898 7381

Email

msahrai@tums.ac.ir

Grant name

Grant code / Reference number

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

Yes

Title of funding source

Tehran University of Medical Sciences

Proportion provided by this source

1

Public or private sector

Public

Domestic or foreign origin

Domestic

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

Academic

2

Sponsor

Name of organization / entity
ROYAN stem cell technology Co

Full name of responsible person
Morteza zarrabi

Street address
No. 24, East Hafez Alley, Bani Hashim Square, Resalat Highway

City
Tehran

Province
Tehran

Postal code
1665666311

Phone
+98 21 8978 1307

Email
info@rsct.ir

Grant name

Grant code / Reference number

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

Title of funding source
ROYAN stem cell technology Co

Proportion provided by this source
99

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
Persons

Person responsible for general inquiries

Contact

Name of organization / entity
Tehran University of Medical Sciences

Full name of responsible person
Morteza Heidari

Position
Assistant Professor

Latest degree
Subspecialist

Other areas of specialty/work
Neurology

Street address
Children's Medical Center Hospital, No 62, Gharib Street, End of Keshavarz Blvd

City
Tehran

Province
Tehran

Postal code
1419733151

Phone
+98 21 6612 9252

Email
mortezah93@gmail.com

Person responsible for scientific inquiries

Contact

Name of organization / entity
Tehran University of Medical Sciences

Full name of responsible person
Mahmoud Reza Ashrafi

Position
Professor

Latest degree
Subspecialist

Other areas of specialty/work
Neurology

Street address
Children's Medical Center Hospital, No 62, Gharib Street, End of Keshavarz Blvd

City
Tehran

Province
Tehran

Postal code
1419733151

Phone
+98 21 6612 9252

Fax
+98 21 6612 9252

Email
mr_ashrafi@yahoo.com

Person responsible for updating data

Contact

Name of organization / entity
Tehran University of Medical Sciences

Full name of responsible person
Anahita Majmaa

Position
Fellowship of PICU

Latest degree
Specialist

Other areas of specialty/work
Pediatrics

Street address
Children's Medical Center Hospital, No 62, Gharib Street, End of Keshavarz Blvd

City
Tehran

Province
Tehran

Postal code
1419733151

Phone
+98 21 6612 9252

Email
majmaaa911@yahoo.com

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
Undecided - It is not yet known if there will be 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
Undecided - It is not yet known if there will be a plan to make this available
Data Dictionary
Undecided - It is not yet known if there will be a plan to make this available
Title and more details about the data/document
All collected data deidentified can be shared
When the data will become available and for how long
Availability of data starting 6 months after publication,without time limitation
To whom data/document is available
Researchers working in academic institutions and people working in businesses
Under which criteria data/document could be used
Documents will be shared for replication of study in other Medical universities
From where data/document is obtainable
Email addresses
What processes are involved for a request to access data/document
Request by email addresses
Comments