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-naive 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 ≥ 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 × 109/L, hemoglobin concentration of >10 g/dL and neutrophil count of >1×109/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

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Hematology

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+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

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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