Clinical Trial Protocol
Iranian Registry of Clinical Trials

06 Nov 2026

A phase I trial evaluating the safety of 3 Times Intratechal Injection of the-MSC, an Allogenic Umbilical cord-derived Mesenchymal Stromal Cell Product, in Children with Autism Spectrum Disorder

Protocol summary

Study aim

This study aims to evaluate the safety of three intrathecal injections (at one-month intervals) of human umbilical cord tissue-derived mesenchymal stromal cells, at a fixed dose of 20 million allografts, to reduce symptoms and Improve function Children with Autism.

Design

Phase II clinical trial, safety evaluation, single group, no control

Settings and conduct

Five autistic children, aged 3 to 7 years, referred to Roozbeh Hospital who met the inclusion criteria were included in the intervention group. The subjects will receive three intrathecal injections (at one-month intervals) of, human umbilical cord tissue-derived mesenchymal stromal cells, at a fixed dose of 20 million.

Participants/Inclusion and exclusion criteria

Children aged between 3 to 7 years diagnosed with Autism (level II) will be eligible to participate. Confirmed clinical DSM-5 diagnosis of Autism using the DSM-5 Checklist with level II; History of unstable or uncontrolled seizure disorder, History of prior cell therapy or participation in other clinical trials.

Intervention groups

One group; subjects will receive three intrathecal injections (at one-month intervals) of, human umbilical cord tissue-derived mesenchymal stromal cells, at a fixed dose of 20 million.

Main outcome variables

Determination of side effects; Symptom severity based on CARS; Impact on evolution based on the ATEC form; Change in severity of behavioral disorder based on BPI-S form; EEG-based changes in electroencephalographic waves; Impact on Mental Health and Parental Stress in Children with Autism Based on SF36 Form.

General information

Reason for update

Due to challenges in patient recruitment, the eligible age range for children diagnosed with Autism Spectrum Disorder has been revised from 3–5 years to 3–7 years. This amendment was approved by the Central Ethics Committee of the Academic Center for Education, Culture, and Research (ACECR).

Acronym

MSCA

IRCT registration information

IRCT registration number: **IRCT20200217046526N3**
Registration date: **2020-04-15, 1399/01/27**
Registration timing: **prospective**

Last update: **2025-08-15, 1404/05/24**
Update count: **1**

Registration date

2020-04-15, 1399/01/27

Registrant information

Name

Masoumeh Nouri

Name of organization / entity

Royan Stem Cell Technology Co

Country

Iran (Islamic Republic of)

Phone

+98 21 2763 5512

Email address

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Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2020-06-21, 1399/04/01

Expected recruitment end date
2020-07-22, 1399/05/01
Actual recruitment start date
empty
Actual recruitment end date
empty
Trial completion date
empty

Scientific title
A phase I trial evaluating the safety of 3 Times Intratechal Injection of the-MSC, an Allogenic Umbilical cord-derived Mesenchymal Stromal Cell Product, in Children with Autism Spectrum Disorder

Public title
Stem Cell Therapy for Autism
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Confirmed clinical DSM-5 diagnosis of Autism Spectrum Disorder using the DSM-5 Checklist with level 2 Parental consent Stable on current psychiatric medication regimen (dose and dosing schedule) for at least 2 months prior to infusion of study product
Exclusion criteria:
Review of medical records indicates Autism diagnosis not likely Presence of any concomitant systemic disease (active infection, any malignancy,...), Central Nervous System Infection, people with Nervous System Inflammation, Cerebral palsy Presence of any psychiatric disorders such as Asperger's syndrome, Depression, Bipolar disorder, Schizophrenia, Tourette's syndrome History of major surgery in the last 30 days History of allogeneic tissue transplantation History of active seizure disorder over the past 6 months History of a primary immunodeficiency disorder, HIV Records indicate that the child has a known genetic syndrome related to Autism History of allergic disease Participate in other clinical trial studies
Age
From **36 months** old to **84 months** old
Gender
Both

Phase
1
Groups that have been masked
No information
Sample size
Target sample size: **5**
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
ACECR Biomedical Research Ethics Committee
Street address
No. 1270, Enghelab St., Tehran., District 11., Tehran Province., Iran
City
Tehran
Province
Tehran
Postal code
14155-4364
Approval date
2020-02-23, 1398/12/04
Ethics committee reference number
IR.ACECR.REC.1398.003

Health conditions studied

1

Description of health condition studied
Autism Spectrum Disorder
ICD-10 code
ICD-10 code description
F84.0

Primary outcomes

1

Description
Adverse events assesment
Timepoint
At the time of each injection, 24 hours after each injection, 7-10 days after each injection, 3 month after the final injection
Method of measurement
Common Terminology Criteria for Adverse Events (CTCAE) Version 4

Secondary outcomes

1

Description
Change in Childhood Autism Rating Scale, CARS
Timepoint
Before injection, 7-10 days after each injection, 3 months after the final injection
Method of measurement
Childhood Autism Rating Scale form

2

Description
Change in Autism Treatment Evaluation Checklist
Timepoint
Before injection, 7-10 days after each injection, 3 months after the final injection
Method of measurement
Autism Treatment Evaluation Checklist

3

Description
Change in the Behavior Problems Inventory
Timepoint
Before injection, 7-10 days after each injection, 3 months after the final injection
Method of measurement
The Behavior Problems Inventory Checklist

4

Description
Change in Electroencephalogram
Timepoint
Before injection, 7-10 days after each injection, 3 months after the final injection
Method of measurement
Change in EEG waves

5

Description
Assess the quality of life base on Short-form 36
Timepoint
Before injection, 7-10 days after each injection, 3 months after the final injection
Method of measurement
SF 36 questionnaire

Intervention groups

1

Description
Intervention group: 5 autistic children receiving mesenchymal cells intrathecally at one month intervals.
Category
Treatment - Other

Recruitment centers

1

Recruitment center
Name of recruitment center
Roozbeh Hospital
Full name of responsible person
Hadi Zarafshan
Street address
No. 606, South Karagar St., District 11
City
Tehran
Province
Tehran
Postal code
13337159140
Phone
+98 21 5541 9151
Email
zarafshan84@gmail.com

Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Tehran University of Medical Sciences
Full name of responsible person
Mohammad Ali Sahraian
Street address
ImamKhomeini Ave., Hasan Abad Sq., Sina Hospital
City
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Province
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١١٣٦٧٤٦٩١١
Phone
+98 21 6670 6142
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., Bani Hashim St., Resalat Highway., Tehran., Iran
City
Tehran
Province

Tehran

Postal code
۱۷۶۵۶۶۶۳۱۱

Phone
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Email
m.zarrabi@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
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
Industry

Person responsible for general inquiries

Contact

Name of organization / entity
Royan Stem Cell Thechnology Co

Full name of responsible person
Morteza Zarrabi

Position
Managing Director

Latest degree
Ph.D.

Other areas of specialty/work
Cell Therapy

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

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Person responsible for scientific inquiries

Contact

Name of organization / entity
Tehran University of Medical Sciences

Full name of responsible person
Mohammad Reza Mohammadi

Position
Professor

Latest degree
Subspecialist

Other areas of specialty/work
Psychiatrics

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Person responsible for updating data

Contact

Name of organization / entity
Royan Stem Cell Technology co

Full name of responsible person
Masoume Nouri

Position
R&D Responsible

Latest degree
Ph.D.

Other areas of specialty/work
Cell Therapy

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

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masoume.nouri2002@gmail.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
Undecided - It is not yet known if there will be a plan to make this available

Clinical Study Report
Undecided - It is not yet known if there will be 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 deidentified IPD can be shared

When the data will become available and for how long

6 months after publication

To whom data/document is available

Researchers and clinicians

Under which criteria data/document could be used

Planning of similar studies in other academic centers

From where data/document is obtainable

eMail

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

1-2 months after request during

Comments