

# Clinical Trial Protocol

## Iranian Registry of Clinical Trials

11 Sep 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

##### Country

Iran (Islamic Republic of)

##### Phone

+98 71 3612 5617

##### Email address

karimim@sums.ac.ir

#### 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 aminotranferase (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 10days 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

**Street address**

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,  
Zand Avenue, Shiraz

#### City

Shiraz

## 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

#### Street address

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## Person responsible for scientific inquiries

### Contact

#### Name of organization / entity

Shiraz University of Medical Sciences

#### Full name of responsible person

Mehran Karimi

#### Position

Professor of Pediatric Hematology-Oncology  
Hematology Research Center

#### Other areas of specialty/work

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## Person responsible for updating data

### Contact

#### Name of organization / entity

Hematology Research Center, Nemazee Hospital,  
Zand Avenue, Shiraz

#### Full name of responsible person

Tahereh Zarei

#### Position

Msc Microbiologist

#### Other areas of specialty/work

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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