# Clinical Trial Protocol
# Iranian Registry of Clinical Trials

10 Jun 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 forthy. 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

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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 deferasirox 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 hypertention, 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 defreasirox 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**
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## Person responsible for scientific inquiries

**Contact**
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## Person responsible for updating data

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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*