Clinical Trial Protocol
Iranian Registry of Clinical Trials

07 Nov 2026

Evaluation of the safety and effectiveness of Haploidentical hematopoietic stem cell transplantation for the treatment of severe aplastic anemia patients who lack an Human leukocyte antigen (HLA)-matched (sibling or unrelated) donor.

Protocol summary

Study aim

The purpose of this study is to test with optimal approaches as post transplant cyclophosphamide in combination with Antithymocyte Globulin (ATG) whether we will achieve acceptable results in haploidentical transplantation in patients with Severe Aplastic Anemia.

Design

Phase 1-2 prospective single-arm interventional pilot study with 10 patients.

Settings and conduct

Severe Aplastic Anemia patients candidates for allogeneic transplantation are admitted at the hematopoietic stem cell transplant department of Shariati Hospital and will receive the conditioning regimen and will be transplanted.

Participants/Inclusion and exclusion criteria

Inclusion criteria: Patients from 18 to 60 years old, with Severe Aplastic Anemia and unresponsive to prior immune suppressive treatments, no full matched (sibling or unrelated) donor available. Exclusion criteria: Prior allogeneic stem cell transplantation Symptomatic coronary artery disease, Karnofsky Performance Score < 60, patients with uncontrolled bacterial, viral or fungal infection, a known life-threatening reaction (anaphylaxis) to Thymoglobin, females who are pregnant or breastfeeding, patients seropositive for human immunodeficiency virus or active hepatitis B or C with detectable viral load, received Antithymocyte globulin drug within 2 weeks before enrolling in the study, previous malignancies.

Intervention groups

Intervention group: Thymoglobulin (Genzyme) 0.5 mg/kg/D, Day -9 and Thymoglobulin 2.5 mg/kg/D, Day -8 and -7, Cyclophosphamide (Baxter) 14.5 mg/kg, Day -6 and -5, Fludarabin (ACTOVERCO) 30 mg/m2, Day -6 to -2, Busulfan (Nanoalvand) 3.2 mg/kg/D, Day -3 and -2. Cyclophosphamide (Baxter) 50 mg/kg, Day +3 and Day +4 after transplantation.

Main outcome variables

- One year overall survival - Incidence of acute and chronic graft versus host disease - Transplant related mortality

General information

Reason for update

Acronym

-

IRCT registration information

IRCT registration number: **IRCT20140818018842N29**
Registration date: **2023-01-31, 1401/11/11**
Registration timing: **prospective**

Last update: **2024-04-12, 1403/01/24**
Update count: **1**

Registration date

2023-01-31, 1401/11/11

Registrant information

Name

Leyla Sharifi Aliabadi

Name of organization / entity

Research Institute for Hematology, Oncology and Stem Cell Transplantation,Tehran University of Medic

Country

Iran (Islamic Republic of)

Phone

+98 21 8490 3691

Email address

ctu@sina.tums.ac.ir

Recruitment status

Recruitment complete

Funding source

Expected recruitment start date
2023-02-20, 1401/12/01
Expected recruitment end date
2024-03-20, 1403/01/01
Actual recruitment start date
empty
Actual recruitment end date
empty
Trial completion date
empty

Scientific title
Evaluation of the safety and effectiveness of Haploidentical hematopoietic stem cell transplantation for the treatment of severe aplastic anemia patients who lack an Human leukocyte antigen (HLA)-matched (sibling or unrelated) donor.

Public title
Haploidentical hematopoietic stem cell transplantation for the treatment of severe aplastic anemia patients.
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Patients from 18 to 60 years old Patients with Severe Aplastic Anemia Patients unresponsive to prior immune suppressive treatments. No full matched (sibling or unrelated) donor available.
Exclusion criteria:
Prior allogeneic stem cell transplantation Symptomatic coronary artery disease Karnofsky Performance Score < 60 Patients with uncontrolled bacterial, viral or fungal infection A known life-threatening reaction (anaphylaxis) to Thymoglobin. Females who are pregnant or breastfeeding. Patients seropositive for human immunodeficiency virus or active hepatitis B or C with detectable viral load. Received Antithymocyte globulin drug within 2 weeks before enrolling in the study. Previous malignancies other than resected basal cell carcinoma or cervical cancer treated in situ.
Age
From **18 years** old to **60 years** old
Gender
Both

Phase
2
Groups that have been masked
No information
Sample size
Target sample size: **10**
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
Ethic committee of Hematology- Oncology and cell therapy Research Institute, Tehran University of Me
Street address
Shariati Hospital, Jalal-e-Al-e-Ahmad Hwy
City
Tehran
Province
Tehran
Postal code
1411713135
Approval date
2023-01-25, 1401/11/05
Ethics committee reference number
IR.TUMS.HORCSCT.REC.1401.025

Health conditions studied

1

Description of health condition studied
Aplastic Anemia
ICD-10 code
D61.9
ICD-10 code description
Aplastic anemia, unspecified

Primary outcomes

1

Description
overall survival
Timepoint
Monthly for 12 months after transplantation
Method of measurement
Visiting the patient and performing monthly lab tests in outpatient clinique

Secondary outcomes

1

Description
Incidence of acute graft versus host disease
Timepoint
Monthly for 4 months after transplantation
Method of measurement
Visiting the patient in person and performing monthly lab tests in outpatient clinique

2

Description
Incidence of chronic graft versus host disease
Timepoint
Monthly for 12 months after transplantation
Method of measurement
Visiting the patient in person and performing monthly lab tests in outpatient clinique

3

Description
Incidence of infectious complications post-transplant
Timepoint
Monthly for 12 months after transplantation
Method of measurement
Visiting the patient in person and performing monthly lab tests in outpatient clinique

4

Description
Transplant Related Mortality
Timepoint
Monthly for 12 months after transplantation
Method of measurement
Visiting the patient in person and performing monthly lab tests in outpatient clinique

Intervention groups

1

Description
Intervention group: patients with Severe Aplastic Anemia who are candidates for allogeneic Hematopoietic Cell Transplantation and are between 18-60 years old and are unresponsive to prior immune suppressive treatments and no have no full matched (sibling or unrelated) donor. They will be transplanted according to the reduced-intensity conditioning regimen. The conditioning regimen consists of: Thymoglobulin (Genzyme) 0.5 mg/kg/D, Day -9 and Thymoglobulin 2.5 mg/kg/D, Day -8 and -7, Cyclophosphamide (Baxter) 14.5 mg/kg, Day -6 and -5, Fludarabin (ACTOVERCO) 30 mg/m2, Day -6 to -2, Busulfan (Nanoalvand) 3.2 mg/kg/D, Day -3 and -2. Cyclophosphamide (Baxter) 50 mg/kg, Day +3 and Day +4 after transplantation.
Category
Treatment - Other

Recruitment centers

1

Recruitment center
Name of recruitment center
Research Institute for Oncology, Hematology and Cell Therapy, Tehran University of Medical Sciences
Full name of responsible person
Tanaz Sayar Bahri
Street address
Shariati Hospital, Jalal-e-Al-e-Ahmad Hwy
City
Tehran
Province
Tehran
Postal code
14117 13135
Phone
+98 21 8800 4140
Email
tanaz.bahri@gmail.com

Sponsors / Funding sources

1

Sponsor
Name of organization / entity
Tehran University of Medical Sciences
Full name of responsible person
Dr. Akbar Fotouhi
Street address
Poursina St., 16 Azar St., Keshavarz Blvd.
City
Tehran
Province
Tehran
Postal code
1416634793
Phone
+98 21 81631
Email
afotouhi@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
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
Tehran University of Medical Sciences
Full name of responsible person
Leyla Sharifi Aliabadi
Position
Research Assistant
Latest degree

Master

Other areas of specialty/work

Epidemiology

Street address

Shariati Hospital, Jalal-e-Al-e-Ahmad Hwy

City

Tehran

Province

Tehran

Postal code

1417713135

Phone

00982288004140

Email

ctu@sina.tums.ac.ir

Person responsible for scientific inquiries

Contact

Name of organization / entity

Tehran University of Medical Sciences

Full name of responsible person

Tanaz Sayar Bahri

Position

Assistant professor

Latest degree

Subspecialist

Other areas of specialty/work

Hematology

Street address

Shariati Hospital, North Kargar Ave.

City

Tehran

Province

Tehran

Postal code

1411713135

Phone

-

Email

tanaz.bahri@gmail.com

Person responsible for updating data

Contact

Name of organization / entity

Tehran University of Medical Sciences

Full name of responsible person

Tanaz Sayar Bahri

Position

Assistant Professor

Latest degree

Subspecialist

Other areas of specialty/work

Hematology

Street address

Shariati Hospital, Jalal-e-Al-e-Ahmad Hwy

City

Tehran

Province

Tehran

Postal code

1411713135

Phone

+98 21 8490 2635

Email

tanaz.bahri@gmail.com

Sharing plan

Deidentified Individual Participant Data Set (IPD)

No - There is not a plan to make this available

Justification/reason for indecision/not sharing IPD

There is no further information

Study Protocol

No - There is not a plan to make this available

Statistical Analysis Plan

No - There is not a plan to make this available

Informed Consent Form

No - There is not a plan to make this available

Clinical Study Report

No - There is not a plan to make this available

Analytic Code

No - There is not a plan to make this available

Data Dictionary

No - There is not a plan to make this available