

Clinical Trial Protocol

Iranian Registry of Clinical Trials

21 Jul 2026

A prospective single arm study to assess the efficacy and safety of deferasirox 20 mg/kg BID in transfusion dependent beta-thalassemia patients unresponsive to current treatment with doses > 35 mg/kg QD or with intolerance to single dose therapy

Protocol summary

Summary

The aim of this study was evaluation safety and efficacy of deferasirox used as divided dose in transfusion dependent beta-thalassemia patients unresponsive to current treatment or with intolerance to single dose therapy. Inclusion of study: Aged 2 years; Serum ferritin>2000 ng/ml; unresponsive to current treatment with doses>35 mg/kg QD(decrease in ferritin level less than 10%);patients who did not tolerate single dose therapy due to adverse effect. Exclusion of study: Patients will be excluded from this trial if they have one of the following criteria: positive serology for HBS Ag , HCV Ab, HIV Ab; Abnormal liver or kidney function tests;An alanine aminotranferase (ALT) level greater than 250 U/L;Also serum creatinine level above the upper limit of normal, and a urinary protein-creatinine ratio of greater than 0.5 mg/mg); a history of ocular toxicity related to iron chelation therapy. Target sample size was forty. Intervention studied was deferasirox 20 mg/kg BID in transfusion dependent beta-thalassemia patients unresponsive to current treatment with doses > 35 mg/kg QD or with intolerance to single dose therapy. Variation in serum ferritin (every three months for 12 months) was evaluated.

General information

Acronym

IRCT registration information

IRCT registration number: **IRCT2014122920051N1**
Registration date: **2014-12-29, 1393/10/08**
Registration timing: **prospective**

Last update:
Update count: **0**

Registration date

2014-12-29, 1393/10/08

Registrant information

Name

Mehran Karimi

Name of organization / entity

Hematology Research Center, Shiraz University of Medical Sciences

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Recruitment status

Recruitment complete

Funding source

Shiraz University of Medical Sciences

Expected recruitment start date

2015-02-20, 1393/12/01

Expected recruitment end date

2015-03-21, 1394/01/01

Actual recruitment start date

empty

Actual recruitment end date

empty

Trial completion date

empty

Scientific title

A prospective single arm study to assess the efficacy and safety of deferasirox 20 mg/kg BID in transfusion dependent beta-thalassemia patients unresponsive to current treatment with doses > 35 mg/kg QD or with intolerance to single dose therapy

Public title

A prospective single arm study to assess the efficacy and safety of deferiasirox in transfusion dependent beta-thalassemia patients unresponsive to current treatment or with intolerance to single dose therapy

Purpose

Treatment

Inclusion/Exclusion criteria

Inclusion: Aged 2 years; Serum ferritin >2000 ng/ml and unresponsive to current treatment with doses > 35 mg/kg QD (decrease in ferritin level less than 10%); patients who did not tolerate single dose therapy due to adverse effects
Exclusion: Patients will be excluded from this trial if they have one of the following criteria: positive serology for HBS Ag, HCV Ab, HIV Ab. Abnormal liver or kidney function tests; An alanine aminotransferase (ALT) level greater than 250 U/L; serum creatinine level above the upper limit of normal, and a urinary protein-creatinine ratio of greater than 0.5 mg/mg; a history of ocular toxicity related to iron chelation therapy; uncontrolled systemic hypertension, a prolonged corrected QT interval; systemic infection within 10 days prior to enrollment; gastrointestinal absorption problem; Patients who found complication of chelation therapy during trial as well as breast feeding and pregnant women.

Age

No age limit

Gender

Both

Phase

3

Groups that have been masked

No information

Sample size

Target sample size: 40

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**

Shiraz University of Medical Sciences

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Zand Avenue, Shiraz University of Medical Sciences, Shiraz - Iran

City

Shiraz

Postal code**Approval date**

2014-10-19, 1393/07/27

Ethics committee reference number

CT-P-9375-7645

Health conditions studied**1****Description of health condition studied**

Thalassemia

ICD-10 code

D56.1

ICD-10 code description

Beta thalassaemia

Primary outcomes**1****Description**

efficacy as reduction in serum ferritin levels as well as change in T2MRI.

Timepoint

Base line, three month, six month, nine month, end of study

Method of measurement

For better evaluation of serum ferritin changes during one year, we define an index: Mean of Relative Change of Serum Ferritin (MRC-SF) from the baseline levels that will be calculated as the percentage of difference between mean serum ferritin levels of each occasion from mean baseline serum ferritin levels.

Secondary outcomes**1****Description**

gastrointestinal symptoms and skin rash fatal, acute, irreversible renal failure

Timepoint

Baseline, three months, six months, nine months, End of study

Method of measurement

Exact follow up of the patients examination, laboratory data

2**Description**

safety

Timepoint

12 years

Method of measurement

exact follow up of the patients examination and laboratory data

Intervention groups

1

Description

Deferasirox 20 mg/kg BID ,During on year,all patients took deferasirox during the study

Category

Treatment - Drugs

Recruitment centers

1

Recruitment center

Name of recruitment center

Hematology Research Center

Full name of responsible person

Mozhgan Rezaei

Street address

Hematology Research Center, Nemazee Hospital,
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Sponsors / Funding sources

1

Sponsor

Name of organization / entity

Novartis

Full name of responsible person

Pooneh Heidari

Street address

2551, 10th Floor, KIAN Bldg, Valiasr Ave

City

Tehran

Grant name

Grant code / Reference number

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

Yes

Title of funding source

Novartis

Proportion provided by this source

100

Public or private sector

empty

Domestic or foreign origin

empty

Category of foreign source of funding

empty

Country of origin

Type of organization providing the funding

empty

Person responsible for general inquiries

Contact

Name of organization / entity

Hematology Research Center, Nemazee Hospital,
Shiraz

Full name of responsible person

Seazaneh Haghpanah

Position

Assistance professor of community medicine

Other areas of specialty/work

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Tahereh Zarei

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Sharing plan

Deidentified Individual Participant Data Set (IPD)
empty

Study Protocol
empty
Statistical Analysis Plan
empty
Informed Consent Form
empty
Clinical Study Report
empty
Analytic Code
empty
Data Dictionary
empty