



Study to Evaluate Treatment Compliance, Efficacy and Safety of an Improved Deferasirox Formulation (Granules) in Pediatric Patients (2-<18 Years Old) With Iron Overload (CALYPSO)

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Main Information

Primary registry identifying number

LBCTR2019020197

Protocol number

ICL670F2202

MOH registration number

6428/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

LCTR was recently initiated, original file was previously submitted by Paper

Date of registration in national regulatory agency

15/07/2015

Primary sponsor

Novartis Pharma Services Inc.

Primary sponsor: Country of origin

Novartis Pharmaceuticals

Date of registration in primary registry

02/11/2023

Date of registration in national regulatory agency

15/07/2015

Public title

Study to Evaluate Treatment Compliance, Efficacy and Safety of an Improved Deferasirox Formulation (Granules) in Pediatric Patients (2-<18 Years Old) With Iron Overload (CALYPSO)

Acronym

CALYPSO

Scientific title

A randomized, open-label, multicenter, two arm, phase II study to evaluate treatment compliance, efficacy and safety of an improved deferasirox formulation (granules) in pediatric patients with iron overload

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

This is a randomized, open-label, multicenter, two arm, phase II study to evaluate treatment compliance and change in serum ferritin of a deferasirox granule formulation and a deferasirox DT formulation in children and adolescents aged ≥ 2 and < 18 years at enrollment with any transfusion-dependent anemia requiring chelation therapy due to iron overload, to demonstrate the effect of improved compliance on iron burden.

Randomization will be stratified by age groups (2 to <10 years, 10 to <18 years) and prior iron chelation therapy (Yes/ No). There will be two study phases which include a 1 year core phase where patients will be randomized to a 48 week treatment period to either Deferasirox DT or granules, and an optional extension phase where all patients will receive the granules up to 5 years. Patients who demonstrated benefit to granules or DT in the core phase, and/or express the wish to continue in the optional extension phase on granules, will be offered this possibility until there is local access to the new formulation (granules or FCT) or up to 5 years, whichever occurs first.





Brief summary of the study: Arabic

دراسة عشوائية التوزيع، مفتوحة اللصاقة، متعددة المراكز، ذات مجموعتين، في المرحلة الثانية لتقييم الامتثال للعلاج بصيغة ديفيرازيروكس محسنة (حبيبات) وفعاليتها وسلامتها لدى الأطفال المرضى الذين يعانون من الحديد الزائد

Health conditions/problem studied: Specify

Pediatric Patients (2-<18 Years Old) With Iron Overload

Interventions: Specify

•Drug: Deferasirox granule formulation
Deferasirox granules will be provided as stick packs containing 90 mg, 180 mg and 360 mg granules for oral use.

Other Name: ICL670

•Drug: Deferasirox DT formulation
Deferasirox DT will be provided as 125 mg, 250 mg and 500 mg dispersible tablets for oral use

Other Name: ICL670

Key inclusion and exclusion criteria: Inclusion criteria

- Written informed consent/assent before any study-specific procedures. Consent will be obtained from parent(s) or legal guardians. Investigators will also obtain assent of patients according to local guidelines.
- Male and female children and adolescents aged ≥ 2 and < 18 years. [France: Male and female children and adolescent aged ≥ 2 and < 18 years old, however children aged ≥ 2 and ≤ 6 years can be enrolled only when deferoxamine treatment is contraindicated or inadequate in these patients as per investigator decision. Applicable to core phase only. Once in the core phase patients can turn 18 years and still be considered eligible, also for participation in the optional extension phase.
- Any transfusion-dependent anemia associated with iron overload requiring iron chelation therapy and with a history of transfusion of approximately 20 PRBC units and a treatment goal to reduce iron burden (300mL PRBC = 1 unit in adults whereas 4 ml/kg PRBC is considered 1 unit for children).
- Serum ferritin > 1000 ng/mL, measured at screening Visit 1 and screening Visit 2 (the mean value will be used for eligibility criteria).
- Patient has to have participated and completed the 48 weeks core phase treatment as per protocol (For optional extension phase eligibility only).

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

2

Key inclusion and exclusion criteria: Age maximum

18

Key inclusion and exclusion criteria: Exclusion criteria

- Creatinine clearance below the contraindication limit in the locally approved prescribing information (using Schwartz formula) at screening visit 1 or screening visit 2.
- Serum creatinine > 1.5 xULN at screening measured at screening Visit 1 and or screening Visit 2
- ALT and/or AST > 3.0 x ULN at screening visit 1 or screening visit 2.
- (Criterion no longer applicable, removed as part of Amendment 1): Prior iron chelation therapy.
- Liver disease with severity of Child-Pugh class B or C.
- Significant proteinuria as indicated by a urinary protein/creatinine ratio > 0.5 mg/mg in a second morning urine sample at screening Visit 1 or screening Visit 2.
- Patients with significant impaired gastrointestinal (GI) function or GI disease that may significantly alter the absorption of oral deferasirox (e.g. ulcerative diseases, uncontrolled nausea, vomiting, diarrhea, malabsorption syndrome or small bowel resection).

Other protocol-defined Inclusion/Exclusion may apply.

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

Randomized controlled trial

Study design: Masking

Open (masking not used)

**Study design: Control**

Active

Study phase

2

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

Worldwide

Name of IMP

Jadenu (ICL670) / Deferasirox

Year of authorization

2017

Month of authorization

10

Type of IMP

Others

Pharmaceutical class

Deferasirox is an N-substituted bis-hydroxyphenyl-triazole, a class of tridentate iron chelators.

Therapeutic indication

Patients with Iron Overload/ Transfusion Dependent Anemia

Therapeutic benefit

- Change in serum ferritin in ICT naïve patients.
-The comparison of means between the two treatment arms of change from baseline to week 24 of treatment in serum ferritin in pediatric ICT naïve patients with iron overload.

Study model

N/A

Study model: Explain model

N/A

Study model: Specify model

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 with DNA**

Biospecimen description



MCHC, MCV, Platelets, Red blood cells, White blood cells(WBC) count with differential, RBC Morphology with Differential (Basophils, Eosinophils, Lymphocytes, Monocytes, Neutrophils) Biochemistry Albumin, Alkaline phosphatase, ALT, AST, Bicarbonate, Calcium, Chloride, Creatinine, Creatine kinase, Direct (conjugated) Bilirubin, Indirect Bilirubin, Total Bilirubin, Total Cholesterol, LDL, HDL, Lactate Dehydrogenase (LDH), Total Protein, Triglycerides, Blood Urea Nitrogen (BUN) or Urea, Uric Acid, C Reactive Protein (CRP), Urinalysis Microscopic Panel: Red Blood Cells, White Blood Cells, Casts, Crystals, Bacteria, Epithelial cells
Macroscopic Panel (Dipstick): Color, Bilirubin, Blood, Glucose, Ketones, Leukocytes esterase, Nitrite, pH, Protein, Specific Gravity, Urobilinogen
Hepatitis markers HbsAg, HbsAb, HbcAb, HCV RNA, Anti-HCV
Additional tests Serum ferritin, creatinine clearance, urine protein/creatinine ratio, serum pregnancy test

Target sample size

23

Date of first enrollment: Type

Actual

Date of study closure: Type

Actual

Recruitment status

Complete

Date of completion

21/12/2017

IPD sharing statement plan

No

Additional data URL

<https://clinicaltrials.gov/ct2/show/NCT02435212?term=2013-004739-55&rank=1>

Admin comments**Trial status**

Approved

Actual enrollment target size

23

Date of first enrollment: Date

13/10/2016

Date of study closure: Date

29/02/2024

Recruitment status: Specify**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 review 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 availability is according to the criteria and process described on www.clinicalstudydatarequest.com

Secondary Identifying Numbers

| Full name of issuing authority | Secondary identifying number |
|--------------------------------|------------------------------|
| Clinical Trials. gov | NCT02435212 |



Sources of Monetary or Material Support

Name

Novartis Pharma Services Inc.

Secondary Sponsors

Name

NA

Contact for Public/Scientific Queries

| Contact type | Contact full name | Address | Country | Telephone | Email | Affiliation |
|--------------|-------------------|------------|---------|------------------------------|-------------------------------|------------------------------|
| Public | Ali Taher | Beirut | Lebanon | 01-350000 ext 5392 | ataher@aub.edu.lb | Chronic Care Center |
| Scientific | Hind Khairallah | Sin El Fil | Lebanon | +961 1 512002 Ext. 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 | Dr Ali Taher | Hematology | Approved |

Ethics Review

| Ethics approval obtained | Approval date | Contact name | Contact email | Contact phone |
|--|---------------|------------------|----------------------------|--------------------------------|
| American University of Beirut Medical Center | 15/06/2016 | Fouad Ziyadeh | fz05@aub.edu.lb | +961 (0) 1 350 000 ext:5445 |
| Chronic Care Center | 11/07/2016 | Michele Abi saad | cccmass@chroniccare.org.lb | +961 3 664 310 |





Countries of Recruitment

| Name |
|--------------------------|
| Lebanon |
| Belgium |
| Bulgaria |
| Egypt |
| Oman |
| United States of America |
| India |
| Italy |
| France |
| Tunisia |
| Turkey |

Health Conditions or Problems Studied

| Condition | Code | Keyword |
|---|------------------------------|------------------------------|
| Patients with Iron Overload/ Transfusion Dependent Anemia | Anaemia, unspecified (D64.9) | Transfusion Dependent Anemia |

Interventions

| Intervention | Description | Keyword |
|---|--|--|
| Physical examination, height, weight, Hematology, Chemistry, Ferritin, Creatinine, Creatinine Clearance, Hepatitis, Pregnancy Test, Urine Dipstick, Microscopic Urinalysis, Proteinuria, Urine Pregnancy Test, Liver function test, Ocular exam, audiometry, ECG, Electrocardiogram, PK sampling, vital signs, Growth and development | ICF, IMP, Lab tests and ECG , diary completion | ICF, IMP, Lab tests and ECG , diary completion |

Primary Outcomes

| Name | Time Points | Measure |
|---|------------------|------------------|
| •Compliance (using stick/pack tablet count). | 24 weeks | 24 wks |
| •Change in serum ferritin in ICT naive patients | baseline, 24 wks | baseline, 24 wks |



Key Secondary Outcomes

| Name | Time Points | Measure |
|---|--------------------------|--------------------------|
| •Compliance (using stick/pack tablet count) | 48 weeks | 48 wks |
| •Change in serum ferritin in ICT naive patients | baseline, 24 wks, 48 wks | baseline, 24 wks, 48 wks |
| •Overall safety, as measured by frequency and severity of adverse | from baseline to 48 wks | from baseline to 48 wks |

Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files