

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

09 Jun 2026

### Assessment of efficacy and safety of Romiplostim in immunosuppressive-naïve children with severe acquired aplastic anemia: a phase II/III open-label study

#### Protocol summary

##### Study aim

Efficacy and safety of Romiplostim in complete or partial remission in children with IST-naïve severe acquired aplastic anemia

##### Design

Open-label, single-arm, phase 2-3 study with posttreatment outcome assessment

##### Settings and conduct

Patients with a confirmed diagnosis of acquired aplastic anemia who are admitted to Amir Oncology Hospital, and are candidates for immune suppressive therapy are treated with Romiplostim. This is a single-arm study and has no blinding.

##### Participants/Inclusion and exclusion criteria

Children 1 month up to the age of 18 years with severe and very severe acquired aplastic anemia who are eligible to be treated with immune suppressive therapy (antithymocyte globuline (ATG) plus cyclosporine) are enrolled. The following patients are excluded: Patients with inherited aplastic anemia, Previously treated with ATG, cyclosporine, Concurrent active infection, Cancer in the past 5 years, paroxysmal nocturnal hemoglobinuria, chromosome aberrations in bone marrow cells, Bone marrow fibrosis, Active or latent human immunodeficiency virus, hepatitis B, hepatitis C, tuberculosis or visceral leishmaniasis infection, Active cytomegalovirus infection, Planned hematopoietic stem cell transplantation during the study

##### Intervention groups

This is a single-group study. Romiplostim is administered to all eligible patients entering the study.

##### Main outcome variables

Primary outcome measure: • Achievement of complete hematologic response (CHR) or partial hematologic response (PHR) at week 27 post-dose

#### General information

##### Reason for update

##### Acronym

##### IRCT registration information

IRCT registration number: **IRCT20221129056655N2**

Registration date: **2023-09-23, 1402/07/01**

Registration timing: **registered\_while\_recruiting**

Last update: **2023-09-23, 1402/07/01**

Update count: **0**

##### Registration date

2023-09-23, 1402/07/01

##### Registrant information

##### Name

Mohammadreza Bordbar

##### Name of organization / entity

##### Country

Iran (Islamic Republic of)

##### Phone

+98 71 3632 3067

##### Email address

mbordbar53@gmail.com

##### Recruitment status

**Recruitment complete**

##### Funding source

##### Expected recruitment start date

2023-06-22, 1402/04/01

##### Expected recruitment end date

2025-03-19, 1403/12/29

##### Actual recruitment start date

empty

##### Actual recruitment end date

empty

##### Trial completion date

empty

## Scientific title

Assessment of efficacy and safety of Romiplostim in immunosuppressive-naïve children with severe acquired aplastic anemia: a phase II/III open-label study

## Public title

Romiplostim in treatment-naïve acquired aplastic anemia

## Purpose

Treatment

## Inclusion/Exclusion criteria

### Inclusion criteria:

Children 1 month up to the age of 18 years with severe and very severe acquired aplastic anemia who are eligible to be treated with IST (ATG plus cyclosporine)

### Exclusion criteria:

1. Patients with inherited aplastic anemia 2. Previously treated with ATG, cyclosporine 3. Diagnosed as having acute myeloid leukemia (AM)L or myelodysplastic syndrome (MDS) 4. Concurrent active infection not adequately responding to appropriate therapy 5. Having active malignancies, or having a history of the treatment of malignancies within 5 years prior to informed consent 6. Concurrent paroxysmal nocturnal hemoglobinuria 7. History of chromosome aberrations discovered in bone marrow cells 8. Bone marrow fibrosis based on reticulin stain 9. Active or latent HIV infection 10. Active or latent HBV infection 11. Active or latent HCV infection 12. Active or latent tuberculous infection 13. Active or latent visceral leishmaniasis 14. Planned hematopoietic stem cell transplantation during the study

## Age

From **1 month** old to **18 years** old

## Gender

Both

## Phase

2-3

## Groups that have been masked

*No information*

## Sample size

Target sample size: **23**

## 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 Shiraz University of Medical Sciences

#### Street address

7th floor- Central building of Shiraz University of Medical Sciences- Zand Blvd- Shiraz

#### City

Shiraz

#### Province

Fars

#### Postal code

7134814336

#### Approval date

2023-07-05, 1402/04/14

#### Ethics committee reference number

IR.SUMS.MED.REC.1402.140

## Health conditions studied

### 1

#### Description of health condition studied

Acquired aplastic anemia

#### ICD-10 code

D61.3

#### ICD-10 code description

Idiopathic aplastic anemia

## Primary outcomes

### 1

#### Description

complete or partial hematologic response at the end of study

#### Timepoint

At week 27 of the study

#### Method of measurement

Check CBC

## Secondary outcomes

### 1

#### Description

The time to achieve complete or partial hematologic response

#### Timepoint

weekly

#### Method of measurement

CBC

### 2

#### Description

The need to platelet or blood transfusion

#### Timepoint

Week 27

#### Method of measurement

The proportion of patients with transfusion independence or reduction in transfusion needs among patients who had received a transfusion within 8 weeks prior to the first romiplostim administration

## Intervention groups

1

### Description

Intervention group: Romiplostim (N-Plate, Amgen company) is administered subcutaneously at a fixed dose of 10 mcg/kg weekly for 4 weeks (weeks 1-4). The drug is administered on day 1 of IST (horse ATG 40 mg/kg/day on days 1-4 plus cyclosporine 10 mg/kg/day PO divided into two equal daily doses). The dosage is titrated to steps of 10, 15, and 20 mcg/kg once weekly for up to 27 weeks (weeks 5-27). The romiplostim dose is adjusted depending on platelet response and toxicity. The dose is increased by one step every 4 weeks until a platelet response is achieved. If the platelet count is  $>200 \geq 109/L$ , the dose is reduced by one step. The romiplostim dose is tapered with the intent to discontinue when trilineage hematopoiesis is achieved. Trilineage hematopoiesis is defined as a platelet count of  $>50 \times 109/L$ , hemoglobin concentration of  $>10 \text{ g/dL}$  and neutrophil count of  $>1 \times 109/L$  maintained for 8 weeks with the same romiplostim dose without transfusion.

### Category

Treatment - Drugs

## Recruitment centers

1

### Recruitment center

#### Name of recruitment center

Amir Oncology Hospital

#### Full name of responsible person

Mohammadreza Bordbar

#### Street address

Amir Oncology Hospital, Farhangshahr street

#### City

Shiraz

#### Province

Fars

#### Postal code

7187915998

#### Phone

+98 71 3632 3532

#### Fax

+98 71 3632 5655

#### Email

mbordbar53@gmail.com

## Sponsors / Funding sources

1

### Sponsor

#### Name of organization / entity

Shiraz University of Medical Sciences

#### Full name of responsible person

Mohammad Hashem Hashempur

#### Street address

7th floor, Central Building, Shiraz University of Medical Sciences, Zand Blvd

### City

Shiraz

### Province

Fars

### Postal code

7134814336

### Phone

+98 71 3235 7282

### Fax

+98 71 3212 2430

### Email

hashempurm@sums.ac.ir

### Grant name

### Grant code / Reference number

### Is the source of funding the same sponsor organization/entity?

Yes

### Title of funding source

Shiraz 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

Shiraz University of Medical Sciences

#### Full name of responsible person

Mohammadreza Bordbar

#### Position

professor

#### Latest degree

Subspecialist

#### Other areas of specialty/work

Hematology

#### Street address

Amir Oncology Hospital- Farhangshahr Street

#### City

Shiraz

#### Province

Fars

#### Postal code

71879-15998

#### Phone

+98 71 3632 3532

#### Email

mbordbar53@gmail.com

## Person responsible for scientific inquiries

### Contact

#### Name of organization / entity

Shiraz University of Medical Sciences  
**Full name of responsible person**  
Mohammadreza Bordbar  
**Position**  
Professor  
**Latest degree**  
Subspecialist  
**Other areas of specialty/work**  
Hematology  
**Street address**  
Amir Oncology Hospital- Farhangshahr Street  
**City**  
Shiraz  
**Province**  
Fars  
**Postal code**  
71879-15998  
**Phone**  
+98 71 3632 3532  
**Email**  
mbordbar53@gmail.com

## Person responsible for updating data

### Contact

**Name of organization / entity**  
Shiraz University of Medical Sciences  
**Full name of responsible person**  
Mohammadreza Bordbar  
**Position**  
Professor  
**Latest degree**  
Subspecialist  
**Other areas of specialty/work**  
Hematology  
**Street address**  
Amir Oncology Hospital- Farhangshahr Street  
**City**  
Shiraz  
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**Postal code**  
71879-15998

**Phone**  
+98 71 3632 3532  
**Email**  
mbordbar53@gmail.com

## Sharing plan

### Deidentified Individual Participant Data Set (IPD)

Yes - There is a plan to make this available

### Study Protocol

Yes - There is a plan to make this available

### Statistical Analysis Plan

Yes - There is a plan to make this available

### Informed Consent Form

Yes - There is a plan to make this available

### Clinical Study Report

Yes - There is a plan to make this available

### Analytic Code

Yes - There is a plan to make this available

### Data Dictionary

Yes - There is a plan to make this available

### Title and more details about the data/document

Patients' data will be shared anonymously.

### When the data will become available and for how long

The data will be accessible after the results are published.

### To whom data/document is available

Academic investigators will have access to the data upon their request.

### Under which criteria data/document could be used

Researchers can use data for research purposes with the permission of the principal investigator.

### From where data/document is obtainable

Text to the PI (Mohammadreza Bordbar) to the following phone number: 09177072149 or send an email to the following address: mbordbar53@gmail.com

### What processes are involved for a request to access data/document

The request will be sent to the Ethics Committee of the University. If there is no ethical concern, they will be sent to them. Usually it takes 30 working days.

### Comments