

# 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

#### **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**

تهران

#### **Province**

Tehran

#### **Postal code**

۱۱۳۶۷۴۶۹۱۱

#### **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**

۱۶۶۵۶۶۶۳۱۱

**Phone**

+98 21 2763 5512

**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

**Street address**

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

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**Email**

m.zarrabi@rsct.ir

**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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**Email**

mohammadimr@tums.ac.ir

**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

**Street address**

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**Email**

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**