Clinical Trial Protocol
Iranian Registry of Clinical Trials

22 Nov 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

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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 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
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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
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Full name of responsible person
Seazaneh Haghpanah
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Person responsible for scientific inquiries

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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