Clinical Trial Protocol
Iranian Registry of Clinical Trials

14 Oct 2026

Evaluation of safety and efficacy of repeated intrathecal and/or intravenous transplantations of Wharton’s jelly derived mesenchymal stromal cells in patients with ALS, clinical trial, phase I

Protocol summary

Study aim

Evaluation of safety and efficacy of repeated intrathecal and/or intravenous transplantations of Wharton’s jelly derived mesenchymal stromal cells in patients with ALS

Design

This study, which is a phase 1 clinical trial, includes 12 patients with ALS.

Settings and conduct

The patient was admitted to the VIP room of Mostafa Khomeini Hospital and the cell suspension prepared from Whartocell allogeneic cell bank produced in the center with good manufacturing products (GMP) grade was injected intrathecally by the neurologist in charge of the project in 6 patients and intravenously by the expert nurse in charge in 6 other patients.

Participants/Inclusion and exclusion criteria

Age: 18-70 Both gender Disease onset at least: 6 months All patients diagnosed with ALS and R/O of differential diagnosis Forced Vital Capacity (FVC) ≥40٪ ALS Functional Rating Scale-Revised (ALSFRS-R)≥ 25 Under treatment with rilozule and edaravone Ability to walk even with aid and ability to tolerate the procedures

Intervention groups

1- Intrathecal transplantations of Wharton’s jelly derived mesenchymal stromal cells 2- Intravenous transplantations of Wharton’s jelly derived mesenchymal stromal cells

Main outcome variables

1- Evaluation of possible side effects after repeated intrathecal transplantation or Intravenous allogeneic cord-derived mesenchymal cells of allogeneic cord in patients with ALS 2- Evaluation of changes in ALSFRS-R score 3- Evaluation of FVC changes 4- Evaluation of year survival

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20080728001031N32**
Registration date: **2022-05-07, 1401/02/17**
Registration timing: **prospective**

Last update: **2022-05-07, 1401/02/17**
Update count: **0**

Registration date

2022-05-07, 1401/02/17

Registrant information

Name

Nasser Aghdami

Name of organization / entity

Royan Institute

Country

Iran (Islamic Republic of)

Phone

+98 21 2356 2000

Email address

nasser.aghdami@royaninstitute.org

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2022-05-20, 1401/02/30

Expected recruitment end date

2022-07-06, 1401/04/15

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title
Evaluation of safety and efficacy of repeated intrathecal and/or intravenous transplantations of Wharton’s jelly derived mesenchymal stromal cells in patients with ALS, clinical trial, phase I

Public title
Evaluation of safety and effectiveness of recurrent umbilical cord-derived stem cell transplantation in patients with ALS

Purpose
Treatment

Inclusion/Exclusion criteria

Inclusion criteria:
Age: 18-70 Both gender Disease onset at least: 6 months All patients diagnosed with ALS and R/O of differential diagnosis FVC ≥40٪ ALSFRS-R≥ 25 Under treatment with rilozule and edaravone Ability to walk even with aid and ability to tolerate the procedures

Exclusion criteria:
Concomitant psychiatric or cognitive disease Concomitant systemic disease (uncontrolled HTN, uncontrolled DM, uncontrolled kidney diseases, HF, ...) Treatment with corticosteroid , IVIg, immunosuppressive and cytotoxic during 12 months Dependency to ventilation History of malignancy Concomitant other neurodegenerative disorders

Age
From **18 years** old to **70 years** old

Gender
Both

Phase
1

Groups that have been masked
No information

Sample size
Target sample size: **12**

Randomization (investigator's opinion)
N/A

Randomization description

Blinding (investigator's opinion)
Not blinded

Blinding description

Placebo
Not used

Assignment
Parallel

Other design features

Secondary Ids

empty

Ethics committees

1

Ethics committee

Name of ethics committee
Medicel Ethics committee of Royan institute

Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran

City
Tehran

Province
Tehran

Postal code
19395-4644

Approval date
2022-03-13, 1400/12/22

Ethics committee reference number
IR.ACECR.ROYAN.REC.1400.169

Health conditions studied

1

Description of health condition studied
Amyotrophic lateral sclerosis (ALS)

ICD-10 code
G12.21

ICD-10 code description
Amyotrophic lateral sclerosis

Primary outcomes

1

Description
safety

Timepoint
Evaluation of possible side effects at intervals of 24 hours, 1 month, 3 months, 6 and 12 months after injection

Method of measurement
side effects questionnaire

Secondary outcomes

1

Description
Changes in ALSFRS-R score

Timepoint
1, 3, 6 and 12 months after transplantation

Method of measurement
ALSFRS-R questionnaire

2

Description
Changes in FVC percent (spirometery)

Timepoint
6 and 12 months after transplantation

Method of measurement
Spirometry

3

Description
One-year survival

Timepoint
one year
Method of measurement
visiting the patients

Intervention groups

1

Description
Intervention groups: Intravenous or intrathecal injection of 60 millions of Wharton's jelly derived mesenchymal stromal cells in two doses/ 3 months- WJMSCs are suspended in 10 ml N/S for intravenous and 5 ml for intrathecal transplantation. Celltech pharmed Co. provided the WJMSCs for this study.
Category
Treatment - Drugs

Recruitment centers

1

Recruitment center
Name of recruitment center
Royan Research Institute Neurology Clinic
Full name of responsible person
Seyed Massood Nabavi
Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran
City
Tehran
Province
Tehran
Postal code
19395-4644
Phone
+98 912 130 9304
Email
seyedmassoodnabavi@gmail.com

Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Celltech Pharmed Co.
Full name of responsible person
Mohammad Hossein Ziraksaz
Street address
CellTech Pharmed Company, Toalidaroo Factory, Yaftabad, Moallem Blvd., Shahid Hamidreza Yadegar St.
City
Tehran
Province
Tehran
Postal code
1371616312
Phone
+98 21 6735 7000
Email
Ziraksaz@celltech.com
Grant name
Grant code / Reference number
Is the source of funding the same sponsor organization/entity?
Yes
Title of funding source
Celltech Pharmed Co.
Proportion provided by this source
90
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

2

Sponsor
Name of organization / entity
Royan Stem Cell Technology Co.
Full name of responsible person
Morteza Zarrabi
Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran
City
Tehran
Province
Tehran
Postal code
19395-4644
Phone
+98 21 2763 5303
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
10
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
Industry

Person responsible for general inquiries

Contact

Name of organization / entity
Royan Research Institute

Full name of responsible person
Hoda Madani

Position
Researcher

Latest degree
Medical doctor

Other areas of specialty/work
Others

Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran

City
Tehran

Province
Tehran

Postal code
19395-4644

Phone
+98 21 2356 2000

Email
hoda62_m@yahoo.com

Person responsible for scientific inquiries

Contact

Name of organization / entity
Royan Research Institute

Full name of responsible person
Seyed Massood Nabavi

Position
Researcher

Latest degree
Specialist

Other areas of specialty/work
Neurology

Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran

City
Tehran

Province
Tehran

Postal code
19395-4644

Phone
+98 21 2356 2000

Email
seyedmassoodnabavi@gmail.com

Person responsible for updating data

Contact

Name of organization / entity
Royan Research Institute

Full name of responsible person
Shahedeh Karimi

Position
Researcher

Latest degree
Medical doctor

Other areas of specialty/work
Others

Street address
Royan Institute, Banihashem Sq., Banihashem St., Ghasem Soleimani highway, Tehran, Iran

City
Tehran

Province
Tehran

Postal code
19395-4644

Phone
+98 21 2356 2000

Email
karimi58shah@gmail.com

Sharing plan

Deidentified Individual Participant Data Set (IPD)
Undecided - It is not yet known if there will be 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
Not applicable

Analytic Code
Not applicable

Data Dictionary
Not applicable