

Clinical Trial Protocol

Iranian Registry of Clinical Trials

04 Dec 2023

A phase I trial evaluating the safety of 3 Times Intrathecal 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 5 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 ages two to 5 years 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

Acronym

MSCA

IRCT registration information

IRCT registration number: **IRCT20200217046526N3**

Registration date: **2020-04-15, 1399/01/27**

Registration timing: **prospective**

Last update: **2020-04-15, 1399/01/27**

Update count: **0**

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

m.nouri@rsct.ir

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 **60 months** old

Gender

Both

Phase

2

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

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13337159140

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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

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Postal code

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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

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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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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

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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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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