

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

10 Jun 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) 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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#### Province

Razavi Khorasan

#### Postal code

9183889341

#### Phone

+98 915 311 6987

#### Email

aa2.latifian@gmail.com

## Sponsors / Funding sources

1

### Sponsor

#### Name of organization / entity

Mashhad University of Medical Sciences

#### Full name of responsible person

Mohsen Tafaghodi

#### Street address

Ghaem hospital, Ahmad abad Ave

#### City

mashhad

#### Province

Razavi Khorasan

#### Postal code

99199-91766

#### Phone

+98 51 3801 2469

#### Email

latifianaa4021@mums.ac.ir

#### 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

#### Street address

Ghaem hospital, Ahmadabad Ave

#### City

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#### Postal code

99199-91766

#### Phone

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#### Email

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## Person responsible for scientific inquiries

### Contact

#### Name of organization / entity

Mashhad University of Medical Sciences

#### Full name of responsible person

Amirali Latifian Alaf

#### Position

Resident

#### Latest degree

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#### Other areas of specialty/work

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**Person responsible for updating data****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

**Street address**

ahmad abad

**City**

mashhad

**Province**

Razavi Khorasan

**Postal code**

99199-91766

**Phone**

+98 51 3801 2469

**Email**

latifianaa4021@mums.ac.ir

**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**