

Clinical Trial Protocol

Iranian Registry of Clinical Trials

18 Sep 2026

Feasibility, safety, effectiveness and toxicity of Post-transplant Expanded, Activated Haploidentical Natural Killer Cells Infusion in Haploidentical Hematopoietic Stem Cell Transplantation for Pediatric Patients with Acute Myeloid Leukemia: A Pilot Study

Protocol summary

Study aim

Infusion of expanded NK cells from healthy haploidentical donors to enhance anti-leukemia effect in pediatric patients with acute myeloid leukemia.

Design

A Pilot single arm clinical trial of 5 Patients

Settings and conduct

1. Haploidentical donors will donate 30 cc/kg (recipient body weight) of blood collected on day -30 for NK-cell production and will undergo peripheral blood apheresis on day 0 of transplant. 2. NK cells will be expanded and activated. 3. The purity of the product would be checked by flowcytometry using NK cell markers. 4. Assessment of activation of NK-cell product would be conducted via evaluation of cytokine secretion including IFN- γ . 5. Assessment of the product viability would be conducted via trypan blue dye exclusion. 6. Assessment of the functionality of the product would be done via cytotoxicity assay. 7. NK-cell product will be infused from cryopreserved aliquots on days +5 post transplant.

Participants/Inclusion and exclusion criteria

1. Age $\leq$ 18 years old. 2. Diagnosis of AML other than acute promyelocytic leukemia (APL) / candidate for first allogeneic haploidentical HSCT. 3. Adequate organ function. 4. Karnofsky or Lansky performance score of greater than or equal to 50. 5. At least two weeks since receipt of any systemic chemotherapy and/or radiation therapy.

Intervention groups

In this single arm study, Haploidentical activated expanded NK cell will be infused after Haploidentical HSCT in pediatric patients with AML.

Main outcome variables

1. To evaluate 1 year overall survival after infusion of expanded haploidentical NK cells. 2. To evaluate 1 year relapse-free survival after infusion of expanded

haploidentical NK cells. 3. To evaluate 1 year relapse rate after infusion of expanded haploidentical NK cells.

General information

Reason for update

Acronym

IRCT registration information

IRCT registration number: **IRCT20140818018842N33**

Registration date: **2023-04-03, 1402/01/14**

Registration timing: **registered_while_recruiting**

Last update: **2023-04-03, 1402/01/14**

Update count: **0**

Registration date

2023-04-03, 1402/01/14

Registrant information

Name

Leyla Sharifi Aliabadi

Name of organization / entity

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

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

2023-03-21, 1402/01/01

Expected recruitment end date

2025-03-21, 1404/01/01 Actual recruitment start date empty Actual recruitment end date empty Trial completion date empty Scientific title Feasibility, safety, effectiveness and toxicity of Post-transplant Expanded, Activated Haploidentical Natural Killer Cells Infusion in Haploidentical Hematopoietic Stem Cell Transplantation for Pediatric Patients with Acute Myeloid Leukemia: A Pilot Study	Randomization (investigator's opinion) Not randomized Randomization description Blinding (investigator's opinion) Not blinded Blinding description Placebo Not used Assignment Single Other design features Secondary Ids empty
Public title Feasibility, safety, effectiveness and toxicity of Post-transplant Expanded, Activated Haploidentical Natural Killer Cells Infusion in Haploidentical Hematopoietic Stem Cell Transplantation for Pediatric Patients with Acute Myeloid Leukemia: A Pilot Study Purpose Treatment Inclusion/Exclusion criteria Inclusion criteria: Age $\leq$ 18 years old. Patients must have a diagnosis of AML other than acute promyelocytic leukemia (APL) according to WHO criteria and should be a candidate for their first allogeneic haploidentical HSCT. All patients must have adequate organ function defined as:a. Renal Function: Age-adjusted serum creatinine $\leq$ to 1.5 x normal for age/gender OR creatinine clearance or GFR greater than or equal to 60 cc/min/1.73m2b. Liver Function: Total bilirubin $\leq$ 1.5 x normal for age, AND Alanine aminotransferase (ALT) is no more than 2 times the upper limit of normal and Aspartate transaminases (AST) is no more than 2 times the upper limit of normal.c. Cardiac Function: Normal ejection fraction documented by either echocardiogram or radionuclide MUGA evaluation OR Normal fractional shortening documented by echocardiogramd. Pulmonary Function: No dyspnea at rest, no oxygen requirement. Karnofsky or Lansky performance score of greater than or equal to 50. Potentially available haploidentical family donor (child/sibling), willing and fit for NK cell donation At least two weeks since receipt of any biological therapy, systemic chemotherapy, and/or radiation therapy. Exclusion criteria: Pregnancy: Quantitative Serum B-HCG must be negative in girls who are post-menarchal Breast feeding women are not eligible. Active or uncontrolled infection Patient declines participation Second HSC	Ethics committees <u>1</u> Ethics committee Name of ethics committee Tehran University of Medical Sciences Street address North kargar st City Tehran Province Tehran Postal code 146767 Approval date 2022-10-10, 1401/07/18 Ethics committee reference number IR.TUMS.MEDICINE.REC.1401.540 Health conditions studied <u>1</u> Description of health condition studied Acute Myeloid Leukemia ICD-10 code C92.0 ICD-10 code description Acute myeloid leukemia
Age To 18 years old Gender Both Phase 1-2 Groups that have been masked No information Sample size Target sample size: 5	Primary outcomes <u>1</u> Description Relapse free survival Timepoint 1 year Method of measurement spss Secondary outcomes empty

Intervention groups

1

Description

Intervention group: Pediatric Patients with Acute Myeloid Leukemia who are candidate to receive Haploidentical Hematopoietic Stem Cell Transplantation 1. Haploidentical donors will donate 30 cc/kg (recipient body weight) of blood collected on day -30 for NK-cell production and will undergo peripheral blood apheresis on day 0 of transplant. 2. NK cells will be expanded and activated. 3. The purity of the product would be checked by flow cytometry using NK cell markers. 4. Assessment of activation of NK-cell product would be conducted via evaluation of cytokine secretion including IFN- γ . 5. Assessment of the product viability would be conducted via trypan blue dye exclusion. 6. Assessment of the functionality of the product would be done via cytotoxicity assay. 7. NK-cell product will be infused from cryopreserved aliquots on days +5 post transplant.

Category

Treatment - Other

Recruitment centers

1

Recruitment center

Name of recruitment center

Research Institute for Oncology, Hematology and Cell Therapy Hematology

Full name of responsible person

Tahereh Rostami

Street address

North kargar st

City

Tehran

Province

Tehran

Postal code

146767

Phone

+98 912 389 0394

Email

trostami@sina.tums.ac.ir

Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Tehran University of Medical Sciences

Full name of responsible person

Marjan Yaghmaie

Street address

North kargar st

City

Tehran

Province

Tehran

Postal code

146767

Phone

+98 912 389 0394

Email

m-yaghmaie@sina.tums.ac.ir

Web page address

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

Azadeh Kiumarsi

Position

Assistant professor

Latest degree

Subspecialist

Other areas of specialty/work

Hematology

Street address

North kargar st

City

Tehran

Province

Tehran

Postal code

146767

Phone

+98 912 389 0394

Email

akiumarsi@sina.tums.ac.ir

Person responsible for scientific inquiries

Contact

Name of organization / entity

Tehran University of Medical Sciences

Full name of responsible person

Tahereh Rostami

Position

Assistant professor

Latest degree
Subspecialist
Other areas of specialty/work
Hematology
Street address
North kargar st
City
Tehran
Province
Tehran
Postal code
146767
Phone
+98 912 389 0394
Email
trostami@sina.tums.ac.ir

Person responsible for updating data

Contact

Name of organization / entity
Tehran University of Medical Sciences
Full name of responsible person
Azadeh Kiumarsi
Position
Assistant professor
Latest degree
Subspecialist
Other areas of specialty/work
Hematology
Street address
North kargar st
City

Tehran
Province
Tehran
Postal code
146767
Phone
+98 912 103 7104
Fax
+98 21 8800 4140
Email
akiumarsi@sina.tums.ac.ir

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

Undecided - It is not yet known if there will be a plan to make this available

Analytic Code

No - There is not a plan to make this available

Data Dictionary

Undecided - It is not yet known if there will be a plan to make this available