



# THETIS- Efficacy and Safety Study of Deferasirox in Patients With Non-transfusion Dependent Thalassemia

22/11/2024 00:13:00

## Main Information

**Primary registry identifying number**

LBCTR2020011375

**Protocol number**

CICL670E2419

**MOH registration number**

9932/ص

**Study registered at the country of origin**

Yes

**Study registered at the country of origin: Specify****Type of registration**

Retrospective

**Type of registration: Justify**

Study was submitted previously before implementation of LBCTR

**Date of registration in national regulatory agency**

03/11/2014

**Primary sponsor**

Novartis Pharmaceuticals

**Primary sponsor: Country of origin**

Novartis Pharmaceuticals

**Date of registration in primary registry**

07/01/2020

**Date of registration in national regulatory agency**

03/11/2014

**Public title**

THETIS- Efficacy and Safety Study of Deferasirox in Patients With Non-transfusion Dependent Thalassemia

**Acronym****Scientific title**

An Open Label, Multi-center, Efficacy and Safety Study of Deferasirox in Iron Overloaded Patients With Non-transfusion Dependent Thalassemia

**Acronym****Brief summary of the study: English**

Assessed the efficacy of deferasirox in patients with non-transfusion dependent thalassemia based on change in liver iron concentration from baseline after 52 weeks of treatment. Provided further assessment of the long-term efficacy and safety of deferasirox in NTDT patients with iron overload (LIC  $\geq$  5 mg Fe/g liver dw and SF  $\geq$  300 ng/mL) for up to 260 weeks.

**Brief summary of the study: Arabic**

دراسة مفتوحة اللصاقه متعدده المراكز حول فعاليتها وسلامة دواء ديفيرازيروكس لدى مرضى التالاسيميا غير المعتمدين على نقل الدم الذين يعانون من زيادة تركيز الحديد (THETIS)

**Health conditions/problem studied: Specify**

Non-transfusion Dependent Thalassemia

**Interventions: Specify**

Drug: deferasirox

Deferasirox dispersible tablets at strengths of 125 mg, 250 mg, and 500 mg were administered by oral daily dosing.

Other Name: ICL670

**Key inclusion and exclusion criteria: Inclusion criteria**

Non-transfusion dependent congenital or chronic anemia inclusive of beta-thalassemia intermedia, HbE beta-thalassemia or alpha-thalassemia





intermedia (HbH disease)/ Liver iron concentration  $\geq 5$  mg Fe/g dw Serum Ferritin  $\geq 300$  ng/mL

**Key inclusion and exclusion criteria: Gender**

Both

**Key inclusion and exclusion criteria: Specify gender**

**Key inclusion and exclusion criteria: Age minimum**

10

**Key inclusion and exclusion criteria: Age maximum**

99

**Key inclusion and exclusion criteria: Exclusion criteria**

HbS-beta Thalassemia, anticipated regular transfusion program during the study, blood transfusion 6 months prior to study start, significant proteinuria..

**Type of study**

Interventional

**Type of intervention**

Pharmaceutical

**Type of intervention: Specify type**

N/A

**Trial scope**

Therapy

**Trial scope: Specify scope**

N/A

**Study design: Allocation**

N/A: Single arm study

**Study design: Masking**

Open (masking not used)

**Study design: Control**

N/A

**Study phase**

4

**Study design: Purpose**

Treatment

**Study design: Specify purpose**

N/A

**Study design: Assignment**

Single

**Study design: Specify assignment**

N/A

**IMP has market authorization**

Yes, Lebanon and Worldwide

**IMP has market authorization: Specify**

China, Greece, Italy, Lebanon, Thailand, Tunisia, Turkey, United Kingdom

**Name of IMP**

Deferasirox (ICL670)

**Year of authorization**

2006

**Month of authorization**

4

**Type of IMP**

Others

**Pharmaceutical class**

Iron chelator

**Therapeutic indication**

Thalassemia

**Therapeutic benefit**

Change in liver iron concentration

**Study model**

N/A

**Study model: Explain model**

**Study model: Specify model**

N/A



N/A

**Time perspective**

N/A

**Time perspective: Explain time perspective**

N/A

**Time perspective: Specify perspective**

N/A

**Target follow-up duration**

**Target follow-up duration: Unit**

**Number of groups/cohorts**

**Biospecimen retention**

Samples without DNA

**Biospecimen description**

Samples are sent to central lab for analysis

**Target sample size**

20

**Actual enrollment target size**

20

**Date of first enrollment: Type**

Actual

**Date of first enrollment: Date**

26/02/2013

**Date of study closure: Type**

Actual

**Date of study closure: Date**

17/01/2019

**Recruitment status**

Complete

**Recruitment status: Specify**

**Date of completion**

31/12/2013

**IPD sharing statement plan**

Yes

**IPD sharing statement description**

Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent expert panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations.

This trial data is currently available according to the process described on [www.clinicalstudydatarequest.com](http://www.clinicalstudydatarequest.com).

**Additional data URL**

<https://clinicaltrials.gov/ct2/show/record/NCT01709838?view=record>

**Admin comments**

**Trial status**

Approved

## Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
clinicaltrials.gov	NCT01709838

## Sources of Monetary or Material Support

Name
Novartis Pharmaceuticals

## Secondary Sponsors

Name
NA

## Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Ali Taher	Beirut	Lebanon	01350000#7908	ataher@aub.edu.lb	Chronic Care Center
Scientific	Hind Khairallah	Sin elfil	Lebanon	+961 1512002 #271	Hind.Khairallah@fattal.com.lb	Khalil Fattal et Fils s.a.l.

## Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Chronic Care Center	Ali Taher	Hematology	Approved



## Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Chronic Care Center	17/11/2012	Michele Abi saad	cccmas@chroniccare.org.lb	+961 3 664 310
American University of Beirut Medical Center	30/11/2012	Fuad Ziyadeh	fz05@aub.edu.lb	961 (0) 1 350 000 ext:5445

## Countries of Recruitment

Name
Lebanon
China
Greece
Italy
Thailand
Tunisia
Turkey

## Health Conditions or Problems Studied

Condition	Code	Keyword
Non-transfusion Dependent Thalassemia	Thalassaemia, unspecified (D56.9)	Non-transfusion Dependent Thalassemia

## Interventions

Intervention	Description	Keyword
ICF, Labs, drug administration , Radiology	ICF, Labs, drug administration , Radiology	ICF, Labs, drug administration , Radiology

## Primary Outcomes

Name	Time Points	Measure
Absolute change in liver iron concentration measured by MRI	baseline, 52 weeks	baseline, 52 weeks



## Key Secondary Outcomes

Name	Time Points	Measure
•Percentage of Participants With Baseline LIC more than 15 Achieving LIC less than 5 mg	5 years	5 years
•Time to Achieving LIC less than 5 mg	5 years	5 years

## Trial Results

### Summary results

#### Study results globally

<https://clinicaltrials.gov/ct2/show/results/NCT01709838?view=results>

Reference study results tab in the above link of clinical trials.gov

#### Date of posting of results summaries

#### Date of first journal publication of results

#### Results URL link

<https://clinicaltrials.gov/ct2/show/results/NCT01709838?view=results>

#### Baseline characteristics

#### Participant flow

#### Adverse events

#### Outcome measures

#### URL to protocol files