Clinical Trial Protocol
Iranian Registry of Clinical Trials

22 Nov 2026

Evaluation of the safety and efficacy of treatment for Ataxia-telangiectasia using stem cell transplantation in children at Mashhad medical university

Protocol summary

Study aim

To evaluate the efficacy of stem cell therapy in improving the Scale for Assessment and Rating of Ataxia(SARA) score in patients with Ataxia-Telangiectasia at 12 month follow up

Design

this study is a single center non randomized, phase1/2 pilot clinical trial to evaluate the safety and efficacy of allogenic mesenchymal stem cell transplantation in 5 patients with Ataxia telangiectasia

Settings and conduct

Study location: Pastor Hospital Method: IV and Intrathecal stem cell infusion in day care unit 24 hours monitoring and periodic clinic assessment

Participants/Inclusion and exclusion criteria

Inclusion criteria; Patient with a confirmed diagnosis of Ataxia-Telangiectasia based on clinical criteria and verified by genetic testing Patients aged between 5 and 15 years old A score of [e.g.,5 to 17] on the scale for the Assessment and Rating of Ataxia(SARA) Providing written informed consent by parents or legal guardians Patients who are medically stable at the time of enrollment Exclusion criteria; Presence of other severe progressive neurological disorder History of or active malignancy Active uncontrolled infections History of severe allergy or anaphylaxis to any component used in the cell preparation or infusion process Unwillingness of the family to comply with study protocol or follow up schedule

Intervention groups

*Treatment with intravenous and intrathecal infusion of allogenic mesenchymal stem cell *Human bone marrow derived mesenchymal stem cell *Intravenous and intrathecal infusion at a dose of 1 million cells per kilogram of body weight *Three infusion, administered one month apart *premedication with antihistamine 30 minutes prior to infusion

Main outcome variables

*Efficacy: change in scale for the assessment and rating of Ataxia(SARA) score from baseline to 12 months post treatment *Safety: incidence of treatment related serious adverse events within 12 months

General information

Reason for update

Acronym

CellTX-AT

IRCT registration information

IRCT registration number: **IRCT20250528065956N1**
Registration date: **2025-09-19, 1404/06/28**
Registration timing: **prospective**

Last update: **2025-09-19, 1404/06/28**
Update count: **0**

Registration date

2025-09-19, 1404/06/28

Registrant information

Name

Amirali Latifian Alaf

Name of organization / entity

Country

Iran (Islamic Republic of)

Phone

+98 51 3841 5352

Email address

latifianaa4021@mums.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date

2025-10-17, 1404/07/25

Expected recruitment end date

2025-12-16, 1404/09/25

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

Evaluation of the safety and efficacy of treatment for Ataxia-telangiectasia using stem cell transplantation in children at Mashhad medical university

Public title

Safety and Efficacy of Stem cell Transplantation in Patients with Ataxia-telangiectasia

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion criteria:

The age of onset is between 5 and 15 years old Informed consent from parents or legal guardians The definitive diagnosis of the disease is based on clinical and genetic criteria

Exclusion criteria:

Presence of other neurodevelopmental disorder Dissatisfaction with study participation History of epilepsy or seizure disorder Adverse reaction to stem cell injection

Age

From **5 years** old to **15 years** 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

Ethics committee of Mashhad University of Medical Science

Street address

no44,16th Qaem, Ahmadabad St.

City

Mashhad

Province

Razavi Khorasan

Postal code

9183889341

Approval date

2025-05-07, 1404/02/17

Ethics committee reference number

IR.MUMS.REC.1404.053

Health conditions studied

1

Description of health condition studied

Ataxia telangiectasia

ICD-10 code

G11.3

ICD-10 code description

Cerebellar ataxia with defective DNA repair

Primary outcomes

1

Description

Primary outcome1: Efficacy : change in SARA score from baseline to 12 months post treatment - Primary outcome2: Safety: Number of patients with treatment related serious adverse events within 12 months

Timepoint

Timepoint for outcome measurement: Baseline(before treatment)-3 months post treatment- 6 months post treatment- 12 months post treatment

Method of measurement

For SARA score: Clinical assessment by neurologist using standardize SARA scale - For adverse events: Monitoring and recording based on CTCAE v5.0 criteria

Secondary outcomes

1

Description

Improved immune function - Improved life function - Improved daily function - Progression-free survival

Timepoint

Baseline(pre treatment)-3months post treatment- 6 months post treatment- 12 months post treatment

Method of measurement

Immunoglobulin levels: ELISA test from venous blood sample - Quality of life: Standard PedsQL questionnaire completed by parents - Daily functioning: Clinical observation and interview by occupational therapist - Disease progression: Clinical assessment by neurologist

Intervention groups

1

Description
Intervention Group: Patients receiving intravenous and intrathecal infusion of allogenic mesenchymal stem cell(3 doses at 1 month intervals) with24hours monitoring post infusion
Category
Treatment - Drugs

Recruitment centers

1

Recruitment center
Name of recruitment center
Qaem Hospital
Full name of responsible person
Mehran Beiraghi
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No.44,16th Qaem, Ahmadabad St.
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Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Mashhad University of Medical Sciences
Full name of responsible person
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Grant name
Grant code / Reference number
Is the source of funding the same sponsor organization/entity?
Yes
Title of funding source
Mashhad University of Medical Sciences
Proportion provided by this source
100
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

Person responsible for general inquiries

Contact
Name of organization / entity
Mashhad University of Medical Sciences
Full name of responsible person
Amirali Latifian alaf
Position
Resident
Latest degree
Specialist
Other areas of specialty/work
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Sharing plan

Deidentified Individual Participant Data Set (IPD)
Yes - There is a plan to make this available
Study Protocol
No - There is not a plan to make this available
Statistical Analysis Plan
Not applicable
Informed Consent Form
Undecided - It is not yet known if there will be a plan to make this available
Clinical Study Report
Yes - There is a plan to make this available
Analytic Code
Not applicable
Data Dictionary
Not applicable
Title and more details about the data/document
Other than identity and contact information
When the data will become available and for how long
Access period starts 6 months after results are published.
To whom data/document is available
Researchers working in academic and scientific institutions
Under which criteria data/document could be used
It is free with attribution.
From where data/document is obtainable
Dr. amirali latifian
What processes are involved for a request to access data/document
The email must be sent to latifianaa4021@mums.ac.ir and if approved, information will be provided within 2 months.
Comments