Clinical Trial Protocol
Iranian Registry of Clinical Trials

17 Sep 2026

Assessment of efficacy and safety of Romiplostim in immunosuppressive-refractory children with severe acquired aplastic anemia

Protocol summary

Study aim
Efficacy and safety of Romiplostim in CR or PR in children with IST-refractory severe acquired aplastic anemia

Design
Single-arm, open-label, phase 2-3 study with posttreatment outcome assessment

Settings and conduct
Romiplostim is administered subcutaneously at a fixed dose of 10 mcg/kg weekly for 4 weeks (weeks 1-4) followed by titrated dose steps of 10, 15, and 20 mcg/kg once weekly 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.

Participants/Inclusion and exclusion criteria
Children 1 month up to the age of 18 years with non-severe transfusion-dependent AA, severe and very severe acquired aplastic anemia who are ineligible to be treated with IST (ATG plus cyclosporine) or are refractory to IST are enrolled. The following patients are excluded: Patients with inherited aplastic anemia, acute myeloid leukemia or myelodysplastic syndrome, malignancies within 5 years prior to informed consent. paroxysmal nocturnal hemoglobinuria, chromosome aberrations discovered in bone marrow cells. bone marrow fibrosis based on reticulin stain, hematopoietic stem cell transplantation during the study

Intervention groups
This is a single-arm study. Romiplostim is administered to all eligible patients entering the study.

Main outcome variables
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: **IRCT20221129056655N1**
Registration date: **2023-06-01, 1402/03/11**
Registration timing: **prospective**

Last update: **2023-06-01, 1402/03/11**
Update count: **0**

Registration date
2023-06-01, 1402/03/11

Registrant information

Name
Mohammadreza Bordbar

Name of organization / entity

Country
Iran (Islamic Republic of)

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+98 71 3632 3067

Email address
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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-refractory children with severe acquired aplastic anemia

Public title
Romiplostim in treatment-refractory acquired aplastic anemia
Purpose
Treatment
Inclusion/Exclusion criteria
Inclusion criteria:
Children up to the age of 18 years with non-severe transfusion-dependent AA, severe and very severe acquired aplastic anemia who are ineligible to be treated with IST (ATG plus cyclosporine) or are refractory to IST are enrolled.
Exclusion criteria:
1. Patients with inherited aplastic anemia 2. Diagnosed as having acute myeloid leukemia (AM)L or myelodysplastic syndrome (MDS) 3. Having active malignancies, or having a history of the treatment of malignancies within 5 years prior to informed consent. 4. Concurrent paroxysmal nocturnal hemoglobinuria 5. History of chromosome aberrations discovered in bone marrow cells. 6. Bone marrow fibrosis based on reticulin stain 7. 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: **24**
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
City
Shiraz
Province
Fars
Postal code
7134814336
Approval date
2023-04-29, 1402/02/09
Ethics committee reference number
IR.SUMS.MED.REC.1402.051

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
The percentage of patients who achieved complete or partial hematologic response at the end of study
Timepoint
Week 27
Method of measurement
To divide the number of patients with complete or partial hematologic response over total number of patients

Secondary outcomes

1

Description
The time to achieve complete or partial hematologic response
Timepoint
weekly
Method of measurement
CBC

2

Description
Reduction in the need to platelet or blood transfusion
Timepoint
Week 27
Method of measurement
The proportion of patients who achieved 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 is administered subcutaneously at a fixed dose of 10 mcg/kg weekly for 4 weeks (weeks 1-4) followed by titrated dose steps of 10, 15, and 20 mcg/kg once weekly 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

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Sponsors / Funding sources

1

Sponsor

Name of organization / entity
Shiraz University of Medical Sciences

Full name of responsible person
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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

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Person responsible for scientific inquiries

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Subspecialist
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Person responsible for updating data

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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
Send an e-mail to the PI to receive the data.
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.
Comments