

Clinical Trial Protocol

Iranian Registry of Clinical Trials

27 Jul 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

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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) with 24 hours monitoring post infusion

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Qaem Hospital

Full name of responsible person

Mehran Beiraghi

Street address

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

Mohsen Tafaghodi

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Ghaem hospital, Ahmad abad Ave

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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

Pediatrics

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Person responsible for updating data**Contact****Name of organization / entity**

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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